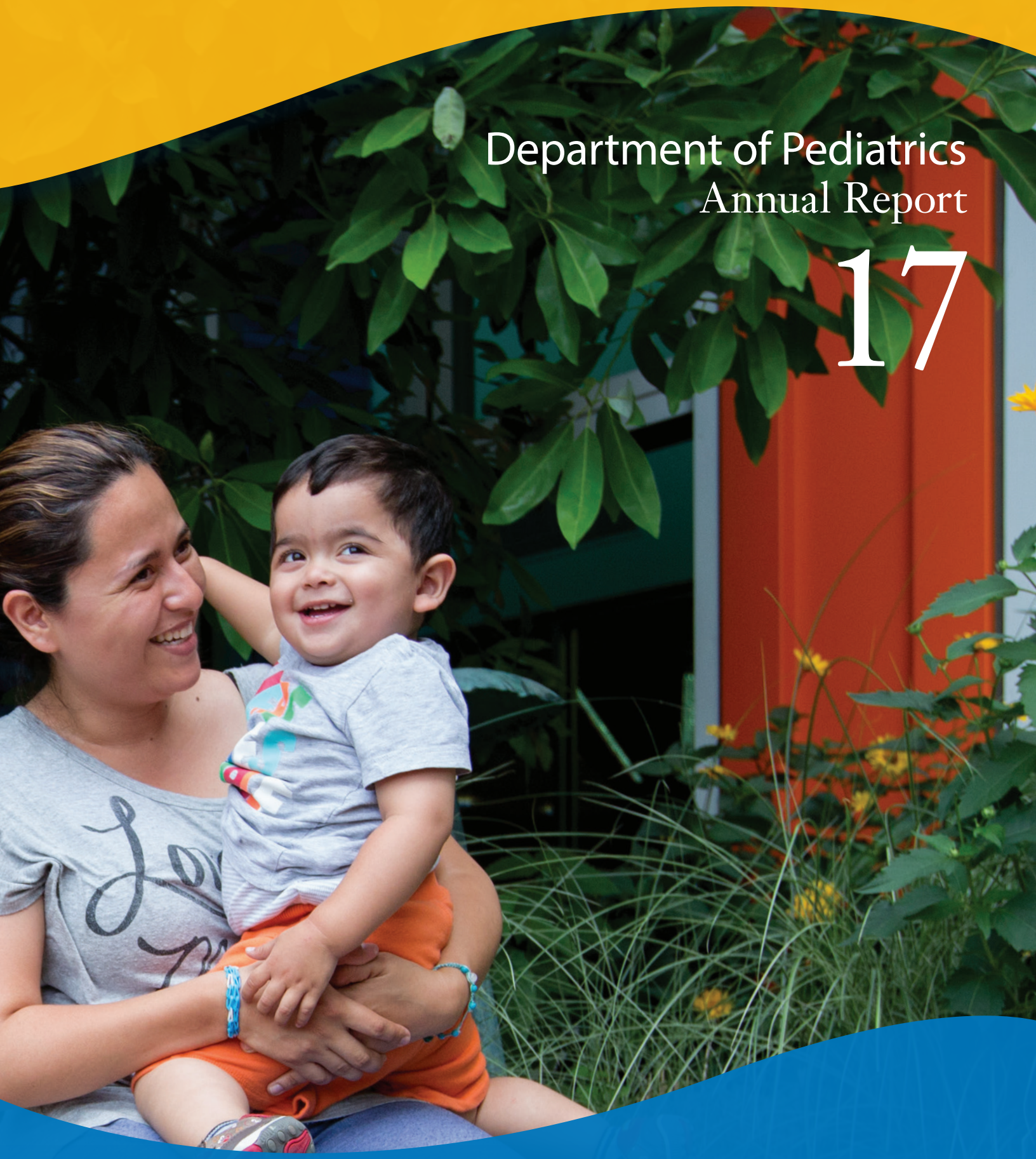


Department of Pediatrics
Annual Report
17

University of Pittsburgh
School of Medicine

Children's Hospital of Pittsburgh of UPMC

CONTENTS

Department of Pediatrics
Annual Report 17

Photography by Denmarsh Photography, Bill Exler Photography, Harry Giglio Productions, Tom Gigliotti, Dennis Marsico, Annie O'Neill, Michael Tarquino, and UPMC Medical Media.

"We are stewards entrusted with futures well beyond our own: the lives of patients, the preparation of our own surpassers, and the advancement of biomedical innovation."

Message from the Chair

My second year began with the graduation of a superlative class of residents, the product of untold hours of study, practice, mentoring, and devotion. Commencement marks the opening of new vistas of possibilities previously unrealizable, literally "beginning now only at the end." I see and feel the inspiration and potential of Pediatrics at Children's Hospital of Pittsburgh of UPMC and know no bounds. And yet, it is humbling to take stock surrounded by so much accomplishment. Even by our standards—to become the worldwide leader in pediatric health care, education, and discovery—so far have we come, and yet so far to go.

The end of all our efforts is child health; tending to the health and well-being of children justifies efforts in education and research. Thus, progress is measured in terms of child health. In Pediatrics, a victory is a young person graduating from high school on time, healthy, and thriving. Children rightly deserve no less.

To the children in our care, victory is all important. It is similar for our students and trainees working to earn the next level of responsibility. But for faculty and staff, the metaphor in which we reveal ourselves is not winning wars or playing to win. We are not heroes in their struggles; we are planters and tenders of trees, under whose shade we do not expect to rest. We are servants, healers, teachers, and seekers. We labor, ultimately, for others. We are stewards entrusted with futures well beyond our own: the lives of patients, the preparation of our own surpassers, and the advancement of biomedical innovation. We work for the benefit of those who need us most while wishing that they never have cause to call on us, but when they do, they spare us not a backward glance as they race off into futures still theirs because we saw them past their need for the hope of doing so.

We suffer no lack of seeds to plant. Tending seedlings is unrelenting and insistent; we do the best we can with what we have and always look to have better to offer. We reach out to the community as many times as it takes to foster relationships that advance child health. We stand meticulously measuring fluid into rows of tubes, working toward providing a better future. We put on one more smile to recruit another family to participant in a study.

"In pediatrics, a win wears a smile and holds a diploma ..."

All too often, we get but one shot per seed, and then, each is not watered but once. So, sometimes we are forced to settle for hope, but, mostly, we make progress. Clinically, we have a decent handle on acute and chronic care, and thus the responsibility to move past decent and pivot toward caring for *health* in addition to tending to illness.

We are seeding research under the slogan, "*Healthy Minds. Healthy Bodies. Healthy Families.*"

- For "*Healthy Minds*," the **Children's Neuroscience Institute** will focus on preservation of brain function following acute brain injury and brain cancer. The thrust will be discovery of diagnostics, therapeutics, and rehabilitation practices.
- For "*Healthy Bodies*," the **Institute for Infection, Inflammation, and Immunity**, the I4Kids, will pursue the complexities of personalized vaccination, diabetes, the microbiome, and congenital infection.
- For "*Healthy Families*," the **Children's Community Health Collaborative** will study the barriers to implementation of evidence-based community health interventions. Targets include acute asthma, violence reduction, nutrition, and opioid issues.
- We are installing a translational core facility called **sciVelo-CHP** to facilitate development and shepherd biomedical discoveries and inventions to bedsides and communities. **Bioinformatics** and **biorepository** core facilities are on the way.

The seeds of *health* research are going into the ground as **The Pittsburgh Study**. We are looking for the determinants of health. The Framingham Study is why everyone knows the word cholesterol. Following that model, the Pittsburgh Study will be why everyone knows the words that determine child health. This will be a longitudinal, birth cohort, population *health* study of Allegheny County with as many cohorts and as broad a spectrum of data as we can assemble. When we find gems, they will be spun off as targeted interventions.

In pediatrics, a win wears a smile and holds a diploma, and with the rest of the graduating class is ready to commence with what comes next. We are looking to run up the score!

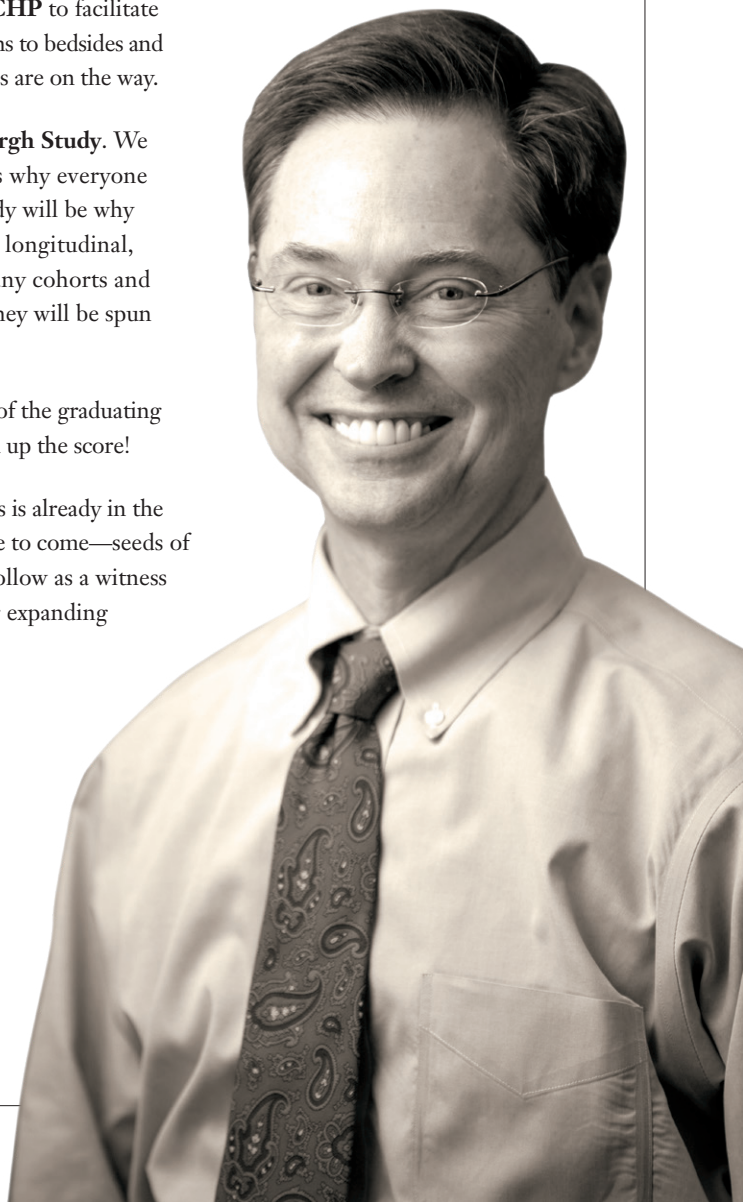
For the year to come, our sleeves are rolled up, an amazing array of seeds is already in the ground, more have been added in the past year, and there are many more to come—seeds of care, seeds of education, and seeds of discovery. I offer the pages that follow as a witness to the works under way and as a glimpse of the fruits to come from our expanding grove. Turn the page and tour the arbor!

Hail to Pitt!

Terence S. Dermody, MD

Vira I. Heinz Professor and Chair of Pediatrics
University of Pittsburgh School of Medicine

Physician-in-Chief and Scientific Director
Children's Hospital of Pittsburgh of UPMC



"We are far from done. We are poised to seize opportunities at the bench, at the bedside, and in the community."

Executive Summary

The Department of Pediatrics continues to advance toward its mission to become the worldwide leader in pediatric health care, education, and discovery. Pediatrics is 1,143 strong, consisting of 21 divisions and centers assembled from more than 320 faculty, 200 clinical trainees, 95 research trainees, almost 200 research staff, more than 300 clinical staff, and only 33 administrators supporting the rest. We recruited 38 faculty, of whom nine are underrepresented minorities and the majority of whom are women. The ten-year revenue trend was 6% growth, which made possible $11 million to support research. Children's Hospital was named to *U.S. News & World Report*'s Honor Roll of America's Best Children's Hospitals for the seventh consecutive year. There is much that makes us proud.

We had unprecedented volume. Children's Hospital is licensed for 315 beds. The average daily census was a record-breaking 276, or 88% of capacity. On our busiest day, our census was 340, 25 over bed count. Total visits for the Emergency Department exceeded 80,000. Express Care came in at 66,700 visits. The NICU count has leveled off, because it is completely and constantly full. We are recruiting to keep pace with demand and, in the future, will be planning a new bed tower.

Excellence in education continued to be a departmental hallmark. We look forward to the accomplishments of our 40 new interns even as we seek to retain as many as possible of this year's 19 University of Pittsburgh School of Medicine seniors who are applying in pediatrics.

The department and the larger biomedical community recognized our faculty's contributions. Thirteen faculty members earned promotions. Total compensation tracked the rise in total number of faculty at approximately a 4.5% growth rate. Accolades for professional service and scientific excellence were bestowed on our faculty, e.g., Juan Celedón became the president-elect of the American Thoracic Society, Kishore Vellody was inducted as the president of the National Down Syndrome Congress Executive Committee, and Stacey Cook and Damara Ortiz earned UMPC ACES Awards as top clinical performers.

Discovery is how we offer hope to children who have little now. Pediatrics published 440 peer-reviewed papers, of which too few can be highlighted here. Carolyn Coyne and her team advanced our understanding of how the placenta impedes Zika virus infection, which may lead to new strategies to increase placental pathogen resistance (*PNAS*). Alejandro Hoberman and his team set the standard of care for acute otitis media (*NEJM*). Jerry Vockley and Lisa Pan found neurometabolomic paths forward in treating previously unresponsive forms of depression (*American Journal of Psychiatry*). Our breakthroughs are big and numerous.

Funding undergirds hope's discovery. The department's extramural budget has held fast at $50 million for the past three years. This year, two-thirds came from the NIH despite tightening federal budgets. This year, our faculty earned three new NIH K awards, three NIH T32 grants, and 10 new grants at or above the half-million-dollar mark. Clinical research funding has increased seven-fold since 2011 to $7 million, supporting more than 200 studies. Pediatrics anticipates future funding growth consistent with our expanding faculty. The Research Advisory Committee's research support budget was about $830,000. The Children's Hospital Foundation's exemplary efforts brought in more than $18.2 million from 35,167 donors this year. The only limits to advancing care for children through research are the limits of our imagination!

Pediatrics added the Division of Health Informatics under the direction of Srinivasan Suresh. We applauded Vivek Allada's interim service in Cardiology as we welcomed Jackie Kreutzer as the new division director. As Tom Diacovo is joining us to direct Newborn Medicine, we laud the heroic efforts of Richard Saladino as interim director. Drew Feranchak is now leading Gastroenterology, Hepatology, and Nutrition, and we owe a debt of gratitude to David Keljo for his interim service.

The department added the Center for Microbial Pathogenesis under the direction of Carolyn Coyne and the Center for Pediatric Research on Obesity and Metabolism under the direction of Silva Arslanian. We are developing the following strategic research initiatives.

- The **Children's Neuroscience Institute** will focus on preservation of brain function following acute brain injury and through brain cancer. The thrust will be discovery of diagnostics, therapeutics, and rehabilitation practices.
- The **Institute for Infection, Inflammation, and Immunity**, the I4Kids, will pursue the complexities of personalized vaccination, diabetes, and congenital infection.
- The **Children's Community Health Collaborative** will study the barriers to implementation of evidence-based community health interventions. Targets include acute asthma, violence reduction, nutrition, and opioid issues.
- We are installing a translational core facility called **sciVelo-CHP** to facilitate development and shepherd biomedical discoveries and inventions to bedsides and communities. **Bioinformatics** and **biorepository** core facilities are on the way.
- **The Pittsburgh Study** will find the determinants of health. It will be a longitudinal, birth cohort, population health study of Allegheny County with as many cohorts and as broad a spectrum of data as we can assemble.

We are far from done. We are poised to seize opportunities at the bench, at the bedside, and in the community. We invite you to follow your curiosity through the pages to follow.

Hail to Pitt!

Terence S. Dermody, MD

Vira I. Heinz Professor and Chair of Pediatrics
University of Pittsburgh School of Medicine

Physician-in-Chief and Scientific Director
Children's Hospital of Pittsburgh of UPMC

Leadership

Chair
Terence S. Dermody, MD

Adolescent and Young Adult Medicine
Elizabeth Miller, MD, PhD

Blood and Marrow Transplantation and Cellular Therapies
Paul Szabolcs, MD

Child Advocacy
Rachel Berger, MD, MPH

Neurology and **Child Development**
Ira Bergman, MD, PhD

Gastroenterology (Interim)
David Keljo, MD, PhD

General Academic Pediatrics
Alejandro Hoberman, MD

Hematology/Oncology
Linda McAllister-Lucas, MD, PhD

Infectious Diseases
John V. Williams, MD

Medical Genetics
Jerry Vockley, MD, PhD

Nephrology
Carlton Bates, MD

Newborn Medicine (Interim) and **Emergency Medicine**
Richard Saladino, MD, PhD

Cardiology (Interim)
Vivek Allada, MD

Endocrinology, Diabetes, and Metabolism
Radhika Muzumdar, MD

Pulmonary Medicine, Allergy, and Immunology
Juan Celedón, MD, DrPH

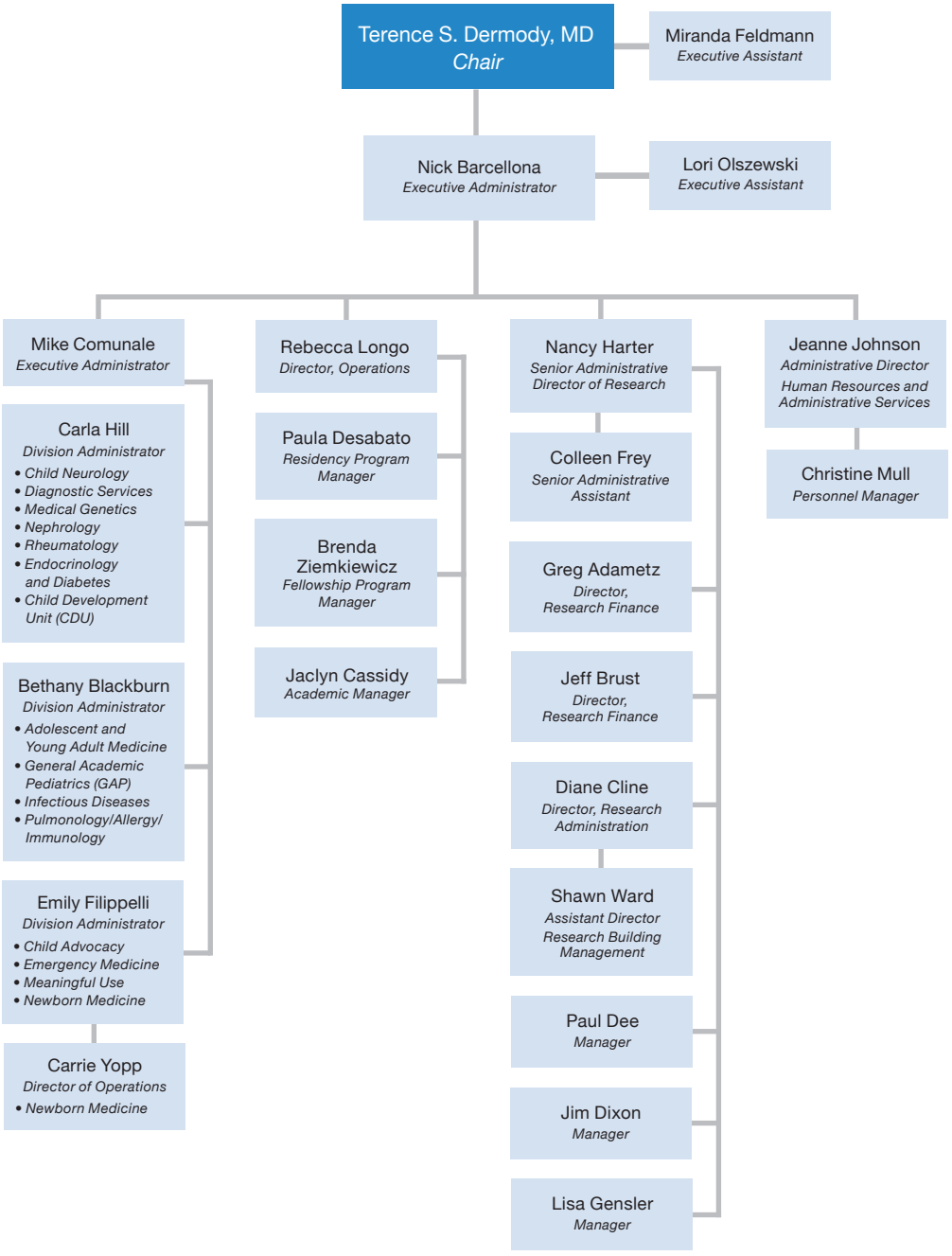
Rheumatology (Interim)
A. Kim Ritchey, MD

Richard King Mellon Foundation Institute
Jay Kolls, MD

Paul C. Gaffney Diagnostic Referral Service
Basil Zitelli, MD

Weight Management and Wellness Center
Silva Arslanian, MD

Administration



DIVISION OF ADOLESCENT AND YOUNG ADULT MEDICINE

Mission

The mission of the Division of Adolescent and Young Adult Medicine, Children's Hospital of Pittsburgh of UPMC, is to improve the health and well-being of youth and young adults through:

- Accessible health care services for adolescents and young adults in the context of their families, cultures, and communities
- Interdisciplinary adolescent health care education for health professional trainees and practitioners, as well as youth, young adults, families, and communities
- Research to increase understanding of disparities in adolescent health, promote adolescent health equity, and improve adolescent health outcomes
- Engagement with youth and young adults in their communities as adult allies and advocates

FACULTY AND STAFF

Elizabeth Miller, MD, PhD*
Professor of Pediatrics, Public Health, and Clinical and Translational Science
Division Director

Carol Byers, CRNP*
Clinical Nurse Practitioner

Brenda Cassidy, DNP, MSN, CPNP-PC
Assistant Professor of Pediatrics

Serena Chan, MD
Assistant Professor of Obstetrics and Gynecology and Pediatrics

Cherie Priya Dhar, MD*
Assistant Professor of Pediatrics

Nancy R. Eligator, MD
Clinical Assistant Professor of Obstetrics and Gynecology

Erika Forbes, PhD
Associate Professor of Psychiatry and Pediatrics

Yukiko Giho, MSN, CRNP*
Clinical Nurse Practitioner

Elissa Gittes, MD*
Assistant Professor of Pediatrics

Joanne Goodall, CRNP*
Clinical Nurse Practitioner

Karen Hacker, MD, MPH
Clinical Professor of Pediatrics

Colleen Krajewski, MD
Clinical Assistant Professor of Obstetrics and Gynecology

Kara Mackinson Peters, LSW, MPH*
Transition Care Coordinator/ Social Worker

Michael Marshal, PhD
Associate Professor of Psychiatry and Pediatrics

Loreta Matheo, MD*
Clinical Associate Professor of Pediatrics
Fellowship Program Director

Ann McCurdy, MSW, LSW*
Transition Care Coordinator/ Social Worker

Gerald Montano, DO, MS*
Clinical Instructor

Ana Radovic, MD, MSc*
Assistant Professor of Pediatrics
Associate Fellowship Program Director

Laura Richardson, PA*
Clinical Physician Assistant

Dana Rofey, PhD
Assistant Professor of Pediatrics, Psychology, and Psychiatry

Joseph Sanfilippo, MD, MBA
Professor of Obstetrics, Gynecology, and Reproductive Sciences

Tahniat Syed, MD*
Assistant Professor of Pediatrics
Clinical Director

Michelle Ziegler, PA*
Clinical Physician Assistant

* Indicates that primary appointment is in the Division of Adolescent and Young Adult Medicine

OVERVIEW OF DIVISION

The Division of Adolescent and Young Adult Medicine is a dynamic, interdisciplinary team of researchers, educators, and practitioners dedicated to the health and well-being of adolescents and young adults. Faculty within the division hold regional and national leadership positions with organizations such as the American Academy of Pediatrics, Academic Pediatric Association, Society for Pediatric Research, Society for Adolescent Health and Medicine, and National Association of Pediatric and Adolescent Gynecology. In addition, they contribute to the editorial leadership of the *Journal of Pediatric and Adolescent Gynecology* and the journal *Violence Against Women*.

Research: Faculty academic interests include prevention of adolescent relationship abuse and sexual violence; prevention of pregnancy, HIV, and sexually transmitted infections; mental health disparities among sexual minorities; innovative health services delivery for marginalized youth; transition to adult care for youth with complex medical conditions; adolescent contraceptive and sexual decision making; and medical and interdisciplinary educational scholarship. Faculty research is funded by the Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD), National Institute of Justice, National Institute of Mental Health (NIMH), National Institute on Drug Abuse (NIDA), National Institute on Alcohol Abuse and Alcoholism (NIAAA), Centers for Disease Control and Prevention (CDC), Department of Health and Human Services Office on Women's Health, and Department of Justice Office of Juvenile Justice and Delinquency Prevention, as well as numerous local and national foundations. The division's faculty and staff have also participated in industry-sponsored research related to contraception and adolescent health. Other research collaborations exist with a variety of divisions within the Department of Pediatrics, including Pediatric Hematology/ Oncology, Pediatric Pulmonary, Pediatric Endocrinology, and Pediatric Infectious Diseases. Robust research, educational, and practice collaborations also exist with faculty in the Departments of Obstetrics and Gynecology, Psychiatry,

Pharmacy, and Sports Medicine, as well as the School of Nursing, School of Social Work, Graduate School of Public Health, and School of Dental Medicine. The division also proudly supports a Youth Research Advisory Board, a group of young people ages 15–26 who provide input into adolescent and young adult research being conducted at the University of Pittsburgh. The youths provide consultation to researchers on study focus and design, recruitment, consent processes, study flow, as well as interpretation of results and research dissemination.

Clinical Care: The division maintains an active Oakland-based practice, supports satellite locations in the provision of adolescent care, and provides consultations at Children's Hospital in Lawrenceville. Clinical services focus primarily on adolescent medicine consultation, as well as primary care, reproductive health, and behavioral health. Faculty members serve a diverse population of youth and young adults, including medical care and leadership at Carlow University; at the county's juvenile detention facility, Shuman Juvenile Detention Center; and at Family Links shelters, the Gay Lesbian Community Center, and the Auberle Foundation's 412 Youth Zone, providing care for youth and young adults who are homeless. Faculty members also work collaboratively with colleagues at Western Psychiatric Institute and Clinic (WPIC) and in the community to treat adolescents with eating disorders. The youth-led CHANGE (Children's Hospital Advisory Network for Guidance and Empowerment) Transition Program focuses on improving support for youth and families in the successful transition to adulthood, including navigating from pediatric health care to the adult system. The Gender and Sexual Development Program (an interdisciplinary collaboration with Behavioral Health and Endocrinology at Children's Hospital) provides support and treatment for sexual minority and gender-fluid children and youth. The division is also responsible for the clinical services and community outreach provided through the Children's Hospital Ronald McDonald House Care Mobile (clinical services provided via a mobile van).

Education: Training for those studying to be health care professionals includes rotations for medical, nurse practitioner, and physician assistant students and opportunities for service and scholarly projects; practicum and internship opportunities for public health, social work, psychology, nursing, nutrition, and pharmacy students; resident rotations for those in pediatrics, psychiatry, obstetrics and gynecology, family medicine, and internal medicine; and the Adolescent Medicine Fellowship Program. Fellows in the Reproductive Endocrinology Fellowship at Magee-Womens Hospital of UPMC also participate in the pediatric and adolescent gynecology sessions.

CLINICAL ACTIVITIES

The Division of Adolescent and Young Adult Medicine provides a diverse program of primary care and consultative adolescent medicine. It provides in-hospital care for adolescents up to age 26 at Children's Hospital, primarily through an inpatient consultation service which allows the division to work closely with medical and surgical professionals, including those in hematology/oncology, endocrinology, gastroenterology, urology, behavioral health, general surgery, and transplant medicine.

Each year, the division has about 7,500 outpatient visits, including approximately 4,100 visits to the Oakland office. Subspecialty clinics include pediatric and adolescent gynecology-focused sessions attended by a gynecologist, as well as insertion of longer-acting, reversible contraceptives. A nutritionist and several psychologists work collaboratively with the adolescent medicine clinicians and see patients in division offices. Clinical interests and expertise of the faculty include smoking cessation, trauma-informed care, sexual violence prevention, contraception in healthy and medically complex patients, pediatric and adolescent gynecology, eating disorders, transitional care for adolescents with chronic conditions, integrated behavioral health, polycystic ovarian syndrome, menstrual disorders, and gender and sexual development (including care for gender-fluid and transgender youth).

Community-based services include the provision of medical care to youth detained at the county's juvenile detention center, the Shuman Center. The division provides health care to homeless youth and young adults in Allegheny County at multiple Family Links shelters, as well as the Gay and Lesbian Community Center and the Auberle Foundation's 412 Youth Zone, as part of a community-wide collaboration to increase services for unaccompanied youth. The Children's Hospital Ronald McDonald House Care Mobile provides clinical services to children, youth, and families in underserved communities in the region. Two suburban hospital satellite locations are the sites of additional adolescent clinics (South Fayette in the south and Pine Center in the north). Medical care and leadership of Carlow University's Health Service are provided by Ana Radovic. Loreta Matheo oversees the integration of adolescent and young adult care into the region's Children's Community Pediatrics practices.

RESEARCH AND OTHER SCHOLARLY ACTIVITIES

Elizabeth Miller, MD, PhD

RESEARCH

Elizabeth Miller is the director of the Division of Adolescent and Young Adult Medicine and maintains an active research program focused on reducing gender-based violence to improve adolescent health; funding comes from the National Institutes of Health (NIH), the CDC, the Office on Women's Health, and foundations. Examples of research include a cluster-randomized, controlled trial of a gender-based violence-prevention program, funded by the CDC, which involves training coaches to encourage their middle-school male athletes to recognize and stop disrespectful and harmful behaviors toward girls. Another CDC-funded study involves testing a gender transformative program (addressing healthy masculinity and sexuality) among African American males ages 13–19 in 20 neighborhoods in Pittsburgh. Another cluster-randomized, controlled trial is testing a brief sexual assault intervention (NIAAA R01) at student health centers on 28 college campuses.

Miller also serves as a research consultant to Futures Without Violence, a national nonprofit organization providing resources to health care providers in their efforts to prevent domestic violence and sexual assault. Project Connect, a national training project funded by the Office on Women's Health, involves work with states and tribal organizations on the integration of partner-violence prevention and intervention into public health programs, specifically reproductive health, adolescent health, and maternal child health programs. Miller has served as the evaluator for this project, as well as a similar project located in community health centers in California, funded by the Blue Shield of California Foundation.

STUDY SECTIONS

- Chair, Scientific Review Panel for Research on Violence Against Women, National Institute of Justice, 2012–2014

EDITORSHIPS

- Editorial Board, *Violence Against Women*

BOARD AND ADVISORY COMMITTEE MEMBERSHIPS

- Chair, Research Committee, Society for Adolescent Health and Medicine
- Member, Violence Subcommittee, Society for Adolescent Health and Medicine
- Member, Advisory Board, National Girls Institute, Office of Juvenile Justice and Delinquency Prevention Programs
- Board member, North American Society for Pediatric and Adolescent Gynecology

Elizabeth Miller, MD, PhD
Division Chief, Adolescent and Young Adult Medicine

MAJOR LECTURESHIPS AND SEMINARS

- "Rethinking Sexual and Dating Violence Prevention: Sex, Gender, and Trauma," keynote address, International Family Violence and Child Victimization Research Conference, Portsmouth, N.H., 2016
- "Trauma-Informed Care and Universal Education, Beyond Screening," Texas Health Summit, Texas Council on Family Violence, Austin, Texas, 2016
- "Rural Adolescent Health Disparities: Is It About Geography and Access?" Rural Behavioral Health Conference, Canonsburg, Pa., 2016
- "Rethinking Relationship Abuse in Sexual Violence Prevention in Adolescents: Sexuality, Trauma, and Gender," 2016 Ohio Adolescent and Young Adult Health Statewide Summit, Ohio State University, Columbus, Ohio, 2016
- "Understanding Intimate Partner Violence (IPV): Health Resources and Services Administration (HRSA) Strategy to Address IPV," IPV Summit, Rockville, Md., 2016
- "Rethinking Dating and Sexual Violence Prevention: Exploring the Roles of Sexuality, Trauma, and Gender," Healthy Relationships Brown-Bag Lecture Series, Penn State New Kensington, New Kensington, Pa., 2017
- "Impact of Violence on Health," National Health Collaborative on Violence and Abuse, Congressional Briefing, Washington, D.C., 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Fellow, Society for Adolescent Health and Medicine, 2014 to the present
- Fellow, Society for Pediatric Research
- Academic Pediatric Association
- Fellow, American Academy of Pediatrics, 2005 to the present
- Council on Community Pediatrics and Section on Adolescent Medicine, American Academy of Pediatrics
- North American Society for Pediatric and Adolescent Gynecology

HONORS

- Distinguished mentor, Institute for Clinical Research Education, University of Pittsburgh, 2014
- Best Doctors, *Pittsburgh Magazine*, 2014 to the present
- Distinguished Professional Award, Center for Victims, Pittsburgh, Pa., 2015
- Community Health Teaching Award, Department of Pediatrics, Pittsburgh, Pa., 2016
- Linda A. Dickerson Award, Homeless Children's Education Fund, Pittsburgh, Pa., 2016
- Health Care Heroes, *Pittsburgh Business Times*, Pittsburgh, Pa., 2016
- Community Partner Award, Auberle Foundation, McKeesport, Pa., 2016
- Philip Troen, MD, Excellence in Medical Student Research Mentoring Award, University of Pittsburgh School of Medicine, Pittsburgh, Pa., 2017

Brenda Cassidy, DNP, MSN, CPNP-PC

Brenda Cassidy is a pediatric nurse practitioner (PNP) who holds a doctorate of nursing practice and is an assistant professor and coordinator of the PNP program at the University of Pittsburgh School of Nursing. She received the Distinguished Clinical Scholar Award for two consecutive years (2013–2015), which provided funding to establish a partnership between the School of Nursing and the Division of Adolescent Medicine and to develop a faculty preceptor model in which she precepts PNP students in the Center for Adolescent and Young Adult Health (CAYAH) and coordinates clinical experiences for them in community sites with other adolescent providers. She has collaborated with doctor of nursing practice (DNP) students on scholarly DNP projects, four of which have utilized the division's research staff to improve patient outcomes in adolescent health. Her research involves strategies to improve human papillomavirus (HPV) vaccine coverage and has produced several funded projects to implement quality-improvement strategies to increase HPV vaccine rates in primary care. She has presented internationally, nationally, regionally, and locally on adolescent health care as well as quality-improvement strategies to increase HPV vaccine coverage, and she is active in the community on advisory boards and task forces for local HPV vaccine initiatives.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Fellow, National Association of Pediatric Nurse Practitioners (NAPNAP)
- Member-at-large, Executive Board, NAPNAP Three Rivers Chapter, 2017
- National Association of Nurse Practitioner Faculty
- National Association of Pediatric Nurse Practitioner Faculty
- Association of Faculty of Pediatric Nurse Practitioner Programs

- Allegheny County Immunization Coalition
- Sigma Theta Tau, Theta Mu Chapter, Eta Chapter, 1983
- Adolescent Health Care Special Interest Group, NAPNAP
- Pennsylvania Coalition of Nurse Practitioners, 2005 to the present
- NAPNAP legislative liaison, 2008–2015
- SERV-PA: State Emergency Registry of Volunteers for Pennsylvania

HONORS

- Dean's Distinguished Teaching Award, 2016
- Distinguished Clinical Scholar Award, 2013–2015
- Community Leadership and Service Award, 2014

Cherie Priya Dhar, MD

Cherie Priya Dhar directs resident and medical student rotations for the division. She is a full-time clinician educator and provides specialty services in menstrual disorders, reproductive health care, and care for transgender and gender-expansive youth. She is a medical consultant for the outpatient child and adolescent eating disorders program at the Children's Hospital Pine Center facility.

RESEARCH

Her research with refugee teenagers, titled "Attitudes and Beliefs Pertaining to Sexual and Reproductive Health Among Unmarried, Female Bhutanese Refugee Youth in Philadelphia," was published in the *Journal of Adolescent Health*.

She has joined the Association of American Medical Colleges' Medical Education Research Certificate Program to become more involved with the division's education research.

MAJOR LECTURESHIPS AND SEMINARS

- "Fertility Preservation for Transgender and Gender-Expansive Youth," Second Annual Conference on Transgender Health, Pittsburgh, Pa., September 2017
- "The Care of Transgender and Gender-Expansive Youth," Perioperative Nurses Conference, Pittsburgh, Pa., September 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Fellow, American Academy of Pediatrics
- Society of Adolescent Health and Medicine
- North American Society of Pediatric and Adolescent Gynecology

Nancy R. Eligator, MD

Nancy Eligator is a clinical assistant professor in the Departments of Medicine and Pediatrics. She is a practicing gynecologist in the Division of Adolescent and Young Adult Medicine in Oakland. She also practices in the General Internal Medicine Clinic at UPMC Montefiore. She is involved with training medical students, residents, and fellows; consults on adolescent gynecology patients; and supervises insertion of long-acting contraception methods.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Fellow, American College of Obstetrics and Gynecology
- Society for Lower Genital Tract Disorders
- American Balint Society
- North American Menopause Society

Erika Forbes, PhD

RESEARCH

Erika Forbes is a clinical and developmental psychologist whose research focuses on the role of neural reward circuitry in adolescent affective psychopathology, typical development, and substance use. She has contributed to the conceptual and empirical literature on reward in developmental psychopathology. She has led developmental neuroscience studies on adolescents and has used a multimethod approach that includes functional magnetic resonance imaging (fMRI), ecological momentary assessment, and behavioral methods. She is currently principal investigator (PI) of two NIH-funded R01 awards: one on the development of anhedonia during adolescence in a high-risk population (R01 MH104418), which includes an administrative supplement–funded study on anhedonia in sexual minority youth; and one on affective and cognitive flexibility in young adults with anorexia nervosa (R01 MH103230). She also is a recipient of a National Alliance for Research in Schizophrenia and Depression (NARSAD) Independent Investigator Award on inflammatory and growth factors, reward circuitry, and risk for depression; a past recipient of a NARSAD Young Investigator Award on neural reward circuitry and clinical course of adolescent depression; and a co-investigator on numerous federally funded studies (NIH and Department of Defense).

She recently completed an NIMH-funded R01 on the neurobiology of depression in high-risk adolescent girls; an NIDA-funded R01 (DA026222) to examine genetic, neural, and developmental factors in substance use in late adolescence; and an NIDA-funded R21 (DA033612) to develop a novel, ecologically valid fMRI paradigm to assess adolescents' neural responses to peer reward and test its value for predicting reward-related problems.

She has also been the recipient of a K01 award (MH074769) to study reward-related brain function and positive affect in natural settings in adolescent depression, and she has served as a co-investigator on an NIMH-funded Center for Intervention Development and Applied Research (CIDAR; P50 MH080215) grant on treatment for anxiety in young adolescents.

STUDY SECTIONS

- External referee, VIDI Grant Programme, Netherlands Organisation for Health Research and Development (also known as ZonMw)
- Ad hoc reviewer, NIH Cognition and Perception Scientific Review Group
- External referee, TOP Grant Programme, VENI Grant Programme, VICI Grant Programme, Netherlands Organisation for Health Research and Development
- External referee, Research Talent Grant Programme, Netherlands Organisation for Scientific Research
- Ad hoc reviewer, NIH Child Psychopathology and Developmental Disabilities Scientific Review Group
- Ad hoc reviewer, Children's Hospital of Michigan Foundation
- Ad hoc reviewer, Simons Foundation
- Ad hoc reviewer, NIH Conflict Study Section for Adult Psychopathology and Disorders of Aging
- Ad hoc reviewer, NIH Special Emphasis Panel for NICHD Program Project Applications
- Ad hoc reviewer, Medical Research Council of the United Kingdom, Career Development Award Program
- Ad hoc reviewer, NIH Special Emphasis Panel for Program Announcement on Accelerating the Pace of Drug Abuse Research Using Existing Data
- Ad hoc reviewer, NIH Behavioral Genetics and Epidemiology Study Section
- Ad hoc reviewer, NIMH BRAINS Program Study Section
- Ad hoc reviewer, NIH Conflict Study Section for Cognition and Perception

EDITORSHIPS

- Associate editor, *Psychological Science*
- Editorial Board, *Journal of Abnormal Psychology*
- Editorial Board, *Journal of Clinical Child and Adolescent Psychology*
- Editorial Board, *Journal of Abnormal Child Psychology*

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Psychological Association
- Society for Research in Child Development
- Society of Biological Psychiatry
- Society for Research in Psychopathology
- Full member, American College of Neuro-psychopharmacology
- International Society for Research on Child and Adolescent Psychopathology
- Association for Psychological Science
- Psychiatric Research Society

HONORS

- Young Investigator Award, NARSAD
- Travel Award, American College of Neuropsychopharmacology Emerging Mentor Award, WPIC
- Independent Investigator Award, NARSAD
  Eva King Killam Award, American College of Neuropsychopharmacology

Yukiko Giho, MSN, CRNP

Yukiko Giho is a new addition to the division (August 2016). She is a women's health nurse practitioner who excels in contraceptive counseling and management, providing patient-centered guidance for the full range of contraceptive options. Prior to joining the division, she served undergraduate and graduate students at the University of Pittsburgh Student Health Service, as well as provided ambulatory OB/GYN services in underserved communities in Western Pennsylvania through Adagio Health. Her expertise has increased accessibility to long-acting reversible contraception, including critically important opportunities for same-day placement for patients seen in the division's clinics. Her clinical duties include being a member of the division's collaborative practice to embed adolescent subspecialty care into Children's Community Pediatrics, where she enhances access to reproductive health services in the community setting. She has given lectures on contraceptive counseling to nurse practitioner students at the University of Pittsburgh for the past three years. At the division's Adolescent Medicine Conference in May 2017, she introduced Laughter Yoga, a unique practice to enhance resilience.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Vice president, Nurse Practitioner Association of Southwestern Pennsylvania
- Pennsylvania Coalition of Nurse Practitioners
- Association of Reproductive Health Professionals
- National Association of Nurse Practitioners in Women's Health
- American Association of Nurse Practitioners

Elissa Gittes, MD

Elissa Gittes brings to the division expertise in medical assessment and management of eating disorders and clinical experience in a wide range of adolescent-specific health care issues, such as menstrual disorders, mental health, acne, and contraception. She is involved with resident and fellow education at the Adolescent Clinic in Oakland. She oversees the Adolescent Clinic at the Children's Hospital Pine Center facility, offering her expertise to suburban youth. She is a medical consultant for the outpatient child and adolescent eating disorders program, now located in the Children's Hospital Pine Center facility.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- National Eating Disorders Association
- Society for Adolescent Health and Medicine

HONORS

- Best Doctors, *Pittsburgh Magazine*

Joanne Goodall, CRNP

Joanne Goodall is a nurse practitioner who has been with the division for 24 years. She provides acute and primary care to youth at the CAYAH and Family Links shelters. She primarily serves high-risk, vulnerable, and homeless youth and young adults. She collaborates with and coordinates services among many local medical, mental, and social service providers. She is the lead clinician of the Gender and Sexual Development Program, serving gender-expansive and transgender youth and young adults, and she is an active supporter of the division's research with lesbian, gay, bisexual, transgender, and queer (LGBTQ) minority youth. Goodall delivers numerous presentations to a variety of audiences on transgender care.

HONORS

- Unsung Hero Award, Homeless Children's Education Fund, 2014
- ACES Award, UPMC Award for Commitment and Excellence in Service, 2015
- Ambulatory Accolades Award, UPMC, 2015
- Excellence in Patient Experience, UPMC, 2016

Kara Mackinson Peters, LSW, MPH

Kara Mackinson Peters is the transition care coordinator/social worker in the Division of Adolescent and Young Adult Medicine. She coordinates the identification, assessment, and counseling of individuals who have complex medical and psychosocial needs and assists in the transition of adolescents and young adults into adult health care.

Peters leads the Mental Health QI project, which serves as the foundation of the division's integrated behavioral health model. The project involves collecting and analyzing data on the utilization and show rates for mental health and social work appointments to evaluate the effectiveness of the model and to address barriers through iterative modifications.

In her role as transition care coordinator, Peters participates in the hospital-wide Task Force 13, which aims to improve the process by which patients with complex health needs transition from pediatric to adult care. Peters co-leads the Transition Readiness QI project, which implements transition "vital signs" and readiness assessments with patients to promote patient self-efficacy regarding their health and health care transition.

Peters serves as a field instructor with a Health Resources and Services Administration grant for the University of Pittsburgh School of Social Work; the grant aims to develop and expand the workforce in substance abuse and mental health. Peters supervises master's students completing field placements through the grant within the Division of Adolescent and Young Adult Medicine, specifically with training and development in the Integrated Health Care Model. The workforce supports the behavioral health needs of children, adolescents, and transitional-age persons 16–25 years old who are at risk for mental illness, substance abuse, and suicide and who are among the least likely to seek continuous help.

BOARD AND ADVISORY COMMITTEE MEMBERSHIPS

- Task Force 13 for Health Care Transition, Children's Hospital of Pittsburgh

Michael Marshal, PhD

RESEARCH

Michael Marshal's primary research goals are to identify risk and protective factors for mental health problems among sexual and gender minority youth to inform and develop prevention and intervention programs. He is the PI on several studies funded by the NIH that examine longitudinal trajectories of substance use and associated mental health problems over time among sexual and gender minority youth (e.g., DA034619 and DA037958). Both of those studies have directly or indirectly used the division's Oakland clinic as a sampling venue. One study

examines stress-reactivity and long-term outcomes regarding substance use among heterosexual and sexual minority young women. Preliminary findings have been presented at national adolescent-focused conferences. The other study examines substance use and mental health disparities among gender minority youth, as well as outcomes over time. Marshal is the PI on a secondary data-analysis grant focused on examining trajectories of disparities over time regarding substance use among sexual minority females (DA030385). Marshal is a co-investigator or consultant on several other studies that examine mental health and wellness among these vulnerable and marginalized populations.

MAJOR LECTURESHIPS AND SEMINARS

- "Taking Stock of an Emerging Literature: Mental Health Disparities Among LGBTQ Youth and Their Implications for Healthcare Providers," invited talk, Transforming Care: Midwest Conference on LGBTQ Health Equity and HIV/AIDS, Columbus, Ohio, October 2016

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Association for the Advancement of Science
- American Psychological Association
- Research Society on Alcoholism
- Society for Research in Child Development

HONORS

- Predoctoral National Research Service Award, Mentored Research Scientist Development Award, NIAAA
- Early Career K-Award, Mentored Research Scientist Development Award, NIAAA

Loreta Matheo, MD

Loreta Matheo is the adolescent medicine fellowship program director. Until June 2016, she served as the medical leader for Duquesne University's Health Service, and she oversaw training and education in college health for the division's fellowship program. Currently, she is the director of the Transition Services Program in the division. Additionally, she is the Children's Community Pediatrics adolescent and young adult physician liaison, working to enhance educational opportunities for primary care pediatric practices regarding care for adolescents and young adults while also providing embedded subspecialty care in their offices as part of a collaborative care pilot program.

BOARD AND ADVISORY COMMITTEE MEMBERSHIPS

- Admissions Interviewing Committee, University of Pittsburgh School of Medicine
- Admissions Committee, University of Pittsburgh School of Medicine
- Diversity and Inclusion Committee, Children's Hospital of Pittsburgh
- Children's Hospital of Pittsburgh Transition Care Task Force 13
- Retain AYA Task Force

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- College Health Special Interest Group, Society for Adolescent Health and Medicine
- Section on Clinical Medicine, American College Health Association
- Fellow, Section on Adolescent Health, American Academy of Pediatrics

HONORS

- Best Doctors, *Pittsburgh Magazine*, 2014, 2015, 2016

Ann McCurdy, MSW, LSW

Ann McCurdy is a clinical social worker and transition care coordinator within the Division of Adolescent and Young Adult Medicine. She is lead faculty on a Health Resources and Services Administration training grant to train master's in social work students in integrated behavioral health and care for adolescents and young adults. McCurdy coordinates the identification, assessment, and counseling of individuals who have complex medical and psychosocial needs, with emphasis on the LGBTQ population. McCurdy serves as field instructor at the University of Pittsburgh School of Social Work, where she is responsible for the supervision of master's students completing field placements within the division. The role includes the 412 Youth Zone, a drop-in center for young adults 18–24 who are currently experiencing homelessness or who have aged out of the foster care system. McCurdy's primary social work interests include working with adjudicated and dependent youth, working with transgender adolescents and young adults, and connecting patients to resources throughout the community.

BOARD AND ADVISORY COMMITTEE MEMBERSHIPS

- Youth Support Provider Network, Allegheny County Department of Human Services
- Allegheny County Ending Youth Homelessness Initiative
- Allegheny County Department of Human Services Homeless Youth Leadership Group
- Board member, Cannon Fellowship, University of Pittsburgh School of Social Work

Gerald Montano, DO, MS

RESEARCH

Gerald Montano's overarching research aim is to improve the health and well-being of LGBTQ youth through the development of community-based and clinical interventions. His research involves identifying and describing differences in parent-child relationships between heterosexual youth and sexual minority youth—youth who identify as gay/lesbian/bisexual, have same-sex attractions, or have same-sex romantic/sexual partners—and the impact these differences have on the health of sexual minority youth.

Montano is a recipient of two recent grants. The first, titled "Identifying Differences in Parental Monitoring Between Sexual Minority and Heterosexual Girls Throughout Adolescence," is funded by the Children's Hospital of Pittsburgh Research Advisory Committee and seeks to identify how monitoring differences influence substance use between the two groups. The study will conduct a secondary data analysis of the Pittsburgh Girls Study—a community-wide longitudinal study of young women in the city of Pittsburgh—to identify such differences using multilevel regression and Cox models with non-proportional hazards.

The second grant, titled "Sexual Minority Youth Substance Use Prevention Strategies," is funded by the University of Pittsburgh Physician Foundation and seeks to describe the strategies parents use to prevent substance use by their sexual minority children and the experiences the children have with the strategies. Additionally, it seeks input from stakeholders in LGBTQ health to determine new and novel ways to prevent substance use among sexual minority youth. The study will use in-depth interviews and focus groups to achieve its aims.

Montano is also an expert in care for gender minority youth and organizes the annual Pittsburgh Transgender Conference for health professionals, in partnership with the Persad Center.

BOARD AND ADVISORY COMMITTEE MEMBERSHIPS

- Director, Youth Research Advisory Board, Children's Hospital of Pittsburgh of UPMC
- Treasurer/secretary, Ohio Valley Society of Adolescent Health and Medicine
- Medical education codirector, Transgender Care: Basic and Beyond for Providers, Children's Hospital of Pittsburgh of UPMC and Persad Center, Pittsburgh, Pa., September 2016

MAJOR LECTURESHIPS AND SEMINARS

- "The Journey Begins: Initial Medical Management for Transgender Youth," Trans Pride PGH Professional Conference, Pittsburgh, Pa., September 2016
- "Group-Based Trajectories of Parental Monitoring Components Between Sexual Minority and Heterosexual Girls and Their Associations with Substance Use," New Investigator Award Lecture, Society of Adolescent Health and Medicine, March 2017
- "Beyond Acceptance and Support: Parent-Adolescent Relationship Among LGBT Youth," keynote address, Greater Pittsburgh Psychological Association Continuing Education Conference, May 2017
- "Parent-Adolescent Relationship Among Sexual Minority Youth," Fifth Annual Adolescent Medicine Symposium, Children's Hospital of Pittsburgh of UPMC, May 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Society for Adolescent Health and Medicine
- Society of Pediatric Research
- Ohio Valley Society of Adolescent Health and Medicine

HONORS AND AWARDS

- Finalist, New Investigator Award, Society of Adolescent Health and Medicine

Ana Radovic, MD, MSc

RESEARCH

Ana Radovic was awarded an NIMH career-development award (1K23MH111922-01A1). The overarching goal of her research program is to increase use of mental health services by adolescents with depression and/or anxiety. Specifically, she has developed an intervention, SOVA or Supporting Our Valued Adolescents, which aims to address key mechanisms which affect service use using two moderated social media websites (one for parents, one for adolescents), with daily blog posts, peer interactions, and discussion guides. Currently, she is testing methods that increase user engagement with these sites and investigating factors which may affect implementation in primary care settings. She plans to test SOVA in a pilot randomized, controlled trial and learn more parent-adolescent communication factors regarding mental health and relationship quality in the context of a web-based intervention.

BOARD AND ADVISORY COMMITTEE MEMBERSHIPS

- Board member, FamilyLinks
- Mental Health Committee, Society for Adolescent Health and Medicine

MAJOR LECTURESHIPS AND SEMINARS

- "Screening for Depression in Adolescents Presenting to Primary Care," Montana American Academy of Pediatrics: Annual Pediatric Roundup Conference, Chico Hot Springs, Mont., 2016
- "Adolescent Well Care and Immunizations," Gateway Health webinar, June 2017
- "Social Media Use in Depressed and Anxious Adolescents: A Two-Way Street," grand rounds, Children's Hospital of Pittsburgh; Services for Teens at Risk Conference, WPIC; and National Alliance on Mental Illness Keystone Pennsylvania First Child and Adolescent Conference, 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Society for Adolescent Health and Medicine
- Society of Pediatric Research

HONORS

- Fellow, American Academy of Pediatrics, 2014
- Dean's Faculty Advancement Award, 2017

Dana Rofey, PhD

RESEARCH

Dana Rofey's research focus has been on prevention and intervention efforts to assist obese adolescents in increasing functional aspects of their lives. Her work emphasizes the importance of evidence-based interventions and bariatric surgery targeting multiple and synergistic regulatory processes (e.g., weight, mood, sleep). Through her research, she has provided evidence that behavioral and surgical interventions, delivered during adolescence, a time of optimal brain plasticity, can induce broad and positive shifts in behavioral trajectories. Moreover, Rofey has also found that for adolescents with severe obesity (> 150% ideal weight), surgery can improve cognitive, psychopathological, and functional domains. Her current work in this area focuses on the effects of obesity on the brain (both functional and structural) and mechanistic pathways. She has incorporated aspects of community-partnered, stakeholder-engaged obesity work, as well as served as an expert to train nurses and providers within the community to disseminate and implement evidence-based care. She emphasizes key windows of opportunity during the transition period—adolescents moving into adulthood—and the importance of providing high-quality services, as well as understanding how to effectively engage young adults. Rofey has received grant funding to further develop transition programs in the areas of transgender youth/young adults, type I diabetes, and youth without a primary care provider. Generally, Rofey's research focuses on adolescents, especially those transitioning to adulthood, across regulatory processes, with a focus on obesity and gender identity.

BOARD AND ADVISORY COMMITTEE MEMBERSHIPS

- President-elect/president, Clinical Management Section, Obesity Society, 2014–2017
- Chair, Membership Committee, Obesity Society
- Advisory Committee on Childhood Obesity, Pennsylvania Community Health Needs, 2015, 2017
- Membership Committee, Obesity Society, 2015 to the present
- Mental Health Advisory Board, Allegheny County
- NIH Scientific Review Committee, Clinical Endocrinology and Reproduction, 2015
- Advisory Committee on Childhood Obesity, Pennsylvania Community Health Needs, 2015
- Membership chair, Obesity Society, 2017
- President, Obesity Society Clinical Management, 2017
- Advisory Board, Pittsburgh Park Rx, 2017

MAJOR LECTURESHIPS AND SEMINARS

- "Bullying and Childhood Adversity as Predictors of Nonsuicidal Self-Injury Among Sexual Minority Adolescents in the Heathy Allegheny Teen Survey," Society of Adolescent Health and Medicine annual meeting, Washington, D.C., March 2016
- "Motivational Interviewing With Adolescents," Society of Adolescent Health and Medicine annual meeting, Washington, D.C., March 2016
- "Standardization for Diagnosing PCOS in an Adolescent Medicine Division," North American Society for Pediatric and Adolescent Gynecology, Toronto, Canada, April 2016
- "Transgender 101," Pittsburgh Public Schools, Pittsburgh, Pa., May 2016
- "Creating Evidence-Based Group Models for Transgender Adolescents and Young Adults," Annual Adolescent and Young Adult Health Research Symposium, Pittsburgh, Pa., May 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Psychological Association
- Academy for Eating Disorders
- Association for Behavioral and Cognitive Therapies
- Obesity and Eating Disorders Special Interest Group, Association for Behavioral and Cognitive Therapies

- Women's Special Interest Group, Association for Behavioral and Cognitive Therapies Obesity Society
- Eating Disorder Research Society
- Motivational Interviewing Network of Trainers

Joseph Sanfilippo, MD, MBA

RESEARCH

Joseph Sanfilippo is a collaborator on several studies that include epigenetic profiling of human embryonic stem cells and sperm DNA integrity, as well as post-concussion menstrual patterns in adolescent females. Over the past year, in his capacity as executive director of the North American Society for Pediatric and Adolescent Gynecology, he has overseen the work of the society's research committee.

BOARD AND ADVISORY COMMITTEE MEMBERSHIPS

- National Advisory Board, American Association of Gynecologic Laparoscopists
- Fellowship site inspector, Fellowship in Minimally Invasive Gynecology Boards
- Past member, Board of Trustees, American Association of Gynecologic Laparoscopists
- Executive director, North American Society for Pediatric and Adolescent Gynecology
- Member at large, Executive Committee, North American Society for Pediatric and Adolescent Gynecology
- Ex-officio board member, North American Society for Pediatric and Adolescent Gynecology
- Past president, American Society for Reproductive Medicine

EDITORSHIPS

- Editor in chief, *Journal of Pediatric and Adolescent Gynecology*, through April 2016
- Editorial Board, *Fertility and Sterility*
- Editorial Board, *Journal of Minimally Invasive Gynecology*

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Association of Gynecologic Laparoscopists
- Certified by American Board of Obstetrics and Gynecology with subspecialty certification in Reproductive Endocrinology and Infertility
- American College of Obstetricians and Gynecologists
- Accreditation Council for Gynecologic Endoscopy
- American Society for Reproductive Medicine (formerly the American Fertility Society)
- American Society for Psychosomatic Obstetrics and Gynecology
- Association of Professors of Gynecology and Obstetrics
- North American Society for Pediatric and Adolescent Gynecology
- Society for Reproductive Investigation
- U.S. International Foundation for Studies in Reproduction

HONORS

- Best Doctors, *Pittsburgh Magazine*, 2005 to the present
- Distinguished alumnus, Chicago Medical School, Rosalind Franklin University of Medicine and Science

MAJOR LECTURESHIPS AND SEMINARS

- "Text Messaging Yields High Research Response Rates to Track Menstrual Cycles and Patient-Reported Outcomes," Pacific Coast Reproductive Society 64th Annual Meeting: New Frontiers in Reproduction and Genetics, Rancho Mirage, Calif., March 2016
- "Text Messaging Yields High Research Response Rates to Track Menstrual Cycles and Patient-Reported Outcomes in Adolescents," 30th Annual Clinical and Research Meeting, North American Society for Pediatric and Adolescent Gynecology, Toronto, Canada, April 2016
- "Abnormal Menstrual Patterns in Young Women Following Sport-Related Concussion," Magee-Womens Research Day in Reproductive Biology and Women's Health, Pittsburgh, Pa., May 2016
- "A Prospective Examination of Abnormal Menstrual Patterns in Adolescent Female Athletes Following Concussion," American College of Sports Medicine 63rd Annual Meeting, Boston, Mass., June 2016

Tahniat Syed, MD

RESEARCH

Tahniat Syed is the clinical director for the division. She has expertise in adolescent reproductive health and menstrual disorders. She works closely with the front desk staff, nurses, medical assistants, and clinicians on operations of the Oakland CAYAH, south, and north offices. She is involved with teaching pediatric residents and adolescent medicine fellows. She also completed the Webster Leadership training course, which involved a project assessing advanced practice providers' training. She is a member of the Medical Standards Committee at Adagio Health. Syed is working on the development of the Advanced Practice Provider Fellowship Curriculum for the Division of Adolescent and Young Adult Medicine.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Fellow, American Academy of Pediatrics

TEACHING ACTIVITIES

Faculty members are engaged in a wide variety of educational activities geared toward advancing training in adolescent health for students of the health professions (including medicine, nursing, social work, psychology, public health, and pharmacy), residents, and fellows.

The Division of Adolescent and Young Adult Medicine offers a unique interdisciplinary learning environment where learners at various stages from a range of disciplines practice integrated care to improve the health and well-being of adolescents and young adults. This interdisciplinary training is supported through federal grants (such as a grant from the Health Resources and Services Administration to the School of Social Work), internal grants (such as a faculty development award to Cassidy for development of a learning experience for nurse practitioner students), and other foundation funds that encourage integrated care (such as the CHANGE program).

Clinical learning activities occur primarily through teaching sessions at CAYAH in Oakland, the Shuman Juvenile Detention Center, and the Adolescent Consultation Service at Children's Hospital in Lawrenceville. At CAYAH, trainees participate in primary care, family planning, and consultation services for a diverse population of teens and young adults from local neighborhoods and neighboring states. At the Shuman Center, residents provide health screening and acute medical care at the center's medical clinic. All pediatric residents experience a four-week outpatient adolescent medicine rotation, including clinical sessions at Shuman and CAYAH. All residents (and senior medical students) complete a half-day orientation, including a talk on motivational interviewing that is a component of their yearlong motivational interviewing curriculum. Additionally, all residents are scheduled to spend a day at the Center for Overcoming Problem Eating (a WPIC/Children's Hospital collaboration). There, they follow a triple-boarded attending (Alexis Fertig) through the assessment process, inpatient unit rounds, and case discussions. They spend at least one session with an obstetrics/gynecology faculty member, which allows exposure to long-acting reversible contraception for adolescents. They also visit a drop-in center and clinic (overseen by the division) for unaccompanied youth and young adults in downtown Pittsburgh at the Gay and Lesbian Community Center and at the 412 Youth Zone. Finally, all residents are encouraged to provide a summative 15-minute talk on a topic of their choosing regarding an aspect of adolescent health, highlighting the relevant evidence base.

Students participate in clinical education activities for a week during their second-year ambulatory medicine rotations or as senior students in a monthlong elective rotation. In addition to learning at CAYAH, students have opportunities to experience adolescent care at Carlow Health Services, 412 Youth Zone, and other community-based sites.

Outside those clinical teaching activities, the faculty members serve as course directors, facilitators, or instructors in a variety of medical school and public health courses, including Introduction to Medical Interviewing, Advanced Medical Interviewing, Methods and Logic in Medicine, Pediatric Advanced Physical Exam, and courses in community-partnered research. Faculty members serve as advisors and mentors to medical students, residents, fellows, and graduate students from a number of different departments and schools (including family practice, obstetrics/gynecology, internal medicine, public health, education, social work, nursing, and pharmacy). Other teaching activities include presenting noon conferences for residents and students, as well as mentoring research projects for fellows, residents, and students. As noted, faculty members deliver numerous invited presentations locally, nationally, and internationally.

Dhar, Syed (clinical director for the division), and Radovic (associate fellowship program director) oversee a range of teaching activities within the division. Current teaching responsibilities include: directing the required PGY-2 rotation in adolescent medicine for pediatrics, medicine-pediatrics, and triple-board residents; precepting fellows in adolescent medicine; precepting and mentoring interdisciplinary trainees in the Leadership Education in Neurodevelopmental and Related Disorders Program; coordinating the Combined Ambulatory Medicine and Pediatrics Clerkship (CAMP-C) ambulatory care course for second-year students; lecturing for the pediatric resident noon conferences; codirecting Advanced Medical Interviewing, a required four-week course for second-year medical students; providing monthly lectures for third-year medical students (Introduction to Adolescent Medicine); giving lectures for medical students signed up for the pediatric clerkship Introduction to Adolescent Medicine; and mentoring graduate students across disciplines for community-oriented projects sponsored by the Bridging the Gaps Program and the Albert Schweitzer Foundation.

Dhar is actively involved with teaching of adolescent medicine fellows. She has started a monthly lecture series for residents on the adolescent medicine clerkship on topics such as sexually transmitted infections, contraception, abnormal uterine bleeding, and care for gender-expansive youth. She oversees the confidentiality lectures for all CAMP-C students for the University of Pittsburgh School of Medicine. Dhar conducts Advanced Medical Interviewing and Advanced Physical Exam courses for medical students and is a facilitator for the Diversity Seminar Series for the University's class of 2021.

Matheo, the fellowship program director, provides mentorship to students, residents, and fellows in the health professions. She provides clinical supervision of adolescent medicine fellows; pediatric, medicine-pediatrics, and triple-board residents; and medical students in the inpatient setting and in the outpatient Adolescent Medicine Clinic, as well as Duquesne University's Health Service. She serves as the medical advisor for the University's Eating Disorder Team. Additional teaching duties include precepting the PGY-2 residents at the Shuman Center and precepting second-year medical students for the CAMP-C ambulatory care course. From 2011 to the present, she has had an average of four students per session for a total of four sessions per course. Matheo is also an instructor in Advanced Medical Interviewing.

Gittes provides mentorship to first-year medical students through the FAST advising program.

The CRNPs (Goodall, Cassidy, Byers, and Giho) serve as faculty preceptors for pediatric and family nurse practitioner students; the physician assistants (Ziegler and Richardson) serve as faculty preceptors for the physician assistant students from Chatham University. Cassidy oversees the training of nurse practitioner students, including a course at the School of Nursing. Ziegler is working with Syed on the development of the Advanced Practice Provider Fellowship Program.

Faculty members provide clinical teaching to the residents and fellows on a rotating basis. Miller is responsible for teaching school health and trauma-informed care to the pediatric residents during their community health and advocacy rotation.

RESEARCH MENTORSHIP

Miller is a research mentor for multiple junior faculty members in pediatrics, psychiatry, and social work; fellows in adolescent medicine, hematology/oncology, gastrointestinal medicine, internal medicine, and general academic pediatrics; graduate students in public health and social work; and medical and nursing students. She participates in research training for the adolescent fellowship program, and she oversees multiple medical student scholarly projects, doctoral nurse practitioner student capstone projects, and public health and social work practicum projects. Current research mentees have produced multiple publications and presented their work nationally at meetings of the Society for Adolescent Health and Medicine and the American Public Health Association. She received the 2013 Distinguished Mentor Award from the Institute for Clinical Research Education at the University of Pittsburgh as well as the Philip Troen, MD, Excellence in Medical Student Research Mentoring Award from the University of Pittsburgh School of Medicine in 2017.

Marshal is engaged in a host of teaching and mentoring activities, which have produced several training grants funded by the NIH and peer-reviewed publications.

- He is a co-mentor on four K awards and one F31.
- He designed and teaches a graduate-level course on applied multiple regression analysis in the Graduate School of Public Health.
- He teaches quantitative methodology, SPSS programming, and developmental psychopathology to medical students, residents, and fellows in the Department of Pediatrics (Adolescent and Young Adult Medicine Clinic at Children's Hospital).
- He contributes to two research and teaching seminars for the Center for Research on Health and Sexual Orientation and the LGBTQ Health Disparities Certificate Program in the Graduate School of Public Health.

Montano and Radovic provide research mentorship to undergraduate, medical, and public health students, including oversight of scholarly projects.

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wilson

DIVISION OF

BLOOD AND MARROW TRANSPLANTATION AND CELLULAR THERAPIES

Mission

The clinical mission of the Division of Blood and Marrow Transplantation and Cellular Therapies (BMT&CT) is to design and test disease-specific, biologically rational, novel, reduced-toxicity transplantation regimens for patients with high-risk leukemia or lymphoma and for those afflicted with life-threatening inherited conditions that can lead to bone marrow failure, immune deficiency, autoimmune diseases, and neurodegenerative conditions. Less-intense chemotherapy is combined with risk-tailored and pharmacologically personalized immunosuppression. Post-transplant cell therapy is offered to decrease disease recurrence and reduce infectious complications with the overarching goals to improve quality of life and disease-free survival after cord blood or bone marrow transplantation.

The division's translational research mission focuses on developing new cellular therapy programs and testing the use of bone marrow transplantation following solid organ transplantation to establish immunity, extend organ survival, and achieve eventual tolerance. Some patients come to Pittsburgh as their last hope after being too sick to be treated anywhere else.

The division's laboratory-based research mission focuses on elucidating mechanisms of alloreactivity (the biological principle driving graft-versus-host disease [GVHD] and rejection) and mechanisms essential for successful tolerance. These studies will help design new and better drugs and immunotherapy interventions.

FACULTY

Paul Szabolcs, MD
Professor of Pediatrics
and Immunology
Chief, Division of BMT&CT
Program Director, BMT&CT
Medical Director, Blood and Marrow
Processing Laboratory

Jessie Barnum, MD
Assistant Professor of Pediatrics

Craig Byersdorfer, MD, PhD
Assistant Professor of Pediatrics
and Immunology

Beth Carella, MD
Assistant Professor of Pediatrics

Xiaohua Chen, PhD
Research Assistant Professor
of Pediatrics

Randy Windreich, MD
Assistant Professor of Pediatrics
Director, BMT&CT Fellowship
Training Program
Director, Pediatric Hematology/
Oncology/BMT&CT
Outpatient Clinic

OVERVIEW OF DIVISION

The Division of BMT&CT was established in July 2011 upon the arrival of Division Chief Paul Szabolcs from Duke University. Prior to that, it was called the Blood and Marrow Transplantation (BMT) Program, operated as a part of the Pediatric Hematology/Oncology Division, and averaged 20–24 transplants a year.

In 2017, the BMT&CT division had five faculty physicians with clinical activities. Since the arrival in August 2014 of Craig Byersdorfer, a clinician-scientist, all clinical transplant services have been provided by members of the BMT&CT division. The division is poised to perform 40–50 transplants per year over the next two to three years and to open novel clinical protocols that will primarily recruit patients from out of state.

The division is the only center in the world with the ability to successfully engraft children suffering from sickle cell disease, thalassemia, and many other disorders with a reduced-intensity regimen paired with a single-unit, human leukocyte antigen (HLA)-mismatched cord blood graft (ClinicalTrials: NCT01962415). A CliniMACS® device has been successfully implemented since 2012 to support novel clinical trials with T-cell-depleted autologous transplantation for Crohn's disease and T-cell-depleted HLA-mismatched allogeneic bone marrow transplantation, all approved by the U.S. Food and Drug Administration as an Investigational New Drug (IND).

The division is the only center in the world to offer tandem lung and bone marrow transplantation for pediatric and adult patients with immune deficiencies who have progressed to pulmonary failure by recovering organ and marrow from the same deceased HLA-mismatched unrelated donor. This programmatic effort is supported by the National Institute of Allergy and Infectious Diseases (NIAID) in the National Institutes of Health (NIH). Mechanistic laboratory studies will analyze acquisition of mucosal immunity and tolerance. Ongoing collaborations with University of Pittsburgh investigators and UPMC clinicians will extend this concept toward new indications with disease-specific protocols opening in fiscal year 2018.

CLINICAL ACTIVITIES

During the academic year of 2017, the division performed 38 transplants. Most transplant grafts were procured from unrelated allogeneic donors, with unrelated cord blood (UCB) grafts being most common. By 2016, the use of unrelated bone marrow grafts had exceeded the use of HLA-matched sibling marrow transplantation. Autologous mobilized stem cell rescue is performed for children with high-risk neuroblastoma and brain tumors. Haploidentical transplants were performed by both *in vitro* T-cell depletion and by post-transplant cyclophosphamide administration. With all possible transplant modalities on site, the division can find a suitable donor for any patient who may benefit from hematopoietic stem cell transplantation (HSCT).

One of the division's signature protocols (ClinicalTrials: NCT01962415) has attracted patients from two dozen states, ranging from Florida to Alaska. Patients with about 20 unique genetic diagnoses are enrolled on this reduced-intensity conditioning (RIC) trial. Diagnoses range from sickle cell disease, thalassemia, osteopetrosis, Krabbe disease, metachromatic leukodystrophy, to many primary immune deficiency syndromes such as BLS and X-linked inhibitor of apoptosis

deficiency. Day 100 non-relapse mortality has remained exceptionally low; there have been no deaths during this most vulnerable transplant period. With more than three dozen patients enrolled so far, one-year event-free survival exceeds 90% in the unrelated cord blood transplant setting, exceeding the results of centers of excellence worldwide. In 2016, the division opened an institutional prospective trial for children and young adults afflicted with high-risk acute myeloid leukemia (AML), employing RIC and myeloablative conditioning for HSCT in AML/myelodysplastic syndromes (ClinicalTrials: NCT02626715). To bridge the temporary post-transplant immune-deficient state, the division has performed therapeutic T-cell infusions with adenovirus hexon-specific interferon gamma-captured cells under IND/Institutional Review Board (IRB)-approved treatment plans. These will pave the way for new protocols that will open in 2018 and take advantage of a new CliniMACS® Prodigy device and conceptual advances.

RESEARCH AND OTHER SCHOLARLY ACTIVITIES

Paul Szabolcs, MD

RESEARCH

Paul Szabolcs is a physician-scientist whose clinical and research interests are focused on the biology of donor-derived cellular immunity and modulating alloreactivity in recipients of allogeneic hematopoietic cell transplantation (HCT). The overall goal of his efforts is to develop novel diagnostic and therapeutic approaches to accurately diagnose, predict, and therapeutically accelerate post-transplant immune reconstitution without increasing GVHD. He has developed a research program to elucidate mechanisms essential for successful tolerance. These studies will help to develop novel immunotherapy interventions. He continues to focus on unrelated cord blood transplantation (UCBT) as the dominant clinical scenario and laboratory model. His work has continued to influence the global field of transplantation medicine, especially the translational science of HLA-mismatched bone marrow transplantation as it is applied in tandem with solid organ transplantation.

LABORATORY-BASED RESEARCH

Graft Engineering and Immunotherapy After UCBT. The Szabolcs lab studies HCT recipient and donor pairs to analyze the development of protective immunity and to modulate T-cell responses toward amplifying leukemia-reactive T cells. Szabolcs and Xiaohua Chen study active mechanisms responsible for development of tolerance in HLA-mismatched cord blood recipients. This research aims at developing innovative immunomodulatory strategies relevant to multiple disease categories.

Immune Reconstitution After Cord Blood Transplant. Szabolcs and Chen perform correlative studies of T-cell immune reconstitution in ongoing clinical trials utilizing UCBT to identify the predictors of clinical outcomes. Since late 2012, the research has increasingly focused on elucidating mechanisms of tolerance in mixed chimerism and understanding the role of regulatory T cells in those with tolerance and those without, namely those suffering from GVHD.

Paul Szabolcs, MD
Division Chief, Blood and Marrow Transplantation and Cellular Therapies

CLINICAL RESEARCH

Reduced-Intensity UCBT for Children With Rare Metabolic and Primary Immune Deficiency (PID) Disorders. Children with PID syndromes, even those who may have significant comorbidities, can be cured with reduced-toxicity regimens. The CHP BMT&CT medical team works closely with collaborating services, in particular the Program for the Study of Neurodevelopment in Rare Disorders (NDRD) at CHP and Medical Genetics, to identify those who may benefit from HCT. The team offers a novel RIC regimen (ClinicalTrials: NCT01962415) that is testing the hypothesis that it can reduce/eliminate transplant-related mortality and achieve superior neurocognitive outcomes compared to traditional myeloablative conditioning for a wide variety of inherited metabolic disorders, including but not limited to Krabbe disease, metachromatic leukodystrophy, and mucopolysaccharidosis syndromes.

In fiscal year 2017, Szabolcs focused on a new UPMC initiative called the Immune Transplant and Therapy Center (ITTC), where novel auto-transplant protocols will be developed for autoimmune disorders such as inflammatory bowel disease and scleroderma. With Beth Carella, MD, Szabolcs continues to design allogeneic transplant protocols

to address HLA-matched donor availability in sickle cell disease by offering HLA-mismatched unrelated donor and/or haploidentical transplantation in children and adults. Barnum's virus-specific T-cell therapy protocol will play a critical support role in this initiative.

Autologous Stem Cell Transplantation With CD34-Selected Peripheral Blood Stem Cells (PBSCs) in Pediatric and Young Adult Patients With Severe Crohn's Disease and Other Autoimmune Disorders (ClinicalTrials: NCT0692939). This study continues to evaluate the safety and efficacy of high-dose immunotherapy followed by infusion of autologous CD34-selected PBSCs in pediatric and young adult patients who are refractory to all other treatment modalities.

Tandem Solid Organ Transplant and T-Cell-Depleted BMT. This new treatment modality was developed to transplant in stages two organs, both procured from a partially HLA-matched cadaveric donor. The therapy addresses the unmet need to offer meaningful and high-quality life to children and young adults who have pulmonary or other end organ failure and severe immune deficiency. Szabolcs was the sponsored investigator for this protocol; collaborators came from the Thomas E. Starzl Transplant Institute, Mellon Institute, University of Pittsburgh Cancer Center, and Children's Hospital of Pittsburgh. The team continued to enroll patients on a protocol titled "Sequential Cadaveric Lung and Bone Marrow Transplant for Primary Immune Deficiency Diseases" (ClinicalTrials: NCT01852370). One patient received a lung transplant for IL-7R null severe combined immunodeficiency in September 2015 and received the bone marrow transplant from the same cadaveric donor in January 2016. She was the first patient in the world to engraft with one of eight high-resolution HLA-matched cadaveric bone marrow, and she successfully weaned off immunosuppression in May 2017. This is also the first case of engraftment and immune reconstitution with a bone marrow graft prepared from vertebral bodies. Adult and pediatric patients with no other life-saving alternatives have come from as far as Texas and Rhode Island to enroll on this research protocol. This work was supported by NIAID/NIH R34 and UO1 grants to Szabolcs as communicating co–principal investigator (PI).

Cadaveric Donor Lung and Bone Marrow Transplantation in Immunodeficiency Diseases. 1U01AI125050-01: NIH/NIAID, 07/06/16-06/30/21. For patients with PID who develop the complication of end-stage lung disease, neither BMT nor lung transplantation are therapeutic options. This is the first clinical trial to evaluate the safety and efficacy of a *combined-tandem* strategy for lung transplant followed by BMT to correct the defective immune system, using the same organ donor. The lab performs tests to determine whether BMT restores the ability to fight infection and allows acceptance of the lungs, which would permit eventual withdrawal of all immunosuppression medications in these unique, combined transplant recipients. Ancillary mechanistic studies will test for global and mucosal immune competence, and others will test for mechanisms of tolerance. Szabolcs is the communicating PI. John McDyer, MD, UPMC Adult Lung Transplantation, is co-PI. Multiple investigators have joined this translational research proposal from Children's Hospital of Pittsburgh (Geoff Kurland, MD, Marian Michaels, MD, and Jay Kolls, MD, PhD), Presbyterian Hospital of UPMC, and the University of Pittsburgh Department of Immunology (Fadi Lakkis, MD, and Dario Vignali, PhD).

MAJOR LECTURESHIPS AND SEMINARS

- "Studies on Alloreactivity and Tolerance After Peripheral Blood and Cord Blood Transplantation: Is There a Tipping Point?" University of Pittsburgh Cancer Institute, Cancer Immunology Program Seminars, Pittsburgh, Pa., April 2016
- "Lung Transplantation in Tandem with Bone Marrow Transplantation from Partially HLA-Matched Deceased Donors: Clinical and Mechanistic Studies," 2016 STI Scientific Retreat, Pittsburgh, Pa., November 2016
- "Reduced-Intensity Transplantation Is Effective for Multiple Genetic Diseases: The Pittsburgh Protocol," Dubai-Arab Medical Congress, 2017
- "Reduced-Intensity Unrelated Donor Transplantation for Non-Malignant Diseases: A Journey Toward Combined Organ and Marrow Transplantation," hematology grand rounds, UPMC Shadyside Hospital, University of Pittsburgh Cancer Institute, Pittsburgh, Pa., March 2017
- "Mechanisms of Tolerance Following HLA-Mismatched Cord Blood Grafts: A Journey Toward Tandem Cadaveric Organ + Marrow Transplantation," grand rounds, Memorial Sloan Kettering Cancer Center, New York, N.Y., June 2017
- "Mechanisms of Tolerance Following HLA-Mismatched Cord Blood Grafts: A Journey Toward Tandem Cadaveric + Organ Marrow Transplantation," Children's Hospital of Pittsburgh of UPMC Molecular Medicine Research Seminar, June 2017

EDITORIAL BOARDS

- *Cytotherapy*
- *Blood Research*
- *American Journal of Transplantation*

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Society of Hematology
- American Society for Blood and Marrow Transplantation
- Center for International Blood and Marrow Transplant Research (CIBMTR)
- American Association of Immunologists
- Society for Pediatric Research Clinical Immunology Society
- Federation of Clinical Immunology Society
- UPCI
- Fellow, American Academy of Pediatrics
- International Society for Cellular Therapy

Jessie Barnum, MD

RESEARCH

Haploidentical Viral-Specific T Lymphocytes to Treat Persistent Reactivation or Infection With Adenovirus, Cytomegalovirus, and Epstein-Barr Virus After HCT or Solid Organ Transplantation. Jessie Barnum focuses on improving available therapy for patients with serious and often life-threatening viral infections after BMT. Available antivirals are quite toxic and often ineffective. She continues toward an institutional investigator-initiated protocol of T-cell immunotherapy for adenovirus, cytomegalovirus, and Epstein-Barr virus.

Outcomes of Human Adenovirus Infection and Disease in Pediatric Allogeneic Stem Cell Transplant Recipients: A Prospective, Multicenter, Observational Cohort. Barnum is a co-investigator and the BMT lead on this NIAID-funded, prospective, multicenter trial designed to study risk factors for severe adenoviral infections in stem cell transplant recipients. This protocol opened at Children's Hospital of Pittsburgh in July 2017, and it is projected to enroll 86 allogeneic transplant patients in the next five years.

Bilateral Orthotopic Lung Transplant in Tandem With CD3+ and CD19+ Cell-Depleted Bone Marrow Transplant From Partially HLA-Matched Cadaveric Donors. Barnum is a co-investigator for the Tandem Solid Organ Transplant and T-Cell-Depleted Bone Marrow Transplant protocol. This new treatment modality was developed to transplant lungs followed by bone marrow, both procured from a partially HLA-matched cadaveric donor. The therapy addresses the unmet need to offer meaningful and high quality of life for children and young adults who have significant respiratory insufficiency and severe immune deficiency. Barnum is one of two physicians fully trained to procure vertebral bodies from cadaveric donors and has performed this procedure on two occasions. She has trained other faculty in this procedure and wrote a standard operating procedure for use in the operating room.

Naïve T-Cell Depletion for Prevention of Chronic GVHD in Children and Young Adults. Barnum serves as principal investigator on this CIBMTR study, which is a multicenter, phase II, randomized, controlled trial comparing outcomes in pediatric patients receiving allogeneic HCT with either naïve T-cell-depleted peripheral blood stem cells or T-cell-replete bone marrow. This trial has progressed to the initial feasibility phase; the randomized, controlled trial will follow.

Use of Miltenyi Biotec's CliniMACS® CD34 Reagent System as a Humanitarian Use Device for Isolation of Hematopoietic Stem Cells or T-Cell Depletion in Multiple Settings. Barnum wrote this protocol to study novel graft manipulation to decrease the risk of GVHD. It was used successfully for a patient with Fanconi anemia and many comorbidities. It remains open for other patients at Children's Hospital of Pittsburgh.

Primary Immune Deficiency Treatment Consortium Protocol 6901: A Prospective Natural History Study of Diagnosis, Treatment, and Outcomes of Children With Severe Combined Immune Deficiency Disorders. Barnum serves as principal investigator on this multicenter, prospective protocol.

Primary Immune Deficiency Treatment Consortium Protocol 6903: Analysis of Patients Treated for Chronic Granulomatous Disease Since 1995. Barnum serves as co-investigator on this multicenter, prospective, retrospective, and cross-sectional protocol.

Primary Immune Deficiency Treatment Consortium Protocol 6904: Analysis of Patients Treated for Wiskott Aldrich Syndrome Since 1990. Barnum serves as co-investigator on this multicenter, prospective, retrospective, and cross-sectional protocol.

MAJOR LECTURESHIPS AND SEMINARS

- "Infectious Complications After BMT," *Association of Pediatric Hematology/Oncology Nurses* (APHON) course, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., September 2016, February 2017, and June 2017
- "Renal Complications After BMT," APHON course, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., September 2016, February 2017, and June 2017
- "Critical Illness After BMT: Respiratory Complications," Pediatric Intensive Care Unit Fellow Didactic Session, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., November 2016
- "Tolerance Strategies," BMT and liver transplant focus group on immune tolerance, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., November 2016
- "Infections in the Immunocompromised Host," Pediatric Hematology and Oncology Fellow Core Lecture Series, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., March 2017
- "Immune Manipulations in Transplantation," Pediatric Hematology, Oncology, Bone Marrow Transplant, and Cellular Therapies Conference, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., April 2017
- "Successful Engraftment, Immune Reconstitution, and Clinical Evidence of Immune Tolerance Following Cord Blood and Intestinal Transplant for Immunodeficiency and Intestinal Failure," poster presentation, Pediatric Immune Deficiency Treatment Consortium Scientific Workshop, Bethesda, Md., May 2017
- "Successful Engraftment, Immune Reconstitution, and Clinical Evidence of Immune Tolerance Following Cord Blood and Intestinal Transplant for Immunodeficiency and Intestinal Failure," oral presentation, Pediatric Immune Deficiency Treatment Consortium Educational Workshop, Bethesda, Md., May 2017
- "Successful Engraftment, Immune Reconstitution, and Prope Immune Tolerance Following Cord Blood and Intestinal Transplant for Immunodeficiency and Intestinal Failure," poster presentation, XV International Congress of the Intestinal Rehabilitation and Transplant Association, New York, N.Y., June 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Society of Bone Marrow Transplantation
- American Society of Hematology
- American Society of Pediatric Hematology/Oncology
- Transplantation Society
- Primary Immune Deficiency Treatment Consortium
- Children's Oncology Group

Craig Byersdorfer, MD, PhD

RESEARCH

Craig Byersdorfer focuses on the biology of GVHD-causing T cells following allogeneic transplantation to develop novel therapeutics to mitigate GVHD while preserving homeostatic immune reconstitution and graft-versus-leukemia effects. Specifically, Byersdorfer's research program seeks to elucidate the specific metabolic pathways that are upregulated in GVHD-causing T cells. His novel approach to disease pathogenesis continues to aim at innovative therapies. His findings on T-cell metabolism have implications extending beyond BMT to solid organ transplantation and long-term anti-leukemia responses.

LABORATORY-BASED RESEARCH

The Role of AMP-Activated Protein Kinase (AMPK) in Alloreactive T Cells. AMPK is a well-known energy sensor and is activated early in T cells during a GVHD response. Byersdorfer has shown that lack of AMPK leads to decreased rates of GVHD but preserves anti-leukemia responses. Further work has demonstrated that a lack of AMPK has consequences for both effector T cells and the generation of regulatory T cells, favoring a tolerogenic response. Future studies will utilize animal models and AMPK knockout cells to determine the mechanisms of improved GVHD in the absence of AMPK signaling.

Transcriptional Control of Fatty Acid Metabolism in Alloreactive T Cells. The Byersdorfer laboratory has previously demonstrated that GVHD-causing T cells increase their dependence on the oxidation of fat. The laboratory found that transcriptional control of fat oxidation depends on signaling through peroxisome proliferator-activated receptors (PPARs), notably PPAR-δ. His lab continues to generate PPAR-δ-deficient mice to determine its role in GVHD propagation and to define whether PPAR-δ is a potential therapeutic target for GVHD treatment.

Using Metabolic Manipulation to Improve Anti-Leukemia Responses. One of the challenges to treatment with chimeric antigen receptor (CAR) T cells for acute lymphoblastic leukemia is its frequent inability to persist *in vivo*. The Byersdorfer laboratory seeks to improve the *in vivo* persistence of CAR T cells by reprogramming their metabolism through constitutive expression of activated AMPK or PPAR-δ and thus increase anti-leukemia efficacy.

CLINICAL RESEARCH

Reprogramming Human T Cells. Having determined the metabolic pathways present in murine alloreactive T cells, the Byersdorfer laboratory has moved on to examine whether similar metabolic changes occur in human T cells during GVHD and whether their elimination mitigates GVHD. Clinical samples will be collected from human patients at the diagnosis of acute GVHD and evaluated so that the researchers can determine the metabolic pathways present. The laboratory has continued to pursue studies using gene-editing programs like CRISPR-Cas9 to eliminate metabolic proteins in human T cells and then test them in xenogeneic models of GVHD before transitioning them to clinical application.

MAJOR LECTURESHIPS AND SEMINARS

- "An Unexpected Role for AMP-Activated Protein Kinase During Graft-Versus-Host Disease," hematology/oncology/BMT seminar series, Ben Towne Cancer Center, Seattle, Wash., May 2016
- "How I Learned to Stop Worrying and Love the Bomb Biochem," keynote address, annual physician-scientist training program research symposium, University of Pittsburgh, Pittsburgh, Pa., November 2016

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Society of Hematology
- American Society for Blood and Marrow Transplantation
- American Association of Immunologists
- Second appointment, Department of Immunology, University of Pittsburgh

HONORS

- First place, Junior Faculty Basic Science Research, annual Immuno-Oncology Young Investigators Forum, Houston, Texas, March 2016
- Hyundai Hope Scholar, May 2016
- American Society of Hematology Scholar, November 2016

Beth Carella, DO

RESEARCH

Beth Carella's clinical research focuses on expanding utilization of stem cell transplantation for patients with sickle cell disease. Collaborating with Szabolcs, she continues to develop a clinical trial to broaden opportunities for patients lacking matched sibling donors. With mismatched donors, the risk of GVHD is high; however, this protocol will use *in vitro* T-cell depletion to reduce risks. This approach will allow a larger number of severely affected patients to access a curative intervention. With an RIC regimen, the trial aims to allow for successful engraftment while reducing toxicity for this non-malignant disease. Carella is the institutional principal investigator for the BMTCTN STRIDE II protocol, which compares standard of care to myeloablative, matched transplantation for patients with sickle cell disease. She is the institutional principal investigator for the PBMTC SUP1601 protocol, which aims to identify pathogens in stem cell transplant patients with lower respiratory tract infections.

MAJOR LECTURESHIPS AND SEMINARS

- "HLA Typing, Part 1," oral presentation, Hematology/Oncology Fellow Lecture Series, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., February 2017
- "HLA Typing, Part 2," oral presentation, Hematology/Oncology Fellow Lecture Series, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., June 2017

ADVISORY COMMITTEE MEMBERSHIPS

- University of Pittsburgh IRB, June 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Society of Pediatric Hematology/Oncology
- American Society of Blood and Marrow Transplant
- American Society of Hematology
- Children's Oncology Group
- American Academy of Pediatrics

Xiaohua Chen, PhD

RESEARCH

Xiaohua Chen focuses on characteristics and corresponding biomarkers that are essential for human transplant tolerance and their potential for application in organ transplantation. She also monitors immune reconstitution in ongoing clinical protocols.

Cadaveric Donor Lung and Bone Marrow Transplantation in Immunodeficiency Diseases. As a co-investigator, Chen spearheads the tolerance and immune competence studies in the Szabolcs laboratory.

Regulatory T Cells and Tolerance in HSCT. Clonal deletion of alloreactive thymocytes is a critical and central mechanism in forming long-term tolerance; however, hyporeactivity may depend on additional peripheral mechanisms with or without immunosuppression. The degree of influence from peripheral factors is unknown. Chen's studies explore the mechanisms of long-term immune tolerance formed in allo-HSCT and their potential in organ transplantation. Both central (clonal deletion) and peripheral (anergy, Treg, Tr1) tolerance are being examined in the HLA-mismatched cord blood and deceased donor bone marrow transplant setting. She uses high-throughput digital sequencing to track clonal evolution versus deletion. She characterizes Treg cells and evaluates anergy in patients who achieve immune tolerance and those with GVHD to continue toward mapping the longitudinal evolution of immune tolerance mechanisms and plot efficacy of GVHD treatments. Evolution of Treg and alloreactive T-cell clonotypes are studied after tandem lung and bone marrow transplant.

Cellular and Molecular Monitoring of Immune Reconstitution Post-HSCT in Ongoing Clinical Protocols. This project applies reliable approaches in cellular and molecular monitoring of immune reconstitution in ongoing clinical protocols. By using multiple *fluorescence-activated cell sorting* panels, TCRb, TCRgd BCR spectratyping, and sjTREC real-time polymerase chain reaction, Chen monitors immune recovery post-HSCT.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Collaborative Institutional Training Initiative
- American Society of Hematology
- American Association of Immunologists
- Federation of Clinical Immunology Societies

Randy Windreich, MD

RESEARCH

Randy Windreich's clinical research focuses on HSCT for acute leukemias, using drug pharmacokinetics and pharmacodynamics to individualize and optimize therapy, particularly within the pediatric BMT patient population, as well as alternative uses for hematopoietic stem cells.

A Phase II Study of Myeloablative and RIC Regimens for Children With Acute Myeloid Leukemia or Myelodysplastic Syndrome Undergoing Allogeneic HSCT. The objective of this study is to determine safety, preliminary efficacy, and event-free survival at six months in pediatric patients receiving a myeloablative or reduced-intensity preparative regimen prior to HSCT for high-risk acute myeloid leukemia and myelodysplastic syndrome. Windreich is developing an institutional protocol for a dual-arm myeloablative and reduced-intensity transplant conditioning regimen for patients with hematologic malignancies and unrelated donors, with an emphasis on acute myeloid leukemia and acute lymphoblastic leukemia. The protocol will expand eligibility for HSCT, particularly for those with serious pre-transplant comorbidities. Subject enrollment continues.

A Multicenter, Randomized, Double-Blind, Placebo-Controlled, Parallel-Group Study to Evaluate the Efficacy, Safety, and Tolerability of Transendocardial Injection of Ixmyelocel-T in Subjects With Heart Failure Due to Ischemic Dilated Cardiomyopathy. This is an industry-sponsored, multicenter study through Vericel Corporation (formerly Aastrom Biosciences) (Ann Arbor, Mich.), in collaboration with the UPMC Heart and Vascular Institute at UPMC Presbyterian Hospital. The objective of this study is to assess the efficacy, safety, and tolerability of ixmyelocel-T compared to placebo (vehicle control) when administered via transendocardial catheter-based injections to subjects with end-stage heart failure due to ischemic dilated cardiomyopathy who have no reasonable revascularization options (either surgical or percutaneous interventional) likely to provide clinical benefit. Results have been published in *Lancet* (2016;387:2412-21), reporting transendocardial delivery of ixmyelocel-T in patients with heart failure and reduced ejection fraction due to ischemic dilated cardiomyopathy

resulted in a significant reduction in adjudicated clinical cardiac events and improved outcomes. An open-label extension is active for patients who had been randomly assigned to receive placebo during the study period and now have the opportunity to undergo bone marrow harvest again and receive ixmyelocel-T therapy.

A Single-Arm, Prospective Study of Remestemcel-L, Ex Vivo *Cultured Adult Mesenchymal Stromal Cells, for the Treatment of Pediatric Patients Who Have Failed to Respond to Steroid Treatment for Acute GVHD.* This is an industry-sponsored multicenter study through Mesoblast International Sarl (Switzerland). The objective of this study is to evaluate efficacy and gather additional information on safety of remestemcel-L in pediatric patients with grades B-D acute GVHD who have failed to respond to steroid treatment post-allogeneic HSCT. Subject enrollment continues.

MAJOR LECTURESHIPS AND SEMINARS

- "Blood and Marrow Transplantation Nuts and Bolts," oral presentation, Pediatric Hematology/Oncology Fellowship Conference, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., September 2016
- "Pediatric Hematology/Oncology and Blood and Marrow Transplantation: 2016 Updates: Transition and Growth," oral presentation, Katie Swaney Foundation board meeting, Pittsburgh, Pa., October 2016
- "Leukemias and Lymphomas," oral presentation, Pediatric Residency Conference, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., December 2016
- "Neuroblastoma," oral presentation, APHON Foundations and *Certified Pediatric Hematology Oncology Nurse* (CPHON) Review Course, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., March 2017
- "Solid Tumors," oral presentation, APHON Foundations and CPHON Review Course, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., March 2017
- "Pediatric Cancer: Solid Tumors," oral presentation, Pediatric Residency Conference, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., April 2017
- "Safety and Feasibility of Granulocyte Transfusion for High-Risk Allogeneic Stem Cell Transplant Recipients," poster presentation, American Society of Pediatric Hematology/Oncology annual meeting, Montreal, Quebec, Canada, April 2017
- "Germ Cell Tumors," oral presentation, Pediatric Hematology/Oncology Fellowship Conference, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., June 2017
- "Cardiac Complications During Blood and Marrow Transplantation," oral presentation, APHON Blood and Marrow Transplantation Course, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., June 2017

ADVISORY COMMITTEE MEMBERSHIPS

- Division of Hematology/Oncology Fellowship Oversight Committee, Children's Hospital of Pittsburgh of UPMC
- Pediatric Hematology/Oncology/BMT Outpatient Clinic Committee, Children's Hospital of Pittsburgh of UPMC
- Chemotherapy Oversight Committee, Children's Hospital of Pittsburgh of UPMC
- 9B (Oncology/BMT Inpatient Unit) Leadership Committee, Children's Hospital of Pittsburgh of UPMC
- 9B (Oncology/BMT Inpatient Unit) Infection Control Committee, Children's Hospital of Pittsburgh of UPMC
- Chemotherapy Oversight Committee, Children's Hospital of Pittsburgh of UPMC

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Society of Pediatric Hematology/Oncology
- American Society for Blood and Marrow Transplantation
- American Society of Hematology
- Children's Oncology Group

HONORS

- Best Doctors, *Pittsburgh Magazine*, 2017

TEACHING ACTIVITIES

The faculty members in the pediatric BMT&CT division are actively involved in teaching residents and fellows. The BMT&CT Fellowship Program (Windreich, director) established an educational environment to train advanced practice fellows in BMT in 2012. Barnum and Windreich mentor pediatric fellows. Byersdorfer mentors four medical students and one undergraduate. Carella leads efforts to help design and implement the curriculum for the advanced practice providers' fellowship program.

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DIVISION OF
CHILD ADVOCACY

Mission

The mission of the Child Advocacy Center (CAC) at Children's Hospital of Pittsburgh of UPMC is to protect children and promote healthy families through excellence in:

- Assessment of child maltreatment
- Medical and social care for vulnerable children
- Collaboration with child-protection agencies
- Education and research

FACULTY

Rachel Berger, MD, MPH
Chief, Division of Child Advocacy
Professor of Pediatrics and Clinical and Translational Science
Associate Director, Safar Center for Resuscitation Research

Jennifer Clarke, MD
Assistant Professor of Pediatrics

Carmen Coombs, MD
Assistant Professor of Pediatrics

Adelaide Eichman, MD
Assistant Professor of Pediatrics

Janet Squires, MD
Professor of Pediatrics

Jennifer Wolford, DO, MPH
Assistant Professor of Pediatrics
Clinical Director

OVERVIEW OF DIVISION

Physicians of the Division of Child Advocacy have responsibilities of medical service, teaching, research, and administrative leadership for the CAC at Children's Hospital of Pittsburgh of UPMC. The purposes of the clinical center are: (a) to ensure that injured, vulnerable patients receive competent assessment for concerns of maltreatment; and (b) to ensure that there are ongoing medical and social services for children who have been abused or are at risk of abuse. CAC combines two separate but complementary programs: (a) a traditional advocacy center model, including forensic interviewing, referrals, and therapy, as well as support of a community-based multidisciplinary team approach to child abuse; and (b) a hospital-based Child Protection Team program for evaluation and treatment of children when there is concern about abuse or neglect.

GOALS AND OBJECTIVES

CLINICAL SERVICE

- To provide consultative services for children receiving care at Children's Hospital, including forensic medical and social evaluations of children who may be victims of child abuse or neglect. This service involves collaboration with other physicians on the medical staff, including specialists from trauma, neurosurgery, orthopedics, radiology, ophthalmology, emergency medicine, behavioral health, and pediatrics.
- To work with professionals from community-based child-protection agencies and to support the judicial system in the protection of children. This includes providing objective medical information through consultative reports and legal testimony for (a) the judicial system, both in family court and criminal court settings; (b) professionals working in the Office of Children, Youth, and Families (CYF); (c) law-enforcement agencies; and (d) district attorney offices.
- To provide pediatric medical care to vulnerable children in the child-protection system. This includes primary care, if needed, for children in foster care and kinship care; a temporary medical home for children in high-risk social situations, especially when CYF or police intervention is ongoing; identification of ongoing developmental and behavioral problems in children who have been identified as maltreated and/or neglected; and referrals for vulnerable children to appropriate support services in the community.

EDUCATION

- To teach medical students, pediatric residents, and fellows, as well as Children's Hospital medical staff and employees, the principles of recognizing, evaluating, and reporting child maltreatment
- To work with other physicians in recognizing patterns of child maltreatment and meeting the requirement of mandated reporting of maltreatment
- To strengthen community relations through outreach and education for child-protection professionals and to participate in community education aimed at reducing violence toward and injury to children

RESEARCH

- To improve the scientific base to diagnose and treat victims of child maltreatment
- To improve techniques to differentiate abusive and accidental trauma

HIGHLIGHTS FROM THE PAST YEAR

- Continuation of a three-year grant funded by the Patient-Centered Outcomes Research Institute (PCORI): "Using the Electronic Medical Record to Improve Outcomes and Decrease Disparities in Screening for Child Physical Abuse." Rachel Berger is principal investigator.
- Start of a grant funded by the Hillman Foundation to develop a multidisciplinary response to unsafe sleep deaths in Allegheny County
- Twelfth annual conference held during Child Abuse Prevention Month (April) titled Pittsburgh Conference on Child Maltreatment. The two-day conference provided expertise and education for 219 child-protection professionals from the tristate area surrounding Pittsburgh and from 10 counties in Pennsylvania.
- Continuation of the Child Advocacy and Community Health Rotation for second-year pediatric residents, a four-week educational program incorporating specific objectives of residency training program requirements

CLINICAL SERVICE ACTIVITIES

Clinical activities are multifaceted and provided at multiple sites. Data are given for fiscal year July 2016 through June 2017.

INPATIENT CONSULTATIONS

Service includes assessment of children who are hospitalized in the inpatient units of Children's Hospital, usually with concerns of physical abuse and/or neglect. The physicians on the Child Protection Team are available 24/7 for consultation.

There were 396 billable inpatient physician consultations.

- 173 (43.6%) patients were from Allegheny County, 198 were from 26 other Pennsylvania counties, and 25 were from two out-of-state locations (18 from Ohio, seven from West Virginia). The diagnosis of definite/probable abuse was made in 113 of the 396 consultations (26% of all consultations).
- Another 104 children received "nonbillable" consultative service, in which physicians participated in assessment and management decisions without physically examining the patients firsthand.
- Nine children died in cases where there was a concern of abuse or neglect.

Wolford, Eichman, Clarke, and Berger were on call for child-protection consultations on a rotating weekly basis.

EMERGENCY DEPARTMENT CONSULTATIONS

Services include on-site consultation in the emergency department (ED) during workday hours when there are concerns of abuse or neglect. Alternatively, ED staff may handle the initial presentation of a case and request follow-up services, which can include a forensic interview and/or a scheduled visit in the CAC outpatient clinic. By protocol, all cases of children seen in the ED with identified concerns of maltreatment receive next-day review, facilitating appropriate follow-up arrangements.

There were 1,027 initial or follow-up consultations for ED patients. Of those, 717 (70%) were from Allegheny County.

OUTPATIENT CLINIC VISITS (ARCH CLINIC)

Services include primary medical care for selected children who have open CYF cases or who have been placed outside their homes. Episodic consultation services include assessments for abuse, hospital follow-up visits, and CYF-mandated physical examinations.

There were 1,518 outpatient visits to the CAC.

FORENSIC ASSESSMENTS IN THE CAC

Services include oversight of the forensic interview process; consultations referred by child-protection agencies; facilitation of the multidisciplinary team's response to concerns of abuse; and, when applicable, a specialized medical examination, including colposcopy for females.

There were 638 forensic interviews performed at Children's Hospital of Pittsburgh of UPMC. Of those, 564 (88%) were with patients from Allegheny County.

MEDICAL/LEGAL INTERFACE

Services include preparation of documents for legal issues, court testimony, preparation for court, and pretrial meetings.

The six physicians received 440 subpoenas for medical testimony. This was an increase of 101 subpoenas over the previous fiscal year.

- 317 (72%) were from Allegheny County.
- 283 subpoenas involved civil courts, and 157 involved criminal courts.
- Physicians gave court testimony in 88 cases:
    - 37 Allegheny County civil and 19 Allegheny County criminal
    - 24 outside-county civil and eight outside-county criminal
    - Court preparation was required for most of the cases, even those (80%) that ultimately were settled without the necessity for direct medical testimony.

Other activities included the following:

- Monthly Allegheny County child-protection multidisciplinary meetings were held the first Friday of each month.
- Suspected Child Abuse and Neglect (SCAN) meetings with teams of child-protection professionals from other counties were scheduled on individual, as-needed basis.
- Faculty reviewed photographs/videos from confiscated computers in cases of potential child exploitation and prepared court papers.

RESEARCH AND OTHER SCHOLARLY ACTIVITIES

Rachel Berger, MD, MPH

Rachel Berger became the chief of the Division of Child Advocacy in 2013. She joined the division in 2001 after completing a fellowship in general academic pediatrics and earning an MPH degree from the University of Pittsburgh Graduate School of Public Health. She is primarily a clinical researcher, and her predominant research focus is improving the diagnosis of physical abuse injury in young children by using electronic health records and novel types of clinical decision support.

RESEARCH

Berger is the director for the division's research activities.

Using the Electronic Medical Record to Improve Outcomes and Decrease Disparities in Screening for Child Physical Abuse. The goal of this study is to decrease the well-recognized racial and economic disparities with which children are screened by physicians for physical abuse. The objectives of the parallel-group, randomized, controlled trial: (a) compare rates of compliance to evidence-based screening protocols for child physical abuse before and after implementation of a trigger system within the Cerner electronic medical record; and (b) compare the accuracy of screening by patient race (white versus non-white), insurance status (private versus public insurance), and hospital type (community versus academic) when physicians do and do not receive screening prompts that are embedded within the electronic medical record. The study, sponsored by PCORI, began in September 2013 and ended September 2017.

Rachel Berger, MD, MPH
Division Chief, Child Advocacy

Decreasing Unsafe Sleep in Allegheny County: A Multidisciplinary, Multipronged Approach. The goal of this project is to better understand injuries and deaths due to unsafe sleep and to develop a multidisciplinary approach to decrease them. The study, sponsored by the Henry L. Hillman Foundation Opportunity Fund, began in April 2016 and will end in March 2018.

Development and Dissemination of an Electronic Health Record (EHR)-Based Child Abuse Clinical Decision Support System Toolkit. The goal of this project is to expand the use of a validated EHR-based child abuse–related clinical decision support system to other children's hospitals which use Cerner. The study, sponsored by the Beckwith Foundation, began in July 2017 and will end in June 2018.

ADVISORY COMMITTEE MEMBERSHIPS

- Executive Steering Committee, Neurointensive Care T32, funded by the National Institute of Child Health and Human Development. Patrick Kochanek is principal investigator.

EDITORSHIPS

- Editorial Board, *Child Abuse—The Quarterly Update*

MAJOR LECTURESHIPS

- "Decreasing Morbidity and Mortality From Abusive Head Trauma by Improving Early Detection," Fifteenth International Conference on Shaken Baby Syndrome/Abusive Head Trauma, Montreal, Quebec, Canada, September 2016
- "The Abused Child," American Academy of Pediatrics National Conference and Exhibition, Sustainable Recognition of Abusive Head Trauma—Finding Abuse Without Vicarious Trauma, San Francisco, Calif., October 2016
- "Finding Goldilocks: How Do We Screen Enough Children for Physical Abuse Without Screening Too Many?" pediatric grand rounds, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., November 2016
- "Using the Electronic Medical Record to Improve Screening Practices in Children at Risk of Physical Abuse," Patrick S. Pasquariello Jr. Lectureship in Pediatrics, Children's Hospital of Philadelphia, Philadelphia, Pa., November 2016
- "Accident or Abuse? An Ethical Discussion of 'What Would You Do?'" Templeton Pediatric Trauma Symposium, Pittsburgh, Pa., March 2017
- "Engaging State and Federal Policy Around the Prevention of Child Abuse Fatalities During a Time of Change" (with David Rubin, Richard Krugman, and Christopher Greeley), Ray Helfer Society 2017 Annual Meeting, Denver, Colo., April 2017
- "Pediatric Emergency Medicine Cases," Emergency Resource Management Inc., Pittsburgh, Pa., April 2017
- "Decreasing Unsafe Sleep in Allegheny County: A Collaborative Approach," Fifth National Cribs for Kids Conference, Pittsburgh, Pa., April 2017
- "The UPMC Child Abuse Initiative: A Collaborative Approach to Improving Detection, Evaluation, and Reporting of Child Maltreatment," State Emergency Nursing Conference, Pittsburgh, Pa., June 2017

HONORS

- Chair Distinction Award for providing outstanding services, Department of Pediatrics, Children's Hospital of Pittsburgh, 2017

Jennifer Clarke, MD

Jennifer Clarke joined the Division of Child Advocacy in August 2015. She completed her fellowship in child abuse at the University of Texas Health Science Center at San Antonio, and she completed her pediatric residency at Maria Fareri Children's Hospital at Westchester in Valhalla, N.Y. Clarke received her medical degree at the State University of New York Upstate Medical University in Syracuse, N.Y. Her areas of interest include sexual abuse, strangulation injuries, evidence-collection kits, and presentation of bleeding disorders.

ADVISORY COMMITTEE MEMBERSHIPS

- Trauma Medical Advisory Committee
- Children's Hospital of Pittsburgh Sexual Assault Task Force

MAJOR LECTURESHIPS

- "Quarterly Child Maltreatment Update," trauma conference (with Eichman and Wolford), Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., July 2016
- "CAC Case Reviews," pediatric emergency medicine conference (with Eichman and Wolford), Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., August 2016
- "Evidence Kit Case Review," Children's Hospital of Pittsburgh Sexual Assault Task Force, Children's Hospital of Pittsburgh of UPMC, September to December 2016
- "Evidence Kit Case Review," Children's Hospital of Pittsburgh Sexual Assault Task Force, Children's Hospital of Pittsburgh of UPMC, January, March, and April 2017
- "Quarterly Child Maltreatment Update," trauma conference (with Eichman and Wolford), Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., January and March 2017
- "Reviewing Evidence Collection and Injuries," Children's Hospital of Pittsburgh Sexual Assault Task Force, Children's Hospital of Pittsburgh of UPMC, February 2017

HONORS

- Chair Distinction Award for providing outstanding services, Department of Pediatrics, Children's Hospital of Pittsburgh, 2016

Carmen Coombs, MD

Carmen Coombs completed her fellowship in pediatric emergency medicine at Children's Hospital of Pittsburgh of UPMC. She completed her pediatric residency, including pediatric chief residency, at Johns Hopkins University School of Medicine in Baltimore, Md., and received a master's degree in public health from Johns Hopkins Bloomberg School of Public Health. She received her medical degree from Johns Hopkins University School of Medicine. Her research is focused on early detection and management of sentinel injuries. Coombs worked one day per week at the CAC. The remainder of her time was in service in the ED.

MAJOR LECTURESHIPS

- "Physical Child Abuse: What Every Emergency Provider Should Know," pediatric emergency medicine grand rounds, IWK Children's Hospital, Halifax, Nova Scotia, Canada, March 2016
- "Whose Emergency Is It? Working With ED Physicians in Regard to Child Abuse Cases," (with Maria Antonucci), 12th Annual Pittsburgh Conference on Child Maltreatment, Pittsburgh, Pa., April 2017

HONORS

- Content expert and clinical advisor, TREKK (Translating Emergency Knowledge for Kids), Canada: one of three content experts/clinical advisors for the development and implementation of clinical practice guidelines, bottom-line recommendations, and evidence repository for child physical abuse, 2016–2017
- National Board Examination for Pediatric Emergency Medicine Contributor and Question Writer, Saudi Arabia, 2016

Adelaide Eichman, MD

Adelaide Eichman joined the Division of Child Advocacy in July 2013. She was recruited to the CAC after completing her pediatric residency at Children's Hospital of Pittsburgh of UPMC. She received a bachelor of arts degree, a bachelor of science degree, and her medical degree with an area of concentration in women's health from the University of Pittsburgh. Starting in October 2014, Eichman has worked to establish a consultation program with the UPMC Mercy Burn Unit. The program was developed out of a request from the Allegheny County district attorney's office and Pittsburgh police. The CHP CAC now provides consultative services for pediatric burn patients in cases of possible abuse/neglect. In addition, the CAC provides ongoing education to the physicians and staff at Mercy Burn Unit to more effectively and accurately identify cases of abuse/neglect, as well as to perform proper work-up when abuse/neglect is suspected. Her areas of interest include toxic stress, intimate partner violence, and early childhood education.

ADVISORY COMMITTEE MEMBERSHIPS

- Board member, Educating Physicians in Their Community on Suspected Child Abuse and Neglect ("EPIC-SCAN") Advisory Board, American Board of Pediatrics, Pennsylvania chapter

MAJOR LECTURESHIPS

- "Quarterly Child Maltreatment Update," trauma conference (with Clarke and Wolford), Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., July and October 2016
- "CAC Case Reviews," pediatric emergency medicine conference (with Clarke and Wolford), Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., August 2016
- "Child Maltreatment," trauma nursing course, UPMC Mercy Hospital, Pittsburgh, Pa., August and November 2016
- "Physical Abuse Basics for Ophthalmologists," Ophthalmology Department, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., December 2016
- "Child Maltreatment," trauma nursing course, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., December 2016
- "Quarterly Child Maltreatment Update," trauma conference (with Clarke and Wolford), Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., January and March 2017
- "Child Maltreatment," trauma nursing course, UPMC Mercy Hospital, Pittsburgh, Pa., February 2017
- "Child Maltreatment," trauma nursing course, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., April 2017

HONORS

- Chair Distinction Award for providing outstanding services, Department of Pediatrics, Children's Hospital of Pittsburgh, 2016
- Bridges Education Champions Program, Office of Faculty Development, Department of Pediatrics, University of Pittsburgh School of Medicine, 2016–2017

Janet Squires, MD

Janet Squires joined the division as director in 2003. She served in that capacity until she semi-retired in July 2013. During FY 2016–17, Squires worked one day per week except during the 12 weeks when two of the CAC physicians were on medical leave. During those 12 weeks, she worked an average of two and a half days per week.

ADVISORY COMMITTEE MEMBERSHIPS

- Ethics Committee, Children's Hospital of Pittsburgh of UPMC
- Institutional Review Board, University of Pittsburgh
- Residency Applicant Interview Committee, University of Pittsburgh School of Medicine

Jennifer Wolford, DO, MPH

Jennifer Wolford joined the Division of Child Advocacy in 2011, having completed a pediatric residency at Children's Hospital of Pittsburgh of UPMC and served one year as chief resident. In July 2013, Wolford became the clinical director of the CAC. In addition, she became the assistant director of the Children's Hospital of Pittsburgh of UPMC's Pediatric Residency Program in October 2015.

Wolford's specific interests include identification of social determinants of health, cultural competency, impact of domestic violence on children, and international health.

Wolford is the program director for the Child Health Evaluation and Coordination Services (CHECS) program. The CHECS program is a pilot program in which the CAC, in collaboration with the Allegheny County Child Protective Services, provides care coordination and health assessments for medically fragile children involved in the child welfare system.

The presence of nurses from the Children's Hospital of Pittsburgh in Child Protective Services offices enhances the ability to provide real-time consultation and coordinated care for children. The nurses are located in the North Regional, Central Regional, and Mon Valley Regional offices, with plans to expand into two more locations.

ADVISORY COMMITTEE MEMBERSHIPS

- Program director, Pediatric Advocacy Leadership Service Residency Program
- Assistant program director, Pediatric Residency Program
- Faculty advisor, Residency Teaching Program Community-Oriented Resident Education
- Faculty codirector, Residency Global Health Interest Group, Children's Hospital of Pittsburgh
- Advisory member, Near Fatality Review Committee, Allegheny County Child Protective Services
- Director, Pediatric Care Clinic, Pittsburgh Domestic Violence Shelter

MAJOR LECTURESHIPS

- "Quarterly Child Maltreatment Update," trauma conference (with Clarke and Eichman), Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., July and October 2016
- "CAC Case Reviews," pediatric emergency medicine conference (with Clarke and Eichman), Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., August 2016
- "Quarterly Child Maltreatment Update," trauma conference (with Clarke and Eichman), Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., January and March 2017
- "Let's Get Social: Being an Advocate in the Digital World," Southern Society for Pediatric Research, southern regional annual meeting, New Orleans, La., February 2017

HONORS

- Chair Distinction Award for providing outstanding services, Department of Pediatrics, Children's Hospital of Pittsburgh, 2016
- Bridges Education Champions Program, Office of Faculty Development, Department of Pediatrics, University of Pittsburgh School of Medicine, 2016–2017

DIVISION TEACHING ACTIVITIES

MEDICAL STUDENTS AND PEDIATRIC RESIDENT TRAINING

- Direct four-week rotation for every second-year pediatric resident involving Child Advocacy and Community Health
- Oversight of four-week rotation for each first-year emergency room fellow
- Monthly lectures, MS3 curriculum on basics of physical abuse
- Quarterly case presentations to trauma team, ED staff, and pediatric house staff

COMMUNITY EDUCATION

- EPIC-SCAN presentations (two at Children's Hospital, one at Butler Memorial Hospital, and one at Slippery Rock University): "Educating Physicians in Their Communities—Suspected Child Abuse and Neglect"
- American Academy of Pediatrics Physician Preceptorship Program: a teaching program for volunteer community physicians interested in improving skills in assessment of child maltreatment, administered through the Pennsylvania chapter of the American Academy of Pediatrics; includes 60 hours of didactic teaching at statewide meeting and multiple-day Children's Hospital on-site trainings; one physician participated.
- The UPMC Child Abuse Initiative: an educational/peer-review program related to the evaluation of children with suspected maltreatment, which is present in all hospitals within the UPMC system. Training sessions were conducted at Magee-Womens Hospital of UPMC, UPMC East, UPMC Passavant, UPMC Mercy, UPMC Shadyside, UPMC McKeesport, and the ERMI Risk Management Annual Conference: The UPMC Child Abuse Initiative: Improving Detection, Evaluation and Reporting of Child Maltreatment in May of 2017.

THREE-YEAR BIBLIOGRAPHY

2015

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DIVISION OF
CHILD NEUROLOGY

Mission

The mission of the Division of Child Neurology is:

- To provide outstanding clinical care to children with disorders of the central and peripheral nervous system
- To perform clinical and basic research that improves the ability to diagnose and treat children with neurological disease
- To train the next generation of child neurologists and to educate physicians and other medical personnel about childhood neurological disease
- To serve the community with expertise in neurology and pediatric medicine

FACULTY

Ira Bergman, MD, PhD
Chief, Division of Child Neurology
Professor of Pediatrics, Neurology, and Immunology
Program Director, Child Neurology Residency Program
Ronald L. and Patricia M. Violi Endowed Chair in Child Development

Hoda Abel-Hamid, MD
Associate Professor of Pediatrics
Director, Neuromuscular Program and Electromyography

Gulay Alper, MD
Associate Professor of Pediatrics
Director, Clinical Neuro-Immunology Program

Miya Asato, MD
Associate Professor of Pediatrics, Psychiatry, and Clinical and Translational Science
Program Director, Neurodevelopmental Disabilities Program
Associate Program Director, Child Neurology Residency Program
Director, Leadership Education in Neurodevelopmental and Related Disabilities (LEND) Program

Catalina Cleves-Bayon, MD
Assistant Professor of Pediatrics
Director, Idiopathic Intracranial Hypertension Program

Patricia K. Crumrine, MD
Professor of Pediatrics and Neurology
Associate Director, Epilepsy Program

Dana D. Cummings, MD, PhD
Assistant Professor of Pediatrics
Director, Pediatric Stroke Program

Luis De Jesus Fernandez, MD
Assistant Professor of Pediatrics
Associate Director, Epilepsy Program

Robyn Filipink, MD
Clinical Associate Professor of Pediatrics
Medical Director, Fragile X Clinic
Medical Director, Tourette Syndrome Clinic
Medical Director, Movement Disorder Clinic

Jenna M. Gaesser, MD
Assistant Professor of Pediatrics
Associate Director, Neonatal Neurology Program
Codirector, Neurofibromatosis Clinic
Codirector, Advanced Practice Provider Development and Utilization in the Child Development Unit

Yanhua Gao, MD, PhD
Research Assistant Professor

Amy C. Goldstein, MD
Assistant Professor of Pediatrics
Director, Neurogenetics Program
Codirector, Neurofibromatosis Clinic

Anuja Vora Jindal, MD, MPH
Assistant Professor of Pediatrics

Todd Lamitina, PhD
Associate Professor of Pediatrics and Cell Biology

Monica Naik, MD
Clinical Assistant Professor of Pediatrics
Director, Neonatal Neurology Program

Udai Pandey, PhD
Associate Professor of Pediatrics and Human Genetics

Christina Patterson, MD
Assistant Professor of Pediatrics
Director of Epilepsy
Director, Epilepsy Monitoring Unit
Medical Director, Pediatric Epilepsy Surgery Program
Director, Advanced Practice Provider Development and Utilization

Deepa Rajan, MD
Assistant Professor of Pediatrics
Codirector, Neurogenetics Program

Robert Safier, MD
Associate Professor of Pediatrics
Director, Medical Student and Resident Child Neurology Education
Codirector, Child Neurology Inpatient Floor Team

Levi Shelton, MD
Assistant Professor of Pediatrics
Codirector, Advanced Practice Provider Development and Utilization
Director, Visiting Resident Education, Child Neurology Division

Bilal Sitwat, MD
Assistant Professor of Pediatrics
Associate Director, Epilepsy Program

Yoshimi Sogawa, MD
Associate Professor of Pediatrics
Associate Director, Epilepsy Program

Kavita Thakkar, MD
Assistant Professor of Pediatrics
Associate Director, Clinical Neuro-Immunology Program

Inna Vaisleib, MD
Associate Professor of Pediatrics
Associate Director, Epilepsy Program

Rajiv R. Varma, MD
Clinical Professor of Pediatrics and Neurology
Clinical Director, Division of Child Neurology

Shelley Williams, MD
Associate Professor of Pediatrics and Neurology
Director, Pediatric Electrophysiology Fellowship Program
Director, Pediatric Ketogenic Diet Clinic
Director, Pediatric Tuberous Sclerosis Clinic
Associate Director, Epilepsy Program

OVERVIEW OF DIVISION

The division consists of the inpatient consultation and primary admitting services, the Pediatric Epilepsy Monitoring Unit, the Pediatric Electroencephalography (EEG) Laboratory, the Electromyography (EMG) Laboratory, the outpatient neurology clinics, a clinical research component, and a basic research component. The faculty provides training in the diagnosis and management of neurological disorders to medical students, pediatric residents, child and adult neurology residents, child psychiatry residents, physiatry residents, epilepsy fellows, nurses, nurse practitioners, and physician assistants. Clinical investigations are directly related to the clinical and educational components of the division's mission.

Clinical activity has continued to increase, and specialized services have been established in epilepsy, epilepsy surgery, headache, neuro-ophthalmology, idiopathic intercranial hypertension, stroke, movement disorders, Tourette syndrome, neurofibromatosis, tuberous sclerosis, neuromuscular disease, muscular dystrophy, inflammatory brain and spinal cord disease, neonatal neurology, ketogenic diet, infantile spasms/epileptic encephalopathy, metabolic disease, and neurogenetics.

Extensive clinical research studies have been initiated and expanded, including epilepsy drug trials and examination of cannabidiol for refractory seizures, mapping of cognitive skills in epilepsy with structural and functional magnetic resonance imaging (MRI), assessment of neural plasticity in patients following epilepsy surgery, computerized cognitive assessment of medication effects in newly diagnosed patients with epilepsy, study of drugs for status epilepticus in the emergency room, prospective study of children with epileptic encephalopathy, effect of vagus nerve stimulation on children with a history of status epilepticus, sildenafil therapy for cardiac failure in Duchenne muscular dystrophy (DMD), morpholino exon skipping in DMD, anti-inflammatory therapy of DMD, 31P NMR evaluation of adenosine triphosphate production in individuals with mitochondrial disease, North American Mitochondrial Disease Consortium activities, triheptanoin treatment of long-chain fatty acid oxidation disorders, a neurogenetics registry, functional characterization of the genetic disorder GEMIN5, a Fragile X registry, studies of sleep in children with autism, examinations in acute demyelinating disorders of childhood, radiological investigations of various neurological disorders, and use of a new compound to improve language learning in children with Fragile X syndrome. Laboratory studies include cellular and molecular mechanisms of fused in sarcoma (FUS)-related neurodegeneration, molecular basis of FUS/translocated in liposarcoma (TLS)-related amyotrophic lateral sclerosis, molecular library screen for suppressors of FUS proteinopathy, a *Drosophila* model to investigate the role of FUS in amyotrophic lateral sclerosis (ALS), development of a targeted oncolytic virus to treat cancer, prevention of cancer metastases of the brain, bipartite regulation of osmosensitive gene expression in *C. elegans* and biomechanical profiling of *C. elegans* motility, and mechanisms and characterization of modifiers of C9orf72-associated dipeptide toxicity.

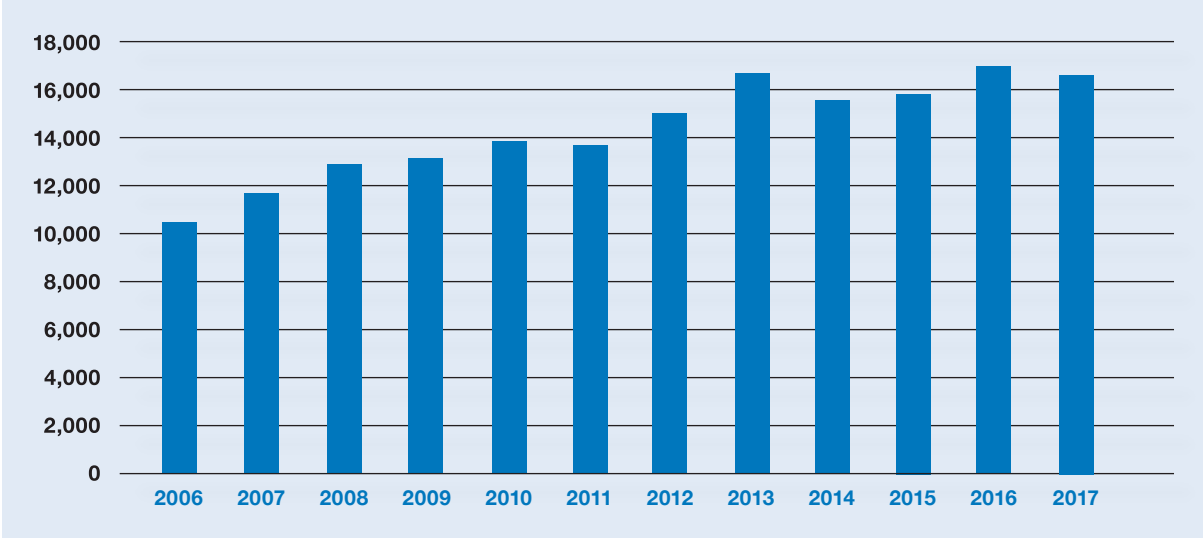
The child neurology training program consists of nine child neurology residents and two neurodevelopmental disabilities residents. Four current PL-2s and four current PL-1s will be entering the child neurology residency. There is one neurophysiology fellow. Educational programs have been established for the medical students, pediatric residents, and visiting neurology and psychiatry residents. The division has partnered with the Epilepsy Foundation of Western/Central Pennsylvania to improve care for children and families with epilepsy and to provide telemedicine visits and educational outreach to rural pediatric practices.

CLINICAL ACTIVITIES

The primary goal of the division is to provide high-quality clinical services to children with neurological disorders. A total of 16,426 outpatients were treated this year in Lawrenceville and at the three satellite facility offices of Children's Hospital of Pittsburgh of UPMC (Wexford, South Hills, and Monroeville), as well as at five outreach facilities (Johnstown, Pa.; Wheeling, W.Va.; Erie, Pa.; Hermitage, Pa.; and Chippewa, Pa.). In addition, the division provided specialty neurology services at the Pediatric Specialty Care Center in Aliquippa, Polk State Center, Mercer County Amish Clinic, and Verland Community Homes. The overall trend over the past 13 years has been a continuing increase in clinical outpatient activity in the division, which varies to some degree because of fluctuations of faculty numbers (see figure below). Children's Hospital neurology physicians had 1,627 inpatient admissions, 1,644 bedded outpatients, and 1,203 inpatient consults in fiscal year 2017.

The comprehensive Epilepsy Program at Children's Hospital is a nationally recognized epilepsy center with a level 4 (highest) designation by the National Association of Epilepsy Centers. The Epilepsy Monitoring Unit has eight beds and is equipped with EEG and audio-telemetric monitoring, which operates 24 hours per day, seven days per week. Video EEG monitoring is also performed in all intensive care units in the hospital, in all rooms on the hospital floors, and in the neonatal intensive care unit at Magee-Womens Hospital of UPMC. All recordings are monitored remotely and continuously by EEG technologists. The monitoring unit is staffed by registered EEG technologists and professional nurses and functions as both an inpatient and outpatient unit. The comprehensive Epilepsy Program is staffed by Board-certified epileptologists; pediatric neurologists; pediatric neurosurgeons; physician assistants; psychologists; a social worker; and associated experts in neuroimaging, dietary, communication, and psychiatric services. Clinical trials for new anti-epileptic drugs are available. There is an active ketogenic diet program with a dedicated team that includes a dietitian, social worker, and nurse. Therapeutic options for patients with refractory seizures not responding to standard anti-epileptic drugs include the use of vagus nerve stimulation, new anti-epileptic drugs in clinical trials, or epilepsy surgery. In fiscal year 2017, the unit performed 2,341 video EEGs, 321 ambulatory EEGs, 37 phase I evaluations, five phase II evaluations, and 18 epilepsy surgeries. Eighteen new vagus nerve stimulators were placed and 16 replaced.

PEDIATRIC NEUROLOGY OUTPATIENT VISITS



The EEG Laboratory staff performs inpatient and outpatient EEG procedures. Procedures are performed in the EEG Laboratory, the intensive care units, the inpatient areas, and the emergency room. EEGs are available around the clock. This fiscal year, the EEG Laboratory performed 2,110 inpatient procedures and 2,150 outpatient procedures. In addition, 124 studies were performed at Magee-Womens Hospital and UPMC Hamot Hospital.

EMG is a study that aids in the diagnosis of neuromuscular disorders. A physician electromyographer performs EMG examination as a diagnostic consultation. The Muscular Dystrophy Program at Children's Hospital provides a multidisciplinary setting for the diagnostic evaluation and follow-up care of infants and children with known or suspected neuromuscular diseases. Hoda Abdel-Hamid is director of the EMG Laboratory and the Neuromuscular Program. This fiscal year, 134 EMG procedures were performed. In addition, Abdel-Hamid administers Botox injections on an outpatient basis; 147 procedures were performed.

RESEARCH AND OTHER SCHOLARLY ACTIVITIES

Ira Bergman, MD, PhD

Ira Bergman is a professor of pediatrics, neurology, and immunology at the University of Pittsburgh; chief of the Division of Child Neurology; interim chief of the Division of Child Development; program director of the Child Neurology Residency Program; and the Ronald L. and Patricia M. Violi Endowed Chair in Child Development.

RESEARCH

- Replicating Recombinant Vesicular Stomatitis Virus, specifically targeting cancer cells and treating cancer by direct killing and by stimulating the immune system to recognize and kill cancer cells
- Viral Immunotherapy to Eradicate Subclinical Brain Metastases, U.S. Department of Defense (DOD), 2015
- Memory Anti-Tumor T Cells Resist Inhibition by Immune Suppressor Cells, anticancer research, 2015

STUDY SECTIONS

- Chair, Peer-Reviewed Cancer Research Program, Pediatric Brain Tumor Review Panel, DOD
- Chair, Tuberous Sclerosis Complex Research Program, Clinical and Population Studies Review Panel, DOD
- Chair, Visionary Postdoctoral Fellowship, DOD

ADVISORY COMMITTEE MEMBERSHIPS

- Research Advisory Committee, Children's Hospital of Pittsburgh of UPMC
- Magnetoencephalography Oversight Board, UPMC

PROFESSIONAL AFFILIATIONS/ SOCIETY MEMBERSHIPS

- Child Neurology Society
- American Academy of Neurology
- American Academy of Pediatrics
- American Association of Immunology
- American Association of Cancer Research
- American Pediatric Society/Society for Pediatric Research

Ira Bergman, MD, PhD
Division Chief, Child Neurology

HONORS

- *Best Doctors*, Pittsburgh Magazine, 2009–2017
- Five-Star Excellence Award, Professional Research Consultants, 2015, 2016
- *Best Doctors in America*, Woodward/White, Inc.
- U.S. patent application 11/227,778, titled "Targeting Viruses Using a Modified Sindbis Glycoprotein"

Hoda Abdel-Hamid, MD

Hoda Abdel-Hamid is an associate professor of pediatrics and neurology and director of the Neuromuscular Program, the Neurology Botox program, the MDA clinic (muscular dystrophy), and the EMG Laboratory at Children's Hospital of Pittsburgh of UPMC.

RESEARCH

- Phase II, Randomized, Double-Blind, Placebo-Controlled, Multiple Ascending Dose Study to Evaluate the Safety, Efficacy, Pharmacokinetics, and Pharmacodynamics of Pf-06252616 in Ambulatory Boys With DMD
- Ataluren in Patients With Nonsense Mutation DMD: A Multicenter, Randomized, Double-Blind, Placebo-Controlled, Phase III Trial
- Phase 3 Efficacy and Safety Study of Ataluren (ptc124) in Patients With Nonsense Mutation Dystrophinopathy: A New Randomized, Double-Blind, Placebo-Controlled, Phase III Trial of Tadalafil for DMD

- Open-Label, Multiple-Dose, Efficacy, Safety, and Tolerability Study of Eteplirsen in Subjects With DMD Who Participated in Study 4658-US-201
- A Multicenter Collaborative Study on the Clinical Features, Expression Profiling, and Quality of Life of Infantile-Onset Facioscapulohumeral Muscular Dystrophy
- Twenty-Year Follow-Up Assessment of the Impact of Newborn Screening on Men With Muscular Dystrophy and Their Families
- Becker Muscular Dystrophy: A Natural History Study to Predict Efficacy of Exon Skipping
- Cardiac Outcome Measures in Children With Muscular Dystrophy
- DMD Tissue Bank for Exon Skipping
- Longitudinal Study of the Relationship Between Impairment, Activity Limitation, Participation, and Quality of Life in Persons with Confirmed DMD
- Clinical Trial of Coenzyme Q10 and Lisinopril in Muscular Dystrophies: Recruitment, Clinical Evaluation of Participants, and Physical Examinations
- Association Study of Exon Variants in the NF-κB and TGFβ Pathways Identifies CD40 as a Modifier of DMD

ADVISORY COMMITTEE MEMBERSHIPS

- Educational Committee for Neurophysiology Fellowship, Children's Hospital of Pittsburgh
- Accreditation Council for Graduate Medical Education, Children's Hospital of Pittsburgh
- Advisory committee, Sarepta Therapeutics
- Advisory committee, Biogen
- Advisory board, Sanofi

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- American Academy of Neurology
- Child Neurology Society
- American Academy of Electrodiagnostic and Neuromuscular Medicine
- World Muscle Society
- Egyptian Medical Syndicate

Gulay Alper, MD

Gulay Alper is an associate professor of pediatrics and neurology and director of the Clinical Neuroimmunology Program.

RESEARCH

- Inflammatory Demyelinating Disorders of the Central Nervous System in Children
- Epidemiologic Study Aimed at Distinguishing Acute Disseminated Encephalomyelitis (ADEM) From Multiple Sclerosis (MS) at Its Earliest Diagnosis in Childhood
- Acute Demyelinating Disorders of Childhood—Pittsburgh Pediatric Demyelinating Cohort Study
- Multicenter Pediatric MS Adherence Study
- Discrimination of ADEM and MS at First Presentation by Clinical Features
- Imaging Characteristics of ADEM Versus MS in Children
- Brainstem Presentation of Pediatric MS
- Non-MS Relapsing Demyelination in Children With Positive Myelin Oligodendrocyte Glycoprotein (MOG) Antibodies

ADVISORY COMMITTEE MEMBERSHIPS

- International Pediatric MS Study Group (IPMSSG)
- Clinical Care Committee (an operational subcommittee of IPMSSG), facilitating studies aimed at optimization of care for children with MS and related disorders
- Expert Panel in Pediatric-Acquired Demyelinating Syndromes, United States
- International Affairs Committee, Child Neurology Society

EDITORSHIPS

- Guest editor, special issue, "Autoimmune Inflammatory Disorders of the Central Nervous System in Children," *Journal of Child Neurology*
- Editorial Board, *Journal of Child Neurology*

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Child Neurology Society
- American Academy of Neurology
- International Pediatric MS Study Group

Miya Asato, MD

Miya Asato is the program director of the Neurodevelopmental Disabilities Fellowship. The fellowship is one of eight accredited programs in the United States that provides clinical and research training to physicians who wish to specialize in neurodevelopmental disabilities. This multidisciplinary specialty includes pediatrics, neurology, and developmental and behavioral medicine. There are four fellowship trainees in different stages of training. Asato also serves on the national level as a leader for neurodevelopmental disabilities training and is the Neurodevelopmental Disabilities Special Interest Group leader for the Child Neurology Society.

Asato is director of the LEND Program. This competitive training grant is funded through the Health Resources and Services Administration (HSRA) and currently funds 17 graduate-level trainees in allied health disciplines so they can gain leadership training related to neurodevelopmental

disabilities. The LEND Program in Pittsburgh has been continuously funded for more than 20 years and is one of 52 programs across the United States. Primary training activities of LEND include a yearlong leadership course and a multidisciplinary clinic.

RESEARCH

- Neurobehavioral Status in Pediatric Epilepsy: This five-year National Institutes of Health (NIH) faculty-development grant examines cognitive and psychiatric comorbidities in children with medically treated epilepsy utilizing MRI methods, including functional MRI and diffusion tensor imaging, diagnostic psychiatric interviewing, and cognitive testing.
- Reward Processing in Adolescence: This NIH-funded longitudinal study assesses the developmental changes related to the effects of incentives on the ability to suppress task-inappropriate responses, using eye movements as a model system, as well as functional and structural MRI.
- Innovating Strategies and Replicating Promising Practices Program: This project, funded by the HSRA, is assessing the cognitive changes associated with epilepsy medical treatments using computerized cognitive testing.
- Non-research funding: The LEND Program at the University of Pittsburgh is a multidisciplinary, graduate-level training program for leadership and clinical training in neurodevelopmental disabilities, funded by the HSRA.
- Infection Unmasking Symptoms of Underlying POLG-Related Disease
- Supporting Transition Education for Families of Youth With Autism Spectrum Disorder

ADVISORY COMMITTEE MEMBERSHIPS

- Professional Advisory Board, Epilepsy Foundation of America
- Scientific Selection and Program Committee, Child Neurology Society
- University of Pittsburgh Graduate Medical Education
- Medical Advisory Board, Epilepsy Foundation of Western/Central Pennsylvania
- Epilepsy Section, American Academy of Neurology
- Medical advisor, Emma Bursick Memorial Fund
- Psychosocial Task Force, American Epilepsy Society
- Non-Epileptic Seizures Task Force, American Epilepsy Society
- Epilepsy Research Benchmarks Committee, American Epilepsy Society and NIH
- Local Advisory Board, Office of Child Development, University of Pittsburgh
- Neurology Education Committee, Children's Hospital of Pittsburgh
- Child Development Unit Education Committee, Children's Hospital of Pittsburgh
- Graduate Medical Education Subcommittee on Program Director Development, University of Pittsburgh

EDITORSHIPS

- Editorial Board, *Journal of Pediatric Epilepsy*
- Editorial Board, *Epilepsy and Behavior*

MAJOR LECTURESHIPS AND SEMINARS

- UPMC Graduate Medical Education 2016 Leadership Conference
- "Cognition and the Developing Brain in Pediatric Epilepsy," New York University neurology grand rounds, New York, N.Y., April 2016
- "Epileptic Syndromes, Autism, and the Developing Brain," American Society of Electroencephalographic Technicians annual meeting, Pittsburgh, Pa., August 2016
- "Epilepsy in Children: Growing Up Healthy," Clinic for Special Children Epilepsy Family Day, Strasburg, Pa., August 2017
- "Unknown Until Genome," 46th Annual Child Neurology Society Meeting, Kansas City, Mo., October 2017
- "Future of Neurodevelopmental Disabilities Training," Johns Hopkins Kennedy Krieger Retreat, Williamsburg, Va., November 2017
- "Co-Management of Pediatric Epilepsy: Primary Care and Specialty Care Partnership Model," American Public Health Association annual meeting, Atlanta, Ga., November 2017
- "Epilepsy and Medical Transition," transition conference sponsored by the Department of Neurology, UPMC, Pittsburgh Pa., December 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Neurology
- American Epilepsy Society
- Child Neurology Society
- Association of Pediatric Program Directors
- Autism Treatment Network

HONORS

- Philip Troen, MD, Excellence in Medical Student Research Mentoring Award, 2015
- Best Doctors, *Pittsburgh Magazine*, 2016
- American Academy of Neurology Transforming Leaders Program Awardee, 2017

Catalina Cleves-Bayon, MD

Catalina Cleves-Bayon is an assistant professor of pediatrics at the University of Pittsburgh and director of the Idiopathic Intercranial Hypertension Program at Children's Hospital of Pittsburgh of UPMC. Cleves-Bayon has also developed an interdisciplinary monthly neuro-ophthalmology conference that offers continuing medical education credit.

RESEARCH

- Clinical Outcomes of Patients With Pseudotumor Cerebri Treated in a Multidisciplinary Clinic
- Pseudotumor Cerebri as a Late Presentation of Craniosynostosis in Children
- Radiological Findings in Neuroborreliosis

MAJOR LECTURESHIPS AND SEMINARS

- "Neurodevelopmental Assessment," UPMC Hamot adult neurology lecture series, 2016
- "Idiopathic Intracranial Hypertension," ophthalmology lecture series, 2016
- "Pseudotumor Cerebri and Optic Nerve Edema," PGY-4 Boot Camp, Child Neurology Residency Program
- "Benign Epilepsies of Childhood," UPMC Hamot adult neurology lecture series, September 2017
- "Idiopathic Intracranial Hypertension: Advances in Diagnosis and Management," X Curso Internacional de Pediatría 2017, Hospital Ángeles, Puebla, Mexico, 2017
- "Childhood Migraine: Advances in Diagnosis and Management," X Curso Internacional de Pediatría 2017, Hospital Ángeles, Puebla, Mexico, 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Neurology
- American Headache Society
- North American Neuro-Ophthalmology Society
- Child Neurology Society
- North Central Headache Society

EDITORSHIPS

- Reviewer, *Headache Journal*

Patricia K. Crumrine, MD

Patricia K. Crumrine is a professor of pediatrics and neurology at the University of Pittsburgh and recently was chair of the Board of Directors of the American Board of Psychiatry and Neurology.

RESEARCH

Crumrine performs clinical research aimed at the study of the safety and efficacy of anti-epileptic drugs for children with seizure disorders.

ADVISORY COMMITTEE MEMBERSHIPS

- Examiner and Board of Directors, American Board of Psychiatry/Neurology
- Board of Directors, Epilepsy Foundation of Western/Central Pennsylvania
- Professional Advisory Board, Epilepsy Foundation of Western/Central Pennsylvania
- Medical Records Committee, Children's Hospital of Pittsburgh of UPMC
- Chair, Epilepsy Exam Writing Committee, American Board of Psychiatry and Neurology, 2011 to the present
- Child Neurology Maintenance of Certification Exam Writing Committee, American Board of Psychiatry and Neurology
- Professionalism Exam Writing Committee, American Board of Psychiatry and Neurology, 2014 to the present
- Task Force on Burnout of Neurologists, American Academy of Neurology, 2015 to the present
- Research Subcommittee, American Academy of Neurology, 2015 to the present
- Engagement Committee, American Academy of Neurology, 2015 to the present

EDITORSHIPS

- Editorial Board, *Journal of Child Neurology*

MAJOR LECTURESHIPS AND SEMINARS

- "Lecture to the Child Neurologists: Update on the Use of the Ketogenic Diet," Children's Hospital of Jagellonian University, Krakow, Poland, May 2015
- "Update of Autism," American Academy of Neurology, Vancouver, British Columbia, Canada, April 2016
- "Drug Effects on the EEG," webinar, American Society of Electrographic Technologists, Pittsburgh, Pa., May 2016
- "Penry Pediatric Epilepsy" four talks and two workshops, Winston Salem, N.C., June 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Child Neurology Society
- Professors of Child Neurology
- American Academy of Neurology
- American Neurological Association
- American Clinical Neurophysiology Society
- American Epilepsy Society

HONORS

- *Best Doctors in America*, Woodward/White, Inc., 2009–2015, 2017
- Best Doctors, *Pittsburgh Magazine*, 2013, 2014, 2015, 2017
- Lifetime Achievement Award, Child Neurology Society, October 2015

Dana D. Cummings, MD, PhD

Dana Cummings is an assistant professor of pediatrics at the University of Pittsburgh and director of the Pediatric Stroke Program at Children's Hospital. After working in international development in pediatric neurology, stroke, and neurorehabilitation in former Soviet Central Asia in collaboration with the U.S. Agency for International Development, Cummings is facilitating a long-term partnership between Children's Hospital/University of Pittsburgh School of Medicine and Nazarbayev University School of Medicine and Kazakhstan National Research Center for Maternal and Child Health (NRCRMC). Cummings was the first Children's Hospital faculty member to give a master training class in Astana, Kazakhstan, at NRCRMC. The long-term goal of the project is to produce measurable improvements in medical education and patient outcomes in Kazakhstan and Central Asia.

RESEARCH

- NIH Thrombolysis in Pediatric Stroke, a multisite clinical trial that demonstrated the first model of a network of pediatric brain attack centers
- Relationship between anemia and non-anemic iron-deficiency states and neurology disorders, including transient ischemic attack and stroke
- Neuroimaging of stroke in children
- Cerebral perfusion changes in atypical migraine with aura mimicking stroke
- Neurovascular coupling abnormalities in pediatric brain attack

ADVISORY COMMITTEE MEMBERSHIPS

- Research Advisory Committee, University of Pittsburgh School of Medicine
- Coordinating Center, National Institute of Neurological Disorders and Stroke (NINDS) Stroke Trials Network
- Child Neurology/Neurodevelopmental Disability Clinical Competency Committee

MAJOR LECTURESHIPS AND SEMINARS

- UPMC 20th Annual Stroke Update, Departments of Neurology and Neurosurgery, University of Pittsburgh Medical Center, September 2016

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Neurology
- World Federation of Neurology
- International Child Neurology Association

Luis De Jesus Fernandez, MD

Luis De Jesus Fernandez is an assistant professor of pediatrics at the University of Pittsburgh and specializes in epilepsy and intensive care unit neurology.

RESEARCH

- EpiBioS4Rx, epilepsy bioinformatics study for antiepileptogenic therapy

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Epilepsy Society
- American Academy of Neurology
- American Clinical Neurophysiology Society

HONORS

- General Academic Pediatrics Primary Care Award, selected by the faculty of Children's Hospital of Pittsburgh

Robyn Filipink, MD

Robyn Filipink is a clinical associate professor of pediatrics at the University of Pittsburgh and a neurodevelopmental disabilities specialist. She is medical director of the Fragile X Clinic, a comprehensive center that serves patients through their life spans. She is also the director of the Tourette Syndrome Clinic, which focuses on all aspects of Tourette syndrome, including comorbidities and various therapeutic approaches. She is director of the Movement Disorders Clinic. Once monthly, she runs the Movement Disorders Clinical Case Conference, during which a patient is presented with his or her family, an examination is performed, and a multidisciplinary plan is formulated with input from several medical specialties.

RESEARCH

- Effects of AFQ056 on Language in Young Children With Fragile X Syndrome, site principal investigator (PI)
- Fragile X Clinical and Research Consortium Registry, PI
- Fragile X Syndrome Registry, PI

ADVISORY COMMITTEE MEMBERSHIPS

- UPMC Autism Taskforce, 2016 to the present
- Pediatric Neurology Education Committee, Children's Hospital of Pittsburgh of UPMC, 2006 to the present
- Clinical Competencies for Child Neurology and Neurodevelopmental Disabilities, 2014 to the present
- LEND affiliated faculty member, 2013 to the present
- Fragile X Clinical and Research Consortium, 2010 to the present
- Co-investigator, NeuroNEXT, University of Pittsburgh

MAJOR LECTURESHIPS AND SEMINARS

- "Tourette Syndrome: Case Dissection," UPMC grand rounds, June 2016
- "Tourette Syndrome Update 2015," Three Rivers Pediatric Update, May 2015
- Movement Disorder Case Conference, first Friday of each month, 60-minute live case presentation of a patient with a movement disorder
- Microcephaly, Medlink, 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Neurology
- American Academy of Pediatrics
- Child Neurology Society

Jenna M. Gaesser, MD

Jenna Gaesser is an assistant professor at the University of Pittsburgh, associate director of the Neonatal Neurology Program, codirector of the Neurofibromatosis Clinic, and codirector of Advanced Practice Provider Development and Utilization in the Child Development Unit.

RESEARCH

- Cardiac neurodevelopmental outcomes
- HEAL Clinical Trial

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Child Neurology Society
- American Academy of Neurology

SERVICE

- Education Committee, Child Neurology Residency, Children's Hospital of Pittsburgh

MAJOR LECTURESHIPS AND SEMINARS

- Practicum in fundoscopic exam for pediatric residents

Yanhua Gao, MD, PhD

Yanhua Gao is a research assistant professor at the University of Pittsburgh.

RESEARCH

Yanhua Gao concentrates on T-cell cancer immunotherapy with the goal of developing therapeutic agents and simple and safe strategies to prevent and treat metastases. She studies methods to generate potent anti-tumor memory T cells and to characterize their functional capabilities. She is dissecting the anti-tumor properties of individual T-cell subsets by isolating them from donor mice that have been cured of tumors and transferring them to host mice with established tumors. She establishes animal brain metastasis models and develops methods to bring anti-tumor T cells into the nervous system to eradicate neoplastic metastatic deposits in the brain and leptomeninges. She studies anti-tumor primary and memory T-cell activation, migration, and interaction with other leukocytes, including stimulatory and inhibitory interactions in tumor microenvironments. The results will lay the basis for clinical trials applying targeted virus and anti-tumor specific memory T cells to control and treat brain metastasis.

Amy C. Goldstein, MD

Amy C. Goldstein is an assistant professor at the University of Pittsburgh, as well as director of the Neurogenetics Subdivision and director of the Neurofibromatosis Clinic of Children's Hospital of Pittsburgh of UPMC.

RESEARCH

- North American Mitochondrial Disease Consortium Patient Registry and Biorepository
- Creating models of rare childhood liver diseases using the human liver on a chip
- Industry-sponsored clinical trial (Reata): RTA 408 Capsules in Patients With Mitochondrial Myopathy, PI: Vockley
- Industry-sponsored clinical trial (Stealth Biotherapeutics): SPIMM-202: A Phase II Randomized, Double-Blind, Placebo-Controlled Crossover Study to Evaluate the Safety, Tolerability, and Efficacy of Subcutaneous Injections of Elamipretide (MTP-131) in Subjects With Genetically Confirmed Mitochondrial Disease Previously Treated in the Stealth BioTherapeutics SPIMM-201 Study, PI: Vockley

STUDY SECTIONS

- Invited ad hoc reviewer, Mitochondrial Disease, Peer-Reviewed Medical Research Program (PRMRP), DOD, February 2016
- Invited ad hoc reviewer, Mitochondrial Disease Discovery Award Program, PRMRP, DOD, November 2016

ADVISORY COMMITTEE MEMBERSHIPS

- Education Committee, Division of Child Neurology, Department of Pediatrics, Children's Hospital of Pittsburgh of UPMC, August 2013 to the present
- Scientific and Medical Board, MitoAction, 2013 to the present
- Scientific and Medical Advisory Board, United Mitochondrial Disease Foundation (UMDF), 2015 to the present

- Board of Trustees, UMDF, 2007–2015
- National Symposium Strategic Planning Steering Committee, UMDF, 2012 to the present
- Clinical Research Committee, UMDF, 2012 to the present
- Symposium Steering Committee, UMDF, October 2011 to the present
- Palliative Care Task Force, Jewish Association on Aging, 2008 to the present
- Autism Treatment Network, 2007 to the present; Genetics-Metabolic Subcommittee, October 2009 to the present
- Abstract Review Committee, UMDF, 2016

EDITORSHIPS

- Editorial Board, *Pediatric Neurology*, August 2010 to the present
- Editorial Board, *Journal of Child Neurology*, October 2007 to the present

MAJOR LECTURESHIPS AND SEMINARS

- "Neurofibromatosis: Lessons From Clinical Experience," course organizer, Neurofibromatosis Symposium, Children's Hospital of Pittsburgh of UPMC, April 2016
- "Genetics of Autism Spectrum Disorder: What We Know and How to Use It," neurology grand rounds, UPMC, May 2016
- "Cryptic X Chromosome Alterations in Patients with Allan-Herndon-Dudley Syndrome," Clinical Genomics Case Conference, Magee-Womens Hospital, May 2016
- "Current Issues in the Management of Primary Mitochondrial Disease," invited speaker, Children's Hospital of Philadelphia, University of Pennsylvania Mitochondria Research Affinity Group, July 2016
- "Current Issues in the Management of Primary Mitochondrial Disease," invited speaker, Pennsylvania-Northeast UMFD Regional Symposium, Children's Hospital of Philadelphia, University of Pennsylvania, Philadelphia, Pa., October 2016
- "Genetics of Autism Spectrum Disorder: What We Know and How to Use It," American Academy of Neurology annual meeting, Vancouver, Canada, April 2016

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Allegheny County Medical Society (ACMS)
- Pittsburgh Pediatric Society
- American Academy of Pediatrics
- American Academy of Neurology
- Child Neurology Society
- Society for Inherited Metabolic Disorders
- President, Mitochondrial Medicine Society, 2014 to the present

HONORS

- Best Doctors: Pediatrics, *Pittsburgh Magazine*, 2012–2016

Anuja Vora Jindal, MD, MPH

Anuja Vora Jindal is an assistant professor of pediatrics at the University of Pittsburgh and specializes in neurodevelopmental disabilities. Her research interests include supporting transitional care for youth with autism spectrum disorder nonsyndromic craniosynostosis mimicking pseudotumor cerebri syndrome.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Child Neurology Society
- American Academy of Neurology
- American Academy of Pediatrics

Todd Lamitina, PhD

Todd Lamitina is an associate professor of pediatric cell biology at the University of Pittsburgh.

RESEARCH

- Bipartite regulation of osmosensitive gene expression in *C. elegans*, 2014–2017
- Biomechanical profiling of *C. elegans* motility, 2012–2017
- Mechanisms of C9orf72-associated dipeptide toxicity, 2015–2018
- Characterization of modifiers of C9orf72-associated dipeptide toxicity in a new *C. elegans* model, 2016–2018

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Society for Neuroscience
- American Society of Nephrology
- Genetics Society of America
- American Physiological Society

MAJOR LECTURESHIPS AND SEMINARS

- "Using *C. elegans* to Identify Conserved Modifiers of C9orf72-Associated Dipeptide Toxicity," Motor Neuron Disease Meeting, Orlando, Fla., 2015
- "Using the Model Organism *C. elegans* to Strikeout Lou Gehrig's Disease," University of Pittsburgh Department of Developmental Biology, 2016
- "Modeling the Complexities of C9orf72 Toxicity in the Simple Organism *C. elegans*," University of Pittsburgh, Live Like Lou Center for ALS Research Conference, 2016
- "A *C. elegans* Model for C9orf72 Toxicity," University of Pittsburgh Division of Neuropathology, 2016
- "Using the Model Organism *C. elegans* to Strikeout Lou Gehrig's Disease," University of Pittsburgh Summer Undergraduate Research Program, 2016

- "Using the Model Organism *C. elegans* to Strikeout Lou Gehrig's Disease," University of Pittsburgh Honors College Health Sciences, 2016
- "Modeling Neurodegenerative Disease Mechanisms in the Model System *C. elegans*," Pittsburgh Institute for Neurodegenerative Diseases, University of Pittsburgh, 2016
- "Novel Translational Products Encoded by Disease-Associated GC-Rich Repeat Expansions Cause Toxicity in *C. elegans*," 15th Annual Pittsburgh Symposium on Intracellular Membrane Traffic, 2017

EDITORSHIPS

- Academic editor, *PLoS ONE*

Monica Naik, MD

Monica Naik is a clinical assistant professor at the University of Pittsburgh and director of the Neonatal Neurology Program. Her interest lies in neonatal neurology and fetal diagnostics. Consultation services are provided at Children's Hospital and Magee-Womens Hospital. Antenatal consultations are provided at Magee-Womens Hospital through the Fetal Diagnostics and Treatment Center. She follows newborns with complex neurological diagnoses in the newborn specialty clinic.

RESEARCH

- Phenobarbital Levels in Neonates: A Comparison Between Blood and Saliva Levels
- Encephalopathy Undergoing Hypothermia
- Placental Origin of Neonatal Brain Injury
- HEAL Study: High-Dose Erythropoietin for Asphyxia and Encephalopathy, proposed multicenter study
- Feasibility of contrast-enhanced transfontanelle ultrasound: comparison with magnetic resonance imaging (MRI) in the neonate
- Evaluation of efficacy and safety of oxcarbazepine for the management of neonatal seizures
- The correlation between a short-term conventional EEG in the first day of life and brain MRI in newborns undergoing hypothermia for hypoxic-ischemic encephalopathy

MAJOR LECTURESHIPS AND SEMINARS

- Talk on basics of neonatal EEG, invited speaker, ASET—the Neurodiagnostic Society Symposium, Pittsburgh, Pa., 2016

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Indian Academy of Pediatrics
- American Academy of Neurology
- Child Neurology Society

Udai Pandey, PhD

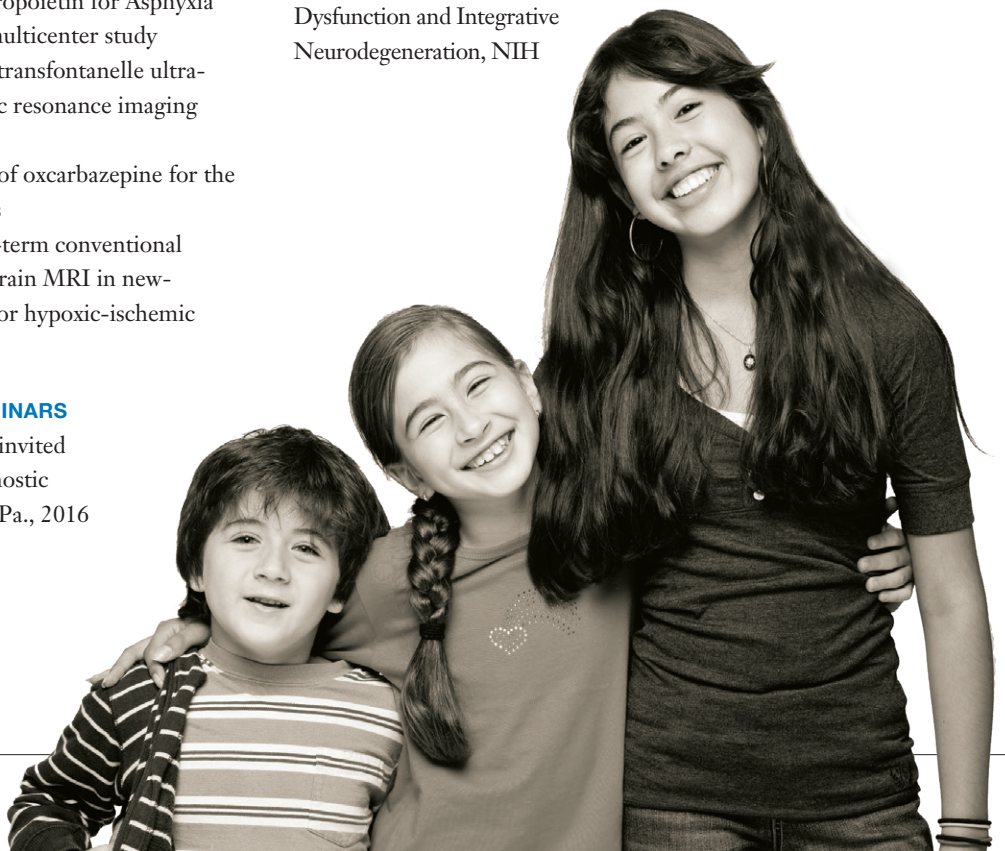
Udai Pandey is an associate professor of pediatrics and human genetics at the University of Pittsburgh.

RESEARCH

Pandey is interested in understanding the molecular basis of ALS and other related motor neuron degeneration diseases so that effective therapies can be developed. Recently, mutations in RNA-binding proteins have been identified in both sporadic and familial forms of ALS. Disease-causing mutations in these RNA-binding proteins indicate that defects in RNA metabolism might play an important role in causing motor neuron degeneration in ALS. Pandey's laboratory has developed *Drosophila* models of ALS that recapitulate several key pathological features of human disease, such as neurodegeneration and behavioral defects. Pandey's laboratory has been utilizing biochemical, cell biological, and genetic tools to dissect molecular mechanisms of ALS in *Drosophila* and mammalian neuronal models.

STUDY SECTIONS

- Ad hoc member, Friedreich Ataxia Research Association, 2010 to the present
- Ad hoc member, Israel Science Foundation, 2014
- Ad hoc member, Chronic Dysfunction and Integrative Neurodegeneration, NIH



- Ad hoc member, Medical Advisory Board, Muscular Dystrophy Association
- Ad hoc member, Special Emphasis Panel, ZRG1 BDCN-W (03), NIH
- Ad hoc member, Molecular Neurogenetics Study Section, NIH
- Ad hoc member, AFM-Telethon Study Section, France
- Ad hoc member, Neural Oxidative Metabolism, Mitochondria, and Cell Death Study Section, NIH

EDITORSHIPS

- Academic editor, *PLOS One*
- Editorial Board, *JSM Genetics and Genomics*
- Editorial Board, *American Journal of Neuroscience*
- Editorial Board, *Austin Neurology*
- Editorial Board, *Scientific Reports*

MAJOR LECTURESHIPS AND SEMINARS

- "ALS-Causing Mutations in FUS Perturb Cytoplasmic Stress Granule Dynamics and Cause Neurodegeneration," Neuropathology Division, Department of Neurology, UPMC, Pittsburgh, Pa.
- "Pur Alpha Ameliorates FUS-Mediated Neuro-degeneration and Regulates Cytoplasmic Stress Granule Dynamics," 16th Annual Robert Packard Center for ALS Research Symposium, Baltimore, Md., March 2016
- "Function and Dysfunction of RNA-Binding Proteins in ALS: Models and Mechanisms," Department of
- Pathology, Case Western Reserve University, Cleveland, Ohio, host: Xinglong Wang, April 2016
- "Molecular Genetic Approaches to Define the Mechanisms of FUS-Mediated ALS," Department of Neurology, University of Massachusetts, Worcester, Mass., host: Fen-Biao Gao, April 2016
- "Identifying Genetic Modifiers of FUS Toxicity in a *Drosophila* Model of ALS," annual Drosophila Research Conference, Orlando, Fla., July 2016
- "Identifying Genetic Modifiers of FUS-Mediated Neurodegeneration," Packard Center for ALS at Johns Hopkins, Baltimore, Md., January 2017
- "Molecular Determinants of RNA-Mediated Toxicity in ALS," Pittsburgh Institute for Neurodegenerative Diseases, Pittsburgh, Pa., January 2017
- "Muscleblind Protects Against FUS-Mediated Neuro-degeneration," Gordon Research Conference on ALS and Related Motor Neuron Diseases, Stowe, Vt., July 2017
- International Conference on Neurology and Stroke, Valencia, Spain, June 2017
- International Conference on Translational Neurosciences and its Application in Protection of Mental Health, Odisha, India, October 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Society for Neuroscience, 2007 to the present
- Genetics Society of America, 2012 to the present

HONORS

- "Best Short Talk," International Conference on Molecular Neurodegeneration, Seoul, South Korea, 2016

Christina Patterson, MD

Christina Patterson is an assistant professor of pediatrics at the University of Pittsburgh. Patterson is the director of the Epilepsy Monitoring Unit, medical director of the Pediatric Epilepsy Surgery Program, codirector of the University of Pittsburgh Comprehensive Epilepsy Center, and director of epilepsy services at Children's Hospital of Pittsburgh of UPMC.

RESEARCH

- Funded R01 project: Recovery of High-Level Visual Function in Patients with Lobectomy or Hemispherectomy, clinical investigator
- Laboratory data for prediction of 30-day hospital readmission of pediatric seizure patients
- Maternal Outcomes and Neurodevelopmental Effects of Anti-Epileptic Drugs study, a multicenter national study investigating antiepileptic drug use in pregnancy and effects on children born to mothers with epilepsy, co-investigator
- Clinical trials for novel treatments for medically intractable epilepsy, co-investigator
- Assessing the accuracy of ictal and interictal single-photon emission computed tomography versus interictal 18F-fluorodeoxyglucose
- Childhood absence epilepsy pharmacokinetics, pharmacodynamics, and pharmacogenetics
- Investigation of CDKL5 and its role in brain ependymal cilia function
- Assessing the accuracy of ictal and interictal SPECT versus interictal fluorodeoxyglucose (FDG) positron emission tomography (PET), an observational data-collection study to assess the accuracy of localization of the epileptogenic zone in ictal and interictal SPECT testing versus interictal FDG PET testing in patients being evaluated for epilepsy surgery

MAJOR LECTURESHIPS AND SEMINARS

- "Diagnosing Pediatric Epilepsy," Three Rivers Pediatric Update, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., May 2015

- "Diagnosing Epilepsy," University of Pittsburgh Nurse Practitioner Training Program, September 2012 and ongoing annually
- "Temporal Lobectomy Versus Hemispherectomy, Invasive Monitoring Case Presentation," International Society for Pediatric Neurosurgery, Denver, Colo., 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Neurology
- American Epilepsy Society
- Child Neurology Society
- National Association of Epilepsy Centers (NAEC)

EDITORSHIPS

- Invited journal referee, *Journal of Clinical Neurophysiology*
- Invited journal referee, *Neurodiagnostic Journal*

Deepa Rajan, MD

Deepa Rajan is an assistant professor of pediatrics at the University of Pittsburgh and codirector of the Neurogenetics Program.

RESEARCH

- Neurogenetics Program Patient Registry: clinical and genetic diagnosis, natural history study, translational research, and biorepository
- Next-Generation Sequencing and Metabolomics in Pediatric Neurogenetic Disorders
- Functional characterization of GEMIN5, establishing a gene-discovery pipeline from variants of unknown significance on next-generation sequencing

ADVISORY COMMITTEE MEMBERSHIPS

- Child Neurology Education Committee, Children's Hospital of Pittsburgh

MAJOR LECTURESHIPS AND SEMINARS

- Neuroanatomy lecture series to the child neurology residents
- "What Do These Orders Really Mean? Simplified Approach to Common Metabolic Tests Ordered in Child Neurology," annual lecture to pediatric residents and medical students, Pittsburgh, Pa., 2016

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Neurology
- Child Neurology Society
- American Epilepsy Society
- Accreditation Council for Graduate Medical Education, setting up milestones for Child Neurology Residency

Robert Safier, MD

Robert Safier is an associate professor of pediatrics at the University of Pittsburgh, director of the Medical Student and Resident Child Neurology Education Program, and codirector of the Child Neurology Inpatient Floor Team.

ADVISORY COMMITTEE MEMBERSHIPS

- Child Neurology Education Committee, Children's Hospital of Pittsburgh
- Codirector, Child Neurology Inpatient Floor Team

MAJOR LECTURESHIPS AND SEMINARS

- "The Impact of Genetic Testing in Infantile-Onset Epilepsy," poster presentation, Child Neurology Society, Vancouver, Canada, fall 2016
- "The Multimodal Approach to Atypical Migraine With Aura Mimicking Stroke: Time Course of Cerebral Perfusion Changes," poster presentation, Child Neurology Society, Vancouver, Canada, fall 2016

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Neurology
- Child Neurology Society

Levi Shelton, MD

Levi Shelton is an assistant professor of pediatrics at the University of Pittsburgh, codirector of Advanced Practice Provider Development and Utilization, and director of Visiting Resident Education in the Child Neurology Division.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Child Neurology Society
- American Academy of Neurology

SERVICE

- Education Committee, Child Neurology Residency, Children's Hospital of Pittsburgh
- Transition of Care Task Force, Children's Hospital of Pittsburgh

MAJOR LECTURESHIPS AND SEMINARS

- "Pediatric Board Review: Neurology," fellow facilitator, 40 pediatric residents, April 2016
- "Fast Facts in Neuro Infectious Diseases," fellow lecturer, 15 child neurology/neurodevelopmental disabilities residents, May 2016
- "Bacterial Infections of the Central Nervous System," invited fellow lecturer, 20 medical residents, Teine-Keijinkai Hospital, Sapporo, Japan, January 2017

- "Headache," invited fellow lecturer, 20 medical residents, Teine-Keijinkai Hospital, Sapporo, Japan, January 2017
- "The Neurologic Exam," invited fellow lecturer, 12 medical residents, Teine-Keijinkai Hospital, Sapporo, Japan, January 2017

Bilal Sitwat, MD

Bilal Sitwat is an assistant professor of pediatrics at the University of Pittsburgh and a member of the epilepsy subdivision. He is interested in using neuromodulation to treat refractory epilepsy.

RESEARCH

- Refractory Status Epilepticus in Children
- Levetiracetam Effectiveness in the Treatment of the Electrical Status Epilepticus During Slow-Wave Sleep
- Determining whether topiramate and zonisamide cause oligohydrosis as well as alter sweat electrolyte concentrations
- A 12-month, open-label study to evaluate the safety and tolerability of pregabalin as adjunctive therapy in pediatric subjects 1 month to 16 years of age with partial-onset seizures and pediatric and adult subjects 5–65 years of age with primary generalized tonic-clonic seizures

ADVISORY COMMITTEE MEMBERSHIPS

- Epilepsy consultant, Epilepsy Foundation of Western/Central Pennsylvania, March 2012 to the present
- Project Access, HSRA

MAJOR LECTURESHIPS AND SEMINARS

- "Sudden Unexplained Death in Epilepsy: Know the Facts! Supporting Parents, Supporting Kids," regional conference for the Epilepsy Foundation of Western/Central Pennsylvania, November 2016
- "Current Practice in Pediatric Epilepsy and Its Management," invited lecturer, Children's Institute of Pittsburgh, May 2017
- "Uncontrolled Seizures: What Every Patient and Parent Should Know!" invited speaker, Epilepsy Foundation of Western/Central Pennsylvania, November 2017
- "Current Practice of Head Imaging in Pediatric Trauma and Epilepsy," Wheeling Hospital, W.Va., November 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Neurology
- American Epilepsy Society
- Child Neurology Society
- American Academy of Pediatrics

Yoshimi Sogawa, MD, MS

Yoshimi Sogawa is an associate professor of pediatrics at the University of Pittsburgh, the divisional director of clinical research, and a member of the epilepsy subdivision of the Child Neurology Division. She has a master's degree in clinical research methods and has more than 20 publications in the field of child neurology and epilepsy. She has started a prospective observational study of infants and young children with epileptic encephalopathy to address their cognitive outcomes and risk factors and has enrolled 81 patients since 2013. She is collaborating on the new NIH-funded Established Status Epilepticus Treatment Trial and is the site PI for a Pennsylvania study of the use of cannabidiol for refractory epilepsy in children.

RESEARCH

- Prospective observational study about epileptic encephalopathy in young children (75 patients enrolled), PI
- The Effect of Vagus Nerve Stimulation in Children With a History of Status Epilepticus, PI
- Use of Vagus Nerve Stimulator on Primary Generalized Epilepsy, PI
- The Impact of Genetic Testing in Infantile-Onset Epilepsy, PI
- Established Status Epilepticus Treatment Trial, co-investigator
- A study on the effect of Epidiolex® in children with intractable epilepsy, site PI
- Subspecialty clinical/clinical research expert (co-investigator) at the University of Pittsburgh in NeuroNEXT network, an NIH-sponsored network to perform multicenter neuroscience clinical research studies (RFA-NS-17-024)

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Epilepsy Society

ADVISORY COMMITTEE MEMBERSHIPS

- American Epilepsy Society
- Pediatric Epilepsy Research Consortium, representing the children's hospital of Pittsburgh, 2017 to the present

MAJOR LECTURESHIPS AND SEMINARS

- "Pediatric Epilepsy Syndromes," annual clinical pharmacology course, University of Pittsburgh School of Medicine, 2014–2017
- "Cognitive Outcome of Infantile Spasms: Are We Making a Difference?" pediatric grand rounds, Children's Hospital of Pittsburgh of UPMC, July 2017

Kavita Thakkar, MD

Kavita Thakkar is an assistant professor of pediatrics at the University of Pittsburgh. She is codirector of the Clinical Neuroimmunology Program. Her research interests include immune-mediated disorders of the nervous system, autoimmune and demyelinating disorders of the brain, and acute brain stem clinical and radiological syndromes in children.

MAJOR LECTURESHIPS AND SEMINARS

- "Immunizations in a Child with Neurological Disorders," Three Rivers Pediatric Conference, University of Pittsburgh, Pittsburgh, Pa., May 2015
- "Acute Ataxia in Childhood," boot camp lecture, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., July 2016

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- The Consortium of MS Centers
- Child Neurology Society
- American Academy of Neurology

Inna Vaisleib, MD

Inna Vaisleib is an associate professor of pediatrics at the University of Pittsburgh and a member of the epilepsy subdivision of the Child Neurology Division.

RESEARCH

- Open-Label, Pharmacokinetics, Safety, and Efficacy Study of Adjunctive Brivaracetam in Children With Epilepsy (N01263)
- Open-Label Long-Term Follow-Up Study of Adjunctive Brivaracetam in Pediatric Subjects With Epilepsy (NO1266)
- Long-Term Follow-Up Partial Epilepsy Study (Sepracor 093-50)
- Randomized, Double-Blind, Placebo-Controlled Study of the Safety and Efficacy of Intranasal Midazolam (USL261) in the Outpatient Treatment of Subjects With Seizure Clusters (Upsher-Smith P261-401A)

ADVISORY COMMITTEE MEMBERSHIPS

- Quality Review Committee in Neurophysiology, Children's Hospital of Pittsburgh
- Director, industry-supported studies and education, Division of Child Neurology
- Director, Intensive Pediatric Epilepsy and Neurophysiology Teaching Program, Children's Hospital
- Physician director of medical services, Epilepsy Camp Frog
- Consultant to pharmaceutical companies on new antiepileptic drugs

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Neurology
- Child Neurology Society
- American Epilepsy Society

Rajiv R. Varma, MD

Rajiv R. Varma is a clinical professor of pediatrics at the University of Pittsburgh and clinical director of the division. He has overseen rapid clinical growth of the division, improvement in access to the physicians, and establishment of multiple satellite and outreach locations. He launched a quality-assessment program. He mentors young faculty. Varma is past president of the ACMS and the ACMS Foundation. The ACMS is a 3,000-physician-member professional organization representing the interests of and advocating for patients and physicians. The foundation raises money to provide grants for scholarships, health-related research projects, and service organizations.

ADVISORY COMMITTEE MEMBERSHIPS

- Professional Advisory Board, Epilepsy Foundation of Western/Central Pennsylvania
- House of Delegates, chair of the International Medical Graduate Section, Pennsylvania Medical Society
- Peer Review Committee, Board of Directors, chair of the Membership Committee, and treasurer, ACMS

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- American Academy of Neurology
- Child Neurology Society
- American Medical Association
- Mitochondrial Medicine Society

MAJOR LECTURESHIPS AND SEMINARS

- "The Global Burden of Epilepsy," Professor Shiv N. Singh Memorial Lecture, Annual Scientific Program of Patna Medical College, Patna, India, February 2017

Shelley Williams, MD

Shelley Williams is an associate professor of pediatrics at the University of Pittsburgh, a member of the epilepsy subdivision of the Child Neurology Division, director of the Pediatric Electrophysiology Fellowship Program, and director of the Pediatric Ketogenic Diet Clinic.

ADVISORY COMMITTEE MEMBERSHIPS

- Advisory Board, Tuberous Sclerosis Alliance Education Committee, Neurology Division

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Child Neurology Society
- American Epilepsy Society

TEACHING ACTIVITIES

CHILD NEUROLOGY RESIDENCY TRAINING PROGRAM

The Child Neurology Residency Training Program is an accredited three-year program. The first year of the residency is spent in the Adult Neurology Program at UPMC. The second and third years of the residency are spent in the Child Neurology Program at Children's Hospital of Pittsburgh of UPMC. Sitwat is a Bridges Program resident mentor.

NEURODEVELOPMENTAL DISABILITIES RESIDENCY TRAINING PROGRAM

One neurodevelopmental disabilities resident each year is accepted for a four-year training program that leads to board eligibility in neurodevelopmental disabilities and in neurology with special competence in child neurology by the American Board of Psychiatry and Neurology.

PEDIATRIC EPILEPSY FELLOWSHIP TRAINING PROGRAM

The Epilepsy Center at Children's Hospital of Pittsburgh of UPMC offers a one-year fellowship in neurophysiology. The program involves working in both inpatient and outpatient settings with the pediatric seizure population. In addition, there are rotations in EEG, prolonged video EEG monitoring, neuromuscular physiology, adult epilepsy, sleep disorders, and evoked responses.

MEDICAL SCHOOL TEACHING 2015–2017

- Clinical teaching in outpatient and inpatient settings (all faculty)
- Lecture series to medical students on clinical rotations (all faculty)
- Core Child Neurology Lecture Series (all faculty)
- Anatomy course/clinical basic skill MS1 (Abdel-Hamid)
- DMD, MS3 and MS4, University of Pittsburgh (Abdel-Hamid)
- Advanced Physical Exam course, MS1, University of Pittsburgh (Abdel-Hamid, Fernandez)
- Methods and Logic in Medicine MS2 (Alper)
- Scientific Management and Leadership course (Asato)
- Neurosciences problem-based learning small-group facilitator, MS1 (Asato)
- Neurosciences lecture, Cerebral Palsy: Neuroanatomic and Clinical Correlates, MS1 (Asato)
- Autism in the Dental Clinic (Asato)
- LEND director for 17 graduate student trainees and course codirector for 2174 HRS_2090 Leadership Seminars in Maternal Child Health and 2181_HRS_2079_Children with Disabilities (Asato)
- Class lecturer, PSYED 2530 course: Applied Developmental Psychology (master's level) lecture on "Neurodevelopmental Disabilities" (Asato)
- Transition to Internship: Focus on Disabilities, seminar organizer and facilitator (Asato)
- UPMC Graduate Medical Education 2017 Leadership Conference (Asato)
- Coordinator and director, Clinical Conference in Child Neurology, University of Pittsburgh (Bergman)
- Pediatric Brain Attack: Assessment and Management Pediatric Emergency Medicine Core Lecture (Cummings)
- Clinical Case in Traumatic Brain Injury (Cummings)
- Coordinator and mediator, Movement Disorder Case Conference (Filipink)
- Tourette Clinic introduction lecture (Filipink)
- Fragile X Clinic introduction and family pedigree lectures (Filipink)

- Grant Writing for Graduate Students (Lamitina)
- Imaging Cell Biology in Living Systems: From Single Molecules to Animal Models (Lamitina)
- Cell Biology of Normal and Disease States (Lamitina)
- Pathobiology of Neurodegeneration (Lamitina)
- Cellular and Molecular Neurobiology course lectures (Pandey)
- Neuroanatomy Workshop facilitator (Pandey)
- Faculty preceptor, Child Neurology Elective–Epilepsy Focus (Patterson)
- EEG record review (Patterson)
- Cranial Nerves, the Neurologic Exam, and Brain Death (Patterson)
- Advanced Physical Examination course, MSII (Rajan)
- Teaching facilitator, Neuroscience Problem-Based Learning (Rajan)
- Oversight of the MS-3 students during their third-year clinical clerkships, two students for each three-week neurology rotation (Safier)
- Director of medical student education, Children's Hospital of Pittsburgh (Safier)
- Pediatric Epilepsy Syndromes, annual course in Clinical Pharmacology (Sogawa)

RESIDENCY TEACHING 2015-2017

- Clinical teaching in outpatient and inpatient settings (all faculty)
- Core Child Neurology Lecture Series (all faculty)
- Bootcamp for new child neurology residents (all faculty)
- Mentor for scholarly projects (many faculty)
- Core neurophysiology teaching and precepting (all epilepsy faculty)
- Critical care fellows, EEG review and lectures, weekly (all epilepsy faculty)
- Preceptor for the first-year child neurology residents (four residents), continuity clinic (Abdel-Hamid)
- Neuromuscular Pathology Conference (Abdel-Hamid)
- Pediatric neuromuscular curriculum lectures (Abdel-Hamid)
- EMG skills and pediatric neuromuscular evaluation (Abdel-Hamid)
- Supervise NDD and child neurology didactic conferences and journal clubs (Asato)
- Western Psychiatric Institute and Clinic (WPIC) psychiatry residents and child psychiatry fellows, "Autism and Epilepsy for Psychiatrists" (Asato)
- Coordinator and director, Clinical Conference in Child Neurology, University of Pittsburgh (Bergman)
- Pseudotumor cerebri curriculum (Cleves-Bayon)
- Neuro-ophthalmology series (Cleves-Bayon)
- Neuro-ophthalmology conference (Cleves-Bayon)
- Cerebrovascular disorder curriculum, Pediatric Critical Care Medicine Fellowship (Cummings)
- Coordinator and mediator, Movement Disorder Case Conference (Filipink)
- Neurodevelopmental disabilities core lectures (Filipink)
- MERCK clinic lecture series, NDD topics (Filipink)
- Molecular Pathobiology course (Lamitina)
- Developing Successful Strategies for NIH K Awards (Lamitina)
- Neurology RITE Exam/Board Review (Patterson)
- Neuro-anatomy curriculum (Rajan)
- Neurophysiology/neuropharmacology curriculum (Rajan)
- Physician Assistant Child Neurology Orientation Program, mentoring (Shelton)
- Journal club faculty facilitator (Sogawa)
- Ketogenic Diet Overview/Troubleshooting, neurology residents, annual course (Williams)
- Tuberous Sclerosis Clinic, monthly, residents and fellows (Williams)

COLLEGE STUDENTS: OBSERVERS

MEDICAL STUDENTS: SECOND, THIRD, AND FOURTH YEARS

- UPMC
- Other national medical schools
- International medical schools

RESIDENCY TRAINING

- University of Pittsburgh Medical School Neurology Residency
- Allegheny General Hospital Neurology Residency
- Allegheny General Hospital Neurophysiology Fellowship
- Allegheny General Hospital Psychiatry Residency
- Hamot Medical Center Neurology Residency
- WPIC Psychiatric Residency
- WPIC Triple-Board Residency Program
- Children's Hospital of Pittsburgh Pediatric Residency Program
- UPMC Physical Medicine and Rehabilitation Residency
- University of Pittsburgh School of Dentistry Residency

THREE-YEAR BIBLIOGRAPHY

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DIVISION OF DEVELOPMENTAL-BEHAVIORAL PEDIATRICS

Mission

The goal of the Division of Developmental-Behavioral Pediatrics is to encourage healthy physical, motor, social, emotional, behavioral, and intellectual growth for children younger than 8 years who have developmental and/or behavioral conditions. The staff endeavors to help parents understand their children's development, behavior, and developmental disorders and/or the long-term consequences of medical conditions, such as premature birth or genetic disorders. The division recommends treatments that will allow children to meet their potential. The Child Development Unit (CDU) places special emphasis on children with autism spectrum disorder (ASD); attention-deficit/hyperactivity disorder (ADHD); Fragile X, neurofibromatosis; or genetic, fetal, or birth-related neurodevelopmental disorders.

The division's mission is:

- To provide exemplary service to children with developmental and/or behavioral concerns, children at risk for long-term developmental challenges, and the families of those children
- To provide training in child development, developmental disabilities, behavioral health, and developmental-behavioral pediatrics to students and practitioners from a wide variety of disciplines
- To complete research aimed at improving quality of life or improving the understanding of developmental and/or behavioral conditions
- To generate and disseminate new knowledge about prevention, identification, etiology, impact, and treatment of developmental and behavioral conditions
- To provide expertise to the hospital, medical school, and community regarding prevention, identification, and treatment of developmental and behavioral conditions in childhood
- To collaborate with community agencies and programs in an effort to improve services for children with developmental or behavioral conditions and their families
- To advocate for the needs of children with developmental or behavioral conditions and their families at all levels

FACULTY

Ira Bergman, MD, PhD
Chief, Division of Developmental-Behavioral Pediatrics
Professor of Pediatrics, Neurology, and Immunology

Miya Asato, MD
Associate Professor of Pediatrics, Psychiatry, and Clinical and Translational Science
Program Director, Neurodevelopmental Disabilities Program
Associate Program Director, Child Neurology Residency Program
Director, Leadership Education in Neurodevelopmental and Related Disabilities (LEND) Program of Pittsburgh

Diego Chaves-Gnecco, MD, MPH
Associate Professor of Pediatrics
Program Director and Founder, Salud Para Niños

Robyn Filipink, MD
Clinical Associate Professor of Pediatrics
Medical Director, Fragile X Clinic
Medical Director, Tourette Syndrome Clinic
Medical Director, Movement Disorder Clinic

Jenna M. Gaesser, MD
Assistant Professor of Pediatrics
Associate Director, Neonatal Neurology Program
Codirector, Neurofibromatosis Clinic
Codirector, Advanced Practice Provider Development and Utilization in the CDU

Sara C. Hamel, MD
Associate Professor of Pediatrics

Kristin Hannibal, MD
Associate Professor of Pediatrics
Clinic Director, General Academic Pediatrics
Clinical Director, Developmental-Behavioral Pediatrics

Beth Heuer, DNP, RN, CRNP, CPNP-PC, PMHS
Nurse Practitioner, CDU
Nurse Practitioner, Tourette Clinic
Nurse Practitioner, Neurofibromatosis Clinic

Anuja Vora Jindal, MD, MPH
Assistant Professor of Pediatrics

Robert B. Noll, PhD
Professor of Pediatrics, Psychiatry, and Psychology
Faculty, LEND Program of Pittsburgh

OVERVIEW OF DIVISION

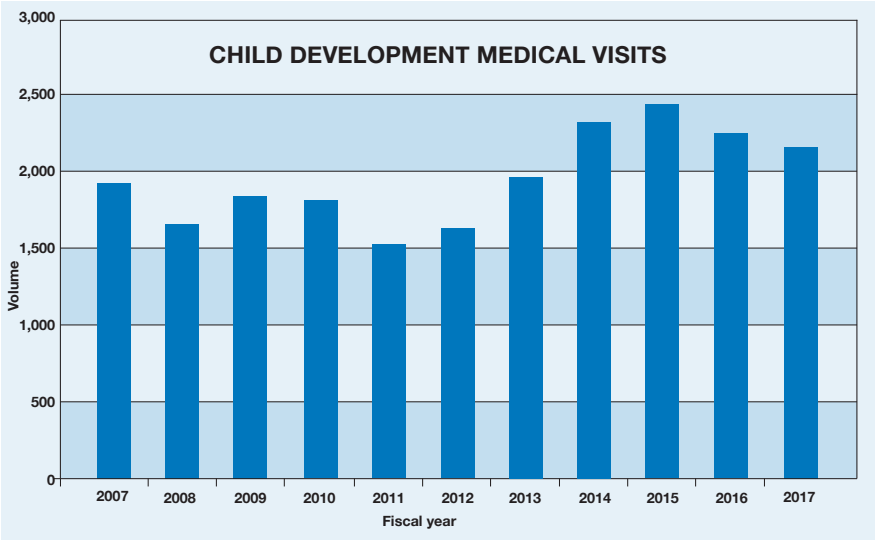
The CDU includes the Autism Center and Fragile X Clinical and Research Consortium, and it provides evaluation and selected treatments for children with a wide range of developmental and behavioral disorders. Patients in Western Pennsylvania and surrounding areas now have rapid access to service.

The Fragile X Clinical and Research Consortium has continued to expand its base of patients. The clinic follows more than 100 patients with Fragile X, providing multidisciplinary care and access to local and national treatment protocols. Pittsburgh is one of 28 sites in the United States participating in the Fragile X Clinical and Research Consortium.

The LEND Program of Pittsburgh is one of 52 training grants within 44 states funded through the Health Resources and Services Administration (HRSA). The LEND center offers a multidisciplinary clinic, opportunities for research, and training in community advocacy. Problem-based learning and a focus on family-centered care are primary mechanisms to create the next generation of leaders who will provide care to children with special needs and their families. The funded LEND faculty represents 14 different training disciplines, including medicine, psychology, audiology, speech-language pathology, public health, occupational therapy, and physical therapy, and the program has students from many of those disciplines each year. This year, the LEND Program of Pittsburgh has 17 long-term trainees. The LEND Program leads a multicenter Case Control Study Supplement to examine outcomes for LEND trainees.

CLINICAL ACTIVITIES

During fiscal year 2017, CDU physicians and nurse practitioners consulted with 2,144 children.



Note: Volume includes medical patients seen at the Oakland and Pine Center sites.

RESEARCH AND OTHER SCHOLARLY ACTIVITIES

Ira Bergman, MD, PhD

RESEARCH

- "Replicating Recombinant Vesicular Stomatitis Virus," specifically targets cancer cells and treats cancer by direct killing and by stimulating the immune system to recognize and kill cancer cells
- "Viral Immunotherapy to Eradicate Subclinical Brain Metastases," U.S. Department of Defense (DOD), 2015
- "Memory Anti-Tumor T Cells Resist Inhibition by Immune Suppressor Cells," anticancer research, 2015

STUDY SECTIONS

- Chair, Peer-Reviewed Cancer Research Program, Pediatric Brain Tumor Review Panel, DOD
- Chair, Tuberous Sclerosis Complex Research Program, Clinical and Population Studies Review Panel, DOD
- Chair, Visionary Postdoctoral Fellowship, DOD

ADVISORY COMMITTEE MEMBERSHIPS

- Research Advisory Committee, Children's Hospital of Pittsburgh of UPMC
- Magnetoencephalography Oversight Board, UPMC

PROFESSIONAL AFFILIATIONS/ SOCIETY MEMBERSHIPS

- Child Neurology Society
- American Academy of Neurology
- American Academy of Pediatrics
- American Association of Immunology
- American Association of Cancer Research
- American Pediatric Society/Society for Pediatric Research

Ira Bergman, MD, PhD
Division Chief, Developmental-Behavioral Pediatrics

HONORS

- Best Doctors, *Pittsburgh Magazine*, 2009–2017
- Five-Star Excellence Award, Professional Research Consultants, 2015 and 2016
- *Best Doctors in America*, Woodward/White, Inc.
- U.S. patent application 11/227,778, titled "Targeting Viruses Using a Modified Sindbis Glycoprotein"

Miya Asato, MD

Miya Asato is the program director of the Neurodevelopmental Disabilities Fellowship. The fellowship is one of eight accredited programs in the United States that provides clinical and research training to physicians who wish to specialize in neurodevelopmental disabilities. This multidisciplinary specialty includes pediatrics, neurology, and developmental and behavioral medicine. There are four fellowship trainees in different stages of training. Asato also serves on the national level as a leader for training in neurodevelopmental disabilities and is the Neurodevelopmental Disabilities Special Interest Group leader for the Child Neurology Society.

Asato is director of the LEND Program. This competitive training grant is funded through the HSRA and currently funds 17 graduate-level trainees in allied health disciplines so they can gain leadership training related to neurodevelopmental disabilities. The LEND Program in Pittsburgh has been continuously funded for more than 20 years and is one of 52 programs across the United States. Primary training activities of LEND include a yearlong leadership course and a multidisciplinary clinic.

RESEARCH

Neurobehavioral Status in Pediatric Epilepsy. This five-year National Institutes of Health (NIH) faculty-development grant examines cognitive and psychiatric comorbidities in children with medically treated epilepsy utilizing magnetic resonance imaging (MRI) methods, including functional MRI and diffusion tensor imaging, diagnostic psychiatric interviewing, and cognitive testing.

Reward Processing in Adolescence. This NIH-funded, longitudinal study assesses developmental changes related to the effects of incentives on the ability to suppress task-inappropriate responses, using eye movements as a model system, as well as functional and structural MRI.

Innovating Strategies and Replicating Promising Practices Program. This project, funded by the HRSA, is assessing cognitive changes associated with epilepsy medical treatments using computerized cognitive testing.

Non-Research Funding. The LEND Program at the University of Pittsburgh is a multidisciplinary graduate-level training program for leadership and clinical training in neurodevelopmental disabilities, funded by the HRSA. In addition, Asato is conducting the following studies: "Infection Unmasking Symptoms of Underlying Progressive External Ophthalmoplegia–Related Disease" and "Supporting Transition Education for Families of Youth With Autism Spectrum Disorder."

ADVISORY COMMITTEE MEMBERSHIPS

- Professional Advisory Board, Epilepsy Foundation of America
- Scientific Selection and Program Committee, Child Neurology Society
- University of Pittsburgh Graduate Medical Education
- Medical Advisory Board, Epilepsy Foundation of Western/Central Pennsylvania
- Epilepsy section, American Academy of Neurology
- Medical advisor, Emma Bursick Memorial Fund
- Psychosocial Task Force, American Epilepsy Society
- Non-Epileptic Seizures Task Force, American Epilepsy Society
- Epilepsy Research Benchmarks Committee, American Epilepsy Society/NIH
- Local Advisory Board, Office of Child Development, University of Pittsburgh
- Neurology Education Committee, Children's Hospital of Pittsburgh
- CDU Education Committee, Children's Hospital of Pittsburgh
- Graduate Medical Education Subcommittee on Program Director Development, University of Pittsburgh

EDITORSHIPS

- Editorial Board, *Journal of Pediatric Epilepsy*
- Editorial Board, *Epilepsy and Behavior*

MAJOR LECTURESHIPS AND SEMINARS

- UPMC Graduate Medical Education 2016 Leadership Conference
- "Cognition and the Developing Brain in Pediatric Epilepsy," New York University neurology grand rounds, New York, N.Y., April 2016
- "Epileptic Syndromes, Autism, and the Developing Brain," American Society of Electroencephalographic Technicians annual meeting, Pittsburgh, Pa., August 2016
- "Epilepsy in Children: Growing up Healthy," Clinic for Special Children Epilepsy Family Day, Strasburg, Pa., August 2017
- "Unknown Until Genome," 46th Annual Child Neurology Society Meeting, Kansas City, Mo., October 2017
- "Future of Neurodevelopmental Disabilities Training," Johns Hopkins Kennedy Krieger Retreat, Williamsburg, Va., November 2017
- "Co-Management of Pediatric Epilepsy: Primary Care and Specialty Care Partnership Model," poster presentation, American Public Health Association annual meeting, Atlanta, Ga., November 2017
- "Epilepsy and Medical Transition," Transition Conference, Department of Neurology, UPMC, Pittsburgh, Pa., December 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Neurology
- American Epilepsy Society
- Child Neurology Society
- Association of Pediatric Program Directors

HONORS

- Philip Troen, MD, Excellence in Medical Student Research Mentoring Award, 2015
- Best Doctors, *Pittsburgh Magazine*, 2016
- American Academy of Neurology Transforming Leaders Program awardee, 2017

Diego Chaves-Gnecco, MD, MPH, FAAP

ADVISORY COMMITTEE MEMBERSHIPS

- Salud Para Niños, Children's Hospital of Pittsburgh of UPMC's first bilingual (Spanish and English) bicultural pediatric clinic
- Reach Out and Read Advisory Board, Leyendo Juntos (Reading Together)
- Board of Directors, Jewish Family and Children's Service Legal Services for Immigrants and Internationals (LSII)

EDITORSHIPS

- *Pontificia Universidad Javeriana*, *Journal Universitas Medica*

MAJOR LECTURESHIPS AND SEMINARS

- "Autism: Diagnosis and Treatment. Managing the Challenges of Clinical Practice," 42nd Annual Refresher Course in Family Medicine, University of Pittsburgh Center for Continuing Education in the Health Sciences, Pittsburgh, Pa., March 2015
- "Unnatural Causes: Place Matters" and "In Sickness and in Wealth," discussant, Global Health Film Series, University of Pittsburgh Graduate School of Public Health, Pittsburgh, Pa., April 2015
- "Providing Health Care to an Invisible Community: Salud Para Niños," 2015 Health Career Scholars Academy, University of Pittsburgh, Pittsburgh, Pa., July 2015
- "Catalyze," Second Toast to Diversity, University of Pittsburgh School of Medicine, Pittsburgh, Pa., September 2015
- "Providing Health Care to an Invisible Community: Salud Para Niños, 14 Years Making a Difference in the Community," Health Sciences Diversity "Diversity in Practice" Speaker Series, University of Pittsburgh, Pittsburgh, Pa., September 2015
- "Contributions and Connections of Latino and Ibero-America to Medicine," University of Pittsburgh School of Medicine, Pittsburgh, Pa., October 2015
- "Keeping Our Children Healthy and Safe," Navigating Pittsburgh Summit, Pittsburgh, Pa., November 2015
- "Providing Health Care for an Invisible Community: Entering Our 15th Year of Services—Focus on Education," pediatric grand rounds, Children's Hospital of Pittsburgh of UPMC, February 2016
- "Providing Social Services for an Invisible Community—A Medical-Social Work Partnership," "Salud Para Niños Celebrating Our 15th Anniversary," and "Race and Child Welfare: National and Local Perspectives on Refugees, Immigrants, Human Trafficking, and Social Services," 2017
- Child Welfare Summer Institute, University of Pittsburgh School of Social Work, June 2017

- "Se Puede Cultivar la Curiosidad," guest panel, City of Asylum @ Alphabet City, Pittsburgh, Pa., November 2017
- "Bilingualism and Child Development," grand rounds, Children's Institute, December 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Society for Developmental and Behavioral Pediatrics
- National Hispanic Medical Association
- American Society of Clinical Pharmacology and Therapeutics
- C.F. Reynolds Medical History Society
- Sociedad Colombiana de Pediatria
- Sociedad Colombiana de Historia de la Medicina

HONORS

- *Best Doctors in America*, Woodward/White, Inc., 2013 to the present
- Best Doctors, *Pittsburgh Magazine*, 2013 to the present
- Hispanic Heritage Leadership Award, Hispanic Heritage Foundation and the National Football League, 2015
- Certificate of Appreciation–Founding Faculty Advisor, Latino Student Medical Association, University of Pittsburgh School of Medicine, 2016–2017
- Fuerza Award, Association of Latino Professionals for America, Pittsburgh chapter, sponsored by Café con Leche, 2016
- F. Edwards Rushton CATCH Award, American Academy of Pediatrics, 2017

Robyn Filipink, MD

RESEARCH

- Site principal investigator (PI), "Effects of AFQ056 on Language in Young Children with Fragile X Syndrome"
- PI, Fragile X Clinical and Research Consortium Registry
- PI, Fragile X Syndrome Registry

ADVISORY COMMITTEE MEMBERSHIPS

- UPMC Autism Taskforce, 2016 to the present
- Pediatric Neurology Education Committee, Children's Hospital of Pittsburgh of UPMC, 2006 to the present
- Clinical Competencies for Child Neurology and Neurodevelopmental Disabilities, 2014 to the present
- Affiliated faculty member, LEND, 2013 to the present
- Fragile X Clinical and Research Consortium, 2010 to the present
- Co-investigator, NeuroNEXT, University of Pittsburgh

MAJOR LECTURESHIPS AND SEMINARS

- "Tourette Syndrome: Case Dissection," UPMC grand rounds, June 2016
- "Tourette Syndrome Update 2015," Three Rivers Pediatric Update, May 2015
- Movement Disorder Case Conference, first Friday of each month, 60-minute live case presentation of a patient with a movement disorder

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Neurology
- American Academy of Pediatrics
- Child Neurology Society

Jenna M. Gaesser, MD

Jenna Gaesser is an assistant professor at the University of Pittsburgh, associate director of the Neonatal Neurology Program, codirector of the Neurofibromatosis Clinic, and codirector of Advanced Practice Provider Development and Utilization in the CDU.

RESEARCH

- Gaesser's research includes cardiac neurodevelopmental outcomes and the HEAL Clinical Trial.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Child Neurology Society
- American Academy of Neurology

SERVICE

- Education Committee, Child Neurology Residency, Children's Hospital of Pittsburgh

MAJOR LECTURESHIPS AND SEMINARS

- Practicum in fundoscopic examination for pediatric residents

Sara Hamel, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Society for Developmental and Behavioral Pediatrics

Kristin M. Hannibal, MD

Kristin M. Hannibal is clinical director of the CDU.

ADVISORY COMMITTEE MEMBERSHIPS

- Educating Practices in Community-Integrated Care,
- Medical Home Implementation Project, Pennsylvania Chapter, American Academy of Pediatrics
- Clinical Effectiveness Committee, General Academic Pediatrics, Children's Hospital of Pittsburgh of UPMC
- Obstetrics Bundling Project, Magee-Womens Hospital of UPMC
- Newborn Committee, Magee-Womens Hospital of UPMC

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Academic Pediatric Association
- Academy of Breastfeeding Medicine
- Society of Developmental Behavioral Pediatrics, with participation in Neonatal Intensive Care Unit Follow-Up Special Interest Group and Early Childhood Special Interest Group
- Fellow, American Academy of Pediatrics

HONORS

- Guest lectureship sponsor, Section on Breastfeeding, American Academy of Pediatrics
- Teaching award, Academic Pediatrics Association
- Level 3 certification, National Committee for Quality Assurance, 2016
- Outpatient Patient Satisfaction Award, Children's Hospital of Pittsburgh, 2017

Beth Heuer, DNP, RN, CRNP, CPNP-PC, PMHS

ADVISORY COMMITTEE MEMBERSHIPS

- Executive/Advisory Board, Neurofibromatosis Clinics Association

MAJOR LECTURESHIPS AND SEMINARS

- "Neurofibromatosis 1 and 2: What You Need to Know for Primary Care," Pennsylvania Coalition for Nurse Practitioners 13th Annual Conference, Lancaster, Pa., 2015
- "Case Report: Diffuse Ganglioneuromatosis in the Small Intestine in a 15-Year-Old Girl with Intestinal Dysmotility and Neurofibromatosis-1: Treatment Considerations," poster presentation, Children's Tumor Foundation, 2015
- Neurofibromatosis Conference, Monterey, Calif., 2015
- "Neurofibromatosis 1 and 2: A Primer for Pediatric Nurse Practitioners," National Association of Pediatric Nurse Practitioners (NAPNAP) 36th Annual Conference on Pediatric Health Care, Las Vegas, Nev., 2015
- "Tic Talk: Everything You Need to Know About Tourette Syndrome for Primary Care," NAPNAP 36th Annual Conference on Pediatric Health Care, Las Vegas, Nev., 2015
- "Tourette Syndrome," Three Rivers NAPNAP 20th Annual Winter Pharmacology Symposium, Pittsburgh, Pa., 2016
- "Clinical Overview of NF-1," Neurofibromatosis/Schwannomatosis Symposium, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., 2016
- "Sensational Pediatric Care: Helping the Child With Sensory Processing Difficulty," NAPNAP 38th National Conference on Pediatric Health Care, Denver, Colo., 2017
- "Primary Care of the Child With Neurological Disorders," Three Rivers NAPNAP 21st Annual Winter Symposium, Moon Township, Pa., 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Society for Developmental Behavioral Pediatrics
- NAPNAP
  - Professional Issues Committee, 2012 to the present
  - Chair, Professional Issues Committee, 2016 to the present
  - Developmental Behavioral Mental Health Special Interest Group, 2013 to the present
  - Secretary, Special Interest Group, 2014–2016
- Sigma Theta Tau, Eta Chapter
- Executive/Advisory Board, Neurofibromatosis Clinics Association, 2013 to the present
- American Association of Nurse Practitioners, 2011 to the present
- Pediatric Nursing Certification Board
  - Item writer, Primary Care Pediatric Nurse Practitioner Certification Exam, 2011–2014
  - Pediatric Primary Care Mental Health Specialist Steering Committee, 2015–2016
  - Pediatric Primary Care Mental Health Specialist Job Task Analysis Workforce Committee, 2016
- Pennsylvania Coalition of Nurse Practitioners, 2002 to the present
- Faculty, School of Nursing and Health Sciences, Robert Morris University
- Adjunct assistant professor of health and community systems, University of Pittsburgh School of Nursing

HONORS

- Inductee, Sigma Theta Tau International Honor Society of Nursing

Anuja Vora Jindal, MD, MPH

Anuja Vora Jindal is an assistant professor of pediatrics at the University of Pittsburgh and specializes in neurodevelopmental disabilities.

RESEARCH

- Supporting transition education for families of youth with ASD, Association of University Centers on Disabilities, poster presentation, December 2016, ongoing study
- Neuropsychological outcomes in pediatric patients with genetically confirmed episodic ataxia type 2, Child Neurology Society, poster presentation, October 2016, ongoing study
- Nonsyndromic craniosynostosis mimicking pseudotumor cerebri syndrome, 2015, ongoing study

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Child Neurology Society
- American Academy of Neurology
- American Academy of Pediatrics

HONORS

- Outstanding Junior Member Award, Child Neurology Society, October 2013

Robert B. Noll, PhD

RESEARCH

Robert B. Noll is leading international efforts within the Children's Oncology Group to better understand the impact of pediatric cancer and its treatments on the quality of life of children facing the disease. The goal of the project is to improve quality of life for survivors of pediatric cancers. A second project is a randomized clinical trial for primary caregivers of children recently diagnosed with cancer to teach coping skills (Bright IDEAS Problem-Solving Skills) that alleviate distress, comparing an electronic approach to face-to-face training. A third project is disseminating Bright IDEAS Problem-Solving Skills training to psychosocial providers providing care in pediatric oncology programs in the United States and Canada. Finally, he is a member of three mentorship committees of funded K awards for junior faculty.

STUDY SECTIONS

- Alex's Lemonade Stand
- Wetenschappelijk Fonds Willy Gepts, Belgium

ADVISORY COMMITTEE MEMBERSHIPS

- Professional Advisory Council, Mattie Miracle Foundation
- Steering Committee, Behavioral Science, Children's Oncology Group
- Steering Committee, Acute Lymphoblastic Leukemia, Children's Oncology Group
- Steering Committee, Supportive Care Committee, Children's Oncology Group
- Emeritus faculty, Cancer Control Committee, Children's Oncology Group

EDITORSHIPS

- Editorial Board, *Journal of Developmental and Behavioral Pediatrics*

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Fellow, American Psychological Society
- Children's Oncology Group
- Society for Research on Child Development
- Society for Developmental and Behavioral Pediatrics
- Society for Pediatric Psychologists

MAJOR LECTURESHIPS, SEMINARS, AND WORKSHOPS

- "Family Support Through Transitions and Family/Peer Influence on Adjustment to Childhood Cancer," National Cancer Policy Forum and the American Cancer Society, Washington, D.C., 2015
- "Neuropsychological Assessments Within the Children's Oncology Group: A Model for Success," International Neuropsychological Society, Denver, Colo., 2015
- "Psychosocial Research in Pediatric Oncology: What Have We Learned?" pediatric grand rounds, Nationwide Children's Hospital, Ohio State University, Columbus, Ohio, 2016
- "Bright IDEAS: Building Evidence in Pediatrics for Family-Centered Behavioral Health Services," pediatric grand rounds, Ruby Memorial Hospital, Morgantown, W.Va., 2017
- "Integration of Behavioral Health Into Pediatric Oncology: Can the Air Force and Navy Work Together?" pediatric grand rounds, University of Texas MD Anderson Cancer Center, Houston, Texas, 2017
- "Meet With the Expert: Stress on Parents/Siblings," concurrent roundtable at the 25th Annual Pediatric Oncology Group of Ontario Multi-Disciplinary Symposium on Childhood Cancer, Toronto, Ontario, 2017

HONORS

- Greater Pittsburgh Psychological Association Legacy Award, 2018

TEACHING ACTIVITIES

The CDU has a vigorous program that trains approximately 40 pediatric residents and a number of medical students per year. Trainees spend four weeks taking part in CDU clinical evaluations and visiting other settings where children with disabilities are evaluated or served in the community. Other hospital-affiliated evaluation sites include children's rehabilitation services clinics, speech and audiology, neonatal high-risk clinics, and child life services. Community-based sites include several schools that provide special education services and programs for children with developmental and behavioral disabilities. The training emphasis is entirely outpatient, focusing on both normal and abnormal development, family-centered practices, culturally sensitive practices, understanding of systems of care, and care coordination. This type of training experience is unique in the residency training program as a whole and vital to preparing pediatricians for practice in a changing health care environment.

THREE-YEAR BIBLIOGRAPHY

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Asato MR, Doss JL, Plioplys S. Clinic-friendly screening for cognitive and mental health problems in school-aged youth with epilepsy. *Epilepsy Behav*. 2015;48:97-102.

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Goldman AM, LaFrance WC, Benke T, **Asato M**, Drane D, Pack A, Syed T, Doss R, Lhatoo S, Fureman B, Dingledine R, American Epilepsy Society/NINDS Epilepsy Benchmark Stewards. 2014 Epilepsy Benchmarks Area IV: Limit or prevent adverse consequence of seizures and their treatment across the lifespan. *Epilepsy Curr*. 2016;16(3):198-205.

Niebler M, Documét P, **Chaves-Gnecco D**, Guadamuz T. Birth experiences of immigrant Latina women in a new growth community. *J Racial Ethnic Health Disparities*. 2016;3:466-77.

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Devine KA, Bukowski WM, Sahler OJ, Ohman-Strickland P, Smith TH, Lown EA, Patenaude AF, Korones DN, **Noll RB**. Social competence in childhood brain tumor survivors: Feasibility and preliminary outcomes of a peer-mediated intervention. *J Dev Behav Pediatr.* 2016 Jul-Aug;37(6):475-82.

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LIFESAVER

DIVISION OF GASTROENTEROLOGY, HEPATOLOGY, AND NUTRITION

Mission

The mission of the Division of Gastroenterology, Hepatology, and Nutrition is:

- To provide up-to-date and compassionate care to children with gastrointestinal (GI) and liver disorders
- To educate the next generation of gastroenterologists and pediatricians
- To advance knowledge of pediatric gastroenterology through research

FACULTY

David Keljo, MD, PhD
Interim Chief, Division of Gastroenterology, Hepatology, and Nutrition (April 1, 2017, to the present)
Professor of Pediatrics
Codirector, Inflammatory Bowel Disease (IBD) Program

Mark E. Lowe, MD, PhD
Chief, Division of Gastroenterology, Hepatology, and Nutrition (until March 31, 2017)
Professor of Pediatrics
Vice Chair of Graduate Medical Education

Feras Alissa, MD
Assistant Professor of Pediatrics
Director, Endoscopy Center

Riha Bhatt, MD
Assistant Professor of Pediatrics
Codirector, Pediatric Gastroenterology Fellowship Program

Kristy Boggs, PhD
Research Assistant Professor

Maria I. Clavell, MD
Associate Professor of Pediatrics

John F. Eisses, MD, PhD
Assistant Professor of Pediatrics

Tamara Feliciano Alvarado, MD
Assistant Professor of Pediatrics

Arjumand Ghazi, PhD
Assistant Professor of Pediatrics, Developmental Biology, and Cell Biology and Physiology

Sohail Z. Husain, MD
Associate Professor of Pediatrics
Director, Exocrine Pancreas Center (EPC)

Zahida Khan, MD, PhD
Assistant Professor of Pediatrics and Pathology

Sandra C. Kim, MD
Associate Professor of Pediatrics
Codirector, IBD Center

Dale King, MD
Assistant Professor of Pediatrics

Douglas Lindblad, MD
Assistant Professor of Pediatrics

Patrick J. McKiernan, MD
Professor of Pediatrics
Director, Pediatric Hepatology Program

Amitava Mukherjee, PhD
Research Instructor

Jeffrey A. Rudolph, MD
Associate Professor of Pediatrics

Wednesday Marie A. Sevilla, MD, MPH, CNSC
Assistant Professor of Pediatrics

Sapana Shah, MD
Assistant Professor of Pediatrics

Leah Siebold, MD
Assistant Professor of Pediatrics
Clinical Director of Gastroenterology

Amina Smajlovic, MD
Clinical Assistant Professor of Pediatrics

James E. Squires, MD, MS
Assistant Professor of Pediatrics
Director, Pediatric Transplant Hepatology Fellowship Program

Robert H. Squires Jr., MD
Professor of Pediatrics
Medical Director, Liver Transplant Program

Arvind Srinath, MD
Assistant Professor of Pediatrics
Codirector, Pediatric Gastroenterology Fellowship Program

Veena Venkat, MD
Associate Professor of Pediatrics

Xunjun Xiao, PhD
Research Assistant Professor (until March 31, 2017)

OVERVIEW OF DIVISION

Over the past year, the division has experienced changes and improvements.

PERSONNEL CHANGES

- Divisional leadership changed at the end of March 2017, when Mark Lowe moved to St. Louis, Mo., and David Keljo took over as interim chief.
- New physician arrivals included the following.
  - Wednesday Sevilla moved from Memphis, Tenn., and is participating in the care of children with intestinal failure.
  - Sandra Kim moved from Nationwide Children's Hospital in Columbus, Ohio, to become the codirector of the IBD Center.

PROGRAM DEVELOPMENT

- Under the leadership of Jeffrey Rudolph and in its seventh year, the Intestinal Care Service continued to increase its patient care activities.
- The EPC, led by Mark Lowe and Sohail Husain, increased its patient numbers and participated in the evaluation and care of children undergoing total pancreatectomy and islet cell auto-transplantation for chronic pancreatitis.
- Under the leadership of Patrick McKiernan and Robert Squires, the inpatient hepatology service now includes joint rounds with Transplant Surgery on patients with liver or small-bowel transplants.
- The IBD Program, codirected by David Keljo and Sandra Kim, cares for almost 1,000 children with Crohn's disease or ulcerative colitis. The center is joining the Improve Care Now initiative and developing a Transition to Adulthood Program in collaboration with the IBD Center at Presbyterian Hospital of UPMC.
- Under the leadership of Arvind Srinath and Riha Bhatt, the fellowship program remains strong and continues to attract applicants who are competitive for the major training programs. Two of the fellows were supported on National Institutes of Health (NIH) T32 training grants.

CLINICAL ACTIVITIES

The following table shows data for the clinical activities of the division. Inpatient visits increased by more than 40%, driven by the hepatology consult service. The outpatient patient volume and procedure volume have been relatively constant, with variations attributable to the number of clinicians. The division staff sees outpatients in nine locations. The total number of outpatient visits was second among all the subspecialty divisions (excluding Emergency Medicine) of the Department of Pediatrics. The division staffs three inpatient services: the general GI service, the intestinal failure service, and the newly launched hepatology consult service.

PATIENT ACTIVITY BY YEAR AND DATE

Activity	Fiscal Year (FY)12	FY13	FY14	FY15	FY16	FY17
Outpatients	17,046	18,535	15,478	16,830	17,622	16,023
Inpatients	5,508	6,699	5,865	6,572	6,899	9,313
Procedures	4,279	4,381	4,045	4,264	4,439	4,337

Because of outstanding clinical and research activities, the division was ranked #5 in pediatric gastroenterology and surgery in the recent *U.S. News & World Report* ratings.

RESEARCH OVERVIEW

Research remains an important facet of the division; four faculty members have NIH funding, and the division continues to attract new research funding from the NIH and other organizations. It participates in multiple national studies for liver disease, pancreatic disease, and IBD.

LABORATORY-BASED RESEARCH

The research laboratories made major advances in the most recent academic year, and they have an even brighter future ahead with an influx of talented researchers.

- Sohail Z. Husain, MD, founded and directs the EPC. Members of the EPC include John F. Eisses, MD, PhD; Amitava Mukherjee, PhD; Kristy Boggs, PhD; and postdoctoral students Li Wen, MD, PhD, and Nayyar Ahmed, PhD. The EPC laboratory manager is Tanveer Javed. This year, the team welcomed a visiting scholar from Chengdu, China: Lihui Deng, MD. The EPC hosted three summer students in 2017. Under Husain's mentorship, five members wrote grants. Last year, EPC members were awarded an R01 competitive renewal (to Husain), a startup grant from the Children's Hospital of Pittsburgh Foundation (to Boggs), and a mid-sized pharmaceutical grant (to Husain).

- Arjumand Ghazi, PhD, and her laboratory joined the division. The Ghazi Laboratory studies the genetics of aging, reproduction, and lipid metabolism using the model system *Caenorhabditis elegans*. The following projects are the focus of the laboratory's research: balancing lipogenesis and lipolysis in health and aging, the role of TCER-1/TCERG1 in immunity and reproductive health, and the role of germline-intrinsic meiotic genes in somatic aging. The Ghazi Laboratory includes postdoctoral students Francis RG Armit, PhD, and Hyeljin Hwang, PhD; graduate students Julia Loose and Nikki Naim; and undergraduate student Thayjas Patil.

CLINICAL AND TRANSLATIONAL RESEARCH

Twenty clinical and clinical/translational research activities are under way in hepatology, IBD, and pancreatic diseases. Eleven pharmaceutical trials are going on within the division.

CHALLENGES

Clinical: The clinical productivity of the division is at capacity and is not adequate to meet the health system mandate of same-day scheduling. The division is addressing this by hiring more advanced practice providers (APPs), so that physicians can focus most of their energies on the most medically advanced aspects of their subspecialty training. To facilitate recruitment and optimize performance and retention of APPs, Srinath and nurse practitioner Leslie Coda are developing a curriculum for a gastroenterology APP fellowship program.

Research: Research is the future of medicine. Research funding is increasingly difficult to obtain. Eisses and Khan have promising K award applications submitted. Transitioning fellows to K awards has not been successful for a few years. One MD/PhD fellowship candidate has been accepted to the fellowship program, and another has been recruited for the combined pediatric residency/GI physician-scientist training program. Two NIH-funded researchers have been recruited for next year. Two fellows are developing new clinical research programs in IBD. Efforts are being made to participate in a larger number of pharmaceutical studies, particularly in IBD.

RESEARCH AND OTHER SCHOLARLY ACTIVITIES

David Keljo, MD, PhD

David Keljo began serving as interim division chief on April 1, 2017.

RESEARCH

David Keljo's research focuses on clinical aspects of pediatric IBD and involves collaborative arrangements with multiple centers.

Sex Differences in Statural Growth Impairment in Pediatric Crohn's Disease. Growth impairment is common in pediatric Crohn's disease and appears to be more marked in males than in females. This study aims to identify the causes of that discrepancy.

Risk Stratification and Identification of Immunogenetic and Microbial Markers of Rapid Disease Progression in Children With Crohn's Disease. An inception cohort study is gathering clinical, genetic, serologic, tissue, and microbiologic samples to try to predict which patients will have rapid progression to stricture or perforation. Enrollment is complete, and 36-month follow-up has been completed. The primary outcome paper has been published. Follow-up continues.

Predicting Response to Standardized Pediatric Colitis Therapy: The PROTECT Study. This multicenter (25 centers), open-label study funded by the National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK) is designed to evaluate the safety and efficacy of standardized initial therapy using either mesalamine or corticosteroids then mesalamine for the treatment of children and adolescents newly diagnosed with ulcerative colitis. The study investigates whether response to the initial four weeks of therapy combined with clinical, genetic, and immune parameters determined during the initial course of therapy will predict severe disease as reflected by need for escalation of medical therapy or surgery. Enrollment and follow-up are complete for this study, and Pittsburgh is one of the top six enrolling centers. The primary outcome paper has been accepted for publication.

A Multidisciplinary Human Study on the Genetic, Environmental, and Microbial Interactions That Cause IBD: The GEM Study. This multicenter study sponsored by the Crohn's and Colitis Foundation of Canada aims to recruit unaffected siblings and offspring of patients with Crohn's disease. Siblings and offspring are 100-fold more likely to develop Crohn's disease than the rest of the population. The study will prospectively measure environmental exposures, changes in enteric microbial flora, and changes in immune responses in relation to the barrier function of the intestine and genetic makeup of these individuals to identify the changes that determine who develops Crohn's disease. Enrollment is ongoing.

David Keljo, MD, PhD
Interim Division Chief, Gastroenterology, Hepatology, and Nutrition

ADVISORY COMMITTEE MEMBERSHIPS

- Information Systems Physician Advisory Committee, Children's Hospital of Pittsburgh of UPMC

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Gastroenterological Association (AGA)
- North American Society for Pediatric Gastroenterology, Hepatology, and Nutrition (NASPGHAN)
- Crohn's and Colitis Foundation of America (CCFA)
- Alpha Omega Alpha

HONORS

- *Best Doctors in America*, Woodward/White, Inc.
- Best Doctors, *Pittsburgh Magazine*

Mark Lowe, MD, PhD

Mark Lowe vacated his position as division chief on March 30, 2017.

RESEARCH

Molecular Mechanisms of Dietary Fat Digestion by Pancreatic Lipases. The long-term goal of Lowe's research is to elucidate the molecular mechanisms of dietary fat digestion by pancreatic lipases. This project focuses on dietary fat digestion by lipases in human newborns. The results will advance knowledge of dietary fat digestion by pancreatic lipases and facilitate bench-to-bedside development of novel nutritional therapies, such as more effective formulas or enzyme replacement that will transform care and improve outcomes of premature infants and critically ill newborns.

Proteotoxicity in the Pathophysiology of Chronic Pancreatitis. This project focuses on a novel mechanism for pancreatitis. Herein, the researchers address the hypothesis that carboxyl ester lipase (CEL) mutants associated with chronic pancreatitis activate adaptive cell signaling pathways and increase susceptibility of cells to injury by metabolic stress. The knowledge gained by the proposed studies will improve the overall understanding of pancreatic injury and provide insight into potential pharmacological interventions directed at a new therapeutic target, protein homeostasis.

International Study Group of Pediatric Pancreatitis: In Search for a Cure 2 (INSPPIRE2) to Study Chronic Pancreatitis. The objective of this application is to determine the natural history of pediatric chronic pancreatitis, identify risk factors and genetic modifiers for its onset and sequelae, and develop approaches to improve clinical outcomes. The long-term goal is to develop diagnostic modalities, prognostic factors, and better treatment approaches for pediatric chronic pancreatitis.

STUDY SECTIONS

- Special Emphasis Panel for the Extramural Loan Repayment Program, NIDDK, NIH
- NIH Special Emphasis Panel for Silvio O. Conte Digestive Diseases Research Core Centers, NIDDK, NIH

ADVISORY COMMITTEE MEMBERSHIPS

- Board of Directors, Western Pennsylvania Chapter of the National Pancreas Foundation
- Board of Directors, National Pancreas Foundation
- Fellow and Resident Leadership Initiative, Department of Pediatrics, University of Pittsburgh
- Ambulatory Care Steering Committee, Children's Hospital of Pittsburgh of UPMC
- Executive Committee, Department of Pediatrics, University of Pittsburgh
- Perioperative Committee, Children's Hospital of Pittsburgh of UPMC
- Resident Performance and Evaluation Committee, Department of Pediatrics, University of Pittsburgh
- Research Task Force, Department of Pediatrics, University of Pittsburgh
- Graduate Medical Education Full Committee, University of Pittsburgh School of Medicine
- Planning Committee, PancreasFest
- Promotions Committee, Department of Pediatrics, University of Pittsburgh
- Visit Coordination Program, Children's Hospital of Pittsburgh of UPMC
- Finance Committee, NASPGHAN
- Strategic Planning Committee, Children's Hospital of Pittsburgh of UPMC
- Pediatric Intern Selection Committee, Department of Pediatrics, University of Pittsburgh
- Center for Rare Disease Therapy, Children's Hospital of Pittsburgh of UPMC

EDITORSHIPS

- Associate editor, *Journal of Pediatric Gastroenterology and Nutrition*

HONORS

- *Best Doctors in America*, Woodward/White, Inc.
- Best Doctors, *Pittsburgh Magazine*
- Carol Ann Kraumer Endowed Chair for Pediatric Research

MAJOR LECTURESHIPS AND SEMINARS

- "Assessing Acinar and Duct Cell Function: Gaps and Opportunities," Chronic Pancreatitis in the 21st Century, NIH/NIDDK Conference, Pittsburgh, Pa., 2016
- "Steatorrhea: What If It's Not Cystic Fibrosis?" World Congress of Pediatric Gastroenterology, Hepatology, and Nutrition, Montreal, Canada, 2016
- "Pancreatitis," learning lunch, World Congress of Pediatric Gastroenterology, Hepatology, and Nutrition, Montreal, Canada, 2016
- "Recurrent and Chronic Pancreatitis: Natural History, Prevention, and Treatment in Pediatrics," American Pancreatic Association annual meeting, Boston, Mass., 2016
- "Pancreatic Lipase-Related Protein 2: From the Lab to the Nursery," Infant Brain annual meeting, Elsinore, Denmark, 2016
- "Recent Advances in Pediatric Acute Pancreatitis," Lviv Children's Hospital Conference, Lviv, Ukraine, 2017
- "Nutrition and Pancreatitis: What To Do When?" fellows symposium, National Pancreas Foundation, Grapevine, Texas, 2017
- "Mechanisms of Lipase Mutation in Chronic Pancreatitis," European Pancreas Club, Budapest, Hungary, 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Pancreatic Association
- American Pediatric Society
- NASPGHAN
- American Association for the Advancement of Science
- AGA
- Society for Pediatric Research
- American Society for Biochemistry and Molecular Biology

Feras Alissa, MD

Feras Alissa's research includes examining the dose-effect relationship of methotrexate and hepatic fibrosis in patients with IBD.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- NASPGHAN

MAJOR LECTURESHIPS AND SEMINARS

- "Line Dancing: Learning How to Obtain Venous Access, Maintain It, and Minimize Complications. Is Loss of Access the Ultimate Failure? And How to Overcome It," Ninth International Pediatric Intestinal Failure and Rehabilitation Symposium, Los Angeles, Calif., 2016

Riha Bhatt, MD

Riha Bhatt is a sub-investigator in several studies within the division.

ADVISORY COMMITTEE MEMBERSHIPS

- Associate program director, Children's Hospital of Pittsburgh Pediatric Gastroenterology Fellowship Program
- Education Committee, NASPGHAN
- Curriculum Committee, University of Pittsburgh School of Medicine
- Codirector of Theme Subcommittee, University of Pittsburgh School of Medicine
- Pediatric Gastroenterology Fellow Clinical Competency Committee, Children's Hospital of Pittsburgh
- Pediatric Gastroenterology Fellow Selection Committee, Children's Hospital of Pittsburgh

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- NASPGHAN
- AGA
- AGA Academy of Educators
- Medical Education Research Certificate, Association of American Medical Colleges

Kristy Boggs, PhD

RESEARCH

Kristy Boggs' research focuses on defining the genomic landscape of pancreatic recovery and regeneration after injury. Through the utilization of next-generation sequencing protocols and molecular biology techniques, her aim is to characterize the transcriptome, genome, and epigenome of the pancreas following pancreatic injury. The exploitation of pancreatic recovery mechanisms may offer therapeutic targets for the treatment of pancreatitis.

Utilizing the murine caerulein hyperstimulation model of mild acute pancreatitis and recovery, she has sequenced the transcriptome of the non-injured and the histologically recovered pancreas by RNA-sequencing (RNA-seq). The RNA-seq data clearly demonstrate differentially expressed genes (DEGs) between the non-injured and the recovered pancreas, although histologically, the pancreas resembles that of the non-injured pancreas. This work has resulted in the preparation and submission to *Scientific Reports* of a manuscript titled "Pancreatic Gene Expression During Recovery After Pancreatitis Reveals Unique Transcriptome Profiles." The manuscript received favorable reviews and following revisions has been re-submitted to *Scientific Reports* (September 2017).

Whether the DEGs identified in the recovered pancreas allow the pancreas to thwart repeated events of stress or injury is currently under investigation. Boggs is assessing whether subsequent attacks of acute pancreatitis in mice are less severe than the initial attack, utilizing the caerulein hyperstimulation model of mild acute pancreatitis and recovery. The severity of recurrent acute pancreatitis is being determined both biochemically and histologically.

In addition to the identification of DEGs in the recovered pancreas, there is the possibility of poised chromatin states in the recovered pancreas. In this case, the gene is not expressed in the recovered pancreas, as determined by RNA-seq, but the chromatin structure is in a transcriptionally permissive state that is primed for rapid gene expression. An altered chromatin state in the recovered pancreas may confer a molecular memory that regulates severity of future organ injury. In future studies, Boggs will assess chromatin accessibility in the recovered pancreas by ATAC-seq (assay for transposase-accessible chromatin sequencing), with a specific focus on identifying poised regions in the chromatin of a recovered pancreas.

Also, in future work, she will determine the epigenetic mechanism(s), focusing on histone modifications, that induce a differential chromatin signature in the recovered

pancreas. Differentially modified histones between the non-injured and recovered pancreas will be assessed by ChIP-seq (chromatin immunoprecipitation and DNA sequencing) to identify specific post-transcriptional modifications of histone tails and the region of DNA associated with these transcriptionally active or repressive modifications. The identification of epigenetic modifications in the recovered pancreas will provided insight into the identified altered gene expression and chromatin structure in the recovered pancreas.

Boggs is affiliated with the Health Sciences Sequencing Core at Children's Hospital of Pittsburgh. She contributes to investigations of the Husain Laboratory and the larger scientific community by developing and optimizing next-generation sequencing technologies.

HONORS AND AWARDS

- Children's Hospital of Pittsburgh of UPMC Research Advisory Committee Grant, "Pancreatic Injury Induces a Unique Chromatin Footprint That Protects the Recovering Pancreas Against Repetitive Injury," 100% effort, $80,000 direct funds over two years, 2017–2019

Maria I. Clavell, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Society for Parenteral and Enteral Nutrition
- NASPGHAN

John F. Eisses, MD, PhD

RESEARCH

John Eisses' research focuses on understanding the role of epigenetic modifiers in regulating injury and recovery of the pancreas as it relates to pancreatic disease. He studies epigenetic regulation of pancreatitis in distinct cell types within the pancreas. Pancreatitis is a life-threatening inflammatory disorder that lacks targeted therapies. Injury and inflammation activate pancreatic stellate cells (PSCs), resulting in the remodeling of the pancreatic microenvironment, which allows pancreatic recovery to occur. Prolonged injury or aberrant regulation of PSCs has been implicated in the development or promotion of several pancreatic diseases, including chronic pancreatitis and pancreatic cancer. The overall goal of the current proposal is to examine novel epigenetic mechanisms by which pancreatic recovery is regulated in response to injury, particularly in the context of PSCs. The hypothesis is that the epigenetic regulation of gene expression in PSCs is crucial for activation and repression of gene expression necessary to promote a microenvironment suitable for pancreatic regeneration. The research is significant because it elucidates for the first time an epigenetic mechanism for regenerative signals important in regulating the pancreatic microenvironment to allow pancreatic recovery. On a much broader level, it opens a new paradigm of possible therapeutic strategies that may enhance the ability of the pancreas to recover, particularly in the context of remodeling the extracellular matrix and the pancreatic microenvironment by PSCs.

ADVISORY COMMITTEE MEMBERSHIPS

- Member, Pancreas Committee, NASPGHAN
- Member, Pediatric Pancreatology Consortium, INSPPIRE

EDITORSHIPS

- Associate editor, *GI Scholarly Newsletter*, Children's Hospital of Pittsburgh

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- NASPGHAN
- European Pancreas Club
- AGA
- American Pancreatic Association
- Pediatric Pancreatology Consortium, INSPPIRE
- Collaborative Alliance for Pancreatic Education and Research

Tamara Feliciano Alvarado, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- NASPGHAN

Arjumand Ghazi, PhD

RESEARCH

The Ghazi Laboratory studies the genetics of aging, reproduction, and lipid metabolism using the model system *Caenorhabditis elegans*. The following projects were undertaken in the past year and are currently the focus of the laboratory's research.

Balancing Lipogenesis and Lipolysis in Health and Aging. Lipid imbalances are characteristic of obesity and a feature of many age-related ailments and reproductive pathologies in humans. Yet the relationship among lipid metabolism, aging, and reproduction remains poorly studied. This project investigates how the balance between lipid synthesis and breakdown influences an organism's rate of aging and health. In *C. elegans*, eliminating the germline extends lifespan, removes fertility, and is a major challenge to lipid metabolism because the animal needs to stop fat deposition into eggs and reorganize its lipid profile. Thus, germline-less worms provide a unique platform to understand how lipid balance is established in the cells and tissues of complex multicellular animals in the

face of major physiological changes. We discovered that fat production and degradation increase simultaneously in response to germline loss. In germline-less adults, a conserved transcription factor, NHR-49, upregulates fatty-acid β-oxidation and desaturation—processes that contribute to lipid breakdown and build-up, respectively. These two, and many other processes involved in lipid synthesis and degradation, are elevated in response to germline removal by the conserved transcription factors DAF-16 and TCER-1. These data suggest that the coordinated enhancement of lipid synthesis and breakdown facilitate adaptation to germline loss by mediating lipid homeostasis. The knowledge obtained from these studies is likely to reveal fundamental insights into obesity and the relationships among lipid metabolism, reproduction, and aging.

Role of TCER-1/TCERG1 in Immunity and Reproductive Health. Reproduction and stress resistance in metazoans are poorly described. We discovered a role for TCER-1, the worm homolog of human transcription elongation, and splicing factor TCERG1 in enhancing reproductive capacity in the face of pathogen attack. In *C. elegans*, eliminating germline stem cells increases lifespan and elevates stress resistance. Previously, we demonstrated that TCER-1/TCERG1 specifically promotes the longevity of germline-less *C. elegans* but is critical for reproductive health in normal, fertile animals. Surprisingly, we have discovered that TCER-1/TCERG1 inhibited stress tolerance in adult worms. *tcer-1* mutants exhibited elevated resistance against multiple biotic and abiotic stressors. TCER-1/TCERG1 impairs stress resistance by inhibiting PMK-1, a conserved innate immunity-promoting kinase. PMK-1-target genes are upregulated in *tcer-1* mutants, and the immunoresistance of *tcer-1* mutants is dependent upon PMK-1. The data suggest that TCER-1/TCERG1 promotes reproductive fitness and represses stress resilience under normal conditions. Under stressful conditions, TCER-1/TCERG1 is repressed, resulting in enhanced stress resilience and reduced reproductive capacity. Unlike most pro-longevity genes, TCER-1/TCERG1 appears to have distinct regulatory effects on lifespan, stress resistance, and fertility, suggesting that the protein may function as a molecular rheostat for coordinating major life-history traits. The phenotypic uncoupling observed in *tcer-1* mutants provides a unique platform to dissect the pathway that governs resource allocation among procreation, stress response, and somatic maintenance in metazoans.

Role of Germline-Intrinsic Meiotic Genes in Somatic Aging. Meiotic chromosomal defects increase dramatically with age and are a major cause of miscarriages, birth defects, and age-related fertility loss in women. Correlative evidence from human studies has indicated that germline fidelity has a role in overall health. This research used the nematode *C. elegans* to address the mechanisms of and cause-effect relationships between germline health and somatic aging. Specifically, the researchers have focused on genes involved in *C. elegans* meiosis, an intrinsically germline-restricted phenomenon that is fundamental to genetic diversity. They have found that mutations in multiple genes that act at different stages of worm meiosis cause lifespan reduction and demonstrate signs of premature aging. Besides lifespan, the researchers have examined the effects of these mutations directly on the rate of organismal aging by testing various measures of healthspan. Preliminary results suggest that meiotic mutants not only exhibit reduced lifespans but also show physiological and molecular signs of accelerated aging. The data suggest that genes that govern meiotic fidelity in germ cells of *C. elegans* impact the rate of aging of the whole animal.

ADVISORY COMMITTEE MEMBERSHIPS

- Chair, Admissions Committee, Interdisciplinary Biomedical Graduate Program, 2016–2017
- Member, Admissions Committee, Interdisciplinary Biomedical Graduate Program, 2017–2018

EDITORSHIPS

- *Scientific Reports*
- *Frontiers in Endocrinology and Aging*

HONORS

- Invited to participate in the National Institute on Aging, NIH, Division of Aging Biology New Investigators Forum, July 2017
- Publication recommended by Faculty of 1000 (Amrit et al., *PLoS Genetics*, 2014)

MAJOR LECTURESHIPS AND SEMINARS

- "Finding Human Longevity Genes in a Spineless Worm," Honors College Health Sciences Undergraduate Summer Fellowship Program, Pittsburgh, Pa., July 2016
- "Aging in Worms … Clinical Relevance?" Breakfast with Mentors, Summer Research Internship Program, Children's Hospital of Pittsburgh, Pittsburgh, Pa., July 2016
- "Concomitant Modulation of Somatic Lipid Production and Breakdown in Response to Germline Signals," Molecular Genetics of Aging meeting, Cold Spring Harbor Laboratory, Cold Spring Harbor, N.Y., September 2016
- "Genes That Link Fat, Fertility, and Aging," Long-Life Family Study Group, Department of Epidemiology, University of Pittsburgh, Pittsburgh, Pa., November 2016

- "Lipid Metabolism and Aging," Division of Endocrinology, Department of Pediatrics, University of Pittsburgh, Pittsburgh, Pa., April 2017
- "Concomitant Modulation of Somatic Lipid Production and Breakdown in Response to Germline Signals," second Interventions in Aging meeting, Cancun, Mexico, March 2017
- "Fat, Fertility, and Aging in *C. elegans*," featured speaker for conference held once every four years, 18th International Society for Developmental Biology meeting, National University, Singapore, June 2017

Sohail Z. Husain, MD

RESEARCH

The overarching goal of Sohail Husain's research is to determine the mechanisms that initiate and propagate pancreatitis, a painful inflammatory disease that accounts for more than 300,000 hospitalizations and 3,200 deaths in the United States annually. Specifically, current advances in the Husain Laboratory are focused on: (1) examining the aberrant calcium signals and calcium targets within the main pancreatic cell, the acinar cell, which are critical to both the initiation and propagation of pancreatitis; (2) determining the role of epigenetic factors in pancreatic recovery and regeneration after injury; and (3) deciphering the mechanisms underlying drug-induced pancreatitis. Husain's work is anticipated to lead to effective treatment or preventive strategies for pancreatitis that target aberrant calcium signaling, enhance pancreatic recovery, or compensate for toxic drug exposures.

STUDY SECTIONS

- Standing member, Clinical, Integrative, and Molecular Gastroenterology Study Section, NIH
- Section member, Peer-Reviewed Medical Research Program, U.S. Department of Defense
- Standing member, Research Advisory Committee, Children's Hospital of Pittsburgh of UPMC

ADVISORY COMMITTEE MEMBERSHIPS

- Chair, Pancreatitis: Inflammation, Fibrogenesis, and Immunology Review Section, AGA
- Chair, AGA Abstract Review for Pancreatitis: Inflammation, Fibrogenesis, and Immunology
- Chair, Pancreas Committee, NASPGHAN
- Councilor, Pancreatic Disorders Section, AGA Institute Council
- Organizing member, National Pancreas Foundation, Western Pennsylvania Chapter
- Site principal investigator, INSPIRRE2: Pediatric Acute and Chronic Pancreatitis Multi-Center, NIH-funded, R01 Consortium

EDITORSHIPS

- Editorial Board, *Pancreapedia*
- Editorial Board, *Journal of Biological Chemistry*
- Editor-in-chief, *GI Scholarly Newsletter*, Children's Hospital of Pittsburgh

MAJOR LECTURESHIPS AND SEMINARS

National/International:

- "Targeting the Calcium Effector Calcineurin in Pancreatitis," invited lecture, APA annual meeting, San Diego, Calif., 2017
- "Why Do Some Drugs Cause Pancreatitis?" invited lecture, Frontiers in Pediatric Pancreatology, NASPGHAN Single Topic Symposium, Las Vegas, Nev., 2017
- "Turning Investigational Therapies into Clinical Reality: Pancreatitis in the Spotlight," visiting professor, pediatrics seminar (host: Teresa Quattrin), University at Buffalo, Buffalo, N.Y., 2017
- "Epithelial Cell Calcineurin Signaling in Pancreatitis," visiting professor, Mayo Clinic Cancer Seminar Series (host: Baoan Ji), Mayo Clinic, Jacksonville, Fla., 2017
- "Pancreatitis," "Pancreatic Anatomy and Anomalies," and "Hereditary Disorders of the Pancreas," invited lectures, NASPGHAN Essentials Pediatric GI Review (course director: Chris Liacouras), Scottsdale, Ariz., 2017

Regional/Local:

- "Advances in Preventing Radiocontrast-Induced ERCP Pancreatitis by Targeting Calcium and Calcineurin," Molecular Medicine Research Seminar (host: Jay Kolls), Pittsburgh, Pa., 2016
- "Preventing Post-ERCP Pancreatitis by Targeting Calcineurin," faculty lecture, internal medicine gastroenterology grand rounds, Pittsburgh, Pa., 2016
- "Getting Published," faculty lecture, Unified Fellows Conference, Pittsburgh, Pa., 2016

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- NASPGHAN
- American Pancreatic Association
- AGA
- INSPPIRE
- National Pancreas Foundation

HONORS

- *Best Doctors in America*, Woodward/White, Inc.
- Best Doctors, *Pittsburgh Magazine*

Zahida Khan, MD, PhD

RESEARCH

Zahida Khan took a sabbatical absence the academic year of 2016–2017 to complete fellowship training in advanced pediatric transplant hepatology at Texas Children's Hospital through Baylor University. Khan completed her advanced pediatric transplant hepatology fellowship in August of 2017. She then returned to Children's Hospital of Pittsburgh of UPMC and the University of Pittsburgh School of Medicine, where she continues to provide her services clinically and research liver diseases, focusing on cellular approaches to liver regeneration and transplantation.

More than 15,000 patients are awaiting liver transplantation, but only 6,729 liver transplants were performed last year. Alpha-1 antitrypsin deficiency (ATD) is the most common hereditary liver disease in children, and it is the most frequent genetic reason for liver transplantation. In classical ATD, the PiZ mutation results in misfolded ATZ protein monomers, which cause proteotoxicity, often leading to chronic liver injury, cirrhosis, and cancer. Not all patients develop severe liver disease, and it is unclear why only some patients need a liver transplant. Khan's research investigates the basic cell biology behind this question, using the PiZ transgenic mouse model. To understand how liver regeneration is impaired in ATD, she is studying intracellular mechanisms of protein processing and cell proliferation in PiZ hepatocytes. She is also studying how bile acid signaling can be utilized as a therapeutic target for ATD-related liver disease. Her research investigates largely unexplored pharmacologic pathways in ATD.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Hepatology Committee, NASPGHAN Research Awards Committee, American Association for the Study of Liver Diseases (AASLD)

HONORS

- Research Presentation Award (Top Abstract of 144), Baylor College of Medicine, Texas Children's Hospital Pediatric Research Symposium, March 2017

MAJOR LECTURESHIPS

- "Bile Duct Ligation Induces ATZ Globule Clearance in a Mouse Model of Alpha-1 Antitrypsin Deficiency," NIH Child Health Research Center annual retreat, Bethesda, Md., September 2016
- "Liver Cell Regeneration in Alpha-1 Antitrypsin Deficiency," Alpha-1 Foundation investigators' meeting, Miami, Fla., October 2016

Sandra C. Kim, MD

RESEARCH

Sandra Kim focuses on clinical care of children with IBD. She has research expertise in basic, translational, and clinical/quality-improvement initiatives in pediatric and adolescent IBD. She is involved in clinical research and quality-improvement initiatives on regional and national levels for children and adolescents with IBD and has authored numerous studies on pediatric and adolescent IBD. She is a member of the Children's Hospital of Pittsburgh's Fellowship Clinical Competency Committee for the Pediatric Gastroenterology Program.

ADVISORY COMMITTEE MEMBERSHIPS

- Crohn's and Colitis Foundation
  - Co-chair, Government/Industry Affairs and Advocacy, National Scientific Advisory Committee
  - Member and past chair, Pediatric Affairs Committee, National Scientific Advisory Committee
- ImproveCareNow (ICN) Pediatric IBD Quality-Improvement Collaborative
  - Chair, Clinical Practice Committee
  - Co-chair, Transition of Care Innovation Community
  - Member, Physician Leadership Group
  - Member, Strategy Council
  - Member, Collaborative Steering Committee, ICN/Crohn's and Colitis Foundation

- NASPGHAN
  - Member, IBD Committee
  - Faculty, NASPGHAN First Year Fellows' Conference
  - NASPGHAN Fellows' Mentoring Program
  - Member, Home Infusion Task Force

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- AGA
- Crohn's and Colitis Foundation
- NASPGHAN
- Alpha Omega Alpha
- Gold Humanism Society

HONORS

- University of Pittsburgh School of Medicine Advanced Faculty Leadership Academy, 2017
- *Best Doctors in America*, Woodward/White, Inc., 2015–2017

MAJOR LECTURESHIPS AND SEMINARS

- "Biosimilars in IBD: What Does the Pediatric Gastroenterologist Need to Know?" invited speaker, NASPGHAN annual meeting, November 2017
- "IBD 2020 at CHP: What Does the Future Hold?" Department of Pediatrics grand rounds, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., October 2017
- "Rheumatologic Issues in Pediatric IBD," and "How Do We Discuss Surgery With Our Pediatric Patients With IBD?" invited speaker, CCFA Advances in IBD, Orlando, Fla., December 2016
- "Diet and IBD: Food for Thought," invited speaker, World Congress for Pediatric Gastroenterology, Hepatology, and Nutrition, postgraduate course, Montreal, Canada, October 2016

Dale E. King, MD

RESEARCH

Dale King participates in several clinical research projects in the department, including studies related to IBD, intestinal failure, parenteral lipid supplements (Omegaven and SMOFlipid), and bowel-preparation regimens.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- NASPGHAN
- AGA
- CCFA

Douglas Lindblad, MD

RESEARCH

Douglas Lindblad is involved with a research proposal (Cystic Fibrosis Foundation) to study the relationship between cystic fibrosis–associated liver disease and cystic fibrosis–related diabetes.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics

Patrick J. McKiernan

RESEARCH

Patrick McKiernan's research interests cover the spectrum of pediatric liver disease but have fallen into three main areas.

- Liver transplantation: His published contributions span clinical outcomes, pharmacokinetics of immunosuppressants, long-term histological outcomes, and guidelines for transition to adult care.
- Portal hypertension: He has published on the use of endoscopic techniques, including variceal band ligation and endoscopic ultrasound, and he has contributed to the development of international guidelines.
- Metabolic liver disease: He has published on the clinical management of metabolic disease, especially tyrosinemia type I, and the evolving role of liver transplantation in metabolic liver disease. He has been involved in clinical trials of stem cell treatment for metabolic disease.

In addition, McKiernan is the local principal investigator for the following first-in-humans multicenter trial: a phase I/II, randomized, open-label, ascending-dose, delayed-treatment concurrent control clinical study to evaluate the safety and preliminary efficacy of AT342, an AAV8-delivered gene transfer therapy in Crigler-Najjar syndrome subjects aged 1 year and older.

Furthermore, he is the local principal investigator for a five-year, multicenter, longitudinal observational study of patients with nonalcoholic fatty liver or nonalcoholic steatohepatitis.

ADVISORY COMMITTEE MEMBERSHIPS

- Center for Rare Disease Therapy, Children's Hospital of Pittsburgh of UPMC

MAJOR LECTURESHIPS AND SEMINARS

- "Liver Transplantation for Metabolic Disease," Society for the Study of Inborn Errors of Metabolism annual scientific meeting, Rome, Italy, September 2016

- "Mitochondrial Hepatopathies in Children: State-of-the-Art of Diagnosis and Treatment," American Association for Study of Liver Disease annual meeting, Boston, Mass., November 2016
- "Improving Outcomes for Metabolic Disease," International Pediatric Medical Congress, Dubai, United Arab Emirates, November 2016
- "Update in Pediatric Liver Disease," Community Liver Alliance, Pittsburgh, Pa., December 2016
- "Current Approach to Neonatal Cholestasis," European Society for Paediatric Gastroenterology, Hepatology, and Nutrition (ESPGHAN) postgraduate course, Prague, Czech Republic, May 2017
- "Liver Transplantation in Mitochondrial Disorders," ESPGHAN annual scientific meeting, Prague, Czech Republic, May 2017
- "Monitoring the Liver Over the Long Term: Structural Assessment," International Pediatric Transplant Association scientific meeting, Barcelona, Spain, May 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- ESPGHAN
- American Association for the Study of Liver Disease
- British Society of Paediatric Gastroenterology, Hepatology, and Nutrition
- British Association for the Study of the Liver
- Society for the Study of Inborn Errors of Metabolism
- British Inherited Metabolic Diseases Group
- Studies of Pediatric Liver Transplantation (SPLIT)

Amitava Mukherjee, PhD

RESEARCH

Amitava Mukherjee's overarching goal is to uncover key molecular signaling pathways for exocrine pancreatic disorders, particularly drug-induced pancreatitis. Currently, his research focuses on deciphering the mechanisms underlying asparaginase-associated pancreatitis. The research suggests that pancreatic asparagine synthetase (ASNS) maintains acinar cell homeostasis and that its upregulation is required to mitigate asparaginase-induced pancreatic cell injury. Therefore, therapies that selectively augment pancreatic ASNS could be used to alleviate asparaginase-associated pancreatitis. He has generated sufficient preliminary data for grant submissions. Mukherjee has published work in collaboration with the Mark Lowe Laboratory on a mutant lipase that is responsible for chronic pancreatitis in the *Journal of Biological Chemistry*. He has written a chapter for *Liver Disease in α1-Antitrypsin Deficiency*.

Mukherjee has been assisted by his research team, consisting of a postdoctoral fellow, Nayyar Ahmed, PhD; a medical fellow, Fateema Rose, MD; and a visiting researcher, Li-Hui Deng. Mukherjee has participated in training two summer students, Chaitanya Srinivasan and Mahedah Rehman.

HONORS

- PancreasFest Travel Scholarship Award: Collaborative Alliance for Pancreatic Education and Research, University of Pittsburgh, June 2017

Jeffrey Rudolph, MD

RESEARCH

Intestinal Care and Rehabilitation Center Database. This retrospective database is designed to collect data that describe the natural history and management practices of intestinal failure and rehabilitation at Children's Hospital.

Compassionate Use of Omegaven in the Treatment of Parenteral Nutrition–Associated Liver Disease. This is a compassionate-use protocol for the use of omega-3 fatty acids as an alternative lipid source in children with total parenteral nutrition–associated liver disease.

A Prospective, Randomized, Controlled, Double-Blind, Parallel-Group, Phase III Study to Compare the Safety and Efficacy of Smoflipid 20% to Intralipid 20% in Hospitalized Neonates and Infants Requiring 28 Days of Parenteral Nutrition. Jeffrey Rudolph is site investigator for a multicenter study comparing the effects of standard parenteral lipid therapy to a novel lipid emulsion.

Biomarkers in Transplant Recipients. This entails obtaining biological samples from intestinal transplant patients during routine endoscopy for assessment of markers that predict the immunological state or organ rejection.

ADVISORY COMMITTEE MEMBERSHIPS

- Codirector, Pediatric Intestinal Failure, Rehabilitation, and Transplant Symposium, Pittsburgh, Pa., 2018
- Nutrition Committee, NASPHAN

MAJOR LECTURESHIPS AND SEMINARS

- "Trace Elements in TPN," invited speaker, Sydra Medical Corporation, Doha, Qatar, April 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- NASPGHAN
- American Society for Parenteral and Enteral Nutrition

HONORS

- Best Doctors, *Pittsburgh Magazine*

Wednesday Marie A. Sevilla, MD, MPH, CNSC

RESEARCH

American Society for Parenteral and Enteral Nutrition New Opportunities for Verification of Enteral Tube Location Project. This project addresses the safety of feeding tube placement. Wednesday Marie A. Sevilla collaborates with the task force on a variety of projects, including development of a position paper on best practices for nasogastric tube placement.

Soluble Fiber Utilization in Pediatric Short Bowel Syndrome: A Survey of Current Practice Trends. This project is a collaboration with a team at Le Bonheur Children's Hospital. The data from the survey will provide an overview of the clinical practice of administering supplemental enteral fiber in pediatric patients with short bowel syndrome. Data have been collected and analyzed. A manuscript is being prepared for submission for publication. Sevilla participated in developing the survey tool, monitoring data collection and analysis, and authoring the manuscript.

ADVISORY COMMITTEE MEMBERSHIPS

- Member, Nutrition Advisory Group, Children's Hospital of Pittsburgh

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Nutrition Committee, NASPGHAN
- AGA
- Pediatrics Section and Pediatric Intestinal Failure Section, American Society for Parenteral and Enteral Nutrition
- Intestinal Transplant and Rehabilitation Association

Sapana Shah, MD

RESEARCH

Predicting Response to Standardized Pediatric Colitis Therapy: The PROTECT Study. This multicenter (25 centers), NIDDK-funded, open-label study is designed to evaluate the safety and efficacy of standardized initial therapy using either mesalamine or corticosteroids then mesalamine for the treatment of children and adolescents newly diagnosed with ulcerative colitis. The study will investigate the hypothesis that response to the initial four weeks of therapy as well as specific clinical, genetic, and immune parameters determined during the initial course of therapy will predict severe disease as reflected by need for escalation of medical therapy or surgery. Enrollment and follow-up are complete, and the Pittsburgh location is one of the top six enrolling centers. The study is in the data-analysis phase.

An Efficacy and Safety Study of Infliximab in Pediatric Participants With IBD. This multicenter study sponsored by Janssen Scientific Affairs, LLC, is designed to evaluate whether trough serum infliximab concentrations at the time of loss of clinical response will identify pediatric participants with IBD who would benefit from dose escalation above the currently approved dose of 5 mg/kg.

Improve Care Now. Improve Care Now is a multicenter, quality-improvement collaborative for patients with pediatric IBD that enables patients, families, clinicians, and researchers to improve knowledge and outcomes related to Crohn's disease and ulcerative colitis. It engages all stakeholders in a learning health network that provides real-time quality improvement, research, and community-building for children with these conditions.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- NASPGHAN

Leah Siebold, MD

RESEARCH

Leah Siebold is principal investigator for the following.

- PRO-KIIDS and NEOPICS Retrospective Cohort Study of Very Early Onset IBD
- The international Early Onset Paediatric IBD Cohort Study
- A Study of Fecal Microbiota Transplantation in Pediatric Patients With Relapsed IBD

ADVISORY COMMITTEE MEMBERSHIPS

- Clinical Care and Quality Committee, NASPGHAN Physical Leadership Team, Children's Hospital of Pittsburgh
- Physician Compensation Committee, Children's Hospital of Pittsburgh
- 8B GI liaison, Children's Hospital of Pittsburgh
- Improve Care Now
- Taskforce for Home Infusions, NASPGHAN

Amina Smajlovic, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics

James E. Squires, MD, MS

RESEARCH AND OTHER SCHOLARLY ACTIVITIES

Potential of Human Induced Pluripotent Stem Cells (iPSCs) in Studies of Progressive Intrahepatic Cholestasis. The hepatocyte canalicular membrane serves as the gateway through which hepatocyte metabolic products pass to form bile.

Disruption of canalicular function, therefore, is expected to have detrimental effects on liver and human health. Autosomal recessive disorders are known to disrupt hepatocyte canalicular function and bile secretion. Progressive familial intrahepatic cholestasis (PFIC) is a group of rare diseases clinically manifested by intrahepatic cholestasis in children and progresses to end-stage liver disease and death or liver transplantation.

The ability to derive patient-specific iPSCs allows opportunities to model hepatic disorders, gain mechanistic insights, and explore cell-based therapies. Briefly, human fibroblasts or lymphocytes are isolated and reprogrammed into hepatocyte-like cells, which bear significant similarities to primary hepatocytes in terms of transcriptional profile and functional properties but retain the unique, patient-specific genetic defects. These patient-derived iPSC cell lines enable the study of individual phenotypes under highly controlled conditions and allow linking the observed defects directly to disease-causing genetic alterations. Liver-specific disease models include ATD, familial transthyretin amyloidosis, glycogen storage disease, Wilson's disease, familial hypercholesterolemia, and Niemann-Pick disease. The present study hypothesizes that patient-specific human iPSCs can be generated and accurately recapitulate the main characteristics of PFIC. Squires is principal investigator.

Graft Injury Group Observing Late-Term Outcomes. This is a multicenter, international collaboration of pathologists, hepatologists, and transplant surgeons looking to better understand and characterize late graft injury in children who receive liver transplants. Squires is site investigator.

Biomarkers of Acute Kidney Injury in Pediatric Liver Transplant Recipients. Acute kidney injury (AKI) is a rapid loss of kidney function, and AKI following liver transplant adversely affects outcomes. Improved detection and management of AKI in the perioperative period would have long-term benefits in reducing the development of chronic kidney disease. Serum creatinine, the current gold-standard marker for kidney injury, is a late and insensitive marker of AKI. Newer biomarkers such as urine IGFBP7, TIMP-2, and NGAL have proven to be superior in the timely detection of renal injury and impairment in critically ill adults, and the ability to sample urine is advantageous. This research will study novel biomarkers of AKI in the pediatric liver transplant population to determine their diagnostic and predictive capabilities relating to the development of AKI in pediatric populations. Squires is principal investigator.

Childhood Liver Disease Research Network. Cholestatic liver diseases are a major cause of morbidity and mortality in children. The most common of these diseases, biliary atresia, is the leading indication for liver transplantation in children. The cause(s), optimal diagnosis, and management of biliary atresia remain unknown. Thus, the NIH has funded a cooperative consortium to conduct a comprehensive study of biliary atresia. The Children's Hospital of Pittsburgh of UPMC is one of 10 clinical centers participating in this collaborative investigation. Squires is co-investigator.

SPLIT. This project is a community of pediatric hepatologists, transplant surgeons, research coordinators, nurse coordinators, and other health professionals across the United States and Canada working together to advance knowledge in pediatric liver transplantation. SPLIT was started in 1995 and has evolved from a research registry into a multifaceted organization focused on improving outcomes for children receiving liver transplantation. Squires is co-investigator.

Clinical Trials in Organ Transplantation in Children (CTOTC): Biomarkers for Post-Transplant Lymphoproliferative Disorders (PTLDs) in Children. This is a clinical trial investigating biomarkers associated with the development of Epstein-Barr virus (EBV)-related PTLDs in pediatric liver, heart, heart with liver, small intestine, and liver with small intestine transplant candidates and recipients. Squires is co-investigator.

ADVISORY COMMITTEE MEMBERSHIPS

- Idiopathic Neonatal Cholestasis Subcommittee, Childhood Liver Disease Research Network (ChiLDReN) Alpha-1-Antitrypsin Deficiency Subcommittee, ChiLDReN
- Mitochondrial Hepatopathy Subcommittee, ChiLDReN
- Education Committee, SPLIT Hepatology Committee, NASPGHAN
- American Board of Pediatric Intensive Care Unit Liver Board Review

EDITORSHIPS

- Associate editor, *GI Scholarly Newsletter*, Children's Hospital of Pittsburgh

HONORS

- Co-author, "Neurocognitive Status in Alagille Syndrome: Results of a Multicenter Prospective Observational Study," AASLD Presidential Poster of Distinction
- AASLD Young Investigators Travel Award for "Liver Transplantation in Pediatric Acute Liver Failure: Practices and Patient Characteristics"

MAJOR LECTURESHIPS AND SEMINARS

- "Liver Dysfunction in Patients With IBD," invited lecture, Cincinnati Children's Hospital Medical Center Pediatric Autoimmune Liver Disease Symposium, Cincinnati, Ohio, October 2017

- "Post-Transplant Lymphoproliferative Disease," International Transplant Nurses Society local chapter symposium, August 2017
- "Anticipating Gene Therapy for Crigler-Najjar Syndrome," invited lecture, Fifth Annual Translational Medicine in Plain Populations Conference, Pittsburgh, Pa., August 2017
- "PFIC: The Clinical Perspective and Modeling Using Patient-Derived iPS Cells," invited lecture, Pittsburgh Liver Research Center Pediatric Cholestatic Liver Disease Focus Group, Pittsburgh, Pa., June 2017
- "Potential of Human Induced Pluripotent Stem Cells in Studies of Progressive Intrahepatic Cholestasis," invited lecture, Pittsburgh Liver Research Center Seminar Lecture Series, Pittsburgh, Pa., March 2017

Robert H. Squires Jr., MD

RESEARCH

A Multicenter Group to Study Acute Liver Failure in Children (NIH/NIDDK). Acute liver failure is a rare condition in which loss of liver cell function occurs rapidly (often less than two weeks), with devastating results. Members of this consortium continue to publish, and important clinical advances have also been made. Ongoing projects utilizing stored biomedical samples coupled with detailed clinical data include: characterizing the role of $CD8^+$ T-cell infiltration and indeterminate pediatric acute liver failure, identifying HMGB1 as an important cytokine mediator and acetaminophen toxicity, characterizing markers of immune activation, identifying genetic causes of indeterminate pediatric acute liver failure, and utilizing biomedical samples for viral discovery. A few recent achievements include: characterization of neurodevelopmental deficits in children following acute liver failure, exploration of liver transplant decisions in the setting of acute liver failure, and identification of a dynamic model using clinical and biochemical markers associated with clinical outcomes. The study is currently in its final no-cost extension phase. Squires is principal investigator.

The Pittsburgh Cholestatic Liver Disease Consortium (NIH/NIDDK). This is a renewal application that is a continuation, expansion, and merging of the Biliary Atresia Research Consortium and the Cholestatic Liver Consortium to form the Childhood Liver Disease Research and Education Network (ChiLDREN). Following enrollment, detailed longitudinal clinical data are collected as well as biomedical samples. The research team is currently enrolling participants with chronic cholestatic conditions and analyzing patient populations. There are two associated clinical trials to assess the efficacy of an apical sodium–dependent bile acid transporter inhibitor in the treatment of cholestatic liver disease in children with progressive familial intrahepatic cholestasis (sponsor: Lumena Pharmaceuticals Inc.) and Alagille syndrome (sponsor: NIH/NIDDK). Squires is principal investigator.

Hepatitis B Clinical Research Network—Data Coordinating Center (NIH/NIDK). This application proposes a multicenter group to study chronic hepatitis B infection to examine natural history and immunopathogenesis. Squires serves as a funded pediatric advisor at the data coordinating center.

ATD Treated by Carbamazepine (CBZ). CBZ decreased the hepatic load of AT granules and hepatic fibrosis in the PiZ mouse model of ATD-associated liver disease. Squires is investigating the effect of CBZ on severe liver disease due to ATD in adolescents and adults. Sponsor: Perlmutter Laboratory. Squires is co-investigator.

Creating Models of Rare Childhood Liver Diseases Using the Human Liver-on-a-Chip. Researchers are focusing initially on *POLG* mutations that cause Alpers Huttenlocher syndrome, one of many rare childhood diseases that involve the liver. This study will expand to include mutations involving the canalicular membrane. Sponsor: NIH. Squires is co-investigator.

Hepatitis C Clinical Trial. The trial has enrollment and is currently monitoring three patients in an open-label, multicenter study to evaluate the pharmacokinetics, safety, and efficacy of glecaprevir/pibrentasvir in pediatric subjects with genotype 1–6 chronic hepatitis C virus infection. Sponsor: AbbVie Inc. Squires is site principal investigator.

Pediatric Intestinal Failure Consortium. Although funding for this project has ended, ongoing sub-analysis of the data continues. A recent publication by Javid et.al. highlighted that the extent of intestinal failure–related liver disease is associated with increased mortality. Sponsor: NIH/NIDDK. Squires is principal investigator.

ADVISORY COMMITTEE MEMBERSHIPS

- 2011-16 International Classification of Disease (ICD)-11 revision process, Pediatric Topic Advisory Group, Gastroenterology Working Group, World Health Organization
- Gastroenterology Project Team, "Best Children's Hospitals," *U.S. News & World Report*
- American Academy of Pediatrics
  - Pediatrics Review and Education Program GI Editorial Board

  - Expert learning community meeting on "Innovative Financing for Health Care Services for Children With Special Health Care Needs: Value-Based Purchasing," American Academy of Pediatrics (Chicago, Ill.) and the Catalyst Center (Boston, Mass.)
  - Subspecialty advisor, Executive Board, Pennsylvania Chapter
- AASLD
  - Steering Committee, Pediatric Liver Disease Special Interest Group
  - Abstract Review Committee, Acute Liver Failure and Artificial Liver Support
- Finance Committee, NASPGHAN
- Advisor to the Executive Committee, INSPPIRE

HONORS

- Ashbel Smith Distinguished Alumnus Award, University of Texas Medical Branch
- Best Doctors, P*ittsburgh Magazine*, annually since 2005
- *Best Doctors in America*, Woodward/White, Inc., annually since 1993

MAJOR LECTURESHIPS AND SEMINARS

- "Acute Liver Failure in Children: A Brief History of Time," World Congress, Pediatric Gastroenterology, Hepatology, and Nutrition, Montreal, Canada, October 2016
- "Acute Liver Failure," breakfast session, World Congress, Pediatric Gastroenterology, Hepatology, and Nutrition, Montreal, Canada, October 2016
- "Acute Liver Failure," Third Annual James Ted Engle Pediatric Liver Transplant Lectureship, Cleveland Clinic, Cleveland, Ohio, October 2016
- "Childhood Liver Disease Research Network (ChiLDReN) and Pittsburgh Liver Research Center (PLRC): Opportunities for Collaboration," University of Pittsburgh, Pittsburgh, Pa., June 2017

Arvind Srinath, MD

RESEARCH

Arvind Srinath's research focuses on subspecialty medical education, pediatric gastroenterology curriculum development, and functional GI disorders.

- A multicenter, randomized, double-blind, placebo-controlled, parallel-group, safety and efficacy study of a range of linaclotide doses administered orally to children ages 7 to 17 years who have IBS with constipation (i.e., Fulfill Rome III Criteria for Child/Adolescent IBS and Fulfill Modified Rome III Criteria for Child/Adolescent Functional Constipation)
- A phase III, multicenter, long-term, safety, efficacy, and pharmacokinetics study of lubiprostone in pediatric subjects aged ≥ 6 years to < 18 years who have functional constipation
- Impact of a nurse practitioner–led teaching program on patient and caregiver knowledge of pediatric IBD, NASPGHAN/Association for Pediatric Gastroenterology and Nutrition Nurses, Susan Moyer 2017 Nursing Research Grant (co-principal investigator)

ADVISORY COMMITTEE MEMBERSHIPS

- NASPGHAN
- Training Committee, NASPGHAN, September 2015 to the present
- Resident Selection Committee, Children's Hospital of Pittsburgh
- Residency Program Leadership, Children's Hospital of Pittsburgh
- Pediatric Gastroenterology Fellow Clinical Competency Committee, Children's Hospital of Pittsburgh
- Pediatric Gastroenterology Fellow Selection Committee, Children's Hospital of Pittsburgh

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- NASPGHAN
- AGA
- CCFA
- American Academy of Pediatrics

HONORS

- *Best Doctors in America*, Woodward/White, Inc.

MAJOR LECTURESHIPS AND SEMINARS

- "Pediatric Gastroesophageal Reflux," general pediatric podcast with Candace Jones, 2017
- "Good Teaching: What Works and What We Can Do," invited presentation, Children's Mercy Hospital, Kansas City, Mo., April 2017

Veena Venkat, MD

RESEARCH

The Pittsburgh Cholestatic Liver Disease Consortium (ChiLDReN). This NIH-funded network conducts detailed clinical and translational investigations of cholestastic liver diseases in children. Veena Venkat is a co-investigator.

A Phase I/IIa Trial of Intravenous Immunoglobulin Therapy Following Portoenterostomy in Infants With Biliary Atresia. This study aims to determine the feasibility, acceptability, tolerability, and safety profile of intravenous immunoglobulin treatment after hepatic portoenterostomy for biliary atresia. Venkat is site principal investigator.

Immunosuppression Withdrawal for Stable Pediatric Transplant Recipients. The long-term objective of this NIH-funded network is to improve outcomes for pediatric liver transplant recipients through discoveries that will guide clinical decision-making related to immunosuppression withdrawal and simultaneously ensure excellent allograft function while minimizing complications of immunosuppression. Venkat is a co-investigator.

Improving Medication Adherence in Children Who Had a Liver Transplant (U34). This is the next phase of a now-completed R01 grant (MALT: Medication Adherence in Children Who Had a Liver Transplant) that validated the use of a marker of medication nonadherence, the Medication Level Variability Index (MLVI), in predicting rejection in children who have had a liver transplant. The researchers are developing a multisite trial in which pediatric liver transplant centers will use the MLVI to identify nonadherent recipients and a telemetric intervention to improve adherence.

A Pilot Study of Frailty in Children With End-Stage Liver Disease. The overall objectives are to validate the concept of frailty in children awaiting liver transplantation and to arrive at an accurate and efficient instrument to gauge the morbidity of transplant patients and to improve post-transplant outcomes beyond survival alone. Venkat is site principal investigator.

Pediatric Primary Sclerosing Cholangitis Consortium. This is a multicenter study with the objective of describing the natural history of primary sclerosing cholangitis in children. Venkat is the site principal investigator.

A Multicenter Collaboration to Study Primary Sclerosing Cholangitis in Children: Primary Sclerosing Cholangitis Partners Seeking a Cure. The objective of this multicenter study is to collect retrospective data on children with primary sclerosing cholangitis that can be used to build a clinical trial and ultimately find an effective therapy. Venkat is the site principal investigator.

CTOTC: Biomarkers for PTLDs in Children. This is a clinical trial investigating biomarkers associated with the development of EBV-related PTLDs in pediatric liver, heart, heart with liver, small intestine, and liver with small intestine transplant candidates and recipients. Venkat is a co-investigator.

ADVISORY COMMITTEE MEMBERSHIPS

- Member, Steering Committee, Pediatric Special Interest Group, AASLD
- Co-chair, Biliary Atresia Natural History and Therapeutics Working Group, ChiLDReN Research Network
- Protocol Committee: A Prospective Database of Older Children with Biliary Atresia, ChiLDReN
- Member, Education Committee, SPLIT, November 2014 to the present

Xunjun Xiao, PhD

Xunjun Xiao served as research assistant professor until March 31, 2017.

RESEARCH

Xiao has been extensively involved in ongoing efforts to better understand how newborns digest dietary fats. His work aims to understand how PLRP2 functions in human newborns. First, he is characterizing the properties of purified PLRP2 in the laboratory. Second, he is translating his findings in newborn mice, demonstrating that PLRP2 is critical for fat digestion. The investigation may assist in finding nutritional therapies to treat the 10% of newborns who have slow weight gain at birth.

Xiao also works to elucidate the mechanism behind chronic pancreatitis. Most children with chronic pancreatitis have genetic mutations that increase their risk, but the contributing mechanisms are not well known. The researchers are using model systems to test the novel hypothesis that expression of the mutant proteins in the pancreas leads to a stress response and cell death. The findings stand to increase understanding of pancreatic injury and provide guidance for developing new pharmacological interventions to prevent the ongoing damage from progressing to chronic changes in the pancreas.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Society of Nutrition Sciences
- American Society of Biochemistry and Molecular Biology

HONORS

- Western Pennsylvania National Pancreas Foundation Research
- Grant Award: "Carboxyl Ester Lipase Hybrid Confers Susceptibility to Chronic Pancreatitis Through Proteotoxicity"

TEACHING ACTIVITIES

NEW TEACHING PROGRAMS AND COURSES (AND OTHER EDUCATIONAL INNOVATIONS)

During the 2016–2017 academic year, the Division of Gastroenterology, Hepatology, and Nutrition has been implementing new educational programs to enhance its faculty and fellows. Under the fellowship direction of Arvind Srinath and Riha Bhatt, as well as the division chiefs, David Keljo (interim) and Mark Lowe (past), the division has augmented and enhanced the curriculum for its fellows, as well as provided more educational opportunities for faculty to increase their knowledge in the many subspecialties that make up the division. Mentoring activities occur in the division's research laboratories and across clinical research teams.

PEDIATRIC EXOCRINE PANCREATIC SEMINAR SERIES

Husain was awarded an unrestricted educational grant from Abbvie to launch a new educational series for fellows and faculty, titled "The Pediatric Exocrine Pancreatic Disorders Seminar Series." The seminar series hosted authorities in the field of clinical and research pancreatology. It included the following visiting professors.

NAME	INSTITUTION	PRESENTATION	DATE OF VISIT
Miklos Sahin-Toth, MD, PhD	Boston University	"A Spotlight on the Genetic Basis for Hereditary Pancreatitis"	November 18, 2016
Vijay P. Singh, MBBS	Mayo Clinic, Scottsdale, Ariz.	"Nutrition in Pancreatitis"	December 16, 2016
Bradley Barth, MD, MPH	University of Texas Southwestern	"Pediatric Pancreatic Interventions"	January 13, 2016
Tanja Gonksa, MD	Hospital for Sick Children and University of Toronto	"CFTR and Pancreatitis"	February 17, 2017
Vikesh Singh, MD, MS	Johns Hopkins University	"Fluids, Pancreatitis Severity, and What's in the Pipeline for Pancreatitis Treatments"	March 17, 2017

The series received overwhelmingly positive feedback, and Husain looks forward to renewed funding.

PEDIATRIC GASTROENTEROLOGY FELLOWSHIP PROGRAM

Srinath and Bhatt, directors of the Pediatric Gastroenterology Fellowship Program, through requested feedback from fellows and faculty, enhanced the program as follows.The directors, along with the division's hepatologists, developed an inpatient rotation specifically to enhance the care of hepatology and transplant hepatology patients. The curriculum was augmented with targeted didactic objectives to be covered in monthly case-based conferences and weekly service patient conferences.

- Sevilla joined the faculty in 2016 and has since codeveloped and implemented (with the fellowship program directors) an advanced nutrition curriculum for the pediatric gastroenterology fellows. Presentations for fellows and faculty occur quarterly.
- A chief pediatric gastroenterology fellow program was piloted. The goal of the program is to offer leadership and mentoring opportunities to the two senior fellows. Feedback from the fellows has indicated success, which has led to its continuation next year.

PEDIATRIC TRANSPLANT HEPATOLOGY FELLOWSHIP PROGRAM

In November 2016, James Squires began the application process to restart the Pediatric Transplant Hepatology Fellowship Program. He received accreditation from the Accreditation Council for Graduate Medical Education (ACGME) for its establishment. He will lead the transplant hepatology team of McKiernan, Robert Squires, Khan, and Venkat and work directly with the pediatric transplant surgeon team to build the program.

RESEARCH LABORATORY TEACHING ACTIVITIES

Ghazi has been very active teaching courses and giving lectures for PhD students, fellows, residents, and medical students in a variety of courses.

TEACHING HONORS AND AWARDS

Hilary Michel was awarded First-Year Fellow of the Year by the UPMC Residency Program. This is the second year in a row that a pediatric gastroenterology fellow has received the award.

QUALITY MEASURES FOR TEACHING ACTIVITES

In addition to continuous bidirectional feedback from faculty, fellows, and staff, the division conducts the following quality measures for teaching activities: fellow evaluations (quarterly), which have been aligned with the ACGME milestones; Clinical Competency Committee evaluations, which include evaluations of fellow teaching activities; resident evaluations (continuous throughout the year for fellows and faculty); and yearly fellow training exam scores.

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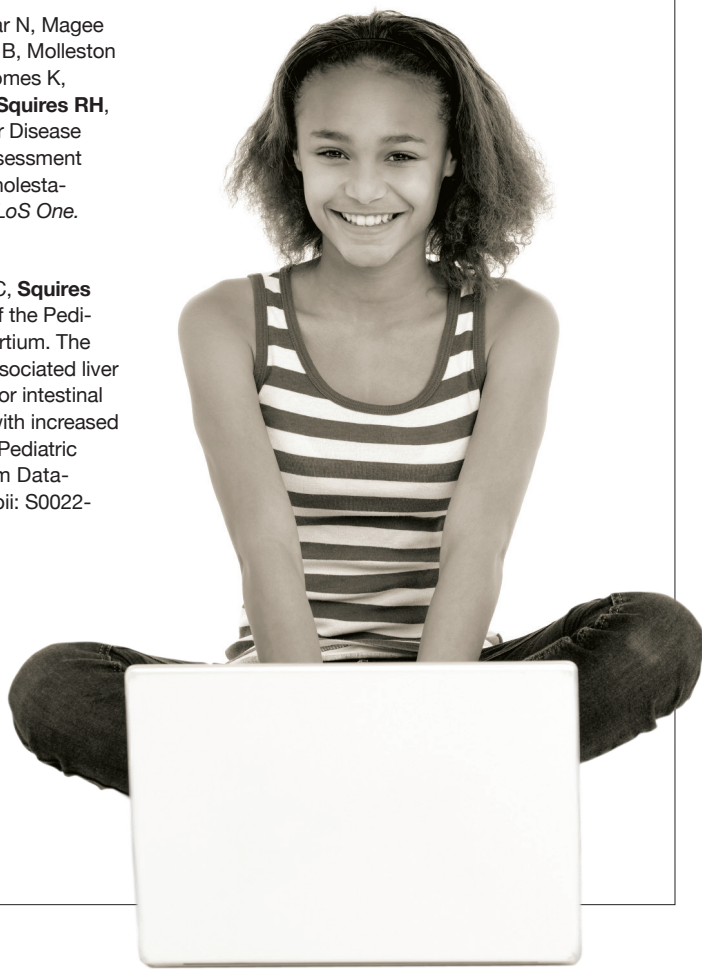

DIVISION OF
GENERAL ACADEMIC PEDIATRICS

Mission

The mission of the Division of General Academic Pediatrics (GAP) is to improve the health and well-being of children, families, and communities through high-quality patient care, leadership in the education of health care providers, high-impact research on common pediatric problems, and advocacy in promoting child and family health. More specifically, the division:

- Provides evidence-based, family-centered, culturally competent pediatric primary care to children living in underserved communities
- Provides innovative, evidence-based education to medical students, residents, fellows, and allied health professionals
- Develops and disseminates high-impact research regarding common pediatric problems, novel treatment approaches, best practices, health outcomes, and innovative models of service delivery
- Advocates through leadership and partnership to promote child and family health at hospital, community, regional, and national levels

FACULTY AND STAFF

Alejandro Hoberman, MD
Professor of Pediatrics and Clinical and Translational Science
Professor of Pediatrics
Jack L. Paradise Professor of Pediatric Research
Vice Chair for Clinical Research
Chief, Division of GAP

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Associate Professor of Pediatrics
Clinical Director, Division of GAP
Clinical Director, Child Development Unit

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Associate Professor of Pediatrics

Aimee Biller, MD
Assistant Professor of Pediatrics
Director, Combined Ambulatory Medicine and Pediatrics Clerkship

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Professor of Pediatrics, Psychiatry, and Clinical and Translational Science
Director, Faculty Development in GAP Fellowship Program

Diego Chaves-Gnecco, MD, MPH
Associate Professor of Pediatrics
Director, Salud Para Niños (Health for the Children)

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Assistant Professor of Pediatrics

Alicia Haupt, MD
Assistant Professor of Pediatrics

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Medical Director, Turtle Creek Primary Care Center (PCC)

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Associate Program Director, Children's Hospital of Pittsburgh of UPMC Residency Program
Medical Director, Resident Continuity Clinic
Director, Primary Care Education

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Assistant Professor of Pediatrics

Jill Radtke Demirci, RN, PhD
Postdoctoral Associate

Emily Barker, PA

Donna Duchene, CRNP

Sandra McRandal, CRNP

OVERVIEW OF DIVISION

The teaching programs and tools that faculty in the division have developed resulted from specific divisional goals of high-quality curricula for learners at all levels. There has been a critical mass of faculty interested in improving medical knowledge and skills, with the outcome being ongoing, active curriculum development by all members, with inspiration from early leaders and mentors. These accomplishments occur in the midst of a thriving clinical environment at the PCCs in Oakland and Turtle Creek and at the Magee-Womens Hospital of UPMC Normal Newborn Nursery (MWH-NNN), as well as in a thriving research program funded by the National Institutes of Health (NIH) in the areas of vitamin D and calcium metabolism; acute bacterial rhinosinusitis; urinary tract infection (UTI); pneumonia; vesicoureteral reflux (VUR); acute otitis media (AOM); and the relationship between children's health and nutrition and maternal mental health issues, such as depression and substance abuse. Research activities are enhanced by Pediatric PittNet, the practice-based research network (PBRN) funded by the CTSI, with research nurses/research assistants who identify and enroll children in multiple physical and mental health studies being conducted in the network.

CLINICAL ACTIVITIES

This year, the Division of GAP had more than 28,215 visits for outpatient services at two PCCs. The PCC at Children's Oakland Medical Building remains the largest program. The PCC in Turtle Creek, which facilitates teaching of residents seeking dual credentialing in medicine and pediatrics, continued to experience growth during the past year. The division also increased the numbers of residents rotating, specifically with additional residents in the Community-Oriented Residency Education (CORE) Program.

Kristin Hannibal continued to co-chair the GAP multidisciplinary Clinical Effectiveness Group, which aims to improve delivery of clinical care at both PCCs. The work group includes nurses, administrators, and clinicians. Their efforts have resulted in a variety of clinical initiatives and educational improvements implemented at each site.

The group also has worked on clinic efficiency, creating a team approach for rooming patients and distributing work. The division has increased clinic efficiency with implementation of practice huddles and the electronic white board tracking system developed by Ana Malinow in collaboration with graduate students from Carnegie Mellon University.

The division has broadened its clinical services with lactation services, dental services, social work/care coordination, medical-legal partnership, and behavioral health services, collocating providers in the clinical space. Dental services are offered through a public health hygienist in collaboration with the Children's Hospital of Pittsburgh of UPMC dental division. The social worker position is funded through a joint venture with Gateway Health Plan for Gateway-insured patients. The division has a UPMC-funded care-coordination position in addition to a practice-supported care coordinator. All are busy serving the needs of an at-risk population while striving to provide a comprehensive medical home for patients. Most recently, the division has partnered with Neighborhood Legal Services Association to form a medical-legal partnership to serve the legal needs of the at-risk patient population. A lawyer from the program is collocated in the clinic three half-days per week, working on a variety of issues from housing, social security, and utility shutoff to educational challenges for families.

The division maintained an extremely busy clinical service at MWH-NNN, where faculty members provide leadership and education to pediatric, medicine-pediatric, and family medicine residents and medical students in the areas of normal newborn assessment, management, discharge planning, and breastfeeding education. The division has increased its participation in administrative responsibilities at MWH-NNN by participating in Newborn Nursery Committee meetings and the ongoing multidisciplinary OB Bundle project at MWH.

Hannibal participated in the implementation and update of the electronic health record on MWH's postpartum floor and in the nursery. The busy service is now responsible for about 1,907 newborns and more than 4,367 total clinical visits to the PCCs annually. As the liaison to MWH-NNN for the Division of GAP, Hannibal has worked in close collaboration with Abeer Azzuga, nursery director. The Newborn Nursery Committee meets every month to discuss newborn issues with other pediatricians, nursing staff, infection-control nurses, lactation specialists, and other invited guests. The division has reviewed newly developed policies such as the safe sleep position program, neonatal abstinence syndrome protocol, pulse oximetry testing of newborns, treatment of hyperbilirubinemia, and the baby-friendly hospital initiative, and it has updated a discharge instruction book for new parents, as well as discussed pertinent newborn issues affecting clinical care in the service.

The patient population at MWH-NNN is quite diverse and is often medically and socially challenging. Patients who choose to receive follow-up care at the PCC in Oakland schedule appointments in an outpatient newborn clinic as soon after discharge as necessary to receive optimal care. The division has expanded to five afternoon newborn follow-up clinics per week and four morning lactation sessions per week. Faculty experts in breastfeeding and newborn care staff the clinics, along with residents and students from the MWH-NNN rotation. In addition, a lactation consultant now serves the newborn follow-up clinic five full days per week. The division now has the availability to expand clinics as needed to accommodate breastfeeding babies, who usually need to be seen within 48 hours after discharge.

Katherine Watson continues to work to establish Healthy Habits for Life (HH4L) as a sustainable clinic at the PCC in Oakland. She has launched research initiatives relating to lifestyle modification and increased activity for patients.

Watson works with the Children's Hospital of Pittsburgh residents in the HH4L clinic during their continuity rotation. The goal is to improve their motivational interviewing skills and increase their medical knowledge about childhood obesity.

Chaves-Gnecco is the director and founder of the program Salud Para Niños, the first pediatric bilingual-bicultural clinic in Southwestern Pennsylvania. At Salud Para Niños, culturally and linguistically competent primary care for children and families is complemented with activities intended to empower the community regarding its own health. Some of the activities of Salud Para Niños include weekly clinics in Oakland at the Children's Hospital of Pittsburgh of UPMC PCC; two monthly free clinics at the Children's Hospital Care Mobile and at the Birmingham Clinic; participation in the organization of community fairs; a literacy program in which children are given bilingual books to promote early literacy and learning of English as a second language; translation of wellness materials into Spanish; and Hispanic/Latino car-seat checks, which emphasize car safety.

RESEARCH AND OTHER SCHOLARLY ACTIVITIES

The division has received support from the CTSI (Clinical Translational Science Award [CTSA] to the University of Pittsburgh) to provide infrastructure for Pediatric PittNet, a PBRN that is currently composed of 20 urban, suburban, and rural practices with more than 200 providers caring for more than 200,000 children. PittNet was created to facilitate the translation of pediatric research discoveries into practice to improve the health of the region's children. Currently, Pediatric PittNet includes more than 50 research protocols covering a range of common childhood behavioral health (e.g., attention-deficit disorder, depression, anxiety) and physical health (e.g., AOM, UTI, immunization) concerns. Reis is the medical director of PittNet; Hoberman and David Brent (Department of Psychiatry) serve as codirectors.

In his role as the Department of Pediatrics vice chair for clinical research, Hoberman has continued to work with the chair on recruiting for various division chief–level positions; participating in Department of Pediatrics committees, including Executive, Scholarship, Promotions and Tenure, and Pediatric Scientist Development Program Steering; fully restructuring CTSA-sponsored programs (PittNet) and the Pediatric Clinical and Translational Research Center; and completing full implementation of a pediatric component of the CTSA research registry. In collaboration with Bogen, Carlton Bates (Nephrology), and Liz Miller (Adolescent and Young Adult Medicine), he developed an aggressive three-year timeline of research expectations and requirements for fellows, as well as a similar two-year outline for junior faculty. Hoberman and Miller established mock K review study sections for fellows and junior faculty submitting applications from the divisions of GAP and Adolescent and Young Adult Medicine.

Alejandro Hoberman, MD

RESEARCH

Completion of Additional Data Analysis on the National Institute of Allergy and Infectious Diseases (NIAID)-Sponsored, Randomized, Placebo-Controlled Study in Young Children With AOM, Leading to a Revised AOM Severity of Symptoms Scale (AOM-SOS v. 5.0). A U.S. Food and Drug Administration (FDA) guidance document for clinical trials in children with AOM has recommended the use of time-to-resolution of symptoms as the primary outcome when evaluating the efficacy of antimicrobial treatment. Following a series of FDA pre–investigational new drug meetings and teleconferences for future studies, Shaikh, Hoberman, and Paradise conducted additional analyses of data collected in the AOM placebo-controlled trial that led to a revision of the AOM-SOS scale, as well as determination of the minimally important clinical difference (MICD), to increase its sensitivity to detect change and determine statistical analysis methods that better discriminate between two treatment groups, rather than between treatment and placebo. A document outlining the analyses and proposed revision to the AOM-SOS scale was submitted to the Division of Antimicrobials, Center for Drug Effectiveness Research, FDA. Three manuscripts were published. The FDA requested further data on parents completing the tool electronically. The researchers completed gathering of data on about 70 children whose parents completed the AOM-SOS elec-

Alejandro Hoberman, MD
Division Chief, General Academic Pediatrics

tronically and planned to submit the completed application to the FDA early in 2018.

Completion of the Broad Agency Announcement (NIAID) Contract on Targeted Interventions to Reduce Antimicrobial Resistance to Compare Short- Versus Standard-Duration Therapy in Children 6–23 Months With AOM. Hoberman was awarded a five-year NIAID contract to evaluate the efficacy of reduced-duration treatment compared with standard-duration treatment and its impact on antimicrobial resistance. This was in response to BAA NIAID-DMID-NIHAI2009058, "Targeted Clinical Trials to Reduce the Risk of Antimicrobial Resistance," and addressed AOM, one of three disease areas of interest, and shorter treatment duration, one of the eligible strategies to reduce antimicrobial resistance (NIAID, $9,005,202). The researchers concluded that in children 6–23 months of age with AOM, reduced-duration antimicrobial treatment resulted in less favorable outcomes than standard-duration treatment, without reduction in the occurrence of adverse events or the emergence of antimicrobial resistance. The manuscript was published in December 2016 in the *New England Journal of Medicine*. A final progress report was submitted to NIAID in November 2016.

Continuation of a Clinical Trial (U01) Sponsored by the National Institute of Deafness and Communication Disorders (NIDCD) Evaluating the Efficacy of Tympanostomy Tubes for Children With Recurrent AOM. This five-year study is a randomized clinical trial of children with recurrent AOM who will be followed over a two-year period to determine whether the surgical therapy compared to nonsurgical therapy results in a substantial reduction in the number of episodes of AOM, whether children are less likely to carry resistant bacteria because of decreased utilization of antibiotics, and whether surgery is cost-effective. Findings will enable evidence-based decision-making by parents. Enrollment in the trial started in November 2015; a total of 789 children have been enrolled during the initial year in phase I (screening phase); 175 have been randomized in phase II at Children's Hospital of Pittsburgh of UPMC, Children's National Medical Center, and Kentucky Pediatric/Adult Research. Monthly teleconferences have been conducted with all participating investigators and an NIDCD scientific monitor to evaluate progression of the study and share successful recruitment strategies.

Monitoring Trends of Antimicrobial Resistance and Serotype Variation in Nasopharyngeal Isolates From Children With AOM. Together with Judith Martin, Hoberman has conducted studies in children with AOM for more than 15 years; the work has permitted the team to follow trends of antimicrobial resistance and serotype variation in nasopharyngeal isolates of *Streptococcus pneumoniae*. Although an initial increase in *H. influenzae* colonization was observed, suggesting an impact of pneumococcal conjugate vaccine, most recent rates of nasopharyngeal colonization with *H. influenzae* and ß-lactamase production are similar to those observed before universal administration of PCV7. A manuscript describing the work has recently been submitted for publication.

Continuing to Contribute to Enhanced Diagnostic Accuracy in Young Children With AOM. In collaboration with Shaikh, Paradise, and Jelena Kovačević (department head, Electrical and Computer Engineering, Carnegie Mellon University), Hoberman continues to contribute articles regarding enhancement of symptomatic assessment in children with AOM and overall outcomes in AOM clinical trials.

Developing a Novel Automated Algorithm for Classifying Diagnostic Categories of Otitis Media. AOM represents a bacterial superinfection of the middle ear fluid, whereas otitis media with effusion (OME) represents a sterile effusion that tends to subside spontaneously. Diagnosing children with AOM is difficult, often leading to over-prescription of antibiotics, as they are beneficial only for children with AOM. This underscores the need for an accurate and automated diagnostic algorithm. In collaboration with colleagues at Carnegie Mellon University, the team developed such an algorithm; it achieved 89.9% classification accuracy and outperformed clinicians who did not receive special training and state-of-the-art classifiers. The researchers also completed a collaboration with Ricoh Innovations, Menlo Park, Calif., (grant support $1,166,402) in developing a prototype light field otoscope. The team published a manuscript, "Light Field Otoscope Design for 3D *In Vivo* Imaging of the Middle Ear," in the *Journal of the Optical Society of America*, which described a light field digital otoscope designed to measure three-dimensional shape of the tympanic membrane (TM) and ear canal. The researchers have completed the collaboration with Ricoh and planned to review with the company a final report of the findings before the end of 2017.

Pitt-CMU-iTM. A team is developing a decision-support diagnostic aid to increase accuracy in diagnosis of AOM, which will reduce unnecessary antibiotics, adverse effects, and bacterial resistance, using off-the-shelf otoendoscopes coupled with smartphones to deliver high-resolution TM images. The overall objective is to complete development, test usability and implementation, and bring to the market

this point-of-care diagnostic aid/decision support with an automated algorithm that exhibited accuracy identifying diagnostic categories comparable to diagnoses made by expert otoscopists. The researchers will also explore a parallel approach of using deep learning to teach diagnostic categories and determine whether that strategy would achieve better results than those currently in use (low 90 percent in terms of classification accuracy), as well as assess usability and implementation in a busy primary care environment. Potential markets for the tool would be emergency departments, urgent care centers, pediatric/family medicine physician offices, and retail clinics. Intellectual property status: U.S. Patent Application Serial No. 15/483,133: "Method and Apparatus for Aiding in the Diagnosis of Otitis Media by Classifying TM Images."

Continuation of Enrollment of Children, in Collaboration With Investigators at Children's Hospital of Philadelphia, for a Study in Response to a Broad Agency Announcement (NIAID) on Targeted Interventions to Reduce Antimicrobial Resistance Contract to Compare Short- Versus Standard-Duration Therapy for UTI in Children (SCOUT, Short-Course Therapy for Urinary Tract Infections in Children). The SCOUT study is a multicenter, double-blind, placebo-controlled, non-inferiority clinical trial of 746 children ages 2 months to 10 years who will be enrolled over six years. Children will be stratified based on presence of fever at the initial presentation of UTI symptoms and by specific antibiotic therapy prescribed by the original treating clinician. Following documented clinical improvement (afebrile and asymptomatic) after the first four to five days of provider-initiated therapy, children are blindly randomized to the standard-of-care antimicrobial therapy arm (continued antibiotic) or the short-course antimicrobial therapy arm (placebo) of five more days to complete 10 days of therapy. Outcomes include treatment failure, UTI recurrences, occurrence of asymptomatic bacteriuria, and gastrointestinal colonization with resistant organisms *Escherichia coli* or *Klebsiella pneumoniae*. Enrollment of children in the study commenced in June 2012 and will continue for 18–24 months; a total of 547 children have been enrolled to date.

UpToDate. In collaboration with Shaikh, Hoberman continued to contribute to UpToDate in the area of UTI in children, with cards on clinical features and diagnosis, epidemiology and risk factors, management, imaging and prognosis, and long-term management and prevention of UTIs in children, all of which are widely accessed and require frequent revisions.

Improvements in Research Methods to Be More Adaptable to the Needs of Participants. Under the leadership of Evelyn Reis, the team continues to contribute to Pediatric PittNet, a practice-based clinical and behavioral health research network under the umbrella of CTSI at the University of Pittsburgh. Both PCCs (Oakland and Turtle Creek) participate in Pediatric PittNet, as do many of the Children's Community Pediatrics practices, where pediatric residents also have their weekly continuity clinic experience. Hoberman is an active member of the Executive Committee of Pediatric PittNet; all of the program's clinical trials enroll children in practices within the network. Accordingly, trainees observe and participate in clinical research; they see the immediate and long-term benefits of research through ongoing exposure.

Mobile Research Service. To bring research as close as possible to the family's actual home, and for research visits that do not require imaging or complex procedures, the team developed a Mobile Research Service. The service consists of research coordinators from various divisions within the Department of Pediatrics who are cross-trained for various protocols and conduct research visits at a family's home, at a pediatrician's office, or at one of Children's satellite locations. Of 567 parents who were offered those options for enrollment and follow-up visits for NIH contracts, of the 466 who chose off-campus visits, 341 (73%) were conducted at home, 72 (15%) at the primary care provider's office, and 53 (11%) at Children's Hospital of Pittsburgh (CHP) satellites. Only 101 (18%) of visits were conducted at the Clinical Trials Unit or at CHP, which were the only options prior to the Mobile Research Service.

eConsent Manager. To empower families and maximize participant recruiting, and in partnership with the CTSI at the University of Pittsburgh School of Medicine, the team developed a software program termed eConsent Manager. The clinical research platform includes a consent document that is approved by the Institutional Review Board. It is presented dynamically, permits video conferencing with the researcher, and shows animated videos about specific study procedures. It also provides alerts when a revised consent document is needed, and at completion, it triggers the data-management system to initiate collection of research data. The team completed in-depth interviews with parents as they used the system and will formally evaluate its impact on consent rates, adherence rates, and parental satisfaction.

Hoberman published 20 articles in the past three years in the *New England Journal of Medicine*, *JAMA Pediatrics*, *Pediatrics*, *Pediatric Infectious Diseases Journal*, *Academic Pediatrics*, *Journal of Pediatric Urology*, and *Journal of Pediatrics*.

ADVISORY COMMITTEE MEMBERSHIPS

- Minority Pre-K Career Education and Enhancement for Health Care Research Diversity Committee, University of Pittsburgh School of Medicine
- Research Advisory Committee, Children's Hospital of Pittsburgh
- Executive Committee, Department of Pediatrics
- Pediatric Development Scientist Steering Committee, Department of Pediatrics
- Promotions and Tenure Committee, Department of Pediatrics
- Non-Tenured Promotions Committee, University of Pittsburgh School of Medicine

EDITORSHIPS

- *Infectious Diseases in Children*

MAJOR LECTURESHIPS AND SEMINARS

- "UTI and VUR: After the Trials," International Pediatric Nephrology Association, Foz do Iguazu, Brazil, September 2016
- "Diagnosis and Management of AOM," visiting professor, Dupont Children's Hospital, Nemours Alfred I. DuPont Hospital for Children, Wilmington, Del., November 2016
- "UTI and VUR: After the Trials," visiting professor, Dupont Children's Hospital, Nemours Alfred I. Dupont Hospital for Children, Wilmington, Del., November 2016
- "Diagnosis and Management of AOM," grand rounds, Children's Hospital of Pittsburgh, Pittsburgh, Pa., December 2016
- "Infecciones Urinarias y Reflujo Vesicoureteral: Despues de los Studios Clinicos," VI Congreso Internacional de Infectologia Pediatrica y Vacunas, Sociedad Argentina de Infectologia Pediatrica, Buenos Aires, Argentina, April 2017
- "Sabemos Diagnosticar y Tratar Otitis Media Aguda?" VI Congreso Internacional de Infectologia Pediatrica y Vacunas, Sociedad Argentina de Infectologia Pediatrica, Buenos Aires, Argentina, April 2017
- "UTIs and VUR," visiting professor, Children's of Alabama and the University of Alabama at Birmingham Department of Pediatrics, clinically focused research roundtable discussion, Birmingham, Ala., May 2017
- "Diagnosis and Management of AOM," visiting professor, Children's of Alabama and the University of Alabama at Birmingham Department of Pediatrics, Grand Rounds Dixon Lecture, Birmingham, Ala., May 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Argentine Pediatric Society
- Academic Pediatric Association
- American Academy of Pediatrics
- Inter-American Society of Pediatric Otolaryngology
- Society for Pediatric Research
- American Pediatric Society

Kristin M. Hannibal, MD

RESEARCH

Kristin Hannibal has worked to facilitate the implementation of a variety of outside research projects within the clinical sites of the PCCs in Oakland and Turtle Creek. In the first of those projects, Hannibal collaborated with the UPMC Department of Family Medicine and continued to recruit participants at a PCC for a study sponsored by the Centers for Disease Control and Prevention (CDC) that helps determine the efficacy of the various influenza vaccines. Efficacy data gathered from children at the PCC resulted in the CDC's recommendation against administration of live attenuated influenza vaccine (FluMist) for children during the 2016–2017 respiratory-infection season.

Hannibal has been credited with developing a culture of quality improvement, building on projects she initiated, which have sparked many others. She has served as clinical director of the Division of GAP since 2004. The clinical services of the PCCs (Oakland and Turtle Creek) have flourished under her leadership. Excellence in clinical practice, improved access for all levels of care, and solid continuity experience as part of the resident educational mission have been prioritized.

Compliance With Early Periodic Screening, Diagnosis, and Treatment Medicaid Visit Requirements. Hannibal has developed office work flow and provider education to ensure that children are receiving developmental and autism screening at the appropriate intervals, as well as being screened for lead and hemoglobin/hematocrit, getting dental home referrals, and following the appropriate preventive visit periodicity. Proper adherence to the latest immunization schedules also has been stressed through modifications in the electronic health record templates as well as continued provider (faculty, resident, and nursing) education.

Dental Home for an At-Risk Population. In cooperation with Hannibal, Deborah Moss, and Amanda Dumas, providers at the PCC in Oakland began the application of dental varnish to at-risk children with Medicaid insurance in March 2011. In coordination with Brian Martin in the dental department, public health dental hygienists come to the PCC to provide dental screenings for patients four days per week. Clinicians promote applications of dental varnish and provide patients with a dental home.

Expansion of Behavioral Health Screening. Providers at all sites have been conducting routine behavioral health screenings of all children ages 4–18 years. The screenings include psychosocial screens at the younger ages, depression and anxiety screening in the teen years, and screenings for attention-deficit and hyperactivity disorder symptoms as needed. Hannibal worked toward embedding a behavioral health specialist at the PCC in Oakland and participated in the selection process. The program added the availability of a psychiatrist to oversee the behavior therapist and be a referral resource for families. The division is also participating in the TIPS program, a telephone support line for providers who see Medicaid patients and have questions about their patients with behavioral concerns.

Reach Out and Read Program. Hannibal serves as medical director of the Reach Out and Read Program at the PCCs in Oakland and Turtle Creek. The program's goals are to promote early literacy through distribution of new books during well-child visits and to improve developmental outcomes in at-risk populations at the two PCC offices.

Liaison to the MWH Administration and the Division of Neonatology as the Director of the Newborn Nursery Rotation. Hannibal facilitates integrated care in the newborn nursery and the specialized care nursery for drug-exposed infants. Hannibal has joined with Jenna Zarit to update the MWH-NNN resident curriculum and enable the Web-based curriculum to serve as a showpiece for pediatrics. Many residency programs across the nation have adopted the Web-based curriculum.

Educating Practices in Community-Integrated Care (EPIC-IC) Pennsylvania Medical Home Initiative. Hannibal also has been instrumental in bringing this initiative to the PCC in Oakland and implementing it. The program is sponsored by the Pennsylvania chapter of the American Academy of Pediatrics and assists practices in the process of improving their functioning as true medical homes for their patients. Hannibal's group has developed a children and youth with special health care needs patient registry for patient care use and earned a care coordination grant to improve care coordination. She also has chaired the pilot project on transition to adult care for children with special health care needs. She assisted the Medical Home Team to achieve National Committee for Quality Assurance level 3 recognition status in 2016 and provides further assistance to maintain the certification.

Clinical Director, Child Development Unit. Along with the division chief, Ira Bergman, Hannibal has improved access for patients requesting urgent evaluation at the Child Development Unit, increasing patient visits and decreasing wait times for appointments. More academic rigor has been established, with weekly seminars on research, clinical, educational, and administrative topics.

ADVISORY COMMITTEE MEMBERSHIPS

- EPIC-IC Medical Home Implementation Project, Pennsylvania Chapter, American Academy of Pediatrics
- Clinical Effectiveness Committee, Division of GAP, Children's Hospital of Pittsburgh of UPMC
- Continuous Quality-Improvement Work Group, Maternal Addictions, MWH
- Newborn Committee, MWH
- OB Bundle Pediatric Work Group, MWH
- Preemie Health Coalition, National Healthy Mothers, Healthy Babies Coalition
- Screening in Primary Care and Neonatal Intensive Care Unit follow-up work groups, Society of Developmental Behavioral Pediatrics

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Academy of Breastfeeding Medicine
- Society of Developmental Behavioral Pediatrics
- Pediatric Academic Association

Sonika Bhatnagar, MD, MPH

RESEARCH

Sonika Bhatnagar's research portfolio includes diagnosis and treatment of common infections in children, including UTI, AOM, sinusitis, and pneumonia. Bhatnagar was a co-investigator and served on the Steering Committee with Hoberman and Shaikh on the multicenter, National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK)-sponsored Randomized Intervention for Children with VesicoUreteral Reflux (RIVUR) and Careful UTI Evaluation (CUTIE) studies.

Bhatnagar presented a poster, "Prognosis of Children with UTI: A Systematic Review," with Shaikh at the International Conference on VUR in Children in Göteborg, Sweden. Bhatnagar, together with Hoberman, Shaikh, and Research Manager Diana Kearney, created a parent toolkit for the NIDDK RIVUR study, which is available online.

Bhatnagar's research role expanded to include analysis of strategies to increase recruitment of children in randomized, controlled trials. She served as a physician consultant for the RIVUR study and presented "Recruitment Strategies for NIH-Sponsored Trial Sites" to the Steering Committee of the NIDDK RIVUR study. She conducted site visits at 18 children's hospitals across the United States and evaluated the use of various recruitment strategies to compose site-specific recommendations and then provide one- and four-month post-intervention reports. She found a significant increase in enrollment at all 17 sites at 120 days post-intervention and determined that repeated interventions may be required to sustain the increases. The results of that work, "Development and Impact of an Intervention to Boost Recruitment in a Multicenter Pediatric Randomized Clinical Trial," were published in *Clinical Pediatrics* and presented at the Pediatric Academic Societies in Washington, D.C., and the NIH Workshop on the Enrollment and Retention of Participants in NIH-Funded Clinical Trials in Bethesda, Md.

Her current research involves assessing the impact of offering a Mobile Research Service on recruitment in pediatric clinical trials. She chairs the Steering Committee that created and launched the Center for Excellence in Child and Adolescent Health Research at Children's Hospital of Pittsburgh of UPMC and the University of Pittsburgh CTSI to promote the health of children, adolescents, and young adults by advancing clinical research. Bhatnagar created an Academic Toolkit for Clinical Researchers, available online through the center, and incorporated the curriculum into the Institute for Clinical Research Education Special Topics in Clinical Trials Course at the University of Pittsburgh. The mission of the center is to bring participants, families, researchers, health care providers, and the community into the research home to learn from one another to develop innovative, collaborative strategies and demonstrate the impact of stakeholder-engaged approaches to translate evidence-based practice into measurable outcomes for children, adolescents, and young adults.

Bhatnagar also studies prevention of disease and injury. On a local level, she has served as one of two Children's Hospital physician contributors to the Allegheny County Child Death Review Team, which also includes law enforcement, child-protective services, the office of the coroner/medical examiner, the district attorney's office, and school superintendents. She contributed to the systematic review of child deaths, identification of preventable contributing factors, and the creation of a public health model of prevention of child fatality that stresses the roles of legislation and public education. With the Child Advocacy Center and the Division of Adolescent Medicine, she created a public health and advocacy continuity clinic curriculum module. On a state level, she has served as a consultant to the Pennsylvania chapter of the American Academy of Pediatrics to analyze statewide child death review findings and propose educational and legislative strategies. On a national level, she was appointed to serve on the CDC Steering Committee for the National Action Plan for Child Injury Prevention. In 2015, Bhatnagar received the American Medical Association Physician's Recognition Award.

ADVISORY COMMITTEE MEMBERSHIPS

- Chair, Steering Committee, Center for Excellence in Child and Adolescent Research
- Representative, Allegheny County Child Death Review Team
- Representative, Federal Advocacy Action Network, American Academy of Pediatrics
- Representative, Mentoring Committee, Drexel University College of Medicine
- Representative, Prevent Child Injury Advisory Committee, CDC
- Representative, Senior Executive Committee, National Action Plan for Child Injury Prevention, CDC

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Academic Pediatric Association
- American Academy of Pediatrics
- Society for Clinical and Translational Science

Aimee Biller, MD

ADVISORY COMMITTEE MEMBERSHIPS

- Director, Combined Ambulatory Medicine and Pediatrics Clerkship, University of Pittsburgh School of Medicine
- Consultant, Ethics Consult Service for Inpatients, Children's Hospital of Pittsburgh of UPMC

- Research ethics consultant, CTSI, University of Pittsburgh
- Clinical Ethics Committee, Children's Hospital of Pittsburgh of UPMC
- Third- and Fourth-Year Retention Committee, University of Pittsburgh School of Medicine
- Stakeholder Advisory Group, CTSI, University of Pittsburgh
- Clerkship Directors Committee, University of Pittsburgh School of Medicine
- Independent Data and Safety Monitoring Board, "Safety and Preliminary Efficacy of Donor-Derived DCreg Infusion and Immunosuppression Withdrawal in Living Donor Liver Transplant Recipients," University of Pittsburgh School of Medicine
- National Advisory Council on Protection of Minors, Conference of Major Superiors of Men

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- American Society of Bioethics and Humanities
- Quality Improvement Innovation Networks, American Academy of Pediatrics
- Council on Clinical Information Technology, American Academy of Pediatrics Council on Medical Student Education in Pediatrics
- Section on Bioethics, American Academy of Pediatrics Children's Hospital of Pittsburgh representative, Southwestern Pennsylvania Consortium Ethics Program

HONORS

- *Best Doctors in America*, Woodward/White, Inc.
- Best Doctors, *Pittsburgh Magazine*
- Patient Satisfaction Award 2016, Children's Hospital of Pittsburgh of UPMC

Debra L. Bogen, MD

RESEARCH

Debra Bogen's research focuses on improving health outcomes for infants born to women with substance use and mood disorders. She serves as a co-investigator on a variety of studies of early-childhood nutrition and breastfeeding, including the following.

- Pamela Morris (New York University) and Daniels Shaw's (University of Pittsburgh, Psychology) multicenter trial funded by the National Institute of Child Health and Development (NICHD): Integrated Model for Promoting Parenting and Early School Readiness in Pediatrics
- Mary Marazita's study funded by the National Institute of Dental and Craniofacial Research: Factors Contributing to Oral Health Disparities in Appalachia
- Richard Beigi's Microbicides Trials Network studies
- Ariadna Forray's (Yale) study funded by the National Institute on Drug Abuse: Progesterone for Postpartum Smokers: Feasibility, Breastfeeding, and Infant Safety
- Melissa Bartick's study funded by the W.K. Kellogg Foundation: Comprehensive Analysis of the Costs of Suboptimal Breastfeeding in the United States, which considers maternal and pediatric health costs
- Ty Ridenour's study funded by the National Institute on Drug Abuse: Substance Use Screening and Prevention for Adolescents in Pediatric Primary Care
- Lori Uscher-Pine's (RAND) study funded by the Health Resources and Services Administration (HRSA) Maternal and Child Health Bureau: Feasibility and Acceptability of Lactation Consultations Via Telehealth: A Pilot Randomized, Controlled Trial in Rural Pennsylvania
- Lisa Maillart's (University of Pittsburgh, Industrial Engineering) study funded by the National Science Foundation: Optimal Management of Donor Milk Banks
- Elizabeth Salisbury's (University of Massachusetts) multisite study funded by the National Institute on Drug Abuse: Stochastic Vibro-Tactile Stimulation: A Non-Pharmacological Intervention for Neonatal Abstinence Syndrome and Drug Withdrawal in Newborns
- Catherine Chappell's (Magee-Womens Research Institute) study funded by NICHD: A Phase I Pharmacokinetic Trial of Ledipasvir/Sofosbuvir Fixed-Dose Combination in Pregnant Women with Chronic Hepatitis C Virus Infection

RESEARCH AND MEDICAL EDUCATION TRAINING

Bogen completed five years of funding (2011–2016) from the HRSA National Research Service Award T32 Primary Medical Care Research Program to train postdoctoral fellows in primary care research. The grant was renewed in July 2016 for another five years (2016–2021). The training program prepares a diverse interprofessional group of primary health care research fellows to conduct research that significantly impacts the nation's primary health care research agenda.

The division recruited fellows for all available slots, and the fellows represent diverse backgrounds and training. All physician fellows have completed a master's degree in either clinical research or public health. Nearly all have received career-development funding to continue their research careers after fellowship, and all have remained at academic medical centers. Current fellows

are: Stacey Engster, MD (completed residency training at St. Christopher's Hospital), Jonathan Arnold, MD (completed internal medicine residency training at UPMC), Anne-Marie Rick, MD (completed residency training at the University of Colorado), and Patricia Bamwine, PhD (completed a doctoral degree in social work from the University of Pittsburgh).

Bogen serves as a research mentor for trainees at all levels, including undergraduates, medical students, residents, fellows, and junior faculty. She serves on the faculty for a number of T32 training programs.

Bogen directs the Newborn Research Support Program (NuRSERy) at MWH for the University of Pittsburgh CTSI (NIH/National Center for Advancing Translational Sciences/CTSA Grant UL1 RR024153). The NuRSERy facilitates research starting in the immediate newborn period to improve the health of the region's children.

ADVISORY COMMITTEE MEMBERSHIPS

- Patient and Family-Centered Care Lactation Experience Working Group
- Allegheny County Breastfeeding Coalition
- Vice president, Three Rivers Mothers' Milk Bank
- Scholarly mentor for CSTP student Gabriela Algarroba

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Member, Hyperbilirubinemia Clinical Practice Guideline Subcommittee
- Member, Section on Breastfeeding
- Pennsylvania Chapter, American Academy of Pediatrics Section on Breastfeeding
- Course director, Educating Physicians in Their Community: Breastfeeding Education Support and Training Program
- Academic Pediatric Association
- Member, Research Committee, Better Outcomes Through Research for Newborns
- Codirector, Fellowship Special Interest Group
- Member, Academic General Pediatric Accreditation Committee
- Member, Governance Committee, Academy of Breastfeeding Medicine
- Member, Research Committee, Organization for Teratology Information Specialists
- Member and Fellow, Academy of Breastfeeding Medicine

HONORS

- Vice chair for education, Department of Pediatrics, Children's Hospital of Pittsburgh
- *Best Doctors in America*, Woodward/White, Inc.

MAJOR LECTURESHIPS AND SEMINARS

- "Cultural Perspectives on Breastfeeding Practices: Latina, African-American, Jewish, and Muslim Context," panel presentation, International Lactation Consultant Association conference, Chicago, Ill., July 2016
- "Moving the Needle: From Initiation to Duration," Annual Breastfeeding Forum, Allegheny County Health Department, Pittsburgh, Pa., August 2016
- "Drugs in Pregnancy and Lactation," breakout session, American Association of Birth Centers Institute, Pittsburgh, Pa., September 2016
- "Reflections on Recovery: A Panel Discussion With Experts in the Field," 14th Annual Perinatal and Neonatal Health Care Conference for Nurses and Allied Health Staff, MWH, Pittsburgh, Pa., November 2016

Diego Chaves-Gnecco, MD, MPH

Diego Chaves-Gnecco is an associate professor in the University of Pittsburgh School of Medicine's Department of Pediatrics. Chaves-Gnecco is also the director and founder of the program Salud Para Niños, the first pediatric bilingual-bicultural clinic in Southwestern Pennsylvania. At Salud Para Niños, culturally and linguistically competent primary care for children and families is complemented with activities oriented toward empowerment of the community regarding its own health. Some of the activities of Salud Para Niños include weekly clinics in Oakland at the Children's Hospital PCC; two monthly free clinics; participation in organizing community fairs; a literacy program in which children are given bilingual books to promote early literacy and learning of English as a second language; translation of wellness materials into Spanish; and Hispanic/Latino car-seat checks, which emphasize car safety.

ADVISORY COMMITTEE MEMBERSHIPS

- Salud Para Niños, Children's Hospital of Pittsburgh of UPMC's first bilingual (Spanish/English), bicultural pediatric clinic
- Reach Out and Read Advisory Board, Leyendo Juntos (Reading Together)
- Board of Directors, Jewish Family and Children's Service Legal Services for Immigrants and Internationals

MAJOR LECTURESHIPS AND SEMINARS

- "Providing Social Services for an Invisible Community—Medical–Social Work Partnership—Salud Para Niños Celebrating Our 15th Anniversary: Race and Child Welfare National and Local Perspectives on Refugees, Immigrants, Human Trafficking, and Social Services," 2017 Child Welfare Summer Institute, School of Social Work, University of Pittsburgh, Pittsburgh, Pa., June 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Society for Developmental and Behavioral Pediatrics
- National Hispanic Medical Association
- American Society of Clinical Pharmacology and Therapeutics
- C.F. Reynolds Medical History Society
- Sociedad Colombiana de Pediatria
- Sociedad Colombiana de Historia de la Medicina

HONORS

- CDC Childhood Immunization Champion
- *Best Doctors in America*, Woodward/White, Inc.
- Best Doctors, *Pittsburgh Magazine*
- Clerkship Preceptor of the Year Award, University of Pittsburgh School of Medicine
- Hispanic Heritage Leadership Award, Hispanic Heritage Foundation and the National Football League
- Founding Faculty Advisor Certificate of Appreciation, Latino Student Medical Association, University of Pittsburgh School of Medicine
- Fuerza Award, Association of Latino Professionals for America, Pittsburgh, sponsored by Café con Leche
- F. Edwards Rushton Community Access to Child Health (i.e., CATCH) Award, American Academy of Pediatrics

Stacey Cook, MD, PhD

Stacey Cook is the medical director of the Complex Care Center (C3 Clinic), which officially opened in July 2017 and acts as a patient-centered medical home for children with special health care needs within the main campus of Children's Hospital of Pittsburgh. The goal of the clinic is to improve patient outcomes, increase family satisfaction, and provide an opportunity for trainees to gain experience caring for our most medically fragile children.

Cook serves as the physician leader for the GAP Medical Home Committee. The PCC is recognized as a Level III Patient-Centered Medical Home by the National Centers for Quality Assurance. Plans for the upcoming year include quality-improvement projects focusing on emergency department utilization and immunization rates in the infant population.

ADVISORY COMMITTEE MEMBERSHIPS

- Combined Ambulatory Medicine and Pediatrics Clerkship Committee, University of Pittsburgh School of Medicine

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics

HONORS

- Award for Commitment and Excellence in Service, UPMC, 2017
- 2014 Clinical Preceptor of the Year Award, University of Pittsburgh School of Medicine

Alicia Haupt, MD

Alicia Haupt is the lead faculty on the Asthma Quality Improvement Project. The project strives to create more consistency among providers with regard to

diagnosing asthma and managing asthmatic patients in the clinic. Haupt works to improve consistent acute and maintenance care of asthmatics in the PCC. Past cycles have included the use of asthma action plans and asthma-control tests during acute visits, well visits, and maintenance visits. Current cycles involve the use of spirometry in conjunction with the CHP Pulmonary Division and the use of healthy home referrals in conjunction with Gateway Health Plan and the Allegheny County Health Department. Under the direction of Haupt, future cycles will include asthma diagnosis/characterization of asthma, correct use of controller medication, flu-vaccine administration, and additional electronic medical record quality-improvement projects.

ADVISORY COMMITTEE MEMBERSHIPS

- Mentor, CORE
- Assistant editor, Children's Hospital of Pittsburgh Continuity Curriculum Committee
- Pediatric support clinician, Early Childhood Education Linkage System
- Reviewer, Oakstone Practical Reviews in Pediatrics

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics

Sanjay Lambore, MD

Sanjay Lambore is the medical director of the Turtle Creek PCC. He joined the faculty about 14 years ago after collecting a wealth of national and international teaching and clinical experience. Under his leadership, the Turtle Creek office is considered to be a stellar practice and a center for clinical and educational excellence. He is admired and adored by his patients and is an asset to the community his office serves. His teaching and clinical acumen is unparalleled, and he represents the front line of the GAP division in providing the bulk of evidence-based clinical care and teaching to medical students and residents while upholding the values and educational mission of the Department of Pediatrics and Children's Hospital of Pittsburgh of UPMC.

Lambore is considered a master clinician-educator by his peers and trainees. He has demonstrated exemplary leadership and outstanding and sustained accomplishments in academic pediatrics in the areas of teaching and patient care. Despite very busy patient and teaching loads, an objective assessment of his billed services consistently reported an audit score of 10/10. He has also consistently exceeded the 75th percentile of productivity for many years. Most recently, he has received a five-star rating on the UPMC doctor profile.

RESEARCH

This year, Lambore was selected as a recipient of the Drue Heinz British Pediatric Fellowship, which will enable him to work as a physician observer at the Royal Manchester Children's Hospital in Manchester, United Kingdom. The purpose of the fellowship is the exchange of ideas between the United Kingdom consultants and trainee doctors with regard to managing various pediatric medical issues. He was also invited to present grand rounds in the Department of Pediatrics at Royal Manchester Children's Hospital on his research interest, "Sudden Cardiac Death in Adolescent Athletes."

Lambore has collaborated with faculty members within GAP on various research projects. His efforts and research interest in sudden cardiac death in adolescent athletes resulted in the adoption of a clinical questionnaire for sports physicals at the PCCs in Oakland and Turtle Creek to detect risk factors for sudden cardiac death in adolescents.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Academy of Medical Educators
- Alpha Omega Alpha Honor Medical Society, University of Pittsburgh

HONORS

- Clerkship Preceptor of the Year Award, University of Pittsburgh School of Medicine, 2016
- William I. Cohen Teacher of the Year Award, University of Pittsburgh School of Medicine
- Award for Commitment and Excellence in Service, UPMC

Ana Malinow, MD, MSHCPM

Ana Malinow has focused her energies on the use of geographic information systems to investigate missed appointments. She completed work on focus groups with funding from the American Academy of Pediatrics Community Access to Child Heath and subsequently received funding from the Beckwith Institute to start a satellite clinic in the Homewood neighborhood of Pittsburgh, a neighborhood with a high no-show rate. The clinic is in the Homewood-Brushton Family Support Center, which works to improve parenting and life skills for parents and school readiness for children. The clinic will allow a second-year pediatric resident to have a continuity clinic there. Malinow is collaborating with the University of

Pittsburgh School of Nursing to offer a community elective at the Homewood clinic for senior nursing students. A community health worker has been hired to ensure the best show rate possible. Malinow is collaborating with the Wolff Center to track metrics in the new clinic.

Malinow has collaborated with several individuals outside pediatrics to author "Transportation Characteristics Associated With Non-Arrivals to Pediatric Clinic Appointments: A Retrospective Analysis of 51,580 Scheduled Visits," which has been accepted for publication in *BMJ Quality and Safety*.

Clinical Effectiveness Committee. Malinow has served for two years as co-chair for this multidisciplinary committee, which addresses issues related to clinical efficiency, improvements in clinical care, and access to care. Along with Hannibal, she has implemented important enhancements, including a focus on retention of staff by developing "Common Grounds," a support group for staff that meets monthly. The committee is currently redesigning the primary care rotation. She continues to use the process of design thinking to enhance group productivity and creativity.

ADVISORY COMMITTEE MEMBERSHIPS

- Codirector, Clinical Effectiveness Committee, Primary Care Clinic, Children's Hospital of Pittsburgh of UPMC
- Co-editor, Pediatric Resident Continuity Clinic Curriculum, Children's Hospital of Pittsburgh of UPMC

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- American Pediatric Association
- Board member, Physicians for a National Health Program

AWARDS

- Best Doctors, *Pittsburgh Magazine*, 2017

Judith M. Martin, MD

RESEARCH

Judith Martin is a clinical researcher who works collaboratively to design clinical studies, then recruit, enroll, and follow participants. She serves as the director of the GAP Clinical Trials Unit. The long-term goal of the unit is to evaluate frequently performed pediatric treatments and procedures that entail substantial use of health care resources but whose value has not been substantiated. The studies evaluate the prevalence, diagnosis, treatment, and follow-up of children with a variety of common outpatient infections, including UTI, pneumonia, sinusitis, and AOM. She also assists with data analysis, manuscript preparation, and grant proposals related to those activities. In addition, she continues her investigations of respiratory tract infections in children, including the epidemiology of infections caused by *Streptococcus pyogenes*. She has received funding in the past from the American Heart Association and the NIH for clinical studies of streptococcal pharyngitis in a local school setting, the results of which were published in the *New England Journal of Medicine* and *Pediatrics*. Other notable work has included a review of the past 20 years of experience at Children's Hospital of Pittsburgh of UPMC regarding acute rheumatic fever, published in the *Journal of Pediatrics*.

Martin has directed numerous clinical studies, including a study of ethanol locks to prevent catheter-related bloodstream infections, a study of children with acute rheumatic fever, and studies describing trends in nasopharyngeal colonization in children with AOM. She has been working with Rick Zimmerman and the CDC on studies of the immune response to the influenza vaccine. The work was published recently and contributed to the recommendation that the nasal influenza vaccine (LAIV-live attenuated influenza vaccine) should no longer be used for immunization. She also serves as a member of the Children's Hospital of Pittsburgh Research Advisory Committee, the UPMC Technology and Innovative Practice Committee, and the Institutional Review Board (IRB) for the University of Pittsburgh. She has served as vice chair of the IRB in the Human Research Protection Office since 2013 and oversees two committee meetings per month. She is involved in educating and mentoring students, residents, and fellows and is a member of several scholarship oversight committees.

The following list represents Martin's grant submissions.

- A Randomized Trial to Evaluate Short-Course Versus Standard-Course Outpatient Beta-Lactam Antimicrobial Therapy of Community-Acquired Pneumonia in Children (SCOUT-CAP); submitted June 2015, funded August 2015, recruitment November 2016; site principal investigator (PI)
- Child Care Knowledge and Readiness for Seasonal and Pandemic Influenza, American Academy of Pediatrics and CDC Survey of Influenza Immunization Practices in Daycare Providers; submitted May 2015, funded September 2015; site co-investigator; study completed and manuscript published
- National Vaccine Surveillance Network, Enhanced Active Surveillance of Pediatric Infectious Diseases and Vaccines and Enhanced Active Surveillance of Pediatric

Respiratory Infections and Vaccine Preventable Diseases; PI: John Williams, MD; Martin sub-investigator for healthy control participants
- CDC, Option C Child and Adolescent, Pittsburgh: Immunological Response to Influenza Vaccination in Different Demographic Groups; submitted and renewed summer 2016; PI: Rick Zimmerman, MD; Martin sub-investigator for healthy control subjects
- Immune Pathways Associated With Influenza Vaccines, CTSI Translational Research Pilot Awards (T3/T4 clinical practice and population health); awarded March 2017, funding through March 2018; PI: Martin
- Efficacy of Tympanostomy Tubes for Children With Recurrent AOM Site Protocol, Tympanostomy Tubes Grant; NIDCD; submitted fall 2013 and resubmitted fall 2014, funded July 2015, recruitment ongoing; PI: Hoberman
- Efficacy of Antibiotics in Children With Acute Sinusitis: Which Subgroups Benefit? NIAID; submitted May/September 2014, funded June 2015, recruitment ongoing; PI: Shaikh

Martin is involved with the following funded clinical studies.
- A Phase 2b, Randomized, Double-Blind, Placebo-Controlled Study to Evaluate the Safety and Efficacy of MEDI8897, a Monoclonal Antibody With an Extended Half-Life Against Respiratory Syncytial Virus, in Healthy Preterm Infants; PI: Martin
- Efficacy of Tympanostomy Tubes for Children With Recurrent AOM, Site Protocol, Tympanostomy Tubes; PI: Hoberman
- Efficacy of Antibiotics in Children With Acute Sinusitis: Which Subgroups Benefit? PI: Shaikh
- 1R01 DK087870-01: Corticosteroids for Children With Febrile UTIs; PI: Shaikh
- CHP Research Advisory Council: Urinary Biomarkers for Pyelonephritis; PI: Shaikh
- BAA-NIAID-DMID-NIHAI2008025: The SCOUT Study: Short-Course Therapy for UTIs in Children; PI: Zaoutis, site PI: Hoberman
- CDC, Option C Child and Adolescent, Pittsburgh: Immunological Response to Influenza Vaccination in Children and Adolescents When No Change in Vaccine Strain Antigens; PI: Zimmerman

Other ongoing projects that Martin is involved with include the following.
- Center for Excellence in Pediatric and Adolescent Health Research (development of Web site)
- Quantitation of Sialic Acid in Individual Salivary Samples; project with saliva samples from the Falk School Streptococcal Study, Patti Ryan, PhD, Rockefeller University in New York

ADVISORY COMMITTEE MEMBERSHIPS
- IRB, University of Pittsburgh
- Vice chair, IRB, University of Pittsburgh
- Technology and Innovative Practice Committee, UPMC
- Codirector, Center for Excellence in Adolescent and Child Health Research
- Research Advisory Committee, Children's Hospital of Pittsburgh of UPMC
- Scholarship Oversight Committee, University of Pittsburgh School of Medicine
- Faculty Mentor Committee, University of Pittsburgh School of Medicine

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS
- Public Responsibility in Medicine and Research
- Society for Pediatric Research
- Lancefield Society
- Infectious Diseases Society of America
- Pediatric Infectious Diseases Society
- American Academy of Pediatrics
- Alpha Omega Alpha Honor Medical Society

Deborah Moss, MD, MPH

SCHOLARLY AND ADVOCACY ACTIVITIES
This year, Deborah Moss continued her work on public and population health issues. Along with her team at UPMC Health Plan, she published findings from a Medicaid payment reform project in the *Journal of Healthcare Quality* in an article titled "Advancing Value-Based Population Health Management Through Payer-Provider Partnerships: Improving Outcomes for Children With Complex Conditions." Moss contributes to educating practicing providers on public health topics such as oral health access and practices and pediatricians' role in addressing the opioid epidemic. She is also leading a project to prevent childhood obesity at UPMC Health Plan as part of a Robert Wood Johnson Foundation–funded national initiative run by the Association of Community Affiliated Plans to address childhood obesity in the Medicaid population.

Moss has extended her public health focus through advocacy activities and has provided testimony for public hearings related to prohibition of e-cigarettes and vaping products in indoor public spaces as well as

legalization of medical marijuana, written letters of support for policies on universal lead screening and the expansion of donor milk use, and advocated in numerous ways to support the continuation of Medicaid and CHIP insurance services for children. Most recently, she was appointed by the county executive to serve on the Allegheny County Lead Task Force to develop best-practice recommendations for addressing lead exposure in the county.

From a teaching perspective, she began mentoring a second-year medical student on her long-term scholarly research project, "Assessing Factors Related to Successful Transition of Care from Pediatric to Young Adult Sickle Cell Disease." She continues teaching a variety of courses at the medical school, including Population Health and Behavioral Health for second-year medical students, Combined Ambulatory Medicine and Pediatrics for third-year students, and a mini-course on health care reform called Practicing Medicine: What Awaits You for fourth-year medical students.

ADVISORY COMMITTEE MEMBERSHIPS

- Allegheny County Health Department Dental Task Force
- Allegheny County Lead Task Force
- Connections4Health Advisory Board
- IRB, University of Pittsburgh
- Healthy Teeth Healthy Children program, Pennsylvania American Academy of Pediatrics
- President-elect, Pennsylvania American Academy of Pediatrics
- Treasurer, Medical Executive Committee

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Vice president of the Pennsylvania chapter, American Academy of Pediatrics
- Academic Pediatric Association

MAJOR LECTURESHIPS AND SEMINARS

- "Limiting Screen Time," 5-2-1-0 program, Phipps Conservatory and Botanical Gardens, Pittsburgh, Pa.

HONORS

- Best Doctors, *Pittsburgh Magazine*, 2017
- *Best Doctors in America*, Woodward/White, Inc., 2017

Gysella Muñiz, MD

RESEARCH

Gysella Muñiz is an assistant professor in the University of Pittsburgh School of Medicine, Department of Pediatrics. Her research interests are focused on the diagnosis and treatment of common pediatric infections, including AOM, UTI, sinusitis, and pneumonia. She also assists with data analysis, manuscript preparation, and grant proposals related to those activities.

Muñiz is spearheading a multicenter study evaluating the ability to diagnose constipation based on scout x-ray associated with performance of a voiding cystourethrography obtained as part of the RIVUR study. The study looks at correlation between UTI diagnosis and the degree of constipation.

Muñiz is leading a study looking at the importance of renal contour change in the diagnosis of UTIs. Renal function and contour change with age. The goal of the study is to determine whether contour change and the diagnosis of renal scarring are correlated, thus whether changes in renal contour are required to diagnose renal scarring.

In close collaboration with Shaikh and medical student T. Elliott, Muñiz works on a study titled "Meta-Analysis to Predict the Risk of High-Grade VUR in Children With First Febrile UTI." The aim is to add data on procalcitonin, age, gender, C-reactive protein, and family history to the already established renal bladder ultrasound in order to create a prediction rule for high-grade VUR in children with first febrile UTI with sensitivity and specificity of 80%.

She is also involved with the following funded clinical studies.

- A Phase 2b, Randomized, Double-Blind, Placebo-Controlled Study to Evaluate the Safety and Efficacy of MEDI8897, a Monoclonal Antibody With an Extended Half-Life Against Respiratory Syncytial Virus, in Healthy Preterm Infants; PI: Martin
- Efficacy of Tympanostomy Tubes for Children With Recurrent AOM, Site Protocol, Tympanostomy Tubes; PI: Hoberman
- Efficacy of Antibiotics in Children With Acute Sinusitis: Which Subgroups Benefit? PI: Shaikh
- 1R01 DK087870-01: Corticosteroids for Children With Febrile UTIs; PI: Shaikh
- CHP Research Advisory Council: Urinary Biomarkers for Pyelonephritis; PI: Shaikh
- BAA-NIAID-DMID-NIHAI2008025: The SCOUT Study: Short-Course Therapy for UTIs in Children; PI: Zaoutis, site PI: Hoberman

- CDC, Option C Child and Adolescent, Pittsburgh: Immunological Response to Influenza Vaccination in Children and Adolescents When No Change in Vaccine Strain Antigens; PI: Zimmerman

In the global health field, Muñiz is the leader of the Remote Village Project in rural Honduras. The project is a sustainable initiative that trains 10–15 community health workers and equips three clinics in isolated mountainous areas to deliver year-round treatment for common health issues like diarrhea, bronchitis, and parasites. The project also provides clean water, smokeless stoves, sanitation, and general health education.

ADVISORY COMMITTEE MEMBERSHIPS

- Assistant editor, Children's Hospital of Pittsburgh Pediatric Residency Program Continuity Curriculum Committee
- Assistant editor, Children's Hospital of Pittsburgh's *Guide to Care for Your Newborn*, Spanish version board member, Hope for Honduras/Virginia Hospital Center Medical Brigade
- Chair, Remote Village Project, Hope for Honduras/ Virginia Hospital Center Medical Brigade

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Peruvian Medical College

MAJOR LECTURESHIPS AND SEMINARS

- "Hope for Honduras: A Model of Sustainable Community Development for Global Health Non-Governmental Organizations," University of Pittsburgh Health Sciences Diversity 2016–2017 Speaker Series, Pittsburgh, Pa., September 2016

Jack L. Paradise, MD

RESEARCH

Jack Paradise continued to consult with faculty in the division concerning their research efforts. He formally retired in September 2005 but remains involved in reporting findings of research studies, submitting novel NIH applications, and serving as scientific advisor and mentor to multiple research endeavors.

ADVISORY COMMITTEE MEMBERSHIPS

- Data and Safety Monitoring Board, National Institute on Deafness and Other Communication Disorders, NICHD, Xylitol Syrup for the Prevention of AOM in Otitis-Prone Children
- Reviewer, Clinical Practice Guideline and Technical Report on Diagnosis and Management of AOM, American Academy of Pediatrics Subcommittee on Diagnosis and Management of AOM

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Public Health Association
- Johns Hopkins Medical and Surgical Association
- American Academy of Pediatrics
- Section on Developmental and Behavioral Pediatrics, American Academy of Pediatrics
- American Cleft Palate Association
- Academic Pediatric Association
- Society for Ear, Nose, and Throat Advances in Children
- American Pediatric Society
- Fellow, American Association for the Advancement of Science

Kumaravel Rajakumar, MD, MS

RESEARCH

Kumaravel Rajakumar's clinical research is focused on vitamin D nutrition in children, with an emphasis on minority health and health disparities, and it is currently funded by an NIH/National Heart, Lung, and Blood Institute R01 grant (R01HL112985, 2013–2018). Rajakumar has received extramural funding support from NIH since 2006 (NIH/NICHD R03 [R03HD053479, 2006–2009] and NIH/NICHD K23 [K23HD052550, 2007–2013]), as well as intramural funding support from Children's Hospital of Pittsburgh's Research Advisory Committee (2008–2010, 2016–2018). In the context of his vitamin D research, he has also been exploring the racial differences in parental trust and their implications for research enrollment of children, as well as other factors pertaining to research participation in children.

Vitamin D and Vascular Function in Obese Children (R01HL112985, 2013–2018). The research goals of this multidisciplinary clinical trial are: (a) to determine the efficacy of enhanced vitamin D3 supplementation in improving vascular endothelial function, arterial stiffness, insulin sensitivity, and metabolic syndrome risk status in obese and overweight vitamin D–deficient 10- to 18-year-old children; and (b) to examine the vitamin D supplementation–induced effects on adipokines and inflammatory markers relevant for cardiovascular disease risk. This study is funded by an R01 grant from the NIH/National Heart, Lung, and Blood Institute (2013–2018).

A total of 229–252 eligible children will be randomized to 600 IU versus 1,000 IU versus 2,000 IU of vitamin D3 once daily for six months. Vascular function assessments and other study outcomes will be completed at randomization and at the three- and six-month follow-up visits. The clinical trial was opened for enrollment on August 19, 2013. As of October 9, 2017, a total of 1,542 children had been assessed for eligibility, 609 of the 986 eligible children had been screened for participation, and 219 of them had been randomized.

ADVISORY COMMITTEE MEMBERSHIPS

- Research Committee, Division of GAP, 2006 to the present
- Center for Excellence in Child and Adolescent Health Research, Division of GAP, 2014 to the present

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Fellow, American Academy of Pediatrics
- American Society for Nutrition
- Society for Pediatric Research
- Endocrine Society
- Editorial Board, *Metabolism—Clinical and Experimental*
- Editorial Board, *Frontiers in Child Health and Human Development*

Kristin Ray, MD, MS

RESEARCH

Kristin Ray joined the faculty of the Division of GAP in 2014 after completing her GAP fellowship at the Children's Hospital of Pittsburgh of UPMC. Ray's research is currently funded by the NICHD. Her overall research goal is to develop and implement strategies to improve the efficiency, equability, and value of pediatric health care systems. Using qualitative and quantitative methods, recent projects have examined family perspectives on the use of telemedicine for pediatric subspecialty care and have documented the growing role nationally of nurse practitioners and physician assistants in the provision of outpatient specialty care. Ray is the director of health systems improvement for Children's Community Pediatrics and is also affiliated faculty within the University of Pittsburgh Health Policy Institute.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Academic Pediatric Association
- Academy Health

HONORS

- CHP Scholar
- CHP Department of Pediatrics Endowed Instructorship

Evelyn Reis, MD

RESEARCH

CTSI Pediatric PittNet. Evelyn Reis serves as the medical director for the University of Pittsburgh CTSI pediatric PBRN, Pediatric PittNet. The network strives to facilitate the translation of pediatric research discoveries into practice to improve the health of the region's children. Pediatric PittNet consolidates and builds on previous physical and mental health research networks that have successfully supported federally and industry-sponsored clinical research studies based in local pediatric primary care practices over the preceding decade. To meet the goal of effective translation, Pediatric PittNet promotes collaboration between pediatric clinical investigators and practitioners, as well as collaboration among PBRNs.

Pediatric PittNet currently includes more than 50 research protocols covering a range of common childhood behavioral health issues (e.g., attention-deficit hyperactivity disorder, anxiety) and physical health concerns (e.g., ear infections, UTIs, vaccine effectiveness). Numerous large-scale clinical trials are conducted in Pediatric PittNet practices each year. From July 2007 to June 2016, more than 4,800 children were enrolled in Pediatric PittNet studies. During fiscal year 2017, 595 participants were enrolled. New studies and investigators are added regularly to the research network, which spans the University of Pittsburgh School of Medicine; the departments of Pediatrics, Psychiatry, Psychology, Neurobiology, Family Medicine, and Obstetrics/Gynecology; the School of Nursing; and the Graduate School of Public Health.

Pediatric PittNet member practices include 24 Children's Community Pediatrics practices, an independent pediatric practice, a federally qualified community health center, the Children's Hospital PCCs (including Oakland and Turtle Creek), and the Children's Hospital Center for Adolescent and Young Adult Health. Together, the 29 practices include 50 office sites in six counties in Western Pennsylvania. Approximately 200 Pediatric PittNet providers serve more than 240,000 privately and publicly insured patients, ages birth to 21 years, in urban, suburban, and rural settings.

Pediatric PittNet actively disseminates research opportunities and findings to providers, staff, and parents in the practice community. Outreach to parents is conducted through original videos and slides on eMessage boards in practice waiting rooms, screen-saver messages on exam room monitors, and posts on the Children's Hospital blog.

Outreach to providers and practice staff is conducted through lunchtime teleconferences; on-site practice visits with investigators; our network newsletter, "The Paw"; and regular email updates, including a featured study of the month.

University of Pittsburgh CTSI. The University of Pittsburgh CTSI serves as the integrative academic home for clinical and translational scientists across Health Sciences, Carnegie Mellon University, UPMC, and the region. CTSI's primary focus is to develop, nurture, and support a cadre of clinical and translational scientists by building on the University's existing clinical research training programs (K30, Roadmap K12). Through integration and innovation, CTSI fosters development of new biomedical knowledge and the translation of that knowledge from the basic and preclinical research settings to individuals, communities, and health practices.

Influenza Surveillance and Vaccine Effectiveness in a Large, Diverse Network. Reis serves as a co-investigator for this high-impact, CDC-funded, national study. It aims to estimate annual seasonal influenza vaccine immunogenicity and effectiveness. Pittsburgh, one of only five participating sites, completed the sixth of its 10 funded years of enrollment. Results led to the CDC recommendation against intranasal vaccine FluMist due to ineffectiveness.

An Enhanced 4 Pillars Toolkit for Increasing Adolescent Immunization: A Randomized, Controlled Cluster Trial in Pediatrics and Family Medicine. Reis serves as a co-investigator for this practice-improvement study in Pediatric PittNet, which focuses on human papillomavirus and influenza vaccination.

Balint Group Experience for Pediatric Residents. Reis is the PI for an annual survey study of Children's Hospital residents to assess their perceived impact of the Balint group experience. Survey findings have revealed that Balint participation enhances perceived professional development and stress management among pediatric residents.

Center for Excellence in Child and Adolescent Research. Reis is a member of this design team, which aims to disseminate high-quality, effective research practices to a wide national audience of children/adolescents, parents, pediatricians, and investigators.

MEDICAL EDUCATION LEADERSHIP

Pediatric Resident Balint Group. Reis is the co-leader of the CHP resident Balint group, a weekly, facilitated case discussion about the doctor-patient relationship. The goals of the experience are to build empathy among residents and to sustain them through the stress of training. Reis conducts the annual resident evaluation of the Balint group experience and has led national presentations on the novel approach of block scheduling of Balint groups.

Pediatric Resident Communication Course. Reis codirects this course, in which residents practice communication skills with simulated patients. She has led national workshops on this experiential skills training.

Pediatric Resident Continuity Clinic Curriculum Committee. Reis is the co-editor of this case-based, biweekly online curriculum, which guides the primary care education of all residents and preceptors, both academic and community based.

Medical Interviewing. Reis has cofacilitated small-group, experiential learning for MS-1 students in the introductory Medical Interviewing course, focusing on the content of the medical interview and communication skills.

Faculty and Students Together (FAST). Reis serves as a leader and faculty advisor of the School of Medicine's FAST groups, which support MS-1 through MS-4 students through both faculty and near-peer mentoring.

ADVISORY COMMITTEE MEMBERSHIPS

- Faculty member, Pediatric and Adolescent Medicine Interest Group, University of Pittsburgh School of Medicine
- Faculty member, Student Wellness Committee, University of Pittsburgh School of Medicine

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Academic Pediatric Association
- University of Pittsburgh School of Medicine Academy of Master Educators
- National Council member and co-chair of the Scholarship Committee, American Balint Society

HONORS

- Top 10 Teaching Faculty, Children's Hospital of Pittsburgh of UPMC
- *Best Doctors in America*, Woodward/White, Inc.
- Best Doctors, *Pittsburgh Magazine*

MAJOR LECTURESHIPS AND SEMINARS

- "A Bolus of Balint: Is Block Scheduling of Balint Groups Effective for Pediatric Residents?" Second National Meeting of the American Balint Society, Oak Brook, Ill., July 2016

Nader Shaikh, MD, MPH

RESEARCH

NIAID Clinical Trial (U01) Evaluating the Efficacy of Antibiotics in Children With Acute Sinusitis. This is a multicenter, randomized trial (five years, $4.9 million) in children meeting clinical criteria for acute sinusitis. The primary aim of the study will be to determine whether some subgroups of children do not benefit from treatment with antibiotics. Enrollment started in February 2015, and 190 children have been enrolled to date. Other sites participating are: Kentucky Pediatrics and Adult Research in Bardstown, Ky.; American Family Children's Hospital in Madison, Wis.; Legacy Pediatrics in Rochester, N.Y.; and General Pediatric and Adolescent Care in Morgantown, W.Va.

NIDDK-Sponsored Clinical Trial (R01) Evaluating the Efficacy of Adjuvant Dexamethasone in Reducing Renal Scarring in Children With Febrile UTI. This randomized, double-blind, placebo-controlled trial (five years, $3.1 million) aims to determine the effects of three days of daily adjuvant dexamethasone on the incidence of renal scarring six months after a first febrile UTI and to determine whether previously identified markers of renal parenchymal involvement, along with clinical information, can accurately predict which children willdevelop renal scarring. Apart from Pittsburgh, children were enrolled from Nationwide Children's Hospital in Columbus, Ohio; the American Family Children's Hospital, which is a children's hospital within the University of Wisconsin system; and Hasbro Children's Hospital in Rhode Island. The study is in its fifth year. Enrollment was scheduled to finish in March 2018. 250 children have been enrolled to date.

Data Analysis on a Study (R21) Sponsored by the NIDDK Aimed at Finding Biomarkers for Acute Pyelonephritis. This study is examining whether blood and urine tests at the time of diagnosis can accurately differentiate children with pyelonephritis from children with cystitis. Transcriptomics was performed with high-throughput methods. Samples were also tested for proteins involved in the immune response to UTI. The study enrolled 202 patients, and data are being analyzed.

SCOUT Study. The SCOUT study is a multicenter, double-blind, placebo-controlled non-inferiority clinical trial of 746 children ages 2 months to 10 years who will be enrolled over four years. As a co-investigator, Shaikh has been enrolling patients.

Additional Data Analysis on the NIAID-Sponsored, Randomized, Placebo-Controlled Study in Young Children With AOM Leading to a Revised AOM-Severity of Symptoms Scale (AOM-SOS v. 5.0). An FDA guidance document for clinical trials in children with AOM has recommended the use of time-to-resolution of symptoms as the primary outcome when evaluating the efficacy of antimicrobial treatment. Shaikh, Hoberman, and Paradise conducted additional analyses of data collected in the AOM placebo-controlled trial that led to a revision of the AOM-SOS scale and the MICD to increase its sensitivity to detect change. The statistical analyses discriminated between two treatment groups, rather than between treatment and placebo. A document outlining the analyses and proposed revision to the AOM-SOS scale was submitted to the Division of Antimicrobials, Center for Drug Effectiveness Research, FDA. Three manuscripts were published.

Additional Data Analysis on the NIAID-Sponsored Observational Study of Children With Acute Sinusitis Aimed at Development of a Symptom Severity Scale. Through several rounds of studies, the team has developed a Pediatric Rhinosinusitis Symptoms Scale (PRSS). The data support the use of the PRSS as a measure of change in symptom burden in clinical trials of children with acute sinusitis. A manuscript is being prepared.

Additional Data Analysis on the NIDDK-Sponsored, Randomized, Placebo-Controlled Study in Children With VUR (RIVUR) Examining the Association Between Delay in Antimicrobial Treatment and Treatment Delay. As published in *JAMA Pediatrics*, delayed treatment was associated with increased risk of renal scarring.

Investigation Examining the Association Between the Presence of Pyuria and Results of Urine Culture. The team used data on 46,158 children evaluated at the Children's Hospital of Pittsburgh emergency department between 2007 and 2013 who had symptoms of UTI and paired urinalysis and urine cultures. As published in *Pediatrics*, uropathogens are less likely to be associated with pyuria in symptomatic children. The study provides a biological explanation for the relatively low sensitivity of pyuria for the diagnosis of UTI and suggests that more accurate biomarkers are needed for screening.

Two Studies Comparing Diagnosis of AOM in Experts Versus Pediatricians and Trainees. The first study showed that bulging of the tympanic membrane (TM) was the main finding that otoscopists used to discriminate AOM from OME; information regarding the presence or absence of other signs and symptoms added little to the diagnostic

process. An algorithm using bulging and opacification of the TM correctly classified 99% of ears in an independent dataset. The second study tested the hypothesis that difficulties in diagnosis were related to differences in the relative importance placed on redness and bulging of the TM in the diagnostic process. The paper reported that experts and non-experts differed in their interpretation of redness of the TM; experts placed much lower importance on redness compared to bulging of the TM.

UpToDate. In collaboration with Hoberman, Shaikh continued to contribute to UpToDate in the area of UTI in children, with cards on clinical features and diagnosis, epidemiology and risk factors, management, imaging and prognosis, and long-term management and prevention of UTIs in children, all of which are widely accessed and require frequent revisions.

Videos in Clinical Medicine. Shaikh was the first author on two *New England Journal of Medicine* videos in clinical medicine. The first video describes the steps involved in tympanocentesis in young children with AOM in the outpatient setting. The second video, titled "Diagnosis AOM," focuses on demonstrating the proper technique for pneumatic otoscopy and cerumen removal. A third video was produced in collaboration with Jennifer Marin, Robert Hickey, Steven Docimo, and Hoberman on suprapubic aspiration of the bladder.

ADVISORY COMMITTEE MEMBERSHIPS

- Sinusitis Guideline Committee, American Academy of Pediatrics
- IRB, University of Pittsburgh

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Academic Pediatric Association
- American Academy of Pediatrics
- Society for Pediatric Research

Timothy R. Shope, MD, MPH

RESEARCH

Timothy Shope's research focuses on the diagnosis and treatment of common pediatric infectious diseases. With colleagues, Shope completed a five-year, NIH-funded study, "Targeted Interventions to Reduce Antimicrobial Resistance to Compare Short- Versus Standard-Duration Therapy in Children 6–23 Months with AOM," which was published in the *New England Journal of Medicine*. He actively enrolled patients in five ongoing federally funded protocols: "Steroids to Actively Reduce Renal Scarring," "Efficacy of Tympanostomy Tubes for Children With Recurrent Otitis Media," "Short-Course Antimicrobial Treatment of UTI," "Short-Course Antimicrobial Treatment of Pneumonia," and "Efficacy of Antibiotics in Children With Acute Sinusitis: Which Subgroups Benefit?"

In addition, Shope is working as a sub-investigator on private-industry studies in the following areas.

Improving the Diagnosis of AOM: The Light Field Otoscope for Diagnosis of AOM (Ricoh). This study involves a three-dimensional technology using an algorithm to aid in diagnosis of AOM. In collaboration with Carnegie Mellon University investigators, he is working on a hand-held cell-phone capture of images of the TM and using image capture to develop an algorithm for enhanced diagnosis of AOM.

Prevention of RSV Infection. Funded by Medimmune, this is a phase IIB, randomized, double-blind, placebo-controlled study to evaluate the safety and efficacy of MED19997, a monoclonal antibody with an extended half-life against respiratory syncytial virus in healthy preterm infants.

Shope is funded by the American Academy of Pediatrics and CDC to do national work on influenza preparedness in early education and child care settings. This ongoing project resulted in a publication in *Pediatrics*. He is evaluating seasonal influenza vaccine requirements for licensed child care centers in the United States.

Shope completed a randomized trial of different methods of cerumen removal in young children, and manuscript preparation is under way. This is the first study in young children evaluating the efficacy of irrigation methods, which do not require the presence of a physician.

Shope also completed a project examining emergency department clinicians' adherence to UTI testing guideline recommendations—the first study of its kind since revision of the American Academy of Pediatrics UTI guideline in 2011. He is also working closely with Shaikh on developing and publishing an online prediction rule for determining when to test and treat febrile young children with possible UTIs.

ADVISORY COMMITTEE MEMBERSHIPS

- National Advisory Committee, National Resource Center for Health and Safety in Child Care and Early Education, 2011 to the present

- Pediatric Residency Intern Selection Committee, Children's Hospital of Pittsburgh of UPMC, 2013 to the present

MAJOR LECTURESHIPS AND SEMINARS

- "Preparing Head Start/Child Care and Communities for Seasonal Influenza," hosted by the American Academy of Pediatrics and the National Center on Early Childhood Health and Wellness, November 2016
- "Who's in and Who Snot?: Exclusion Criteria for Early Education and Child Care Settings," grand rounds, Children's Hospital of Pittsburgh, Pittsburgh, Pa., July 2017

Katherine Watson, DO

Katherine Watson is an associate program director for the pediatrics residency. In that role, she focuses on making resident-focused program improvements and increasing community pediatrician outreach. Watson has increased primary care educational opportunities for residents by coordinating biannual GAP teaching sessions for residents who complete their continuity clinic in a community office.

Watson is partnering with Community Health and Health Improvement Partnerships to create a new rotation for first-year residents, encompassing primary care and community engagement. The rotation will provide educational opportunities on the Care Mobile, during home visits, and within the Pittsburgh Public Schools and Pittsburgh Parks Conservancy.

Watson is leading the CHP Resident and Fellow Well-Being Taskforce and also serves on the UPMC Graduate Medical Education WELL committee. Both groups are working to provide structured educational opportunities for residents to increase physician well-being and decrease burnout.

Clinically, Watson continues to work to establish HH4L as a sustainable clinic model at the PCC in Oakland. She has launched research initiatives relating to lifestyle modification and increased activity for patients. Watson works with CHP residents in the HH4L clinic during their continuity rotation. The goal for the experience is improvement in their motivational interviewing skills and increased medical knowledge about childhood obesity.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Fellow, American Academy of Pediatrics
- Academy of Pediatric Program Directors

HONORS

- Clerkship Preceptor of the Year, University of Pittsburgh School of Medicine
- Top Ten Educators at Children's Hospital of Pittsburgh

SERVICE

- Clinical lead, Let's Move Pittsburgh Clinical Advisory Team, Parks Rx

Jennifer Zarit, MD

Jennifer Zarit is the primary teaching attending for the GAP well newborn service at MWH, which trains medical students from the University of Pittsburgh School of Medicine and residents from the Children's Hospital Pediatric Residency Program and the combined Family Medicine Programs from the UPMC Shadyside, McKeesport, and St. Margaret hospitals. In addition, she is focused on breastfeeding support and education.

Lactation Elective Course Director. Zarit is the director for the lactation elective for medical students, pediatric residents, and family medicine residents. It is a two- to four-week elective with academic and hands-on learning to assist breastfeeding dyads.

ADVISORY COMMITTEE MEMBERSHIPS

- Newborn Committee, MWH of UPMC
- Clinical Effectiveness Committee, Division of GAP, Children's Hospital of Pittsburgh of UPMC
- Allegheny County Breastfeeding Coalition
- Magee Newborn Hospitalist Committee

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Academy of Breastfeeding Medicine
- International Lactation Consultant Association
- Academic Pediatric Association

TEACHING ACTIVITIES

The Division of GAP plays a considerable role in the education and training of medical students, residents (pediatrics, medicine-pediatrics, triple-board, and family medicine), fellows, and faculty. The GAP teaching program is a product of set division goals, including:

- High-quality, standard curricula for learners of all levels
- Faculty development, with emphasis on attaining a critical-mass interest in improving medical knowledge and skills
- Ongoing curriculum development by division members

The goals are inspired by the Accreditation Council for Graduate Medical Education Outcomes Project, adult-learning theory, the importance of evidence-based medicine, and the experience of master educators.

CURRICULUM DEVELOPMENT AND ONLINE OFFERINGS

Because trainees are placed in a wide variety of ambulatory care settings, the division ensures that all trainees have access to standard learning experiences regardless of their clinical home base. Thus, GAP has developed a wide range of formal primary care curricula in more than 45 topics to accompany clinical experiences. The curricula are available online via a dedicated pediatric education Web site. Oversight and editing of the three-year online curriculum are conducted by a faculty team composed of Watson (primary care education director), Reis, Haupt, and Muñiz. Administration of the Web site at the division level allows the division to track and support users, generate reports, and quickly update curricula. The Web site hosts more than 90 offerings within six major topic areas. It is used primarily by CHP of UPMC trainees, but specific curricula also are available to other institutions and the public at large.

At CHP, online offerings accompany the critical supervised hands-on experiences that all trainees receive. In addition, the modules serve as continuing medical education faculty-development opportunities and are made available to all faculty in the division as well as the more than 40 community pediatricians who supervise a subset of residents for their continuity clinic experience.

PRIMARY CARE TRACK (PALS)

Children's Hospital initially received a $1.9 million, five-year grant from HRSA to develop a primary care track in the residency program to prepare pediatricians to provide effective, compassionate, culturally sensitive care to children from underserved urban and rural settings. The program recruits two residents every year, and 75% of PALS graduates practice primary care in a medically underserved area. Watson is establishing a leadership curriculum for PALS residents to be completed as a longitudinal experience.

PRIMARY CARE INTEREST GROUP

This resident-led group is composed of 10–15 individuals every year who are interested in pursuing a career in primary care. The group meets four to six times per year at a faculty member's home for dinner. The goals are to promote collegiality, mentorship, and career guidance and to allow discussion of primary care topics. Topics this year included a scholarly talk by a resident on atopic dermatitis and a panel of community pediatricians on career options. The group has also developed a primary care mentor resource for residents with questions about a career in primary care.

MEDICAL STUDENT TEACHING

GAP faculty members serve in leadership roles and actively participate in teaching medical students. In the preclinical years, faculty members educate students in courses such as Medical Interviewing and Advanced Physical Examination. In the clinical years, Biller directs the Combined Ambulatory Medicine and Pediatrics Clerkship, an eight-week exposure to outpatient medicine. In that course, GAP faculty members have developed and lead didactic sessions for third- and fourth-year medical students. Biller also is a facilitator for the weekly ethics sessions for third- and fourth-year medical students, which are held during the Pediatric Inpatient Medicine Clerkship.

Additionally, faculty members precept students in busy clinical settings, including acute, preventive, and newborn care. Chaves-Gnecco has developed and oversees a one-week elective that focuses on the care of Pittsburgh's Latino population. Bogen has developed and oversees a breastfeeding elective for fourth-year students.
Cook is developing a new one-week rotation in the Complex Care Clinic.

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DIVISION OF HEMATOLOGY/ONCOLOGY

Mission

The core mission of the Division of Pediatric Hematology/Oncology (PHO) is to ensure the care of children with blood diseases and cancer, now and in the future. The division is dedicated to the following goals:

- To provide state-of-the-art, compassionate care to children with blood diseases and cancer
- To perform high-impact basic and clinical research that furthers understanding of the pathogenesis and treatment of childhood blood disorders and cancer
- To train future leaders in pediatric hematology and oncology

FACULTY AND STAFF

Linda M. McAllister-Lucas, MD, PhD
Chief, Division of PHO
Associate Professor of Pediatrics
Associate Professor of Microbiology and Molecular Genetics

Cheryl Hillery, MD
Clinical Director, Pediatric Hematology
Director, Comprehensive Pediatric Sickle Cell Program
Professor of Pediatrics

Louis Rapkin, MD
Clinical Director, Pediatric Oncology
Associate Professor of Pediatrics

Kelly Bailey, MD, PhD
Research Instructor, Department of Pediatrics

Andrew Bukowinski, MD
Director, Phase I/ II Developmental Therapeutics Program
Interim Director, Pediatric Neuro-Oncology Program
Assistant Professor of Pediatrics

Wen-I (Wendy) Chang, MD
Director, Cancer Predisposition Program
Director, Tumor Procurement/Genomics
Assistant Professor of Pediatrics

Jing Cheng, MD, PhD
Research Instructor, Department of Pediatrics

Debra E. Cohen, MD
Assistant Professor of Pediatrics

James D. Cooper, MD, Msc
Clinical Director, Pediatric Coagulation Disorders Program
Director, PHO Medical Student Education
Assistant Professor of Pediatrics

Erika Friehling, MD
Associate Vice Chair for Faculty Development
Associate Director, PHO Fellowship Program
Director, PHO Resident and Fellow Education
Assistant Professor of Pediatrics

J. Anthony Graves, MD, PhD
Assistant Professor of Pediatrics

Scott H. Maurer, MD
Director, Pediatric Palliative Care and Supportive Care Program
Associate Professor of Pediatrics

Amma Owusu-Ansah, MD
Clinical Director, Center for Translational and International Hematology, Pittsburgh Heart, Lung, and Blood Vascular Medicine Institute
Assistant Professor of Medicine

Edward V. Prochownik, MD, PhD
Paul C. Gaffney Professor of Pediatrics
Director, Oncology Research
Professor of Pediatrics
Professor of Microbiology and Molecular Genetics
Professor of Clinical and Translational Science

A. Kim Ritchey, MD
Vice Chair for International Affairs, Department of Pediatrics
Professor of Pediatrics

Jean M. Tersak, MD
Director, Pediatric Cancer Survivorship Program
Program Director, PHO Fellowship Program
Principal Investigator, Children's Oncology Group (COG) Clinical Trials Program at Children's Hospital of Pittsburgh of UPMC
Associate Professor of Pediatrics

Randy M. Windreich, MD
Director, PHO/Bone Marrow Transplantation and Cellular Therapies Outpatient Clinic
Assistant Professor of Pediatrics

Michael R. Wollman, MD
Professor of Pediatrics

Debra Correa, CRNP
Hematology/Coagulation Program

Aimee Costello, CRNP
Pediatric Cancer Survivorship Program
Pediatric Neuro-Oncology Program

Veronica Ekeroth, CRNP
Pediatric Oncology

Maggie Holtz, PA-C
Pediatric Sickle Cell Disease (SCD)/ Hemoglobinopathy Program

Whitney Lerch, PA
Pediatric Oncology

Amanda Moore, PA-C
Pediatric Cancer Survivorship Program

Rachel Raymond, PA
Pediatric Neuro-Oncology Program

Michael Talotta, PA-C
Pediatric Oncology

OVERVIEW OF DIVISION

The Division of PHO includes expert clinicians and scientists, who provide state-of-the-art care to children with blood diseases and cancer, carry out high-impact research, and train future academic leaders in the field. The division is actively involved in both clinical and basic laboratory science research. To achieve its educational and training goals, the division has outstanding and highly acclaimed teachers, as well as a competitive fellowship that is offered in collaboration with the Division of Blood and Marrow Transplantation and Cellular Therapies.

Listed below are some highlights of activities within the division during academic year 2016–2017.

- Linda McAllister-Lucas was invited as the 2016 Research Scholar for the New York University Medical Scientist Training Program.
- McAllister-Lucas was invited as a featured speaker to the 2017 P.O.W.E.R. 4 Precision Medicine Women in Biology Rally.
- McAllister-Lucas was senior author on a manuscript titled "MALT1 Protease Activation Triggers Acute Disruption of Endothelial Barrier Integrity Via CYLD Cleavage." The article was published in *Cell Reports* and was selected as a top article by the Faculty of 1000.
- Cheryl Hillery served as a member of the National Heart, Lung, and Blood Institute (NHLBI) SCD Advisory Committee.
- Hillery was named as a "Top 10 Teacher" by the Children's Hospital pediatric residents.
- Louis Rapkin was invited to speak on "Reducing Racial Disparities in Pediatric Cancer Treatment" at the Gateway Medical Society Symposium.
- Rapkin was invited to join the American Academy of Pediatrics (AAP) committee for PHO policies and guidelines.
- Kelly Bailey was awarded an Alex's Lemonade Stand Foundation Young Investigator Grant to support her work on sarcoma metastasis.
- Bailey received a Vascular Medicine Institute Pilot Grant Award.
- Andrew Bukowinski was appointed as interim director of the section of pediatric neuro-oncology at Children's Hospital of Pittsburgh.
- Bukowinski was selected as the national study chair for an upcoming COG phase I clinical trial.
- Wendy Chang's manuscript titled "Multidimensional Clinomics for Precision Therapy of Children and Adolescent Young Adults with Relapsed and Refractory Cancer: A Report from the Center for Cancer Research" was published in the journal *Clinical Cancer Research*.
- Jing Cheng was invited to give an oral presentation of her work on the MALT1 oncoprotein at the annual American Society of Hematology (ASH) meeting in San Diego, Calif.
- James Cooper was appointed as divisional director of outpatient satellite clinics and outreach.
- Erika Friehling was appointed associate vice chair for faculty development in the Department of Pediatrics.
- Scott Maurer was appointed as director of the newly established Children's Hospital Division of Palliative Medicine and Supportive Care.
- Scott Maurer authored a chapter in *Ethical Issues in Pediatric Hematology/Oncology*, titled "Communicating Prognosis at Diagnosis and Relapse or Progression."
- Amma Owush-Ansah is the principal investigator of a new U.S. Food and Drug Administration R01 grant award.
- Edward Prochownik received a Hyundai Hope on Wheels Scholar Grant Award titled "Ribosomopathy-Like Properties of Cancers."
- A. Kim Ritchey was appointed vice chair for international affairs in the Department of Pediatrics.
- Jean Tersak published "Shared Care of Childhood Cancer Survivors: A Telemedicine Feasibility Study" in the *Journal of Adolescent and Young Adult Oncology*.
- Randy Windreich was appointed as the new director of the outpatient clinic for the PHO and Bone Marrow Transplant and Cellular Therapies (BMT & CT) divisions.
- Cooper, Hillery, Ritchey, Tersak, Windreich, and Wollman were selected for *Pittsburgh Magazine's* Best Doctors, 2017.
- Cooper, Friehling, Maurer, Ritchey, Windreich, and Wollman received awards as top physicians, recognized for spectacular care by patient families at Children's Hospital.

CLINICAL ACTIVITIES OF THE DIVISION

The division provides comprehensive care for children with cancer and blood disease in inpatient and outpatient settings. In addition, the division has specialized programs for the care of long-term survivors of childhood cancer, adolescents and young adults with cancer, patients with brain tumors, patients with hemoglobin disorders, and patients with bleeding disorders.

Clinical activity, both outpatient and inpatient, remained at very high levels for fiscal year 2017.

RESEARCH AND OTHER SCHOLARLY ACTIVITIES

Linda McAllister-Lucas, MD, PhD

RESEARCH

Linda McAllister-Lucas is a physician-scientist funded by the National Institutes of Health (NIH) and investigates how dysregulated intracellular signaling contributes to inflammatory and neoplastic disease. McAllister-Lucas codirects a research laboratory with Peter Lucas, a member of the faculty in the Department of Pathology. Their joint laboratory includes 12 scientists at varying stages of training, and the group is pursuing two related areas of research.

The Molecular Pathogenesis of B-Cell Lymphoma. The laboratory focuses on a complex of signaling proteins referred to as the CARMA-Bcl10-MALT1 (CBM) signalosome. The McAllister-Lucas group, along with several other laboratories around the world, first discovered this complex because the genes encoding its components are targeted by mutation and chromosomal mutation in lymphoid malignancy. This CBM signalosome plays a critical role in normal lymphocyte function by mediating antigen receptor–dependent activation of the pro-inflammatory, pro-survival NF-κB transcription factor. The Mcallister-Lucas laboratory has gone on to make major contributions to understanding of the mechanisms by which deregulation of the CBM complex promotes B-cell non-Hodgkin lymphoma. The group discovered that the API2-MALT1 fusion oncoprotein possesses unique gain-of-function MALT1 proteolytic activity that drives the proliferation and survival of malignant B-cells (*Science*, 2011;331:468-72 and *Nature Communications*, 2015;6:5908). The group is now also focusing on the mechanisms by which deregulated MALT1 activity promotes diffuse large B-cell lymphoma.

The CARMA-Bcl10-MALT1 Signaling Complex in Inflammatory and Neoplastic Disease. The team discovered that a CBM signalosome also functions outside of lymphocytes by operating downstream of specific G-protein coupled receptors (GPCRs) in non-immune cells. In this case, the nonlymphoid-specific homologue CARMA3 substitutes for the lymphocyte-specific CARMA1. The laboratory is now actively studying the contribution of GPCR-induced, CBM-dependent stimulation of NF-κB to a variety of inflammatory and neoplastic diseases and exploring the utility of inhibiting the CBM complex as a new approach to treatment of these conditions. Specifically, the researchers have elucidated a new role for the CBM complex in mediating progression and metastasis of a variety of solid tumors, including breast cancer and sarcoma. The work was recently accepted for publication in the high-impact journal *Cancer Research*. In addition, the McAllister-Lucas research group has discovered a new role for MALT1 proteolytic activity in promoting GPCR-induced endothelial permeability, a finding that was recently published in and featured on the cover of *Cell Reports*.

Linda M. McAllister-Lucas, MD, PhD
Division Chief, Hematology/Oncology

ADVISORY BOARD AND COMMITTEE MEMBERSHIPS

International:

- Award reviewer, Helmholtz Association of German Research Centres
- Grant reviewer, Swiss National Science Foundation
- Grant reviewer, Research Foundation Flanders
- International Scientific Committee, Fifth International Symposium on Childhood, Adolescent, and Young Adult Non-Hodgkin Lymphoma

National:

- Pediatric Non-Hodgkin Lymphoma Biology Research Group
- Program Planning Committee, American Society of Pediatric Hematology/Oncology (ASPHO)

University/Health System:
- Oncology Steering Committee
- Vascular Medicine Institute
- UPMC Hillman Cancer Center Director Search Committee
- UPMC Hillman Cancer Center Research Executive Leadership Advisory Committee

Departmental:
- Molecular Medicine Research Committee

EDITORSHIPS
- Editorial Board, *Pediatric Blood and Cancer*

MAJOR LECTURESHIPS AND SEMINARS
- "Precision Medicine in Pediatric Oncology," Women in Biology Rally: P.O.W.E.R. 4 Precision Medicine, Pittsburgh, Pa., 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS
- AAP
- ASH
- ASPHO
- COG
- Society for Pediatric Research
- American Association for Cancer Research (AACR)
- Midwest Society for Pediatric Research
- American Society for Clinical Investigation

HONORS
- *Best Doctors in America*, Woodward/White, Inc.
- American Society for Clinical Investigation
- American Pediatric Society
- Norman J. Siegel Outstanding Science Award, American Pediatric Society

Andrew Bukowinski MD, MS

RESEARCH
Developmental Therapeutics/COG. Bukowinski is an active co-investigator for developmental therapies for the COG phase I program. Children's Hospital is one of the few hospitals in the country conducting clinical research on novel therapies for children with cancer. Bukowinski has been selected as national study chair for an upcoming COG phase I trial scheduled to open in spring 2018.

Pediatric Brain Tumor Consortium. Bukowinski is an active co-investigator for developmental therapies for the Pediatric Brain Tumor Consortium. Children's Hospital is one of the few hospitals in the country conducting clinical research on novel therapies for children with brain tumors.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS
National:
- COG, 2012 to the present
- American Society of Clinical Oncology (ASCO), 2012 to the present
- ASPHO, 2012 to the present

University/Health System
- Protocol Review Committee, University of Pittsburgh Cancer Institute

Divisional:
- Chemotherapy Oversight Committee, Children's Hospital

MAJOR LECTURESHIPS AND SEMINARS
- Children's Hospital of Pittsburgh fellow lecture on medulloblastoma
- Children's Hospital of Pittsburgh fellow lecture on clinical trial design

HONORS
- Conquer Cancer Award Trainee Travel Award, ASCO annual meeting, Chicago, Ill.

Wen-I (Wendy) Chang, MD

RESEARCH
Cancer Predisposition Clinic. This is a multidisciplinary effort to set up a clinic for pediatric oncology patients who have a strong family history of cancer. Chang has arranged to meet with Jerry Vockley, chief of the Division of Medical Genetics, as well as Elena Infante, a genetics counselor, to discuss the implementation of a clinic time and space to see patients together. The two divisions are looking to work jointly in providing counseling, genetic testing, and long-term follow-up screening for patients and their family members who may be genetically predisposed to the development of cancer.

Pediatric Oncology Sequencing and Precision Medicine. This is multidisciplinary effort to sequence the exome of patients' tumors and germline DNA in their blood to try to identify the mutations driving their cancers. Together with Kelly Bailey, Chang is setting up meetings with vari-

ous directors of other departments (Precision Medicine, Pediatric Pathology, Genomic Medicine, Bioinformatics, and Pediatric Surgery) to discuss a way to incorporate tissue banking and sequencing of pediatric oncology patients seen in the clinical setting. With the setup of the hospital's own repository of tissue samples and the ability to sequence within the UPMC system, the team will be able to determine targetable mutations in these patients and have the ability to possibly tailor their clinical therapy based on findings. This work is immediately translatable from bench to bedside, with immense potential for patient care.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- ASPHO
- COG
- AAP
- AACR

Jing Cheng, PhD

RESEARCH

Jing Cheng is a research assistant professor in the McAllister-Lucas Laboratory. Her primary research focuses on identifying novel binding partners for MALT1, the effector molecule of the CARMA-Bcl10-MALT1 (CBM) signalosome, which plays an important role in normal lymphocyte function and lymphomagenesis.

Debra E. Cohen, MD

RESEARCH

Model Systems for Newborn Screening and Comprehensive Care for SCD. Over the past eight years, the sickle cell team has been grant-funded by the Health Resources and Services Administration to develop model systems of health care delivery, including newborn screening and comprehensive care for patients with hemoglobinopathies. Work has focused on improving the functioning of newborn screening system; improving access to care; improving transition to adult care; and using information systems, including electronic medical records, to improve outcomes.

Distance Strategies to Improve Follow-Up of Patients with SCD Detected on Newborn Screening. This is a project to improve care and outcome of patients with SCD who have limited access to care, through telehealth.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- ASH
- COG
- Foundation for Sickle Cell Disease Research
- American Balint Society

James D. Cooper, MD

RESEARCH

James Cooper is the director of the Clinical Pediatric Coagulation Program at Children's Hospital. He also is the founder of CureRock, a concert to benefit pediatric and adolescent oncology.

His area of clinical expertise is pediatric hemostasis and thrombosis, heavy menstrual bleeding, and coagulation complications.

ADVISORY BOARD AND COMMITTEE MEMBERSHIPS

- Medical Executive Committee, Children's Hospital of Pittsburgh

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

National:

- ASPHO
- ASH
- COG
- Critical Mass Young Adult Alliance
- American Thrombosis and Hemostasis Network
- AAP
- Foundation for Women and Girls with Bleeding Disorders

Divisional:

- Clinical Competency Committee, Fellowship Program
- Program Evaluation Committee, Fellowship Program
- Pediatric Anticoagulation Committee

HONORS

- Patient Satisfaction Award, Children's Hospital, 2016, 2017
- Best Doctors, *Pittsburgh Magazine*, 2013–2017

Erika Friehling, MD

RESEARCH

Erika Friehling was recently named the associate vice chair for faculty development in the Department of Pediatrics. The goals are to recruit, retain, promote and nurture a vibrant and diverse faculty. The team designed and implemented a successful workshop series. Friehling will be creating a faculty mentoring program for the department to promote professional advancement, well-being, and productivity throughout the entire career cycle.

As director of the Associate Fellowship Program, Friehling's responsibilities include overseeing the educational components of the fellowship program, as well as developing ways to adapt the program to changes in training regulations, the PHO workforce, and health care delivery models.

Improving Subspecialty Education for Residents and Fellows. This is a multifaceted approach to formalize and enhance the medical education of residents interested in pursuing subspecialty careers and of PHO fellows. Friehling has designed and implemented a yearlong career-development curriculum for pediatric residents. She has developed a new and improved fellow core lecture curriculum and is a leader of career-development curriculum for residents. Friehling has also developed and implemented longitudinal experiences for pediatric residents.

ADVISORY BOARD AND COMMITTEE MEMBERSHIPS

National:

- Certification and Continuing Education Committee, ASPHO

University/Health System:

- MedEd Day Planning Committee, University of Pittsburgh School of Medicine
- Abstract reviewer/judge, MedEd Day, University of Pittsburgh School of Medicine
- Fertility Preservation Group

DepartmentalFaculty Development Leadership Group:

- Bridges Mentoring Program Development Group
- Diversity and Inclusion Committee
- Clinical Competency Committee, Pediatric Residency Program
- Intern Selection Committee, Pediatric Residency Program

Divisional:

- Clinical Competency Committee, Fellowship Program
- Program Evaluation Committee, Fellowship Program
- Chemotherapy Oversight Committee

MAJOR LECTURESHIPS AND SEMINARS

- "Fertility Preservation: Considerations for the Pediatric Oncology Patient," Our Clubhouse, Pittsburgh, Pa., November 2016
- "Academic Career Paths," Pediatric Residency Career Week Noon Conference, Pittsburgh, Pa., September 2016
- "CV Workshop," Pediatric Residency Career Week Noon Conference, Pittsburgh, Pa., September 2016
- "Hematologic Emergencies," Pediatric Residency Noon Conference, Pittsburgh, Pa., August 2016
- "Making the Most of Mentoring for Career Success," Department of Pediatrics Faculty Development Workshop, Pittsburgh, Pa., August 2016

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- ASPHO
- COG
- ASH
- AAP
- ASCO
- Academic Pediatric Association
- Association of Pediatric Program Directors

HONORS

- Patient Satisfaction Award, Children's Hospital, 2016
- Clinician Educator Award, ASPHO, 2015–2017
- Educational Scholars Program, Academic Pediatric Association, 2015–2017

J. Anthony Graves, MD, PhD

RESEARCH

Myc Oncoprotein-Dependent Effects on Mitochondria. Mitochondrial structure and function in various tumors are the focus of the work performed in the Graves Laboratory. Mitochondria are essential to virtually every aspect of cellular life and death, not just "the powerhouses of the cell." They are central to production of energy, synthesis of fatty acids, nucleotides, and amino acids and regulation of apoptosis (programmed cell death). The laboratory investigates the role of specific mitochondrial proteins in the pathogenesis of pediatric tumors such as neuroblastoma and medulloblastoma.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- ASPHO
- ASH
- AAP
- AAACR

HONORS

- Young Investigator Award, CureSearch, 2015–2017

Cheryl Hillery, MD

RESEARCH

Cheryl Hillery is an NIH-funded physician-scientist who treats patients with SCD and has been involved with basic and translational research programs focused on vascular and organ pathologies in human and mouse models of SCD, as well as novel mechanisms of pain. Hillery was recruited to the division in May 2015 and has been working with Linda McAllister-Lucas to build a strong clinical and translational benign hematology program. Hillery has also continued her NIH-funded collaborative research activities with scientific colleagues from the Medical College of Wisconsin.

SCD is caused by a genetic disorder of hemoglobin that predisposes sickle hemoglobin to form long crystals that damage the red cell membrane and cause it to block blood vessel flow (vaso-occlusion). The major cause of pain, suffering, and death in SCD is tissue injury and inflammation due to repeated vaso-occlusion that results in progressive organ damage. Much remains unknown regarding exactly how sickle red cells injure the blood vessels and how this leads to the blood vessel and organ damage observed in patients. Because of this, there are currently few choices for effective treatment of this disease. Thus, there is a great need to develop novel therapeutic approaches for the prevention and treatment of vaso-occlusion and resultant complications in SCD.

Role of the Clotting and Inflammatory Pathways in SCD. Abnormal adhesion of sickle red cells to the blood vessel damages the vessel wall and slows or stops blood flow. Increased activity of the clotting and inflammatory pathways further injures blood vessels and organs in SCD. Hillery's team's studies are trying to discern the exact role of the clotting and inflammatory pathways and to determine whether agents that thin the blood or decrease inflammation may help patients who suffer from SCD.

Novel Mechanisms of Pain in SCD. Pain is the major cause for urgent medical care and admission to the hospital in children with SCD. The pain and disability are even more severe in adults. In this project, Hillery and her collaborator, Cheryl Stucky, study the precise nerve cells and pathways that sense the pain and carry the message to the brain so that they can develop new methods to treat SCD more safely and effectively. This study is currently funded by NIH R01 "Nociceptive Mechanisms Underlying Sickle Cell Pain." Multiple principal investigators, Stucky and Hillery, through 2019.

Role of Inflammation in the Morbidities Associated with SCD. SCD is characterized by chronic inflammation. Asthma is an inflammatory comorbid condition that is commonly found in children with SCD and may further contribute to sickle hemoglobin–induced vascular and organ pathologies. A diagnosis of asthma among individuals with SCD is associated with twice the rate of pain and increased mortality when compared to SCD individuals without asthma. The researchers have explored the role of asthma and asthmatic inflammatory pathways in complications of human and mouse SCD. In ongoing studies, Hillery and her collaborator, Kirkwood Pritchard, are now exploring the role of high-mobility group box 1 (HMGB1), a nuclear protein that is important for maintaining DNA structure and function. During inflammation and injury, HMGB1 is released from the cells; once released, it can act as a potent inflammatory agent that increases endothelial cell injury and death. The team hypothesizes that SCD increases HMGB1 to increase endothelial cell injury and risk of RBC congestion and crisis. The goal of these studies is to learn how HMGB1 increases inflammation, injures endothelial cells, and increases vaso-occlusive crises in SCD. These studies are funded by NIH R01 "Mechanisms of Inflammation in Sickle Cell Disease" (multiple principal investigators, Pritchard and Hillery) to continue with a focus on HMGB1. Hillery is also a collaborator for ongoing U.S. Food and Drug Administration R01 "Phase 2 Study of Montelukast for the Treatment of Sickle Cell Anemia" (principal investigator Joshua Field).

ADVISORY COMMITTEE MEMBERSHIPS

- NIH Small Business Hematology Special Emphasis Panel, July 2016
- NHLBI Special Emphasis Panel for Zika virus R21 grants, 2016
- Director, Comprehensive Pediatric Sickle Cell Program, Children's Hospital of Pittsburgh

EDITORSHIPS

- Editorial Board, *Blood Advances*, 2016 to the present

MAJOR LECTURESHIPS AND SEMINARS

- "Clinical, Vascular, and Hematological Aspects of Sickle Cell Pain," Pain Mechanisms and Therapeutics Conference, Taormina, Sicily, Italy, June 2016

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- ASH
- Society for Pediatric Research
- ASPHO

HONORS

- *Best Doctors in America*, Woodward/White, Inc., 2001 to the present

Scott H. Maurer, MD

RESEARCH

Scott Maurer is an associate professor of pediatrics within the division and chief of the Division of Palliative Medicine and Supportive Care at Children's Hospital.

Maurer provides palliative care consultative services to patients on all services in the hospital. He focuses on advanced care planning, decision-making support, pain and symptom management, care coordination, end of life, and bereavement care for pediatric patients with life-limiting illness. Together with colleagues

from the supportive care team, obstetrics, genetics, and neonatology, Maurer co-founded a perinatal palliative care program at the University of Pittsburgh. He also serves as the pediatric palliative care course director for the University of Pittsburgh palliative care fellowship program, and he provides teaching to pediatric residents and medical students. Maurer has mentored numerous medical students, residents, and fellows in clinical and research palliative care projects. He is active in both institutional and cooperative research projects in pediatric palliative medicine, and he has spoken at national and international conferences about how to improve palliative care for children.

His projects include the following.

- PedPRO-CTCAE: Patient-Related Outcomes in Children Undergoing Chemotherapy for Malignant Disease, investigator in multisite study, local principal investigator, 2015 to the present
- A Pilot Study to Evaluate the Effects of Vaccinations with HLA-A2-Restricted Glioma Antigen Peptides in Combination with Poly-ICLC for Children with Newly Diagnosed Malignant or Intrinsic Brainstem Glioma or Non-Brainstem High-Grade Glioma or Recurrent Unresectable Low-Grade Glioma or Recurrent High-Grade Glioma, co-investigator, served as interim principal investigator from 2014–2015, 2016–2017
- A Pilot Study to Evaluate the Effects of Vaccinations with HLA-A2-Restricted Tumor Antigen Peptides in Combination with Imiquimod for Children with Recurrent Ependymoma, co-investigator, served as interim principal investigator from 2014–2015, 2016–2017
- Investigating Spiritual Interventions in Children Referred to the Supportive Care Program: A Quality-Improvement Project, principal investigator, Children's Hospital, 2013 to the present (now in second phase of this study)
- Creating, Implementing, and Testing Standards for Bereavement Care in the Neonatal Intensive Care Unit, principal investigator, Children's Hospital, 2011 to the present
- Quality-Improvement Process for Death Notification to Resident Physicians, mentoring principal investigator for a pediatric resident, 2016 to the present

ADVISORY BOARD AND COMMITTEE MEMBERSHIPS

- Pharmacy and Therapeutics Committee, Children's Hospital of Pittsburgh, 2014 to the present
- Pain Management Advisory Committee, UPMC
- Member, Ethics Committee, Children's Hospital of Pittsburgh, September 2012 to the present
- Palliative Care Fellowship Steering Committee, University of Pittsburgh, July 2012 to the present
- Member, Pediatric Resident Wellness Committee, Children's Hospital of Pittsburgh, 2011 to the present

MAJOR LECTURESHIPS AND SEMINARS

- "Neonatal Pain Management," Medical Ethics Update 2014, University of Pittsburgh School of Medicine, Pittsburgh, Pa., April 2014
- "Ketamine PCA for Treatment of End-of-Life Neuropathic Pain in Pediatrics," platform presentation, Pediatric Academic Societies Annual Assembly, Vancouver, British Columbia, Canada, 2014
- "Supporting Children and Families with Life-Limiting Illness: Old Roads with New Directions," pediatric grand rounds, Children's Hospital, Pittsburgh, Pa., September 2014
- "Decision-Making Support for Families and Children with Life-Limiting Illness," keynote address, Pennsylvania Pediatric Palliative Care Coalition statewide educational conference, Harrisburg, Pa., November 2015

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- COG
- American Academy of Hospice and Palliative Medicine
- ASPHO
- AAP

HONORS

- Outstanding alumnus, Niles Senior High School, 2016
- Outstanding Patient Care Award, UPMC-wide, 2016
- Outstanding Quality of Patient Care Award, Children's Hospital of Pittsburgh, 2016

Amma Owusu-Ansah, MD

RESEARCH

Amma Owusu-Ansah's passion is to improve access to state-of-the-art care for individuals with benign hematologic disorders worldwide, through research, timely translation of novel therapeutics into clinical care, and capacity building. As such, she dedicates 50% of her time and effort to research and capacity-building activities in Ghana, West Africa, in collaboration with other multinational hematologists and scientists. Her current focus is on SCD.

Owusu-Ansah is involved with the following studies.

- Plasma surrogates of nrf2 activation, funded by an Investigator Research Supplement from the NHLBI
- Coordinator (Pittsburgh), Organ Damage in SCD Study, Kumasi, Ghana
- Principal investigator: A phase Ib, dose-finding, pharmacokinetic, and pharmacodynamic study of NVX-508 in SCD (performance site: Noguchi Memorial Institute for Medical Research, University of Ghana, Legon, Accra, Ghana)

MAJOR LECTURESHIPS AND SEMINARS

- "Pediatric Sickle Cell," SCD Champion Symposium, Pittsburgh, Pa., September 2016

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- ASH
- ASPHO

Edward V. Prochownik, MD, PhD

RESEARCH

Edward Prochownik is interested in cancers resulting from the deregulated expression of the c-Myc oncoprotein. He is using animal models of pediatric and adult liver cancer (hepatoblastoma and hepatocellular carcinoma) to ascertain the molecular, biochemical, and metabolic changes that accompany tumor progression, regression, and recurrence. Prochownik is utilizing overexpression and knockout models to determine how genes that cooperate with or are affected by Myc, such as ChREBP and pyruvate dehydrogenase, specifically contribute to the metabolic and molecular landscapes of those tumors.

MAJOR LECTURESHIPS AND SEMINARS

- "Cancer as a Ribosomopathy," Pittsburgh Liver Research Center, Pittsburgh, Pa., November 2016
- "Cancer as a Ribosomopathy," invited speaker and session chairman, International Conference on Genomic Medicine, Baltimore Md., February 2017
- "Cancer as a Ribosomopathy," Molecular Medicine Research Seminar, Children's Hospital, Pittsburgh, Pa., June 2017
- "Eucaryotic Molecular Genetics," invited lecturer, UPMC, Pittsburgh, Pa.
- "Advanced Topics in Gene Expression," invited lecturer, UPMC, Pittsburgh, Pa.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Association for the Advancement of Science
- ASH
- Society for Pediatric Research
- American Society for Microbiology
- American Society for Clinical Investigation
- American Pediatric Society

HONORS

- Hyundai Hope on Wheels Scholar Hope Grant, September 2017
- Summer Undergraduate Research Program poster session, second place presented to Ari P. Dash, Edward Prochownik, MD, PhD, mentor, Children's Hospital of Pittsburgh, 2017
- David G. Nathan Research Award, Society for Pediatric Research, presented to Laura Jackson, MD, third-year neonatal medicine fellow, UPMC, Edward Prochownik, MD, PhD, mentor, 2017
- University of Pittsburgh Medical Student Research Mentoring Merit Award, 2016

Louis B. Rapkin, MD

RESEARCH

Louis Rapkin worked at Emory University School of Medicine and Children's Healthcare of Atlanta for 15 years, caring for pediatric patients with rare tumors. In 2017, he became the clinical director of oncology at Children's Hospital of Pittsburgh and began building the adolescent and young adult oncology program.

Clinically, Rapkin is interested in rare tumors within the pediatric population (tumors that occur fewer than 100 times in the United States per year), including adult carcinomas and soft-tissue sarcomas, treatment of adolescent and young adult patients, and coordination of care between pediatric and adult facilities.

Rapkin's other interests include medical student and resident education.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- ASPHO

HONORS

- Subspecialist Teacher of the Year, Department of Pediatrics, Emory University School of Medicine
- Best M1/M2 Teaching Module, Emory University School of Medicine
- Dean's Teaching Award, Emory University School of Medicine
- Alpha Omega Alpha Honor Medical Society

A. Kim Ritchey, MD

RESEARCH

COG. A. Kim Ritchey is an active clinical investigator and local principal investigator (PI) for a number of COG trials at Children's Hospital. The local PI of a COG trial is responsible for overseeing the research activity of that protocol at the institution. One such trial is testing the efficacy of the tyrosine kinase inhibitor dasatinib when it is added to standard chemotherapy for children with Philadelphia chromosome–positive acute lymphoblastic leukemia. This trial is partially funded by Bristol-Myers Squibb. Ritchey is very involved with oversight of the development and performance of pediatric cancer clinical trials at the national level. He is a member of the National Cancer Institute (NCI) Pediatric Leukemia/Lymphoma Steering Committee, which reviews all COG proposals for clinical research in the areas of leukemia and lymphoma. As chair of the COG Data Safety Monitoring Committee, he leads the group which has oversight of all phase 2 and 3 clinical trials.

Ritchey served as local PI for the Pediatric Brain Tumor Consortium, a collaboration of 12 institutions funded by the NCI to offer potentially practice-changing Phase I research for children with brain tumors.

Ritchey served as the local PI for a study of the thrombopoietin agonist romiplostim for children with chronic idiopathic thrombocytopenic purpura.

ADVISORY BOARD AND COMMITTEE MEMBERSHIPS

National:

- Chair, Data Safety Monitoring Committee A, COG
- Pediatric Leukemia/Lymphoma Review Committee, NCI, National Institutes of Health (NIH)

Departmental (Department of Pediatrics)

- Center for Rare Diseases Committee
- Council
- Leadership Team
- Executive Committee
- Promotion and Tenure Committee

Divisional: (Hematology/Oncology)

- Fellowship Oversight Committee
- Chair, Clinical Competency Committee
- Chair, Advanced Practice Provider Committee

Hospital-based:

- Global Health Service Committee
- Medical Executive Committee

Regional:

- Board of Directors, Hemophilia Center of Western Pennsylvania
- Board of Directors, Make-a-Wish Foundation

EDITORSHIPS

- Editor-in-chief, Pediatric Treatment Board, Physician Data Query, NCI, NIH

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- ASCOASH
- ASPHO
- COG
- Thrombosis and Hemostasis Research Society

HONORS

- *Best Doctors in America*, Woodward/White, Inc.
- Best Doctors, *Pittsburgh Magazine*
- UPMC Honor Roll: Excellence in Patient Care (Top 3 of 70)
- Patient Satisfaction Award, Children's Hospital
- Top 10 Press Ganey Faculty Scores

Jean M. Tersak, MD

RESEARCH

COG. Jean M. Tersak serves as principal investigator of the COG research program at Children's Hospital of Pittsburgh. She is also the principal investigator of the COG phase I program, making Children's Hospital of Pittsburgh

one of the few hospitals in the country conducting clinical research on novel therapies for children with cancer.

Clinical Research Infrastructure. Tersak is also the principal investigator of the Alex's Lemonade Stand Foundation grant for infrastructure support of phase I and phase II research at Children's Hospital of Pittsburgh, as well as a St. Baldrick's infrastructure grant. New research infrastructure initiatives include enhancing the division's Web site to improve ability to communicate information regarding available clinical trials and utilizing a more extensive clinical trials management system for clinical trial activity.

Childhood Cancer Survivor Study. This multicohort study focuses on the health outcomes of the majority of children and young adults diagnosed with cancer who become long-term survivors. Children's Hospital of Pittsburgh is one of 27 institutions participating in the study. The project initially followed more than 14,000 childhood cancer survivors who were diagnosed between 1970 and 1986. Because therapies change over time, a static cohort has limited long-term benefit to patient care and survivors. Accordingly, the cohort is being expanded to include an additional 14,000 patients diagnosed between 1987 and 1999. Areas of study include but are not limited to secondary malignancies, cardiac failure, pulmonary complications, neurocognitive deficits, hearing loss, metabolic syndrome, and quality of life in childhood cancer survivors.

Solid-Organ Transplant in Five-Year Survivors of Pediatric Cancer. Morbidity and death from cardiac, pulmonary, and other medical conditions occur in pediatric cancer survivors as early as five years from diagnosis. Some life-threatening late complications of treatment for childhood cancer are treated with solid-organ transplantation. Long-term outcomes of childhood cancer survivors after solid-organ transplantation are largely unknown. The Childhood Cancer Survivors Study cohort is large enough to provide data for a descriptive analysis of these rare transplants. The primary aim of the study is to evaluate outcomes following solid-organ transplantation in five-year survivors of pediatric cancer diagnosed from 1970 to 1986, focusing on subsequent mortality, recurrence, and second malignant neoplasms. A matched group of subjects who did not receive solid-organ transplantation will also be included in this descriptive analysis.

The Leukemia Physical Functioning Study: Longitudinal Assessment of Peripheral Neuropathy and Motor Function in Children Treated for Average-Risk Acute Lymphoblastic Leukemia. This is a limited institution study embedded within a national COG therapeutic trial for children with acute lymphoblastic leukemia. At scheduled time points, participants will undergo a functional and parent-reported evaluation by a pediatric physical therapist in the pediatric oncology clinic. Peripheral neuropathy is a toxicity that was previously underappreciated and has only recently been understood to impact long-term functioning and quality of life in cancer patients. Interventions are available to address these impairments and, if implemented early, may ameliorate long-term functional loss. Increased understanding of the pathophysiology causing such impairments is crucial to developing early-intervention programs to prevent these deficits in children diagnosed with cancer.

Web-Based Interaction to Improve Long-Term Follow-Up of Childhood Cancer Survivors. Advances in treatment and supportive care have resulted in long-term survival rates of about 80% for individuals diagnosed with cancer during childhood and adolescence. Scientific literature clearly documents that survivors are at risk of treatment-related adverse effects that become apparent months or years after completion of therapy. This project involves the development and use of a multifaceted, interactive Web site that will enhance patient education; provide peer-to-peer emphasis on importance of follow-up through written and video testimony; and provide interactive capabilities, including appointment and testing reminders, as well as ongoing contact with providers. The goal of the project is to improve and make more accessible critical educational resources and follow-up information to a highly mobile, technology-efficient young adult population.

Caring for Cancer Survivors at the Virtual Bedside. This project is funded through the Pitt Innovation Challenge grant and involves the development of a telemedicine model to improve the education of primary care providers regarding the unique medical needs of childhood cancer survivors. This novel approach permits simultaneous communication between the survivor and the survivorship team to optimize the information exchange and transition process. Measurable outcomes will be utilized in this pilot study to provide critical information regarding feasibility and efficacy for future research trials in this area.

ADVISORY COMMITTEE MEMBERSHIPS

Departmental:

- Clinical Resource Management Committee

Divisional:

- Program director, Hematology Oncology Fellowship
- Chair, Chemotherapy Oversight Committee
- Member, Clinical Competency Committee

National:
- Developmental Therapeutics Protocol Development Committee, COG
- Diversity Health Care Disparities Committee, COG

Community:
- Our Clubhouse Medical Resource Council
- Catholic Youth Association Board

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS
- American Medical Association
- ASPHO
- ASCOCOG

HONORS
- *Best Doctors in America*, Woodward/White, Inc.
- Best Doctors, *Pittsburgh Magazine*

Randy Windreich, MD

RESEARCH
Randy Windreich's clinical research focuses on hematopoietic stem cell transplantation (HSCT) for acute leukemias; the understanding of drug pharmacokinetics and pharmacodynamics as a means of individualizing and optimizing therapy, particularly within the pediatric blood and marrow transplantation (BMT) patient population; and alternative uses for hematopoietic stem cells.

A Phase II Study of Myeloablative and Reduced-Intensity Conditioning Regimens for Children with Acute Myeloid Leukemia or Myelodysplastic Syndrome Undergoing Allogeneic HSCT. The objective of this study is to determine safety and preliminary efficacy and to achieve acceptable rates of event-free survival at six months in pediatric patients receiving a busulfan/fludarabine/thiotepa (myeloablative) or alemtuzumab/hydroxyurea/fludarabine/ melphalan/thiotepa (reduced-intensity) preparative regimen prior to HSCT for high-risk acute myeloid leukemia and myelodysplastic syndrome. Developing an institutional protocol for a dual-arm, novel, myeloablative, and reduced-intensity transplant conditioning regimen for hematologic malignancies with unrelated donors with emphasis on acute myeloid leukemia and acute lymphoblastic leukemia will allow more patients, particularly those with serious pre-transplant comorbidities, to become eligible to undergo HSCT. Subject enrollment is ongoing.

A Multicenter, Randomized, Double-Blind, Placebo-Controlled, Parallel-Group Study to Evaluate the Efficacy, Safety, and Tolerability of Transendocardial Injection of Ixmyelocel-T in Subjects with Heart Failure Due to Ischemic Dilated Cardiomyopathy. This is an industry-sponsored, multicenter study through Vericel Corporation (formerly Aastrom Biosciences) (Ann Arbor, Mich.), in collaboration with the UPMC Heart and Vascular Institute at UPMC Presbyterian Hospital. Results have been published in *Lancet* (2016;387:2412-21), demonstrating that transendocardial delivery of ixmyelocel-T in patients with heart failure and reduced ejection fraction due to ischemic dilated cardiomyopathy resulted in a significant reduction in adjudicated clinical cardiac events compared with placebo, leading to improved outcomes. An open-label extension is currently active for patients who had been randomly assigned to receive placebo during the study period and now can undergo bone marrow harvest again and receive ixmyelocel-T therapy.

A Single-Arm, Prospective Study of Remestemcel-L, Ex Vivo Cultured Adult Mesenchymal Stromal Cells, for the Treatment of Pediatric Patients Who Have Failed to Respond to Steroid Treatment for Acute Graft-Versus-Host Disease. This is an industry-sponsored multicenter study through Mesoblast International Sarl (Switzerland). The objective is to evaluate the efficacy and gather additional information on safety of remestemcel-L in pediatric patients with grades B–D acute graft-versus-host disease who have failed to respond to steroid treatment after allogeneic HSCT. Subject enrollment is ongoing.

Windreich also directs the Division of BMT & CT Fellowship Program and mentors a PHO fellow.

ADVISORY COMMITTEE MEMBERSHIPS
- Division of Hematology/Oncology Fellowship Oversight Committee, Children's Hospital of Pittsburgh
- PHO/BMT Outpatient Clinic Committee, Children's Hospital of Pittsburgh
- Chemotherapy Oversight Committee, Children's Hospital of Pittsburgh
- 9B (Oncology/BMT Inpatient Unit) Leadership Committee, Children's Hospital of Pittsburgh
- 9B (Oncology/BMT Inpatient Unit) Infection Control Committee, Children's Hospital of Pittsburgh
- Chemotherapy Oversight Committee, Children's Hospital of Pittsburgh

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS
- ASPHO
- American Society for BMT
- ASH
- COG

MAJOR LECTURESHIPS AND SEMINARS
- "Graft-Versus-Host Disease," oral presentation, Association of Pediatric Hematology/Oncology Nurses (APHON) BMT Course, Children's Hospital, Pittsburgh, Pa., September 2016

- "Graft-Versus-Host Disease Pharmacology," oral presentation, APHON BMT Course, Children's Hospital, Pittsburgh, Pa., September 2016
- "Blood and Marrow Transplantation Nuts and Bolts," oral presentation, PHO Fellowship Conference, Children's Hospital, Pittsburgh, Pa., September 2016
- "Pediatric Hematology/Oncology and Blood and Marrow Transplantation: 2016 Updates: Transition and Growth," oral presentation, Katie Swaney Foundation Board Meeting, Pittsburgh, Pa., October 2016
- "Leukemias and Lymphomas," oral presentation, Pediatric Residency Conference, Children's Hospital, Pittsburgh, Pa., December 2016
- "Cardiac Complications during Blood and Marrow Transplantation," oral presentation, APHON BMT Course, Children's Hospital, Pittsburgh, Pa., February 2017
- "Hepatic Complications during Blood and Marrow Transplantation," oral presentation, APHON BMT Course, Children's Hospital, Pittsburgh, Pa., February 2017
- "Engraftment," oral presentation, APHON BMT Course, Children's Hospital, Pittsburgh, Pa., February 2017
- "Leukemias," oral presentation, Mondays with Mindy (oncology/BMT nursing education series), Children's Hospital, Pittsburgh, Pa., February 2017
- "Neuroblastoma," oral presentation, APHON Foundations and Certified PHO Nurse Review Course, Children's Hospital, Pittsburgh, Pa., March 2017
- "Solid Tumors," oral presentation, APHON Foundations and Certified PHO Nurse Review Course, Children's Hospital, Pittsburgh, Pa., March 2017
- "Pediatric Cancer: Solid Tumors," oral presentation, Pediatric Residency Conference, Children's Hospital, Pittsburgh, Pa., April 2017
- "Safety and Feasibility of Granulocyte Transfusion for High-Risk Allogeneic Stem Cell Transplant Recipients," poster presentation, ASPHO annual meeting, Montreal, Quebec, Canada, April 2017
- "Lymphomas," oral presentation, Mondays with Mindy (oncology/BMT nursing education series), Children's Hospital, Pittsburgh, Pa., May 2017
- "Germ Cell Tumors," oral presentation, PHO Fellowship Conference, Children's Hospital, Pittsburgh, Pa., June 2017
- "Cardiac Complications during Blood and Marrow Transplantation," oral presentation, APHON BMT Course, Children's Hospital, Pittsburgh, Pa., June 2017
- "Hepatic Complications during Blood and Marrow Transplantation," oral presentation, APHON BMT Course, Children's Hospital, Pittsburgh, Pa., June 2017
- "Engraftment," oral presentation, APHON BMT Course, Children's Hospital, Pittsburgh, Pa., June 2017

HONORS

- Best Doctors in Pittsburgh, *Pittsburgh Magazine*, 2016–2017
- Patient Satisfaction Award for Outstanding Achievements in Patient Care, Children's Hospital of Pittsburgh, 2016

Michael R. Wollman, MD

RESEARCH

Michael Wollman is an investigator for COG.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- ASCO
- ASPHO

HONORS

- *Best Doctors in America*, Woodward/White, Inc.
- *America's Top Doctors*, Castle Connolly Medical, Ltd.
- Best Doctors, *Pittsburgh Magazine*
- Teacher of the Year (twice chosen), pediatric house staff, Children's Hospital of Pittsburgh
- Howard A. Mermelstein Award for Excellence in Pediatrics. Children's Hospital of Pittsburgh

TEACHING ACTIVITIES OF THE DIVISION

Members of the division are actively involved with the education of students, residents, fellows, and other professionals. Many of the faculty have been teachers in the following medical school courses or curricula: the hematology course, the neoplasia course, and the integrated life sciences course. Others have served as lecturers and mentors in the clinic for students in the Combined Ambulatory Medicine and Pediatrics Clerkship curriculum, as lecturers and preceptors for students on their pediatric clerkships, and as mentors to fourth-year students on their electives in hematology/oncology.

Pediatric resident education is a high priority, and faculty members provide many hours of direct teaching on rounds with the residents and in the clinic.

Maurer, in pursuit of furthering medical education among pediatric residents, has developed a journal club, enhanced didactic teaching in palliative care, and established a palliative care resident rotation. He directs the pediatric rotation for the Palliative Care Fellowship Program at the University of Pittsburgh. As a result, the Department of Pediatrics is able to accommodate clinical teaching and mentorship of palliative care fellows who are trained in internal medicine and internal medicine-pediatrics. This is in addition to his extensive research mentorship service.

Friehling is director of resident educational activities of the division and is a leader in developing improved subspecialty educational curriculum for pediatric residents. Friehling has developed a new curriculum for residents who are specifically interested in pursuing pediatric hematology and oncology fellowship training.

The division's fellowship program has consistently attracted outstanding fellow candidates. Faculty members are committed to providing an outstanding clinical and research experience for the fellows in training. All faculty participate in the program by serving as mentors in the clinic and on the ward, providing lectures during didactic conferences, teaching at the microscope, and serving as role models. Friehling has created a new didactic lecture series for the fellows. The fellowship program is one of the few to have a Balint group. Cohen had led the group, and Brittani Seynnaeve will be assuming the role moving forward.

Cooper has created a new fellowship rotation in hemostasis and thrombosis.

Faculty members have provided didactic sessions, as well as mentorship, to genetics students, graduate students, nurses, visiting international observers, practicing pediatricians, pediatric surgeons, and adult oncologists.

Medical students, residents, and fellows receive dedicated teaching in subspecialty foci within hematology/oncology, such as neuro-oncology, SCD, and survivorship care, under the direction of the clinical leadership of those respective programs.

THREE-YEAR BIBLIOGRAPHY

2015

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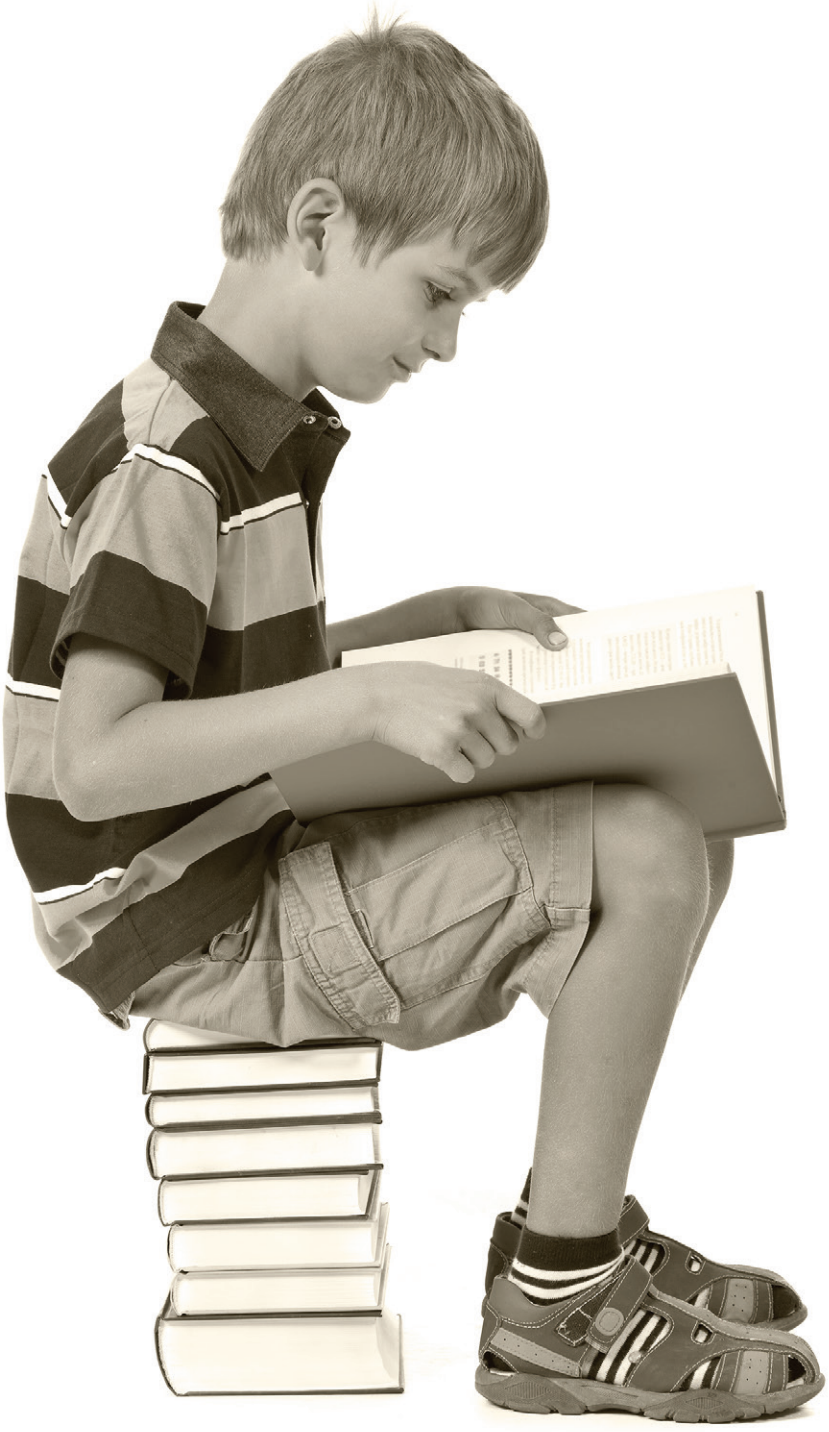

DIVISION OF
INFECTIOUS DISEASES

Mission

The mission of the Division of Infectious Diseases is:

- To excel in clinical care, education, and research
- To conduct and publish basic and clinical research on infectious diseases in children
- To serve as a national resource for pediatric transplant infectious diseases
- To recruit and train outstanding pediatric infectious disease fellows

FACULTY

John V. Williams, MD
Professor of Pediatrics
Henry L. Hillman Endowed Chair in Pediatric Immunology
Chief, Pediatric Infectious Diseases
Affiliate, Center for Vaccine Research
Faculty Member, Graduate Programs in Molecular Virology and Microbiology and Immunology

Brian T. Campfield, MD
Assistant Professor of Pediatric Infectious Diseases

Carolyn B. Coyne, PhD
Associate Professor of Pediatrics
Associate Professor of Immunology
Associate Professor of Microbiology and Molecular Genetics
Director, Center for Microbial Pathogenesis

Terence S. Dermody, MD
Vira I. Heinz Professor and Chair, Department of Pediatrics
Professor of Microbiology and Molecular Genetics
Physician-in-Chief and Scientific Director

Michael Green, MD, MPH
Professor of Pediatrics, Surgery and Clinical and Translational Science
Codirector, Transplant Infectious Diseases
Medical Director, Infection Prevention
Director, Antimicrobial Stewardship Program

Philana Ling Lin, MD, MSc
Associate Professor of Pediatrics
Program Director, Infectious Diseases Fellowship Training Program
Affiliate, Center for Vaccine Research
Faculty Member, Graduate Program in Molecular Virology and Microbiology and Immunology

Marian G. Michaels, MD, MPH
Professor of Pediatrics and Surgery
Director, Pediatric Human Immunodeficiency Virus (HIV) Center
Codirector, Transplant Infectious Diseases

Andrew Nowalk, MD, PhD
Assistant Professor of Pediatric Infectious Diseases
Codirector, Pediatric Residency Program, Department of Pediatrics
Advisory Dean, University of Pittsburgh School of Medicine
Professional Staff President, Children's Hospital of Pittsburgh of UPMC

Laurie A. Silva, PhD
Research Assistant Professor

Gwen Taylor, PhD
Research Instructor of Pediatrics

OVERVIEW OF DIVISION

The Division of Infectious Diseases provides consultation in the diagnosis and management of infectious diseases in hospitalized and outpatient children. The division consists of academically and clinically renowned faculty who contribute in many areas, including basic and clinical research, antimicrobial and vaccine development, microbial pathogenesis, outcomes research in clinical infectious diseases, hospital infection control and epidemiology, and transplant infectious disease. The division oversees an Antibiotic Stewardship Program, working with pharmacists to streamline and optimize antimicrobial use at Children's Hospital of Pittsburgh of UPMC. The goals of the program are to ensure that patients receive cost-efficient antimicrobials with the lowest risk of side effects and decreased risk for antimicrobial resistance. The division also supervises the Children's Hospital Infection Prevention Program, which works to develop guidelines to prevent hospital-acquired infections and manage potential outbreaks. The division conducts both practice-based and hospital-based clinical studies of infectious diseases, including surveillance of acute respiratory and gastrointestinal infections; new vaccines and antimicrobials; *Streptococcus pneumoniae*, cytomegalovirus (CMV), influenza, and other respiratory viral infections; epidemiology and clinical features of Lyme disease; and prophylaxis and treatment of infections in transplant patients. The division has several active basic researchers focused on microbial attachment and cell entry, immunity, and pathogenesis; lung immunobiology and inflammation; and vaccine development. Pathogens studied by investigators in the division include adenovirus, chikungunya virus (CHIKV), human metapneumovirus (MPV), influenza virus, *Mycobacterium tuberculosis*, reovirus, and rhinovirus.

CLINICAL ACTIVITIES

The Division of Infectious Diseases serves the pediatric population of Pittsburgh and the surrounding regions by providing consultation and treatment for children with rare, complicated, and difficult-to-treat infections. The service completed 3,582 consultative visits for inpatients and 875 for outpatients during fiscal year 2016. The Pediatric HIV Clinic provides ongoing care to HIV-infected children in Western Pennsylvania and the tristate region, as well as expert pre- and postnatal consultative services for HIV-infected pregnant women and their newborns.

In addition to treating children with complex infections, the Division of Infectious Diseases is a vital resource for health care providers and the public, both locally and nationally. Locally, the Division of Infectious Diseases is a collaborative partner with Pittsburgh Public Schools regarding influenza vaccination and with the Allegheny County Health Department on matters of communicable infectious diseases in the pediatric population of the Pittsburgh area, especially HIV and tuberculosis (TB). Faculty members routinely provide educational outreach to community health care providers in the tristate region, such as the Children's Institute of Pittsburgh, and educate the public about community-acquired and emerging pediatric infectious diseases through frequent interviews with local news channels, such as KDKA-TV and radio (for example, Middle East respiratory syndrome virus, otherwise known as MERS). Nationally, the faculty serves on committees that establish guidelines for institutions, such as the Centers for Disease Control and Prevention (CDC), the U.S. Food and Drug Administration (FDA), the Organ Procurement Transplant Network (OPTN)/United Network for Organ Sharing (UNOS), the American Society for Transplantation, the National Institutes of Health (NIH), the Pediatric Infectious Diseases Society, and the Society for Pediatric Research. Members of the faculty give invited talks at numerous meetings of medical and scientific societies, such as the American Association of Pediatrics, the Infectious Diseases Society of America, the American Society for Microbiology, the American Transplant Congress, and the Society for Pediatric Research.

RESEARCH AND OTHER SCHOLARLY ACTIVITIES

John V. Williams, MD

John V. Williams joined the division as chief in April 2015 after 12 years on the faculty at Vanderbilt University Medical Center. Williams is an international authority on the epidemiology, immunity, and pathogenesis of respiratory viral infections.

Williams is a graduate of the University of Virginia and completed medical school at the Medical College of Virginia/Virginia Commonwealth University. He trained in pediatrics at Children's Hospital of Pittsburgh/University of Pittsburgh and then in infectious diseases at Vanderbilt. During his fellowship, he began working on human MPV just after the virus was discovered. Since then, Williams' team has described the clinical features and epidemiology of MPV. Articles have been published in top journals, including the *Journal of Clinical Investigation*, the *Journal of Infectious Diseases*, *Nature Structural and Molecular Biology*, the *New England Journal of Medicine*, *Proceedings of the National Academy of Sciences*, and *PLOS Pathology*, among others. For this superb body of work on MPV, Williams won the 2014 E. Mead Johnson Award for Pediatric Research, the most prestigious research award in academic pediatrics. Williams has been very successful in extramural research funding, currently including R01, R21, and U01 grants.

Williams has extensive experience in other academic activities, including NIH study sections; leadership in national and international academic societies; and membership on the editorial boards of the *Journal of Infectious Diseases*, the *Journal of the Pediatric Infectious Diseases Society*, and the *Journal of Virology*. He has been an active mentor of students, residents, and fellows. He received the inaugural Caroline B. Hall Award for Translational Research from the Pediatric Infectious Diseases Society in 2015 and was instilled as the Henry L. Hillman Professor of Pediatric Immunology in 2017.

John V. Williams, MD
Chief, Pediatric Infectious Diseases

RESEARCH

The focus of Williams' research is the basic and clinical investigation of respiratory viruses. A major area of investigation is the immunity and pathogenesis of human MPV. MPV is a recently discovered paramyxovirus that is a leading cause of acute lower respiratory tract illness in infants and children worldwide. Williams published molecular epidemiologic studies establishing the importance of MPV, in the process isolating dozens of field strains collected over 20 years. His team developed rodent models, used those systems to study MPV immunity and pathogenesis, and showed that the fusion (F) protein is the sole determinant of antibody-mediated protection. Williams' group identified RGD-binding integrins as receptors for MPV and showed that MPV uses integrins to enter cells by endocytosis, a novel mechanism of entry for

this type of virus. His laboratory discovered that MPV and other acute respiratory viral infections cause impairment of lung $CD8^+$ T cells via PD-1 signaling, a pathway previously associated with chronic infections and cancer. Williams' laboratory has generated candidate vaccines and monoclonal antibodies against MPV and identified mechanisms by which MPV subverts the host innate immune response.

Williams also leads a large CDC-funded surveillance study of acute respiratory and gastrointestinal infections in children based at Children's Hospital of Pittsburgh, one of only seven sites nationally. He conducts collaborative research with clinical investigators at the University of Pittsburgh and international sites. He has participated in studies of respiratory virus epidemiology in North America, South America, the Middle East, and Africa. His group has published studies on coronaviruses, influenza virus, MPV, parainfluenza viruses, respiratory syncytial virus, and rhinoviruses in diverse populations.

Williams has active research projects in several areas.

- Epidemiology and burden of acute respiratory and gastrointestinal infections
- Effectiveness of influenza and rotavirus vaccines in children
- Mechanisms by which MPV and other respiratory viruses impair lung $CD8^+$ T cells
- Candidate vaccines and therapeutic monoclonal antibodies against MPV
- High-throughput screening for small-molecule inhibitors of MPV
- Human T-cell responses to MPV

The overarching goals of Williams' research on MPV are to elucidate mechanisms of viral pathogenesis, understand the contribution of host immune responses to pathogenesis, and guide the development of interventions against this important human pathogen.

ADVISORY COMMITTEE MEMBERSHIPS

- Scientific Review Group 08 ZHD1 DSR-K1 NIH Loan Repayment Program
- NIH MID-B B Study Section
- Scientific Advisory Board, Quidel
- Independent Data Monitoring Committee, GlaxoSmithKline
- Education Committee, American Society for Virology
- Chair, Fellowship Awards Committee, Pediatric Infectious Diseases Society
- Research Committee, Pediatric Infectious Diseases Society
- Chair, Young Investigator Coaching Program, Society for Pediatric Research

EDITORSHIPS

- Editorial Board, *Journal of Infectious Diseases*
- Editorial Board, *Journal of the Pediatric Infectious Diseases Society*
- Editorial Board, *Journal of Virology*

MAJOR LECTURESHIPS AND SEMINARS

- "Human MPV: The End of the Beginning," pediatric grand rounds, Emory University School of Medicine, Atlanta, Ga., 2016
- "Human MPV: From Holland to the Bronx," pediatric grand rounds, Children's Hospital at Montefiore, Albert Einstein College of Medicine, New York, N.Y., 2017
- "Violets Are Blue: Human MPV Pneumonia," pediatric grand rounds, Hassenfeld Children's Hospital of New York, NYU Langone Medical Center, New York, N.Y., 2017
- "Human MPV: Haciendo tos de Buenos Aires," invited talk, Fundación INFANT, Buenos Aires, Argentina, 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Alpha Omega Alpha, selective honorary society, by election
- Society for Pediatric Research, selective honorary society, by election
- American Association of Immunologists
- American Society for Virology
- Pediatric Infectious Diseases Society
- Infectious Diseases Society of America
- American Society for Microbiology
- American Association for the Advancement of Science

HONORS

- E. Mead Johnson Award in Pediatric Research, Society for Pediatric Research
- Vanderbilt Mary Ann and John H. Hash Award for Outstanding Teaching of Graduate Students in Microbiology and Immunology
- Inaugural Caroline B. Hall Lecture, Infectious Diseases Society of America
- Henry L. Hillman Endowed Chair in Pediatric Immunology

Brian T. Campfield, MD

RESEARCH

Brian T. Campfield joined the division as assistant professor of pediatrics in 2014, providing patient care and conducting research investigating the role of follistatin-like protein 1 (FSTL-1) in host defense and immunity, centered on the role of FSTL-1 in the lung.

Campfield is a graduate of the University of Virginia and completed medical school at the University of Pittsburgh. He completed his pediatrics residency and pediatric infectious diseases fellowship at the Children's Hospital of Pittsburgh of UPMC/University of Pittsburgh. During fellowship, he reported the first study of FSTL-1 in response to infection in the murine model of Lyme disease. Subsequently, under the mentorship of Jay Kolls, he identified a novel and critical role for FSTL-1 in lung homeostasis. That work is the basis of his NIH K08 Career Development Award, which was funded in 2015.

The Campfield Laboratory focuses on host-pathogen interaction in the lung, viewed through the lens of FSTL-1 function, with a focus on innate and adaptive immune responses. His laboratory has identified a role for FSTL-1 in regulation of IL-17 signaling pathways.

A Critical Role for FSTL-1 in Lung Homeostasis. This work aims to investigate the temporospatial expression of FSTL-1 in the lung and the effect of FSTL-1 loss in a murine model of lung inflammation and emphysema development. This study additionally aims to examine the role of interleukin 17RA and CCR2 signaling in FSTL-1-dependent lung disease. A further aim of this research is to determine whether the role of FSTL-1 in lung homeostasis is intrinsic to lung tissue or due to circulating FSTL-1 protein. Through this Career Development Award, Campfield receives mentorship from John V. Williams, Steven D. Shapiro, Prabir Ray, and Jay Kolls. Additional studies under way are investigating the role of FSTL-1 in acute lung injury, utilizing novel tools developed in his laboratory to identify FSTL-1 function at the cellular level. This research is funded through the spring of 2020.

Mechanisms of FSTL-1-Mediated Inflammatory Signaling. Cellular signaling pathways of FSTL-1-mediated inflammation are largely unknown. Campfield has developed several *in vitro* systems to assess the role of FSTL-1 in cellular signaling, employing genetic and molecular suppression, overexpression, and tagged expression. These studies have identified a novel function for FSTL-1 as a modulator of transcriptional regulation.

ADVISORY COMMITTEE MEMBERSHIPS

- Member, Pediatric Scientist Training Program Steering Committee, Children's Hospital of Pittsburgh of UPMC
- Founding member, Pittsburgh Pediatric Physician Scientist Peer Mentoring Group, Children's Hospital of Pittsburgh of UPMC, University of Pittsburgh School of Medicine
- Member, Infection Control Committee, Children's Hospital of Pittsburgh of UPMC
- Member, Physician Wellness Task Force, Children's Hospital of Pittsburgh of UPMC
- Member, Career Mentoring Program, University of Pittsburgh School of Medicine
- Interviewer, Residency Program, Children's Hospital of Pittsburgh of UPMC
- Interviewer, Infectious Diseases Fellowship Program, Children's Hospital of Pittsburgh of UPMC

MAJOR LECTURESHIPS AND SEMINARS

- "A Follistatin-Like Protein 1 Puzzle: IL-17, Inflammation, and the Lung," Child Health Research Center (K12) 10th Annual Retreat, Children's Hospital of Pittsburgh, Pittsburgh, Pa., April 2017
- "Follistatin-Like Protein 1 Modulates IL-17 Signaling Via IL-17RC Regulation in Stromal Cells," Immunology 2017, Washington, D.C., May 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Fellow, American Academy of Pediatrics
- Infectious Diseases Society of America
- Pediatric Infectious Diseases Society
- American Association of Immunology
- American Thoracic Society
- American Association for the Advancement of Science

HONORS

- Pediatric Loan Repayment Program Award, NIH
- Mentored Career Development Award, NIH
- Early Career Faculty Travel Award, American Association of Immunologists

Carolyn B. Coyne, PhD

RESEARCH

The Coyne Laboratory studies the pathways by which viruses cross cellular barriers and the mechanisms by which these barriers restrict viral infections. Research primarily focuses on the polarized epithelium that lines the gastrointestinal tract and placental trophoblasts, which comprise the primary cellular barrier of the human placenta. The laboratory focuses on delineating the pathways targeted

by RNA viruses (e.g., enteroviruses and flaviviruses) to promote their replication and spread. The work is highly multidisciplinary and encompasses aspects of cell biology, tissue engineering, immunology, and microbiology.

The Placental Barrier. The placenta is unlike any other human organ. Given its essential role in protecting the fetus, the placenta must function as a barrier and conduit between the maternal and fetal environments and serve as an active immunological tissue that responds to microbes present in maternal circulation. The research program asks two central questions: (1) What are the mechanisms by which the placenta restricts the vertical transmission of micro-organisms and (2) How do micro-organisms associated with congenital disease breach the placental barrier? The laboratory established a new and important paradigm that, in addition to its role as a physical barrier, the placenta is a dynamic and highly reactive chemical barrier that uses multiple classes of molecules, including type III interferons and microRNAs, to protect the fetus and maternal host from viral infections. However, several key questions remain, including: (1) Are there differences in the mechanisms employed by the placenta to restrict microbial access at different stages of gestation, (2) What mechanisms are used by the placenta to defend against non-viral pathogens, and (3) What is the influence of the systemic maternal immune response on placental antimicrobial defenses?

The Gastrointestinal Barrier. The human gastrointestinal (GI) tract is a complex organ, with an epithelial surface that provides a protective and immunological barrier in a complex and diverse microbial environment. Enteroviruses are leading causes of human infections worldwide, particularly in infants and children, and they infect primarily via the fecal-oral route. These viruses, which include poliovirus, coxsackievirus, echovirus, enterovirus D68 (EV-D68), and enterovirus 71 (EV71), are small, single-stranded RNA viruses belonging to the *Picornaviridae* family. The events that surround enterovirus infections of the human GI epithelium remain poorly understood. The research team recently developed two human models of the GI epithelium to better define enterovirus-GI interactions. These include a cell line–based three-dimensional model and a human primary stem cell–derived enteroid model. Ongoing research in the laboratory is focused on defining the mechanisms by which enteroviruses bypass the GI barrier to initiate infection using these organotypic three-dimensional cell models, with a specific focus on the cell biological and immunological events associated with enterovirus infections of the GI tract.

Cellular Pathways Targeted by RNA Viruses to Promote Their Replication. RNA viruses usurp a variety of host cell pathways to facilitate their replication. Studies focus on identifying the pathways targeted by RNA viruses (including enteroviruses and flaviviruses) to promote their replication and spread. An obligate step in the life cycle of positive-sense RNA viruses is the formation of membrane-enriched organelles, termed replication organelles, which provide the structural support for viral replication. Multiple mechanisms have been proposed for the generation of these membranes, including manipulation of the host autophagic pathway, a process that removes damaged organelles via the formation of double membrane-bound vesicles. Current studies in the laboratory are focused on the identification and characterization of novel regulators of host cell autophagy and on the identification of mechanisms employed by RNA viruses to specifically modulate the host autophagic pathway.

Coyne has active research grants in the following areas. Enterovirus infection of polarized epithelial cells (National Institute of Allergy and Infectious Diseases [NIAID], NIH R01AI081759)

- The response of placental cells to teratogenic viruses during human pregnancies (25 Club of Magee-Womens Hospital)
- Primary human trophoblasts and the transfer of viral resistance (National Institute of Child Health and Development [NICHD], NIH R01 HD075665)
- The actin cytoskeleton and innate immune signaling (Burroughs Wellcome Fund)

ADVISORY BOARD AND COMMITTEE MEMBERSHIPS

Intramural:

- Faculty Executive Committee, University of Pittsburgh School of Medicine
- Executive Committee, Program in Microbiology and Immunology
- Promotions Committee, Department of Pediatrics

Extramural:

- Standing member, NIAID Virology A, NIH Study Section
- Member, Special Emphasis Review Panel, Rapid Assessment of Zika Virus Complications (R21), NIAID
- Member, Scientific Organizing Committee, Viruses 2018: Breakthroughs in Virus Biology, Barcelona, Spain
- Member, Microbe Program Committee, American Society for Microbiology

EDITORIAL BOARDS

- Editorial Board, *Journal of Virology*
- Editorial Board, *Virology*
- Editor, mini-reviews, *Journal of Virology*
- Opinions editor, *PLoS Pathogens*
- Editorial Board, *mBio*

MAJOR LECTURESHIPS AND SEMINARS

- Invited speaker, Maternal-Fetal Crosstalks Keystone Symposia, Washington, D.C., 2017
- Invited speaker, NIH Conference on Innate Immunity: Sensing, Signaling, and Selection, Washington, D.C., 2017
- Invited speaker, Vector-Borne Viruses Symposium, Rocky Mountain Laboratories, Hamilton, Mont., 2017
- Invited speaker, "How To": A Career Planning Satellite Symposium for Junior Virologists, American Society for Virology, Madison, Wis., 2017
- "Crossing the Placenta," invited speaker and convener, special symposium, Microbe 2017, American Society for Microbiology, New Orleans, La., 2017
- Keynote speaker, Mount Sinai–New York University School of Medicine Joint Training Program in Virus-Host Interactions, New York Academy of Medicine, New York, N.Y., 2017
- Invited speaker, Gordon Research Conference on Viruses and Cells, Il Ciocco, Italy, 2017
- Invited speaker, "Cell Biology of Pathogen Entry Into Host Cells," Microbiology Society annual meeting, Edinburgh, United Kingdom, 2017
- Invited speaker, mini-symposium on pathogenic human viruses, Duke University, Durham, N.C., 2017
- Invited speaker, Placenta Satellite Symposium, annual meeting of the Society for Reproductive Investigation, Orlando, Fla., 2017
- Invited speaker, 32nd Congress of the International Society for Advancement of Cytometry, Boston, Mass., 2017
- Virology Seminar Series, Harvard University, Boston, Mass., 2017
- Indiana University Department of Microbiology, Indianapolis, Ind., 2017
- St. Jude Children's Research Hospital, Children's Infection Defense Center, Memphis, Tenn., 2017
- University of Illinois–Urbana Champaign Department of Microbiology, 2017
- University of Maine Department of Molecular and Biomedical Sciences, Orono, Maine, 2017
- University of North Carolina at Chapel Hill Department of Microbiology and Immunology, Chapel Hill, N.C., 2017
- University of New Mexico Department of Microbiology, Albuquerque, N.M., 2017
- University of Wisconsin Department of Microbiology, Madison, Wis., 2017
- Western University, London, Ontario, Canada, 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Society for Microbiology
- American Society for Virology
- Program Planning Committee: Abstract Selection, American Society of Virology
- Communications Committee, American Society of Virology
- Standing member, NIAID Virology A, NIH
- Study Section Member, Scientific Organizing Committee: Viruses 2018: Breakthroughs in Virus Biology, Barcelona, Spain
- Member, Microbe Program Committee, American Society for Microbiology

Terence S. Dermody, MD

RESEARCH

Terence Dermody is a virologist with interests in viral pathogenesis and vaccine development. He has focused mainly on reovirus, an important experimental model for studies of viral encephalitis in infants and young children, and CHIKV, a mosquito-borne virus that causes massive epidemics of febrile arthritis.

The work in the Dermody laboratory has encompassed several interrelated themes, including the structural basis of viral attachment and cell entry, mechanisms of genome replication and packaging, patterns of cell signaling and gene expression occurring in response to viral infection, mechanisms of virus-induced apoptosis and its significance in the viral life cycle, and the role of viral receptor distribution and utilization in disease pathology. The laboratory also is developing viral vectors for oncolytic and vaccine applications.

Dermody has active research grants in several areas.

- The molecular basis of reovirus pathogenesis (NIAID, NIH R01 AI038296-22 to -25)
- Cell biology of reovirus infection (NIAID, NIH R01 AI032539-21 to -25)
- Viral infections and celiac disease pathogenesis (National Institute of Diabetes and Digestive and Kidney Diseases, NIH R01 DK098435-01 to -04)
- Reovirus attachment mechanisms (NIAID, NIH R01 AI118887-01 to -05)
- CHIKV replication and pathogenesis (NIAID, NIH R01 AI123348-01 to -05)

- Molecular basis of pediatric disease (NICHD, NIH K12 HD052892-10)
- Basic and translational research training for Children's Hospital of Pittsburgh pediatric fellows (NICHD, NIH T32 HD071834-04 to -05)
- International Congress of Virology travel grant (NIAID, NIH R13 AI131504-01)

ADVISORY BOARD AND COMMITTEE MEMBERSHIPS

Intramural:

- Chair Management Committee, UPMC
- University of Pittsburgh Physicians Council of Clinical Chairs, UPMC
- Board of Trustees, Children's Hospital of Pittsburgh of UPMC
- Executive staff, Children's Hospital of Pittsburgh of UPMC
- Chair, Research Strategic Planning Committee, Children's Hospital of Pittsburgh of UPMC
- Faculty Executive Committee, University of Pittsburgh School of Medicine
- Search Committee, Center for Vaccine Research
- Chair, Appointments and Promotions Committee, University of Pittsburgh Department of Pediatrics
- Chair, Research Advisory Committee, University of Pittsburgh Department of Pediatrics

Extramural:

- Chair, Virology Division, International Union of Microbiological Societies
- Reoviridae Study Group, International Committee on Taxonomy of Viruses
- Board of Governors, American Academy of Microbiology
- Board of Directors, Burroughs Wellcome Fund
- External Advisory Board, Medical Scientist Training Program, University of Cincinnati College of Medicine
- External Advisory Committee, MD-PhD Training Program, University of Florida College of Medicine
- Scientific Advisory Committee, Autophagy Modulators as Novel Broad-Spectrum Anti-Infective Agents, Center of Excellence for Translational Research, Washington University, St. Louis, Mo.
- External Advisory Board, Medical Scientist Training Program, Duke University School of Medicine
- External Advisory Board, Department of Microbiology, Icahn School of Medicine at Mount Sinai
- External Advisory Committee, Molecular Dissection of Norovirus Replication and Pathogenesis to Develop Therapeutics, Baylor College of Medicine
- Scientific Program Vice chair, 17th International Congress of Virology, Singapore, 2017

EDITORIAL BOARDS

- Editor, *Journal of Virology*
- Spotlight Editor, *Journal of Virology*
- Member, Biology, Microbiology Faculty, Virology Section, Faculty of 1000 Editor, *mBio*
- Associate Editor, *Annual Review of Virology*

MAJOR LECTURESHIPS AND SEMINARS

- "Formation and Function of Reovirus Replication Organelles," Forefront of Virus-Host Interactions Conference, Basel, Switzerland, 2016
- "A Fascination With Viral Homing," Medical Scientist Training Program, University of North Carolina at Chapel Hill, Chapel Hill, N.C., 2016
- "Homing of Reovirus From Intestine to Brain: It Takes Two Receptors," Department of Immunology and Microbial Sciences, Scripps Research Institute, La Jolla, Calif., 2016
- Facilitator, Infectious Diseases Workshop, Cornell Leadership Program for Veterinary Students, New York State Veterinary College, Cornell University, Ithaca, N.Y., 2016
- "How a Virus Travels From Intestine to Brain," Department of Molecular and Biomedical Sciences, University of Maine, Orono, Maine, 2016
- "A Neural Targeting Receptor for Reovirus," Department of Infectious Diseases, Heidelberg University, Heidelberg, Germany, 2016
- "Homing of Reovirus From Intestine to Brain: It Takes Two Receptors," keynote speaker, Immunobiology and Immunopathogenesis Symposium, Department of Immunobiology, University of Arizona, Tucson, Ariz., 2016
- "Following the Journey of Reovirus From Intestine to Brain," plenary speaker, American Society for Microbiology annual meeting, Boston, Mass., 2016
- "A Way Forward for Physician-Scientists," keynote speaker, Medical Scientist Training Program annual retreat, University of Pittsburgh School of Medicine, Pittsburgh, Pa., 2016
- "A Way Forward for Physician-Scientists," keynote speaker, Medical Scientist Training Program annual retreat, Pennsylvania State University College of Medicine, State College, Pa., 2017
- "AOA and the Healthcare Transformations of the 21st Century," David Seegal Alpha Omega Alpha Lecture, Columbia University College of Physicians and Surgeons, New York, N.Y., 2017
- "CHIKV: A Global Emerging Viral Threat," Harold C. Neu Lecture, Department of Medicine, Columbia University College of Physicians and Surgeons, New York, N.Y., 2017

- "Cell Biology of Reovirus Infection," keynote speaker, Virus-Host Interactions Symposium, Mount Sinai–New York University School of Medicine Joint Training Program in Virus-Host Interactions, New York, N.Y., 2017
- "Function of Reovirus Replication Organelles," Department of Microbiology and Immunology, Stanford University School of Medicine, Stanford, Calif., 2017
- "A Neural Targeting Receptor for Reovirus," Division of Infectious Diseases, Department of Medicine, Columbia University College of Physicians and Surgeons, New York, N.Y., 2017
- "CHIKV: A Global Emerging Viral Threat," Department of Microbiology and Immunology, University of Arkansas for Medical Sciences, Little Rock, Ark., 2017
- Facilitator, Infectious Diseases Workshop, Cornell Leadership Program for Veterinary Students, New York State Veterinary College, Cornell University, Ithaca, N.Y., 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Fellow, Infectious Diseases Society of America
- Fellow, American College of Physicians
- American Society for Clinical Investigation
- Society for Pediatric Research
- Association of American Physicians
- Fellow, American Academy of Microbiology
- American Pediatric Society
- Fellow, American Association for the Advancement of Science
- Fellow, Pediatric Infectious Diseases Society

HONORS

- D.C. White Research and Mentoring Award, American Society for Microbiology, 2016

Michael Green, MD, MPH

RESEARCH

The major focus of Michael Green's laboratory has been on the epidemiology of antimicrobial resistance in children in hospital and community settings. His laboratory is currently funded through multiple NIH grants and contracts to carry out the work. Green's interest in antibiotic resistance is further reflected in the efforts of the Children's Hospital of Pittsburgh of UPMC Antimicrobial Stewardship Program (ASP), which he directs. More recently, he has expanded his efforts regarding his longstanding interest in infections in immunocompromised hosts, including transplant patients and children receiving immune suppression for autoimmune disease.

Short-Course Therapy for Urinary Tract Infection (UTI) in Children (SCOUT). This NIH-funded study is a multicenter, randomized, controlled trial to determine whether short-course antimicrobial therapy (five days) is non-inferior to standard-course antimicrobial therapy (10 days) in children with UTI. Green's role in the study is to evaluate serial surveillance cultures from participants to track potential differences in the development of antibiotic resistance in isolates of *Escherichia coli* and *Klebsiella pneumoniae*. Resistant isolates will undergo evaluation for mechanism of resistance. Green is also performing surveillance screening for colonization with carbapenem-resistant *Enterobacteriaceae* to provide a sense of the prevalence of colonization in children with these increasingly important gram-negative bacteria.

Difference in Infecting and Colonizing Enterobacteriaceae *from Short-Course Versus Standard Therapy.* This R21 study (principal investigator [PI]: Scott Weissman, University of Washington) is an NIH-funded ancillary study whose goal is to carry out a detailed molecular analysis of bacterial isolates recovered as part of the SCOUT study to evaluate the impact of antibiotic drug and length of therapy on the indigenous flora and the likelihood of antimicrobial resistance and to assess the presence of virulence factors associated with UTI. Although accrual of specimens and funding for this study were completed in spring 2015, analysis of results from this ancillary study cannot begin until accrual for the primary study is completed.

Type 1 Diabetes TrialNet/University of South Florida (Data Coordinating Center). Green continues to serve as one of two infectious disease consultants for the NIH Type I Diabetes TrialNet. This clinical trial network evaluates potential interventions aimed at modifying the natural history of insulin-dependent diabetes mellitus in children. Many of these interventions include immunosuppressive regimens and are therefore potentially associated with the development of opportunistic infections. Green's role as infectious disease consultant remains formally integrated into the protocol-development and approval process for TrialNet. Additionally, the work has resulted in several infectious diseases–related publications, and the infectious diseases group within TrialNet continues to develop ancillary studies to evaluate the impact of immunosuppression in subsequent TrialNet protocols.

Improving Diagnosis and Treatment of Pediatric Candidiasis. The goal of this NIH-funded, multicenter study is to develop new evidence-based treatment guidelines for invasive candidiasis in children. It retrospectively collects data to compare the effectiveness of echinocandin versus

amphotericin B or triazole antifungal therapy for pediatric invasive candidiasis, as well as to characterize the incidence of all invasive candidiasis infections in pediatric patients. Green has served as the site PI since Children's Hospital of Pittsburgh's involvement in this study began in 2015.

Fungal Biomarkers for Diagnosis and Response to Therapy for Pediatric Candidemia. The goals of this NIH-funded, prospective, multicenter study include defining the operating characteristics of fungal biomarker assays in pediatric patients at high risk for developing invasive candidiasis, determining how fungal biomarkers change in response to antifungal treatment, and creating a biobank of blood samples from pediatric patients who are at high risk for or have invasive candidiasis for future testing of fungal biomarker assays and development of new fungal biomarker assays. Green has served as the site PI since Children's Hospital of Pittsburgh's participation in this study began in 2015.

A Multicenter Prospective Study of Human Adenovirus Infection and Disease in Pediatric Human Stem Cell Transplant (HSCT) Recipients. The goal of this NIH-funded, multicenter Broad Agency Announcement (BAA) is to prospectively study the epidemiology of adenovirus after pediatric HSCT during the first six months after HSCT for development of adenovirus infection and disease. Green is the site co-PI for this study.

A Phase II, Multicenter, Prospective, Randomized, Double-Blind Study to Assess the Clinical and Antiviral Efficacy and Safety of Nitazoxanide for the Treatment of Norovirus in Hematopoietic Stem Cell and Solid Organ Transplant Recipients > 6 Years of Age. The purpose of this NIH-funded, phase II, multicenter, prospective, randomized, double-blind study is to assess the clinical and antiviral efficacy and safety of nitazoxanide for the treatment of acute and chronic norovirus in hematopoietic stem cell and solid organ transplant recipients. Green is site co-PI for this study.

NIH/University of Pittsburgh Clinical and Translational Science Institute. The goal of the Clinical and Translational Science Institute is to provide the clinical research infrastructure for medical scientists who conduct patient-oriented, research-related care for disorders of infancy, childhood, and adolescence. Green's current role continues to be to provide individual support and workshops on informed consent to new clinical investigators and their research staff.

ADVISORY COMMITTEE MEMBERSHIPS

- Chair, Infection Control Committee, Children's Hospital of Pittsburgh of UPMC
- Director, ASP, Children's Hospital of Pittsburgh of UPMC
- Executive Committee, Antimicrobial Stewardship Committee, Pediatric Infectious Diseases Society
- Transplant Infections Organizing Working Group, Pediatric Infectious Diseases Society
- Chair, Infectious Disease Committee, International Pediatric Transplant Association
- Ex-officio member, Patients Safety Advisory Group (subcommittee), Operations and Safety Committee, Organ Procurement and Transplantation Network, UNOS
- Non-Tenure-Stream Promotion Committee, University of Pittsburgh School of Medicine
- Antimicrobial Drug Advisory Committee, FDA
- Member, Transplantation Society's International Consensus Conference on Cytomegalovirus in Solid Organ Transplantation (third edition)
- Member, Sub-Board on Pediatric Infectious Diseases, American Board of Pediatrics

EDITORSHIPS

- Associate editor, *Pediatric Transplantation*
- Editorial Board, *Liver Transplantation*
- Associate editor, *Journal of the Pediatric Infectious Diseases Society*

MAJOR LECTURESHIPS AND SEMINARS

- "Epstein-Barr Virus (EBV) and Post-Transplant Lymphoproliferative Disorder (PTLD): Prevention, Diagnosis, and Management," transplant grand rounds, University of Pittsburgh School of Medicine, Pittsburgh, Pa., October 2016
- "EBV and PTLD: Essential Concepts and Breaking News," pediatric grand rounds, Cleveland Clinic, Cleveland, Ohio, November 2016
- "Infectious Complications of Organ Transplantation in Children," Cleveland Clinic, Cleveland, Ohio, November 2016
- "EBV and PTLD: Essential Concepts and Breaking News," infectious diseases grand rounds, University of Pittsburgh School of Medicine, Pittsburgh, Pa., February 2017
- "Infections in Children on Extrinsic Immune Suppression: Advice to the Primary Care Physician and Non-Transplant Subspecialist," Amos Christie Annual Lectureship, Vanderbilt pediatric grand rounds, Nashville, Tenn., May 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Pediatric Society
- American Society for Microbiology
- International Pediatric Transplantation Association
- Fellow, Infectious Diseases Society of America
- Fellow, American Society of Transplantation
- Society for Pediatric Research
- Pediatric Infectious Diseases Society

HONORS

- *Best Doctors in America*, Woodward/White, Inc.
- Best Doctors, *Pittsburgh Magazine*, 2017
- Clinical Science Established Investigator Award, American Society of Transplantation
- Visiting professor, Department of Pediatrics, Vanderbilt University
- Visiting professor, pediatric infectious diseases, Cincinnati Children's Hospital
- Visiting professor, pediatric infectious diseases, Cleveland Clinic
- Visiting professor, pediatric infectious diseases, Department of Pediatrics, University of Virginia School of Medicine
- 2017 Amos Christie Annual Lectureship, Department of Pediatrics, Vanderbilt University

Philana Ling Lin, MD, MSc

RESEARCH

Philana Ling Lin's research program is focused on the immunologic mechanisms of *M. tuberculosis* infection, the bacterium that causes TB. Her work has examined the role of various immunological factors (e.g., CD4 T cells, CD8 T cells, and tumor necrosis factor) involved in the host response to control both primary and latent *M. tuberculosis* infection. She has shown that positron emission tomography–computed tomography (PET-CT) characteristics of TB can be used to predict outcome soon after infection, as a modality of early treatment response and risk of reactivation. Her work has shown that latent infection is a spectrum of disease and is associated with risk to reactivation, which has important implications in human TB. Her recent studies are focused on co-infection with TB and simian immunodeficiency virus (SIV), a surrogate for HIV. The overall goals of her research program are to improve the current understanding of disease progression, identify predictors of infection outcome, distinguish risk factors for reactivation after latency, develop better strategies for vaccine development, and devise more targeted methods of curing disease from reservoirs and improving treatment outcomes. Her research has been funded by the Bill and Melinda Gates Foundation; American Lung Association; Center for AIDS Research; Otis Foundation; University of Pittsburgh MIDAS National Center of Excellence; and the NIH, including current R01, R21, and R03 funding formats. She has published in high-impact journals, including *Nature Medicine*, the *Journal of Clinical Investigation*, *Proceedings of the National Academy of Sciences*, and *PLOS Pathogens*. She is the program director for the Pediatric Infectious Diseases Fellowship Training Program at Children's Hospital of Pittsburgh of UPMC and has mentored a number of medical students, residents, and fellows on scholarly projects.

Her ongoing research activities include the following.

- Developing blood transcriptional signatures of reactivation risk
- Recognizing innate and early adaptive host responses in the airway associated with infection outcome
- Characterizing reservoirs of reactivation during pre-existing latent infection and subsequent SIV infection
- Identifying immune mechanisms of reactivation TB during SIV-TB co-infection
- Distinguishing immunologic and PET-CT imaging risk factors of treatment relapse
- Determining immunologic mechanisms of increased susceptibility to severe TB during SIV infection with and without antiretroviral treatment
- Examining the impact of TB on SIV viral diversity
- Identifying TB vaccine candidates to advance into human clinical trials
- Surveilling the epidemiology of pediatric invasive pneumococcal infection after universal conjugate pneumococcal vaccination

ADVISORY COMMITTEE MEMBERSHIPS

- Program director, Pediatric Infectious Diseases Fellowship Training Program, Children's Hospital of Pittsburgh of UPMC
- Scholarship Oversight Committee, Children's Hospital of Pittsburgh of UPMC
- Antimicrobial Stewardship Committee, Children's Hospital of Pittsburgh of UPMC
- Infection Prevention Committee, Children's Hospital of Pittsburgh of UPMC
- Ad hoc, AIDS-Associated Opportunistic Infections and Cancer Study Section, NIH ad hoc, Collaborative Research at NIH Clinical Center (U01), NIH ad hoc, Wellcome Trust

EDITORSHIPS

- Editorial Board, *Infection and Immunity*

MAJOR LECTURESHIPS AND SEMINARS

- "Animal Models" speaker, "Smoldering TB: Exploring the Full Spectrum of Infection with TB," scientific symposium, American Thoracic Society, Washington, D.C., May 2017
- "Immunopathogenic Mechanisms of *Mycobacterium Tuberculosis*," Symposium on Synthetic Immunity, Sante Fe, N.M., July 2017
- "TB: Lessons From a Jigsaw Puzzle," NIH Symposium on Molecular Mechanisms and Immune Consequences of Pathogen Reservoirs: Battling Unseen Enemies, Bethesda, Md., September 2017
- "TB Infection: Building a Framework for Eradication," sponsored by NIH and Harvard Medical School Center for Global Health Delivery, Dubai, United Arab Emirates, September 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Fellow, American Academy of Pediatrics
- Pediatric Infectious Diseases Society
- Infectious Diseases Society of America
- Society for Pediatric Research
- American Association of Immunology
- American Society for Microbiology

Marian Michaels, MD, MPH

RESEARCH

Marian Michaels' work has focused on the following major areas: congenital/newborn infections; infections in immunocompromised hosts, including patients receiving solid-organ and bone marrow transplants, children on immunosuppressive medications, and children with HIV; immunizations; more general pediatric infectious diseases, including acute viral infections; and antibiotic treatment and antimicrobial stewardship.

Michaels is funded by the CDC through a subcontract to the Department of Family Medicine (PI: Richard Zimmerman) to evaluate influenza vaccine efficacy in hospitalized patients. The CDC funded five sites across the country for ambulatory vaccine efficacy, but only two sites (Pittsburgh under Michaels and the University of Michigan) were funded for the inpatient study. The division was the only pediatric program, and the work led to the funding listed below as part of the pediatric consortium. As an outgrowth of this study, Michaels is analyzing data that should lead to three papers this coming year: influenza vaccine efficacy for inpatients compared to ambulatory patient groups, attitudes toward influenza vaccination and plans for future vaccines, and a survey on decision making for obtaining respiratory viral panels.

Michaels will be co-PI on a CDC-sponsored grant submission along with John Williams to be a center for the New Vaccine Surveillance Network. The study conducts prospective, population-based surveillance for acute gastroenteritis and acute respiratory illness among hospitalized children to elucidate the epidemiology of acute infections in the pediatric population and to evaluate vaccine efficacy, including inpatient influenza vaccine efficacy.

The "Natural History of CMV-Related Hearing Loss and Feasibility of CMV Screening as Adjunct to Hearing Screening in the Newborn (CHIMES)" study was funded through the National Institute on Deafness and Other Communication Disorders. The study is the largest congenital CMV screening study to date. Although the funding period has ended, the study continues to be a source of publications and presentations at international meetings (e.g., the Pediatric Academic Societies meeting).

Michaels continued her NIAID-supported congenital CMV work as the site PI for "A Phase III, Randomized, Placebo-Controlled, Blinded Investigation of Six Weeks Versus Six Months of Oral Valganciclovir Therapy in Infants With Symptomatic Congenital Cytomegalovirus Infection" (Collaborative Antiviral Study Group 112). The study showed longer therapy to be superior for symptomatic infants with congenital CMV infection. The study has led to two further investigations. The first is sponsored by NIAID as a BAA, with Michaels as a subcontractor to the University of Alabama, and will address treatment of asymptomatic infants with congenital CMV to prevent hearing loss. The second BAA funded by NIDCD will be a treatment trial for infants with hearing loss as the sole manifestation of congenital CMV infection. Michaels is co-investigator overseeing treatment and clinical management of all enrolled infants (site PI: David Chi, Otolaryngology).

The collaboration with the University of Alabama has been quite fruitful, and Michaels is the site PI for two other studies as a subcontractor regarding infants and infection. One is a BAA to look at the pharmacokinetics of ganciclovir for very premature infants. The other is a BAA to do long-term follow-up on newborns with herpes encephalitis. Michaels is acting as a no-cost consultant for a third University of Alabama study at Magee-Womens

Hospital that is performing rapid screening of women in labor for herpes simplex virus shedding. It is anticipated that after this first screening study, a larger study will follow the infants born to those women. Michaels will serve as the co-PI with Harold Wiesenfeld.

Michaels has been the site PI for a study out of Duke University looking at hospital-acquired, ventilator-associated pneumonia in children at risk in the pediatric intensive care unit. Study enrollment has just completed, with more than 100 subjects. The study seeks to inform practice for preventing pneumonia, as well as future treatment strategies.

Outpatient Early-Intervention Services With Respect to HIV Disease for Children, Women, Youth, and Families (2H76HA00079). The major goals of this project are planning, capacity building, and providing care and preventive strategies for HIV infection in children and adolescents in Western Pennsylvania. The work continues to support the HIV pediatric center, providing a 50% full-time-equivalent social worker dedicated to caring for these families and 10% of a faculty salary to oversee the care of these children. Starting in July 2013, the funding source moved from Pitt to UPMC.

HIV Early-Intervention Project for Children, Youth, Women, and Families (2H12HA23029-07). The major goal of this project is to improve access to care and supportive services for HIV-infected women, infants, children, and youth. The grant supports the HIV pediatric center, supporting a 25% full-time-equivalent social worker dedicated to caring for these families and 10% of a faculty salary to oversee the care of these children. Starting in July 2013, the funding source moved from Pitt to UPMC.

Optimizing Outcomes After Pediatric Heart Transplant (National Heart, Lung, and Blood Institute, NIH 1P50HL74732-01). The major goal of this project is to better understand the occurrence of allosensitization in pediatric heart transplant recipients and develop strategies for treatment. Michaels' role has been to develop uniform monitoring protocols to be used across centers regarding infections, prevention, and treatment and to review and analyze results.

Michaels and Green serve as site co-PIs for a BAA out of Children's Hospital of Philadelphia to better understand adenovirus infections in pediatric stem cell recipients. The study has just started enrollment.

Green and Michaels are co-PIs for a BAA out of Northwestern University that will be a double-blinded, placebo-controlled treatment study for immunocompromised hosts infected with norovirus. Enrollment was anticipated to start early in 2018.

Antimicrobial Stewardship. Michaels serves on the steering committee for Children's Hospital of Pittsburgh's ASP. One of the division's recently graduated pediatric residents completed a scholarly project analyzing Children's Hospital's use of piperacillin/tazobactam; the group examined the appropriateness of initially starting and subsequently continuing its use beyond day three. Results of the study were published in the *Journal of the Pediatric Infectious Diseases Society* in 2015. In addition, a manuscript describing Michaels' novel ASP Therapeutic Drug Monitoring Program was recently published in the *Journal of the Pediatric Infectious Diseases Society*.

Michaels collaborates with the Pennsylvania Department of Health's "Get Smart" program, designed to reduce antimicrobial use, particularly in children. As part of the program, Michaels mentored Tabitha Reefer, a graduate student in the University of Pittsburgh's Graduate School of Public Health. Reefer and Michaels collaborated with Michael Morowitz, MD, Department of Surgery, to assess a new strategy to manage children with both complicated and uncomplicated appendicitis to ensure safe management with shorter courses of intravenous and oral antibiotics. Reefer recently moved, and a surgical research fellow will continue as the PI of the project. Michaels will remain a collaborator and mentor.

Michaels' studies sponsored by pharmaceutical companies follow.

GSK/Zanamivir. This study is an open-label, phase II, multicenter, single-arm study to evaluate the safety and tolerability of intravenous zanamivir in hospitalized subjects with laboratory-confirmed influenza infection. Michaels is the site PI. The study has reached full enrollment. Michaels enrolled five patients, beyond the goal of three per site. As one of the more successful investigators, Michaels reviewed the data and co-wrote a manuscript on outcomes, which was presented at 2016 ID Week and accepted for publication in *Pediatrics*.

Merck/Cubist, Daptomycin Study of Bacteremia. This is a single-blinded study of daptomycin versus standard therapy for children with *S. aureus* bacteremia. Michaels has enrolled seven subjects, beyond the goal of five. Pittsburgh is among

the highest-enrolling sites in the United States. The study is close to completing enrollment, and the company has requested that Michaels be among the coauthors for manuscripts stemming from the study.

Optimer: *Fidaxomicin Treatment of* C. Difficile. This was an open-label study testing the safety and efficacy of fidaxomicin for children with *C. difficile* colitis. Michaels enrolled seven subjects, and Pittsburgh was the second-highest-enrolling site. The study is closed and has been presented in abstract form at ID Week. A manuscript titled "A Safety and Pharmacokinetic Study of Fidaxomicin in Children With *Clostridium difficile*–Associated Diarrhea: A Phase IIa Multicenter Clinical Trial" received a positive peer review from the *Journal of the Pediatric Infectious Diseases Society* and was sent back with minor revisions. The authors are waiting for a response regarding acceptance.

Pfizer/Anidulafungin in Children with Invasive Candidiasis. This study examines the safety, pharmacokinetics, and pharmacodynamics of the antifungal anidulafungin in a pediatric population. The study is ongoing but is likely to finish enrollment soon.

Michaels also is involved in some unfunded work. A productive collaboration has developed over the past several years among pediatric infectious disease physicians in the United States who are interested in answering research questions that cannot be answered at a single site. The collaboration grew out of the St Jude's/Pediatric Infectious Diseases Society annual meeting and has led to collaborations looking at respiratory viral infections (paper in revision). In addition, the members have finished entering data on *C. difficile* infections in the population to look at the epidemiology, and they anticipate presenting and publishing in 2018. Finally, the group just launched an investigator-initiated project in collaboration with industry to look at CMV T-cell responses that should prove very exciting and lead to preliminary data for future NIH support.

Michaels has mentored many medical students and residents on scholarly projects, including students investigating problems with families refusing vaccinations, infections in immunocompromised hosts, antimicrobial stewardship, and CMV and EBV infection.

ADVISORY COMMITTEE MEMBERSHIPS

- Board member, American Society of Transplantation
- Data Safety Monitoring Committee, Division of Allergy, Immunology, and Transplantation, NIAID
- Sub-board, Pediatric Infectious Diseases Society, American Board of Pediatrics
- Chair, sub-board, Credentials Committee, Pediatric Infectious Diseases Society, American Board of Pediatrics
- Vice chair, Disease Transmission Advisory Committee, Organ Procurement and Transplantation Network, UNOS
- Chair, Pharmacy and Therapeutics Committee, Children's Hospital of Pittsburgh of UPMC
- Chair, Innovative Use Medication Committee, Pharmacy and Therapeutics Committee, Children's Hospital of Pittsburgh
- Pharmacy and Therapeutics Committee, UPMC
- Chair, Pediatric Pharmacy and Therapeutics Committee, UPMC
- Pharmacy and Therapeutics Antibiotic Advisory Committee, UPMC
- Pharmacy and Therapeutics Pain Management Advisory Committee, UPMC
- Ad hoc co-chair, Infection Control Committee, Children's Hospital of Pittsburgh of UPMC
- Clinical Quality Oversight Committee, Children's Hospital of Pittsburgh of UPMC
- Solutions for Patient Safety Leadership Committee, Children's Hospital of Pittsburgh of UPMC
- Director, Pediatric HIV Center, Children's Hospital of Pittsburgh of UPMC
- Scholarship Oversight Committee, Children's Hospital of Pittsburgh of UPMC
- Medical advisor, laboratory affairs, Planned Parenthood of Western Pennsylvania

EDITORSHIPS

- *Pediatric Transplantation*
- *Transplant Infectious Disease Journal*
- Pediatric Infectious Diseases Publication Committee, *Journal of the Pediatric Infectious Diseases Society*

MAJOR LECTURESHIPS AND SEMINARS

- "Infections and Extracorporeal Membrane Oxygenation (ECMO)," Eighth Annual Neonatal and Pediatric ECMO Educational Conference, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., August 2016
- "Emerging Infections After Pediatric Solid Organ Transplantation," invited speaker, Fifth World Congress on Pediatric Gastroenterology, Hepatology, and Nutrition, Montreal, Canada, October 2016
- "Pediatric Antimicrobial Stewardship: Lessons Learned," invited speaker, One Health Seminar, Annual Get Smart Week 2016, State College, Pa., November 2016
- "Increased-Risk Donors in the Era of a Drug Overdose Epidemic," Organ Donation Symposium, Finger Lakes Donor Recovery Network and University of Rochester Medical Center, Rochester, N.Y., May 2017

- Chair, session on infectious diseases, International Pediatric Transplant Association, Barcelona, Spain, May 2017
- Speaker, CMV update plenary session, International Pediatric Transplant Association, Barcelona, Spain, May 2017
- Chair, session on EBV, International Pediatric Transplant Association, Barcelona, Spain, May 2017
- "Increased Risk Donors: Putting It All Together," Fellows Symposium on Transplantation, American Society of Transplantation, Grapevine, Texas, September 2017
- "Matching the Patient to the Donor in Pediatric Transplantation," Fellows Symposium on Transplantation, American Society of Transplantation, Grapevine, Texas, September 2017
- "The Microbiome, Infection, and Alloimmunity," Fellows Symposium on Transplantation, American Society of Transplantation, Grapevine, Texas, September 2017
- "Rapid-Fire Transplant ID Cases,"panelist/speaker, Meet the Professor, annual ID Week, Infectious Diseases Society of America, San Diego, Calif., October 2017
- "And the Infectious Disease Consultant Says, 'What?'" panelist/speaker, annual ID Week, Infectious Diseases Society of America, San Diego, Calif., October 2017
- "Infectious Issues of Xenotransplantation," annual ID Week, Infectious Diseases Society of America, San Diego, Calif., October 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Alpha Omega Alpha Honor Medical Society
- American Academy of Pediatrics
- Infectious Diseases Society of America
- Pediatric Infectious Diseases Society
- American Society of Transplantation
- American Society for Microbiology
- Society for Pediatric Research
- International Pediatric Transplantation Association
- International Society of Heart and Lung Transplantation

HONORS

- *Best Doctors in America*, Woodward/White, Inc., annually since 2003
- Best Doctors, *Pittsburgh Magazine*, annually since 2008
- Elected to the Board of the American Society of Transplantation

Andrew Nowalk, MD, PhD

RESEARCH

Lyme Disease Epidemic in Western Pennsylvania. Andrew Nowalk's primary focus is the study of the Lyme disease epidemic that has been affecting Western Pennsylvania since 2005. He continues to study the local epidemiology of the Western Pennsylvania Lyme disease epidemic through collaborative projects at Children's Hospital (examining local and national rates of Lyme carditis with Cheyenne Beach, MD, in Pediatric Cardiology; variant presentations of neurologic Lyme disease associated with cranial nerve palsies with Catalina Cleves-Bayon, MD, in Child Neurology; and atypical arthritis presentations with Sriram Ramgopal, MD, in Pediatric Emergency Medicine), as well as with groups at Carnegie Mellon University and Indiana University of Pennsylvania, investigating the geographic spread of Lyme into Western Pennsylvania.

Molecular Characterization of Acinetobacter *Resistance*. Nowalk is a co-investigator with Yohei Doi, MD, PhD, on a project examining proteomic changes in *Acinetobacter baumannii* during evolution of colistin resistance. *Acinetobacter* is a leading cause of multidrug-resistant, hospital-acquired infection. The focus is bacterial adaptation that leads to these phenotypes.

Genomics and Proteomics of Renal Scarring in Pediatric UTI. In collaboration with Nader Shaikh, MD, Nowalk and Doi's laboratory acts as the sample repository for the study titled "Steroids to Actively Reduce Renal Scarring." This NIH-supported grant tests whether corticosteroids decrease rates of renal scarring in children with febrile UTI. Nowalk provides reference laboratory support for the study and supervises the molecular analyses of the bacterial and human samples. The study has expanded into analysis of the microbiome of the subjects, and the laboratory provides data to correlate bacterial virulence factors and biome constituents that may predispose patients to severe scarring in UTI.

Probabilistic Disease Surveillance and Predictive Models of Infectious Disease. In conjunction with Michael Wagner, MD, of the Department of Biomedical Informatics, Nowalk has acted as a consultant to this National Library of Medicine R01, which examines the potential for natural language processing (NLP) algorithms for the early identification of infectious disease epidemics. The joint project, shared with Intermountain Health Care in Salt Lake City, Utah, examines NLP-based detection of respiratory viral infections such

as influenza using a novel instrument to detect cases from electronic health record data from emergency room visits. The project has yielded two recent manuscripts in *PLoS One* and *Applied Clinical Informatics*.

ADVISORY COMMITTEE MEMBERSHIPS

- Clinical Resource Management Committee, Children's Hospital of Pittsburgh of UPMC
- Pediatric Intern Selection Committee, Children's Hospital of Pittsburgh of UPMC
- Professional staff past president, Children's Hospital of Pittsburgh of UPMC
- Member, Medical Executive Committee, Children's Hospital of Pittsburgh of UPMC
- Member, Credentials Committee, Children's Hospital of Pittsburgh of UPMC
- Program director, Pediatric Residency Program, Children's Hospital of Pittsburgh of UPMC
- Advisory dean, University of Pittsburgh School of Medicine

EDITORSHIPS

- Associate editor, *Atlas of Pediatric Physical Diagnosis*, seventh edition

MAJOR LECTURESHIPS AND SEMINARS

- "Lyme Disease: Dos and Don'ts from the Pittsburgh Epidemic," medical grand rounds, Excela Westmoreland Hospital, Greensburg, Pa., September 2016
- "Mosquito-Borne Infections From Arbovirus to Zika," family medicine grand rounds, UPMC St. Margaret's Hospital, Pittsburgh, Pa., September 2016
- "Lyme Disease: Dos and Don'ts from the Pittsburgh Epidemic," First Annual John Govi, MD, Memorial Conference, Excela Latrobe Hospital, Latrobe, Pa., December 2016
- "Mosquito-Borne Infections and Lyme Disease," UPMC Shadyside Urgent Care, Pittsburgh, Pa., March 2017
- "Common and Emerging Pediatric Infections," Pittsburgh Continuing Medical Education Conference and UPMC 44th Refresher Course in Family Medicine, Pennsylvania Academy of Family Physicians, Pittsburgh, Pa., March 2017
- "Tickborne Disease Update," Southwestern Pennsylvania Chapter, National Association of Pediatric Nurse Practitioners, Pittsburgh, Pa., October 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Society for Pediatric Research
- American Society for Microbiology
- National Physicians Alliance
- Pediatric Infectious Diseases Society
- Infectious Diseases Society of America
- American Academy of Pediatrics
- Alpha Omega Alpha Honor Medical Society
- American Medical Student Association

HONORS

- Best Doctors, *Pittsburgh Magazine*, 2014–2017
- UPMC Patient Satisfaction Award, 2016
- Excellence in Education Award: lecturer, University of Pittsburgh School of Medicine Curriculum Colloquium, 2017

Laurie A. Silva, PhD

RESEARCH

Laurie Silva joined the division as a research scientist in the Dermody laboratory in May 2016. She leads the team focused on CHIKV. She also serves as the Rangos BSL3 facility manager and is involved in overseeing the renovation of the BSL3 laboratory on the ninth floor of Rangos Research Building.

Silva's research focuses on replication and pathogenesis of CHIKV, an arthritogenic alphavirus that causes debilitating musculoskeletal inflammatory disease. It is transmitted by *Aedes albopictus* and *Aedes aegypti* mosquitoes and is capable of epidemic urban transmission between mosquitoes and humans. Since 2004, CHIKV has caused epidemics involving millions of persons and expanded into new areas, including Europe, the Middle East, the Pacific region, and most recently in the Americas. No licensed vaccines or specific therapeutics are available for this globally important pathogen.

The CHIKV research program seeks to discover mechanisms by which host cell factors contribute to CHIKV replication and pathogenesis. Silva's main project focuses on elucidating the role of COP-I trafficking pathway proteins in the replication cycle of CHIKV. In a genome-wide siRNA screen to identify host factors that are required for efficient CHIKV replication, COP-I coatomer and regulatory factors were some of the top putative hits. Validation experiments using golgicide A, a specific inhibitor of the COP-I regulatory factor GBF1, supported a function for this host factor in the replication cycle of CHIKV. Further experiments suggest that GBF1 may be acting in a non-canonical manner to enhance CHIKV replication. Microscopy experiments suggest that GBF1 localizes to sites of CHIKV RNA

synthesis early in infection and may be critical for the establishment or maturation of viral replication factories. Current experiments aim to determine the precise stage in the CHIKV replication cycle GBF1 is required and whether GBF1 is necessary for the virus to form viral replication factories in the infected cell. Genetic experiments to determine which domains of GBF1 contribute to CHIKV replication are also under way. Collectively, the experiments will illuminate mechanisms by which an important host factor is usurped by CHIKV, provide support for a noncanonical role for GBF1 in viral replication and perhaps host cell function, and guide intervention strategies based on the host target.

Silva mentors two graduate students, Anthony Lentscher and Nicole McAllister, and a research assistant, Adam Brynes. Other CHIKV projects to which Silva contributes include the following.

- Identification of specific host cell glycosaminoglycans to which CHIKV virions bind
- Elucidation of the role of glycosaminoglycans in the pathogenesis of CHIKV
- Validation of other host proteins that were putative hits for CHIKV proviral factors in a high-throughput siRNA screen
- Determination of specific tissues within an infected host that contribute to the immunopathology of CHIKV
- Candidate vaccines for CHIKV

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Society for Virology

Gwen Taylor, PhD

RESEARCH

Gwen Taylor joined the division as a research instructor and laboratory manager in the Dermody Laboratory in May 2016. She serves as team lead for Mammalian reoviruses, neurotropic viruses that are highly virulent in young mammals.

Taylor focuses on reovirus pathogenesis and aims to elucidate the process of NF-κB-dependent apoptosis by reovirus in the central nervous system, to identify the contribution of cell type–specific NF-κB signaling to reovirus neural pathogenesis, and to define factors under NF-κB control that mediate apoptotic cell death in the central nervous system. Ultimately, this research enhances the understanding of pathogen-host interactions and may lead to the development of broadly applicable therapeutics for neurotropic virus infections.

Taylor mentors five graduate students, Judy Brown, Jon Knowlton, Christopher Lee, Danica Sutherland, and Paula Zamora, who also work on reovirus. Taylor provides experimental guidance on all other reovirus projects, including the following.

- Defining functions of reovirus nonstructural protein σNS
- Elucidating pathways used by reovirus to exit infected cells
- Defining the contribution of glycan engagement to reovirus neurologic disease
- Determining the post-attachment functions and associated conformational changes of the reovirus σ1 attachment protein
- Elucidating the mechanism of reovirus assortment
- Determining the viral factors involved in celiac disease

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Society for Virology

THREE-YEAR BIBLIOGRAPHY

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DIVISION OF MEDICAL GENETICS

Mission

The mission of the Division of Medical Genetics is:

- To provide state-of-the-art diagnostic evaluations, testing, and genetic counseling for children and families with genetic, teratogenic, metabolic, and dysmorphic conditions at Children's Hospital of Pittsburgh of UPMC and throughout the entire UPMC health system
- To provide or recommend therapeutic interventions to maximize clinical outcomes for each child
- To provide excellence in teaching
- To advance knowledge in pediatric genetics and metabolism through ongoing research and collaborations

FACULTY

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Professor of Human Genetics
Division Chief, Medical Genetics
Director, Center for Rare Disease Therapy

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OVERVIEW OF THE DIVISION

In 2017, the Division of Medical Genetics clinical team consisted of 10 MDs, nine clinical genetic counselors, two metabolic dieticians, three clinical nurses, two research nurses, and three genetic counselors dedicated to special projects. Three additional tenure-track and four non-tenure-track research faculty provide a strong basic science foundation. The division's goal is to provide state-of-the-art medical genetics services, as well as to generate new information and rapidly incorporate that information into care for children. Basic and clinical research is an intrinsic goal of the division. Faculty members are internationally recognized and play highly visible roles in national and international academic societies and government advisory panels. They participate in graduate-level education within the University of Pittsburgh's School of Medicine and Graduate School of Public Health. The division integrates genetic counseling duties with counselors based in the Division of Laboratory Medicine and the Center for Rare Disease Therapy.

In addition to basic and clinical research, the Medical Genetics team is devoted to the provision of medical genetic diagnostic and counseling services. Members of the division serve as consultants, manage inpatients with inborn errors of metabolism, and provide diagnostic and counseling services to additional Children's Hospital genetic disease clinics, including neurofibromatosis, cleft lip and palate, cystic fibrosis, and fragile X clinics, as well as to providers throughout Western Pennsylvania, eastern Ohio, and northern West Virginia. The clinics for inborn errors of metabolism, lysosomal storage disease, mitochondrial disease, and PKU provide care for patients with chronic, rare metabolic disorders and function as the follow-up center for Pennsylvania's state-mandated newborn screening. The division's physicians provide genetic services for the Hereditary Telangiectasia Center of Excellence at UPMC and are active in the newly constituted Center for Rare Disease Therapy at Children's Hospital. In 2017, the Neurodevelopmental Research Program joined the division, bringing an expanded focus on neurodegenerative disorders.

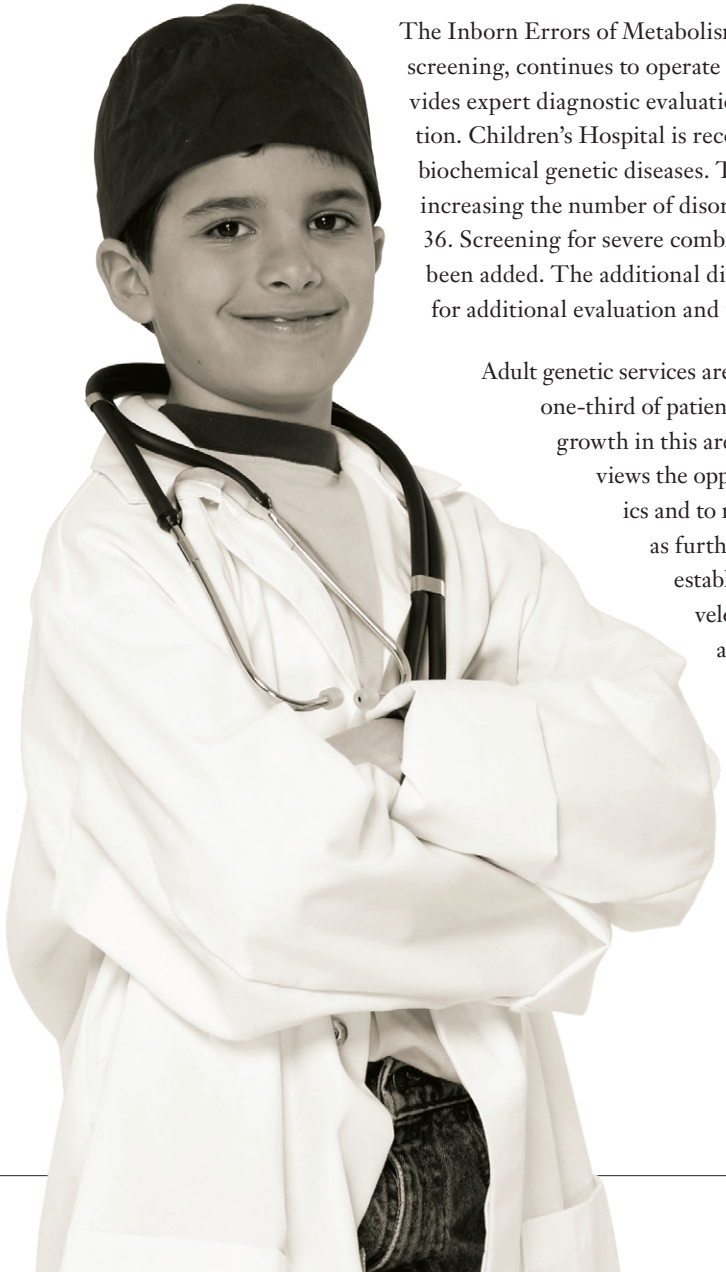
The physicians utilize a team approach to evaluate children thought to have metabolic disorders as well as congenital anomalies, teratogenic disorders, and/or genetic disorders, and they strive to engage patients and families with necessary

support and specialists to optimize clinical and emotional outcomes. Families are provided with state-of-the-art laboratory diagnostic testing. Genetic counseling is provided to optimize the outcomes of children, to provide information to help parents make individualized decisions for family planning, and to initiate support from the community. The division strives to provide realistic hope to families and to provide services in a compassionate and empathetic manner.

CLINICAL ACTIVITIES

An eight-year history of faculty growth in the division has kept pace with increasing patient volume. There were 1,941 visits (981 new and 960 returns) in 2017. Additional visits to the PKU and Program for the Study of NDRD subspecialty clinics totaled 178 and 32, respectively. 1,003 inpatient consultations were performed. The division remains committed to seeing critical inpatients within 24 hours and provides 24/7 on-call coverage for genetic emergencies. Noncritical consultations may be referred to the outpatient clinic upon patient discharge.

The division's specialty clinics offer treatment for lysosomal storage disease, PKU, mitochondrial disorders, and connective tissue disorders. The Neurogenetics Clinic, staffed in conjunction with the Division of Child Neurology, provides comprehensive team care for patients with a known diagnosis of neurogenetic disease, including mitochondrial myopathies. A Connective Tissue Disorders Clinic serves as a regional referral center for patients with such disorders, and the division participates in a nationally recognized Cutis Laxa Clinic. A Plain Communities Translational Medicine Program focuses on the extensive Amish and Mennonite population in Western Pennsylvania and eastern Ohio.



The Inborn Errors of Metabolism Clinic, including its follow-up center for state newborn screening, continues to operate at capacity and is among the largest in the country. It provides expert diagnostic evaluations and ongoing metabolic management to a national population. Children's Hospital is recognized as a national center for the study and treatment of biochemical genetic diseases. The state of Pennsylvania recently passed new legislation increasing the number of disorders in the standard newborn screening panel from seven to 36. Screening for severe combined immune deficiencies and congenital heart disease has been added. The additional disorders are inborn errors warranting referral to the division for additional evaluation and treatment.

Adult genetic services are an integral part of the division's clinical mission, and nearly one-third of patients are adults. The division's leadership anticipates continued growth in this area, with two internal medicine faculty members. The division views the opportunity to demonstrate growing capabilities in adult genetics and to represent Children's Hospital and the University of Pittsburgh as further evidence of its leadership role across UPMC. A proposal to establish a formal adult genetics clinic in internal medicine is in development. In addition, several outside institutions have inquired about possible outreach or telemedicine clinics. The division also looks to explore outreach activities as staffing increases.

DIVISION HIGHLIGHTS

Research and teaching constitute major activities within the division. All division faculty members participate in research and receive at least some external funding (see individual faculty member entries). Division faculty members were awarded more than $7.78 million in research funding in 2017: $5.03 million in faculty grants and $2.75 million in clinical trials funding.

The entire faculty and many genetic counselors participate in and have salary support from externally

funded projects. The division anticipates continued and expanding support. The division has more than 50 protocols approved by the Institutional Review Board. Two PhD students from the Department of Human Genetics are currently performing their thesis research under the direction of Medical Genetics faculty. Multiple genetic counseling students are pursuing thesis projects. The division's faculty and counselors are much in demand for a wide range of continuing medical education, as well as for lectures for fellows and residents. Members of the division have published 60 articles in peer-reviewed journals in the past three calendar years.

Jerry Vockley's service at the state, national, and international levels raises the prominence of the division. He is chair of Pennsylvania's Newborn Screening and Follow-Up Technical Advisory Board, and he serves on the Department of Health's Committee on Lead Exposure and its Birth Defects Registry. He served on the Therapeutics Committee of the American College of Medical Genetics and Genomics (ACMG) and served as co-chair for the PKU clinical guideline work group. Vockley chairs the research career development ("K") award Metabolomics Study Section for the National Institutes of Health (NIH). Vockley is founder and chair of the International Network for Fatty Acid Oxidation Research and Management (INFORM). He is a distinguished editor for the NIH Director's New Innovative Editorial Board. Vockley is past president of the Society for Inherited Metabolic Disorders and is founder and director of its national training program for medical genetics residents, the North American Metabolic Academy. This important program reaches half of the medical genetics residents in the country annually, as well as many from Canada and Mexico. He serves on the European Registry and Network for Intoxication Type Metabolic Diseases, for which he co-chairs the working group on isovaleric acidemia guidelines.

Georgianne L. Arnold is the chair of the Maintenance of Certification Committee for the American Board of Medical Genetics and Genomics. Robert Nicholls and Eric Goetzman have both received major new research grants in the past year.

The division runs an Accreditation Council for Graduate Medical Education (ACGME) training program for the newly established Medical Biochemical Genetics Fellowship, one of only seven in the country.

RESEARCH AND OTHER SCHOLARLY ACTIVITIES

Vockley, MD, PhD

RESEARCH

Jerry Vockley's research focuses on mitochondrial energy metabolism, branched chain amino acid metabolism, inborn errors of metabolism, and development of novel therapies for inborn errors of metabolism. He also has a strong interest in the genetics of the Plain Communities (Amish and Mennonites) and in identification of novel genetic disorders in the general population.

Characterization of a Multifunctional Fatty Acid Oxidation Complex. Fatty acid β-oxidation (FAO) and oxidative phosphorylation (OXPHOS) are key pathways involved in cellular energetics. Genetic disorders of FAO and OXPHOS are among the most frequent inborn errors of metabolism. Patients with deficiencies of either FAO or OXPHOS often have clinical or biochemical findings indicative of a disorder of the other pathway. This study examined the physical and functional interactions between these pathways. It provided evidence of a multifunctional FAO complex within mitochondria that is physically associated with OXPHOS supercomplexes and promotes metabolic channeling.

Jerry Vockley, MD, PhD
Division Chief, Medical Genetics

Very-Long-Chain Acyl-CoA Dehydrogenase (VLCAD) Deficiency. More than 100 cases of VLCAD deficiency have been documented in the literature, with three different disease phenotypes. A severe infant-onset form is characterized by acute metabolic decompensation with hypoketotic hypoglycemia, dicarboxylic aciduria, liver dysfunction, and cardiomyopathy. A second form of the disease presents later in infancy or childhood but has a milder phenotype without cardiac involvement. The third form is of adolescent or adult onset and is dominated by muscle dysfunction often induced by exercise. Not surprisingly, a study found that children with the severe phenotype tended to have null mutations (71% of identified alleles in these patients), whereas patients with the

two milder forms of the disease were more likely to have missense mutations (82% and 93% of identified alleles for the milder childhood form and the adult form, respectively). Nevertheless, a few missense mutations were clearly associated with the severe phenotype. Although the data suggest that missense mutations in VLCAD might obviate clinical symptoms due to some degree of residual activity, no correlation was seen between the mutations identified and residual VLCAD activity in fibroblasts. Moreover, the function effects of few of the known VLCAD missense mutations have been directly characterized.

Team researchers have previously used prokaryotic expression systems to express, purify, and characterize the biochemical properties of several ACAD enzymes. Several have been crystallized and studied by X-ray diffraction, yielding informative three-dimensional models. The team has used its prokaryotic expression system to study six previously missense mutations described in VLCAD-deficient patients (T220M, V243A, R429W, A450P, L462P, and R573W). T220M and V243A are the most frequently reported missense mutations in VLCAD-deficient patients. R429W and R573W are among the few missense mutations believed to result in the severe clinical phenotype. A450P and L462P are located in the C-terminal domain unique to VLCAD and ACAD9. Characterization of purified wild-type, A450P, and L462P VLCAD proteins confirmed the long-held assumption that the C-terminus plays a key role in mitochondrial membrane association. The prokaryotic system developed will greatly facilitate investigation of VLCAD structure and function. Funding for this project is included in the above-referenced grant.

Based on this progress, the laboratory is developing therapeutic agents for treatment of long-chain fatty acid oxidation disorders. Several potential drugs have already been patented and are moving toward clinical trials.

ACAD9. Mitochondrial β-oxidation of long-chain fatty acyl-CoAs is a primary metabolic pathway for maintenance of energy homeostasis and body temperature. It also recycles carbons from many long-chain fatty acids for lipid synthesis. Little is known about the mechanistic role of the latter in the pathogenesis of symptoms in genetic defects of β-oxidation, and its derangement may, in part, explain features of these disorders, such as neurological dysfunction and acute respiratory distress syndrome, which respond poorly to treatment with alternative energy sources. VLCAD is considered the dominant long-chain acyl-CoA dehydrogenase (LCAD) in energy generation in human muscle and heart cells. In contrast, this study provides evidence that acyl-CoA dehydrogenase 9 (ACAD9) and LCAD more likely function in lipid recycling and synthesis in human brain and lung, respectively, given their unique substrate utilization and tissue distribution pattern. Furthermore, the team has identified a new genetic deficiency of LCAD presenting with congenital surfactant deficiency. This disorder represents the first in an α, β-oxidation enzyme primarily involved in lipid recycling or synthesis, revealing a new mechanism of pathogenesis in human disease. Funding for this project by the NIH has been renewed, and a supplement through the economic stimulus grant program has been received.

Human ACAD10. In the last half of the 20th century, the incidence of type 2 diabetes mellitus (T2DM), previously unrecognized in the Pima Indians, began to rise. Multiple factors were postulated to be responsible, including environmental factors, such as diet and resultant obesity, along with a number of genetic determinants. ACAD10 was one of 30 genes examined after demonstrating a significant signal for diabetes in a genome-wide association study. To characterize the physiologic role of *ACAD10* in intermediary metabolism and its possible link to T2DM, the researchers have characterized an *ACAD10* gene trap mouse model. Aging animals become obese on a normal diet and develop insulin-resistant hyperglycemia in response to an intraperitoneal glucose challenge. Tissue and blood acylcarnitine profiles are similar to those previously described for adult humans with T2DM. The findings identify *ACAD10* deficiency as a new monogenic cause of T2DM in mice and provide valuable insight into its potential role in the development of T2DM in Pima Indians.

A New Disorder in Sterol Metabolism. Defects in cholesterol synthesis result in a wide variety of symptoms, from neonatal lethality to the relatively mild dysmorphic features and developmental delay found in Smith-Lemli-Opitz syndrome (SLOS). The team identified mutations in *SC4MOL* as the cause of a newly recognized autosomal recessive syndrome that includes psoriasiform dermatitis, arthralgias, congenital cataracts, microcephaly, and developmental delay. This gene encodes a sterol-C4-methyl-oxidase, catalyzing demethylation of C4-methylsterols in the cholesterol synthesis pathway. C4-methylsterols are members of the meiosis-activating sterols (MAS) family of molecules. MAS are ligands of the liver X receptors α and β, which are important in regulating not only lipid transport in epider-

mis but also the innate and adaptive immunity. They were first found in high concentration in testis and ovary cells and play roles in meiosis activation. The team found that MAS affect cell proliferation in both skin and blood. They also found that inhibition of sterol-C4-methyl-oxidase significantly altered immune regulation in immunocytes. Deficiency of *SC4MOL* represents a new biochemical defect in the cholesterol synthesis pathway, the clinical spectrum of which remains to be defined.

Sterol Rare Disease Consortium. Vockley is the site principal investigator for a new, unique clinical research consortium (the Sterol and Isoprenoid Diseases Research Consortium, or STAIR) to study a group of diseases bound by common biochemistry, impact on health, and rarity: cerebrotendinous xanthomatosis (CTX), hyperimmunoglobulinemia D with periodic fever syndrome (HIDS), Niemann-Pick disease type C (NPC), sitosterolemia, Sjögren-Larsson syndrome (SLS), and SLOS. STAIR activities will be performed by a team of investigators chosen for their clinical research strengths and resources, diverse geographic access to potential research subjects, and the commitment of their institutions to support the consortium. In five years, STAIR will conduct two major clinical studies (a longitudinal natural history study of NPC and a therapeutic trial to evaluate the efficacy of antioxidant therapy in SLOS) and six pilot research studies involving patients with SLS, SLOS, CTX, HIDS, or sitosterolemia. Together with the intramural NIH program, the consortium will support a full-scale training program in the field of sterol and isoprenoid diseases and share its resources and data with the NIH Rare Diseases Clinical Research Network. Participating institutions include Oregon Health and Science University (OHSU), Eunice Kennedy Shriver National Institute of Child Health and Human Development, Children's Hospital of Pittsburgh of UPMC, Cincinnati Children's Hospital Medical Center, University of Nebraska Medical Center, and University of Manitoba (Canada). OHSU will be the administrative home of the consortium. Patient-support organizations (Smith-Lemli-Opitz/RSH Foundation, Hide and Seek Foundation, Ara Parseghian Medical Research Foundation, Dana's Angels Research Trust, Foundation for Ichthyosis and Related Skin Types, and United Leukodystrophy Foundation) will participate in consortium activities. STAIR will foster multidisciplinary clinical research, promote training and education, and support projects to explore promising leads in the mechanisms, diagnostics, and treatments of sterol and isoprenoid diseases. This grant is in its third year of funding from the NIH.

A Pig Model of PKU. Phenylalanine hydroxylase (PAH) deficiency, traditionally known as PKU, results in accumulation of phenylalanine (PHE) in the blood of affected individuals and was the original motivation for population-based newborn screening. This project is developing a miniature pig model for greater genetic homology and more clinical relevance. The new model system will elucidate biomedical bases, facilitating development of therapeutic approaches, especially for mental retardation and neurological and neuropsychological features. Using bioinformatics and phylogenetic comparison to humans, the researchers initially assembled the entire pig *Pah* gene encoding a 452 amino acid enzyme and confirmed high expression of PAH in pig liver and kidney. Furthermore, they successfully targeted deletions and inversions of the *Pah* gene using a CRISPR/Cas9 RNA-guided nuclease approach. Studies over the first eight months of the National PKU Alliance funding period have utilized that *in vitro* model system and successfully optimized the genome editing reagents and mutation-detection assays for the pig *Pah* locus. Working with Missouri collaborators, the researchers have shown that the CRISPR gRNAs function *in vivo* in pig pre-implantation embryos, and a pregnancy has been obtained from embryo transfers of genome-edited embryos (~ 35% modified alleles). Affected animals have now been identified, and clinical characterization is proceeding.

Development of a Home PHE Meter. Early identification of PKU and dietary treatment prevent neurological devastation, but neurodevelopmental and psychological problems are regularly diagnosed even in patients who are identified early and treated continuously. The ACMG recently published a clinical treatment guideline that recognizes the difficulty of lifelong compliance to therapeutic regimens. The guideline reports that nearly all adolescents and adults have blood PHE levels that are out of the recommended therapeutic range, leading to diminished executive function and other neurologic and neuropsychiatric problems. To improve PKU patient outcomes, the ACMG guideline says: "Better tools and strategies are required to optimize care for the individual and improve long-term outcomes." Vockley has recently been awarded two NIH grants to develop and test a home PHE meter for use in treatment of this disease.

Characterizing the Burden of Genetic Disease in Old Order Amish. The Old Order Amish communities (Plain People) of North America have altered health risks that stem from unbalanced population sampling of European founders followed by genetic drift in derivative generations. The population effects have resulted in a high prevalence

of specific genetic disorders that vary from the general population and from each other. Several characteristics of those communities facilitate genetic analysis. Most isolates keep excellent historical and genealogical records. Due to their sociologic and/or geographic isolation, there is usually little or no migration into the group, and the members of the group exhibit relatively homogeneous lifestyles. Large nuclear families are frequent, which provides adequate numbers of affected and unaffected siblings within a sibship for blood samples. The primary genetic advantage, however, results from the interaction of two overlapping phenomena: the founder effect and inbreeding. In collaboration with Ghaloul-Gonzalez, Vockley has developed a new program to characterize the genetic variability between the Amish population in Mercer County and Amish populations in other counties by doing whole-exome and mitochondrial DNA sequencing. This will be crucial to determining the phenotype and frequency of other known and unknown genetic disorders in the populations. This project will allow the researchers to characterize the genetic disease load in Old Order Amish of Mercer County and identify disorders that can benefit from early treatment.

Metabolic Imbalance in Treatment-Resistant Depression. Treatment-refractory depression, defined as depression that has no response to three or more maximum-dose treatment trials of adequate duration, is a devastating clinical problem with significant morbidity and mortality that affects at least 15% of adolescents and adults with depression. Its etiology remains unclear. Several metabolic disorders affect neurotransmitter pathways and are associated with psychiatric symptoms, including depression. The team recently published a report of a sentinel patient with severe and unremitting depression and multiple suicide attempts who was unresponsive to pharmacotherapy or electroconvulsive therapy. A neurometabolic evaluation identified a severe deficiency of all metabolites of biopterin. Treatment with sapropterin, a BH4 analog, led to a dramatic remission of his depression and suicidal ideation that has lasted four years and is still maintained. This finding triggered an exploratory case-control trial in which researchers found 24 of 40 additional patients with treatment-refractory depression to have central nervous system neurometabolic abnormalities. Additional therapeutic options were identified for 23 of the 24 patients based on their metabolic findings. Fourteen patients were identified as having cerebral folate deficiency, and subsequent treatment with folinic acid resulted in a sustained improvement of depressive symptoms in 11 of the individuals. The experience has allowed streamlining of the experimental testing protocol in this population. None of the current tools aimed at developing personalized strategies for the treatment of depression (e.g., functional neuroimaging, pharmacogenetics) would have identified the defects or led to effective therapy. The research continues, funded by a generous donor to the Children's Hospital of Pittsburgh of UPMC Foundation.

Clinical Research. Vockley continues to coordinate a vigorous program in clinical research for the treatment of inborn errors of metabolism. Expanded detection of inborn errors of mitochondrial fatty acid oxidation via tandem mass spectrometry has placed them among the most frequently identified errors. Vockley formed and leads INFORM, which provides a collaborative framework for clinicians and investigators to exchange information on the disorders and their global effect on metabolism. Two medications for fatty acid oxidation disorders that were developed in the Vockley laboratory have reached phase II and III U.S. Food and Drug Administration trials, including triheptanoin, the first drug in development to treat long-chain fatty acid oxidation disorders. Additional diseases under study include lysosomal storage diseases, disorders of sterol metabolism, disorders of the urea cycle, and abnormalities of bone mineralization.

STUDY SECTIONS

- Review Panel, NIH Pioneer Award
- Review Panel, NIH Young Innovators Award
- Ad hoc panels to review Big Data project, Human Mutant Cell Repository Application, NIH
- Chair, NIH K Award Metabolomics Study Section
- Basic to Clinical Collaborative Research Program, University of Pittsburgh

ADVISORY COMMITTEE MEMBERSHIPS

- Collaborating partner, European Registry and Network for Intoxication Type Metabolic Diseases
- Chair, Therapeutics Committee, ACMG Work Group on Evidence-Based Clinical Practice Guidelines, ACMG
- Founder and chair, International Network for Fatty Acid Oxidation Disorders Research and Management
- Chair, INFORM annual meeting, 2017
- Scientific Advisory Board, United Mitochondrial Disease Foundation
- Chair, Pennsylvania State Newborn Screening Advisory Committee
- Member, Pennsylvania State Birth Defects Registry Committee
- Member, Pennsylvania State Lead Screening Committee

- Medical advisor, Organic Acidemia Family Foundation
- Medical advisor, Fatty Acid Oxidation Family Support Group
- Medical advisor, Saving Lives Through Screening Foundation
- Director's Young Investigator Award Second Tier Review Group, NIH ad hoc review committee, Director's Award, NIH, 2017

EDITORSHIPS

- Assistant editor, *Molecular Genetics and Metabolism*
- Founder and managing editor, *North American Metabolic Academy*
- Communicating editor, *Journal of Inherited Metabolic Disease*

MAJOR LECTURESHIPS, SEMINARS, AND TEACHING

University of Pittsburgh:

- School of Medicine Biochemistry and Molecular Medicine course lecturer
- Graduate School of Public Health Biochemical and Molecular Genetics course lecturer
- Medical genetics and pediatric resident lectures

National:

- Course creator and director, North American Metabolic Academy, a weeklong course on inborn errors of metabolism given to medical genetics residents from across the United States and Canada. The course has trained more than half of the medical genetics residents nationally in the past four years.
- "Positive Response to Infliximab in a Patient with VLCAD Deficiency," ACMG annual meeting, Phoenix, Ariz., 2017
- "Newborn Screening for Heavy Metals," Newborn Screening Translational Research Network annual meeting, Baltimore, Md., 2017

International:

- Course faculty, academy course on fatty acid oxidation defects, Society for the Study of Inborn Errors of Metabolism, Lyon, France
- "Triheptanoin in the Treatment of Long-Chain Fatty Acid Oxidation Disorders: Report of Two FDA Phase II Trial Results," Portuguese Society of Metabolic Disorders annual meeting, Lisbon, Portugal, 2017
- "Fatty Acid Oxidation Update," plenary lecture, International Congress on Inherited Metabolic Disorders, Rio De Janeiro, Brazil, 2017
- "Results from a 78-Week Single-Arm, Open-Label Phase II Study to Evaluate UX007 in Pediatric and Adult Patients With Moderate to Severe Long-Chain Fatty Acid Oxidation Disorders," International Congress on Inherited Metabolic Disorders, Rio De Janeiro, Brazil, 2017
- "Phase II Long-Term Pegvaliase Treatment for Adults with PKU: Updated Year 5 Safety and Efficacy Data From the PAL-003 Extension," International Congress on Inherited Metabolic Disorders, Rio De Janeiro, Brazil, 2017
- "Endoplasmic Reticulum-Mitochondria Crosstalk and Redox Homeostasis Disruption in VLCAD-Deficient Fibroblasts," International Congress on Inherited Metabolic Disorders, Rio De Janeiro, Brazil, 2017
- "Antioxidant Therapy as an Adjunct for Treatment of Long-Chain Fatty Acid Oxidation Disorders," International Congress on Inherited Metabolic Disorders, Rio De Janeiro, Brazil, 2017
- "Novel Mechanisms of Pathogenesis in Mitochondrial Trifunctional Protein Deficiency: Implications for Clinical Outcome and Treatment," International Congress on Inherited Metabolic Disorders, Rio De Janeiro, Brazil, 2017
- "Elucidating the Mitochondrial Architecture of Branched-Chain Amino Acid Metabolism Enzymes," International Congress on Inherited Metabolic Disorders, Rio De Janeiro, Brazil, 2017
- "PKU Research Update," Asia-Pacific PKU Summit, Osaka, Japan, 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- ACMG
- American Society of Human Genetics
- American Academy of Pediatrics
- American College of Pediatrics
- American Association for the Advancement of Science
- Society for the Study of Inborn Errors of Metabolism
- Society for Inherited Metabolic Disorders
- American Society for Clinical Investigation

HONORS/AWARDS

- Haworth Visiting Professor, University of Manitoba and Children's Hospital of Manitoba, April 2017
- Boston Children's Hospital Grand Rounds Manton Lecture, Boston, Mass., October 2017
- Past president, Society for Inherited Metabolic Disorders
- Champion for Babies Award, March of Dimes, 2015
- Distinguished editor, NIH Director's New Innovator Award Editorial Board, 2016
- Past chairman, International Congress on Metabolic Disorders
- Postdoctoral trainee Emir Tas, 2016 Fellow Basic Research Award, Society for Pediatric Research, 2016
- Faculty Honors, University of Pittsburgh, 2014

Georgianne L. Arnold, MD

RESEARCH

Clinical Outcomes in FAO Disorders. Georgianne Arnold is part of a collaborative, funded by the NIH and Health Resources and Services Administration, that collects information on clinical management protocols and outcomes studies for common disorders of FAO, including VLCAD, long-chain 3-hydroxyacyl-CoA dehydrogenase (LCHAD), medium-chain acyl-CoA dehydrogenase, and short-chain acyl-CoA dehydrogenase deficiencies. In aggregate, the disorders are fairly common, and expanded newborn screening has profoundly changed the landscape regarding patient outcomes and recommended management. LCHAD and VLCAD deficiencies were initially believed to be life-threatening disorders in infancy, yet many patients diagnosed by newborn screening remain asymptomatic. Furthermore, the severe fat restriction formerly recommended for older patients may be harmful to the developing brain. Some studies suggest that the use of carnitine, a staple in the management of FAO disorders, might actually be harmful in long-chain FAO disorders. At present, Arnold's team is seeking information regarding which patients require more rigorous management versus those who might actually be harmed by over-management. Arnold has published Delphi-based clinical practice protocols for patient management (for which the VLCAD deficiency protocol was awarded the Emmanuel Shapira Award for best paper in *Molecular Genetics and Metabolism* by a member of the Society for Inherited Metabolic Disorders). Her outcomes study on MCAD deficiency was presented to the International Congress of Inborn Errors of Metabolism and published in *Molecular Genetics and Medicine*. She is research funded for participation in gathering outcomes data through the Inborn Errors of Metabolism Information System and is actively seeking funding to conduct outcomes studies on the data.

3-Methylcrotonyl-CoA Carboxylase (3-MCC) Deficiency. This is a disorder in the catabolism of the amino acid leucine. Before the advent of expanded newborn screening, 3-MCC deficiency was believed to be a rare and life-threatening disorder, causing acidosis, failure to thrive, and mental retardation. It is now apparent that most infants who have the disorder detected by newborn screening appear healthy, and some countries have removed the disorder from the newborn screen. However, data collected by Arnold suggest that a subset of affected individuals may indeed be at higher risk of poor outcomes. The study database is being expanded, and a multicenter outcomes study is also under way. Arnold published a Delphi-based management protocol for this disorder and is seeking funding for additional long-term studies for the development of evidence-based guidelines. If some infants are at risk from this disorder, it is important to identify the at-risk infants and offer them appropriate evidence-based treatment.

A Clinical Trial of Sapropterin Dihydrochloride for Cognitive Dysfunction in PKU. Well-managed patients with PKU have long been known to have neuropsychological difficulties and depression even though intelligence quotient is preserved by diet therapy. The drug sapropterin dihydrochloride is a commercial preparation of biopterin, the cofactor for PAH. It appears that a larger than expected number of patients achieves better control of PHE levels while taking supplemental biopterin as sapropterin dihydrochloride. However, the biochemistry of biopterin as a cofactor for tyrosine hydroxylase (important in dopamine metabolism) and tryptophan hydroxylase (important in serotonin metabolism) suggests a biological basis for improved well-being and neurocognitive function reported by patients taking sapropterin dihydrochloride, even if PHE levels do not improve. Arnold is currently funded as a part of a national study to determine the effects of sapropterin dihydrochloride on cognitive and psychological functioning in patients with PKU.

Additional Clinical Investigation Studies. Arnold is an active participant in other clinical studies in the division, including a phase II clinical trial on the use of an investigational medication (triheptanoin) for treatment of FAO defects. Arnold is now leading the UPMC site for phase III trials investigating the safety and efficacy of new enzyme-replacement therapies in the treatment of PKU.

ADVISORY COMMITTEE MEMBERSHIPS

- Database Advisory Board, PKU Demographics, Outcomes, and Safety registry
- Chair, Maintenance of Certification Committee, ACMG
- Program Committee, Evidence-Based Review Group, ACMG
- Board of Directors and program chair, Society for Inherited Metabolic Disorders
- Pennsylvania Newborn Screening Lysosomal Disorders Advisory Board
- Board of Directors, program chair, and president-elect, Society for Inherited Metabolic Disorders

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Society for Inherited Metabolic Disorders
- Society for the Study of Inborn Errors of Metabolism
- ACMG
- American Society of Human Genetics
- International Society for Neonatal Screening

MAJOR LECTURESHIPS, SEMINARS, AND TEACHING

- Core biochemical genetics curriculum, University of Pittsburgh, Pittsburgh, Pa., 2016

HONORS/AWARDS

- Best Doctors, *Pittsburgh Magazine*, 2012–2015
- *Best Doctors in America*, Woodward/White, Inc., 2007–2017
- *Top Doctors*, Castle Connolly, 2009–2017
- Visiting professorship, Children's National Medical Center, Washington, D.C., July 2013
- Visiting professorship, University of Colorado, September 2013
- Visiting professorship, Greenwood Genetic Center, Greenwood, S.C., February 2017

Jane Breck, MD

RESEARCH

Jane Breck is involved in the recruitment and preparation of PKU patients for several research grants. They include the use of hepatocyte transplant for the treatment of metabolic diseases; the use of enzyme-replacement therapy (Peg-Pal) to treat PKU with alternative nondietary treatment; and the neuropsychological effects in addition to the biochemical effects of a PKU medication, sapropterin (Kuvan®).

As a research co-investigator, Breck works with Steve Dobrowolski as he studies the DNA gene mutations of the many patients who attend the PKU Clinic and develops a gene database tracking the effects of gene interaction on various responses to PKU treatment.

Breck's teaching activities include precepting medical students, lecturing graduate students, and interacting with pediatric residents who rotate through an elective in medical genetics. Breck is also active in welcoming genetic counseling students and postdoctoral fellows to observe and participate in the PKU Clinic.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- American Medical Association
- Physicians for Social Responsibility

ADVISORY COMMITTEE MEMBERSHIPS

- Consultant, Head Start Programs of Fayette and Green Counties
- Professional Advisory Committee, Epilepsy Foundation of Western Pennsylvania
- Board of Trustees, Easter Seals Society of Allegheny County
- Advisory Board, Parental Stress Center
- Advisory Board, Caring Foundation, Center for Grieving Children and Adolescents
- Consultant in family communication, WQED TV
- Medical consultant, Shuman Center Juvenile Justice Center

Areeg El-Gharbawy, MD

RESEARCH

Currently, Areeg El-Gharbawy participates as a co-investigator on industry-sponsored clinical trials being conducted in the division on novel pharmaceuticals for inborn errors of metabolism. They include trials that address new therapies for patients with PKU; fatty acid oxidation and glycogenosis disorders; hypophosphatasia; urea cycle disorders; and lysosomal storage disorders, including Pompe, Fabry, and Gaucher disease, and mucopolysaccharidoses. In Vockley's laboratory, she has begun a series of experiments examining cell lines from patients with LCHAD/trifunctional protein (TFP) deficiency and Barth syndrome who share overlapping features with patients with complex 1 respiratory chain defects, including cardiomyopathy, fatigue, exercise intolerance, hypoglycemia, and lactic acidosis, to study the relationships between cellular bio-energetic pathways. Preliminary results on the mechanisms involved and availability of new treatments that target the mitochondria and stabilize cardiolipin are encouraging. El-Gharbawy has submitted an investigator-initiated clinical trial proposal to study the use of the novel anaplerotic agent triheptanoin in patients with glycogen storage disease type 1.

MAJOR LECTURESHIPS AND SEMINARS

- "Fatty Acid Oxidation Disorders," Endocrine Seminar, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., 2016
- "Clinical and Translational Insights in Pompe Disease: Disease Pathology, Patient Identification, and Treatment," Pompe Disease Continuing Medical Education Symposium, American Society of Human Genetics meeting, Orlando, Fla., 2017
- "Energy Defects: More to Learn, More To Do," molecular medicine grand rounds, Oregon Health and Science University, Portland, Ore., 2017
- "Understanding the Underlying Mechanisms Involved in Energy Defects Associated With Overlapping Features," Molecular Medicine Research Symposium, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., 2017
- "Glycogen Storage Disorders: Overview and Updates," endocrine grand rounds, University of Pittsburgh, Pittsburgh, Pa., 2017

- "Study of the Relationship Between Fatty Acid Oxidation, the Electron Transfer Chain, and Cardiolipin in Patients' Cells with LCHAD/TFP Deficiency and Barth Syndrome: Implications for New Patho-Mechanisms and Therapeutic Targets," abstract, United Mitochondrial Foundation meeting, Alexandria, Va., 2017
- "The Biochemical Basis for Overlap of Clinical Features of LCHAD/TFP Deficiency With Mitochondrial Respiratory Chain Defects: Implications for New Therapeutic Approaches," poster presentation, International Congress of Inborn Errors of Metabolism, Rio de Janeiro, Brazil, 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Society for Inherited Metabolic Disorders
- American Medical Association

HONORS/AWARDS

- Research grant award, Promoting Academic Talent in the Health Sciences, 2014
- Nominee, Climb Metabolic Hero Award, 2017

Maria Escolar, MD, MS

RESEARCH

Maria Escolar recently transitioned from the Division of Neurology to the Division of Medical Genetics. Her research takes a multidisciplinary approach focused on the interactions of genes, brain, and behavior and their influences on aberrant development in rare neurodegenerative diseases. Escolar is an expert in leukodystrophies, such as Krabbe disease, metachromatic leukodystrophy (MLD), and adrenoleukodystrophy, as well as other metabolic conditions that can affect the brain, such as Sanfilippo syndrome, Hurler's syndrome, and Hunter's syndrome, among others.

Natural History of Krabbe Disease. Krabbe disease, also known as globoid cell leukodystrophy, is a rare autosomal recessive metabolic disorder characterized by the deficiency of galactocerebrosidase, a lysosomal enzyme responsible for the degradation of psychosine to galactose and sphingosine. The subsequent accumulation of psychosine destroys oligodendrocytes and Schwann cells, causing the formation of multi-nucleated globoid cells, severe demyelination, axonopathy, and neuronal death. The degradation of the central and peripheral nervous systems clinically manifests as progressive neurodegeneration, spasticity, irritability, blindness, deafness, seizures, and premature death. The incidence of Krabbe disease has been estimated as 1 in 100,000 live births. The disease is divided into four subgroups based on age at symptom onset: early infantile (birth to 5 months), late infantile (6–47 months), juvenile (4–17 years), and adult (> 18 years).

As a leading center in the study and treatment of Krabbe disease, the NDRD team has evaluated more than 180 patients, providing the largest single-center database on Krabbe disease in the world. Because of the large and comprehensive database, Escolar has been able to advance research on the different phenotypes. With 97 early-infantile patients and 39 late-infantile patients, Escolar has designed natural history studies to evaluate variability in progression. In Escolar's natural history design, children are evaluated prospectively following a protocol of standardized multidisciplinary testing. Evaluations are completed by a multidisciplinary team of specialists, including neurodevelopmental pediatricians, neuroradiologists, ophthalmologists, speech pathologists, audiologists, physical therapists, genetic counselors, and psychometricians.

Overall, the data collected help establish the initial symptoms and characteristics of disease progression, which can be beneficial in staging of the disease. Resolution of early symptoms can be used for monitoring disease progression and assessing the efficacy of therapeutic interventions. Further work is necessary to understand the correlations among symptoms, function, and genetic mutations. A better understanding of the presenting symptoms of Krabbe disease will increase awareness among pediatricians and result in earlier diagnostic referrals and recruitment for future clinical trials. Such knowledge will become increasingly important as methodological and legislative advances in newborn screening practices continue. Ultimately, natural history data will enable researchers to establish rate and severity of disease progression, allowing clinicians to make better decisions regarding the management and treatment of patients diagnosed via newborn screening.

Psychosine, a Marker of Krabbe Phenotype and Treatment Effect. Although the phenotypic spectrum associated with this condition is broad, the most common form, known as early-infantile Krabbe disease, results in rapid neurodegeneration and death within the first few years of life. Because the only treatment for this condition, hematopoietic stem cell transplantation (HSCT), is most effective if performed prior to the onset of neurological deterioration, it is essential that affected patients be diagnosed during the presymptomatic or minimally symptomatic period. Biomarkers are urgently needed to aid in the prediction of phenotype and assessment of clinical course in patients who are diagnosed with or determined to be at risk for Krabbe disease. Two small prior studies have found evidence that the concentration of psychosine, a substrate of the galactocerebrosidase enzyme, is elevated in patients with early-infantile Krabbe disease. However, given that the prior studies were limited to cross-sectional measurements in a small number of patients,

additional data are needed to assess the value of dried blood spot (DBS) psychosine as a potential biomarker for Krabbe disease.

In July 2017, Escobar reported the findings of the largest study of DBS psychosine concentrations in patients with Krabbe disease to date. The team was the first to longitudinally assess DBS psychosine concentrations in a phenotypically diverse cohort of patients with early-infantile, late-infantile, and juvenile-onset Krabbe disease, along with carriers and at-risk patients who screened positive for Krabbe disease at birth but remain asymptomatic. Substantially elevated DBS psychosine during the newborn period has 100% specificity as a biomarker for infantile-onset Krabbe disease. The findings suggest that measuring psychosine as a second-tier newborn screening test could aid in phenotypic prediction and help clinicians to determine which patients require urgent treatment with HSCT. Future work is aimed toward examining the relationship between psychosine increase and disease progression in later-onset patients and the implications of psychosine concentration changes over time.

Developmental Outcomes of Cord Blood Transplantation for Krabbe Disease: A 15-Year Study. Currently, Krabbe disease is without cure, but umbilical cord blood transplantation (UCBT) has been shown to significantly improve neurological outcomes in asymptomatic neonates. However, when transplantation is performed in symptomatic patients with early-infantile disease, the neurologic insult remains severe. The purpose of this prospective study, which was accepted for publication in *Neurology* in August 2017, was to summarize long-term neurodevelopmental outcomes of 18 children with early-infantile Krabbe disease who were transplanted in the first 7 weeks of life. Long-term outcomes of transplanted patients were assessed with a standardized protocol. Despite failing to cure Krabbe disease, HSCT performed before the onset of severe symptoms delayed disease progression and improved length and quality of life. Children who survived the peritransplant period functioned at a much higher level than untreated patients. Gross motor function responded less well to treatment. Fine motor skills were generally preserved. Cognition was normal or developed at a slightly slower rate than that of typical children. The clinically and statistically significant associations between age at transplantation and expressive language and gross motor function highlight the importance of transplantation as soon as possible for children with early-infantile disease, ideally before 2 weeks of age.

Improvements in Brain Development Following HSCT in Krabbe Disease. Krabbe disease causes severe demyelination of the brain, with rapidly progressing atrophy of certain regions. HSCT is the only treatment available that can halt disease progression. In this study, the NDRD team investigated the development of cerebral myelination by magnetic resonance imaging (MRI) and propose a more sensitive and objective tool to assess the effects of this treatment. The team also compared MRI data from patients who underwent HSCT against natural history. Diffusion tensor imaging (DTI), which utilizes fractional anisotropy to measure the water diffusion property of the white matter and reflects the direction of the axonal microstructure, was used to assess the white matter integrity of the brain. Combined with Escolar's neuroimaging protocol and algorithm, the methods allow for precise quantification of myelin content. A total of 55 patients with early-infantile Krabbe were analyzed, 14 of whom underwent transplantation and 31 who had natural disease progression. Patients treated with HSCT mostly followed the normal developmental trajectory of the corticospinal tract, albeit in the lower part of the normal range. Patients who were not treated with HSCT started with lower-than-normal fractional anisotropy, and the measure decreased significantly within two years after an initial increase. The fractional anisotropy values are consistent with the motor function as measured in behavioral testing. Diffusion-based brain MRI quantitates abnormalities in white matter integrity in patients with early-infantile Krabbe disease. Patients who are treated with HSCT early in life retain corticospinal tract integrity and follow a normal trajectory over time. More broadly, the findings support using diffusion tensor imaging as a tool to measure white matter integrity and effects of treatment in persons with neurodegenerative diseases.

Treatment of Krabbe Disease With Intravenous Adeno-Associated Virus Gene Therapy. Treatment with UCBT can extend life and preserve cognitive function in presymptomatic patients with early-infantile Krabbe disease and in presymptomatic/minimally symptomatic patients with the late-infantile form of the disease. However, all treated children experience some degree of motor disability post-UCBT due to a combination of peripheral nerve disease and early, irreversible damage of the corticospinal tracts. Intravenous gene transfer has been shown to correct myelination of both central and peripheral nervous systems in mice, addressing the critical problem that HSCT is not able to correct—progressive deterioration of the peripheral nerves. Furthermore, UCBT offers no significant benefit once a patient is already symptomatic

because of the extensive early damage to the motor tracts. Therefore, no effective treatments are available once a child manifests signs or symptoms of Krabbe disease. In mice, as explained above, intravenous gene therapy administered shortly after HSCT rapidly improves myelination in both brain and peripheral nerves while the immune system reconstitutes after myeloablative conditioning. In this way, adding gene therapy may shorten the interval between diagnosis and delivery of normal galactocerebrosidase enzyme to oligodendrocytes and Schwann cells, potentially extending the benefits of treatment to patients who have begun to show symptoms.

There is renewed interest in gene therapy, as the development of new viral vectors has reduced the risk of immunogenicity and insertional mutagenesis. Results of recent clinical trials evaluating gene therapy for genetic diseases such as adrenoleukodystrophy and MLD are highly encouraging. Recent advances in gene delivery technologies and the team's newly patented treatment combining gene therapy with reduced-conditioning UCBT may hold the key to effective treatment for more infants and children affected by Krabbe disease. If successful, the proposed clinical trial may transform the standard of care for patients with Krabbe disease. Currently, the project is in the preclinical stages, the toxicology studies. Once those studies are completed, the team intends to move into a phase I/II clinical trial, which will test for safety and explore efficacy in humans.

Krabbe Translational Research Network. Escolar began holding the annual Krabbe Translation Research Network (KTRN) meeting in 2010 while at the University of North Carolina. The KTRN is a consortium of scientists and clinicians who are dedicated to helping children with Krabbe disease live longer and healthier lives. The KTRN brings together the complementary knowledge and skills of investigators who are working in different disciplines at different institutions; it aims to accelerate the development of research findings into new treatments. KTRN members share data and resources to identify promising therapeutic approaches for further development. Specific goals of this year's meeting will include addressing the challenges of gene therapy and effective designs for future trials, discussing the use of gene therapy in patients who were previously transplanted, and considering the treatment of peripheral nerve disease.

Natural History of MLD. MLD is an inherited lysosomal storage disease caused by deficient activity of arylsulfatase A, an enzyme involved in the degradation of 3'-O-sulphogalactosylceramide, otherwise known as sulfatide. Sulfatide exists as a membrane-bound sphingolipid abundant in the myelin sheath and predominantly found in oligodendrocytes in the central nervous system and Schwann cells in the peripheral nervous system. In MLD, the gradual accumulation of undegraded sulfatide in these myelin-producing cells results in progressive demyelination and neurodegeneration in both the central nervous system and peripheral nervous system. The disease is fatal, with a variable clinical course, and it is typically classified into subtypes based on age of onset. The limitations of previous cohort studies inspired the team to carry out a larger, more comprehensive (prospective and retrospective) study of the onset, prevalence, and severity of clinical manifestations of MLD, enabling physicians to recognize early symptom onset and to select patients likely to benefit from current and future therapies. The data will be used to design clinical trials and to assess treatment outcomes. Because all evaluations were conducted at a single site with

a standardized protocol, the data are the most reliable to date. They also favor patients with early onset, the time of greatest therapeutic opportunity. The substantially larger cohort—totalling 134 patients—enabled arraying the population on a continuous spectrum as opposed to discrete subtypes. The approach allows physicians to conceive of the disease as a diverse set of symptoms and varying forms of clinical presentation, instead of distinct, nonoverlapping phenotypes. In sum, the prospective, longitudinal study provides an improved understanding regarding the clinical course of the insidious disease. The findings will lead to improved clinical outcomes by facilitating earlier diagnoses and assisting in the treatment and management of MLD. Additionally, they will provide a baseline for investigators designing clinical trials for novel therapies.

NDRD Brain and Tissue Bank. This biorepository is housed in Rangos Research Center, and post-mortem specimens are housed at the Alzheimer's Brain Bank at the University of Pittsburgh. It currently houses more than 1,000 specimens collected since 2010. The project has been funded by grants awarded by the Legacy of Angels Foundation and a donation from the Believing for Bryleigh Foundation and Partners for Krabbe Research. The tissue bank provides a plethora of research opportunities for multiple collaborations, as most specimens are connected to clinical data in the NDRD database. Escolar works closely with Julia Kofler to draw connections among histological, pathological, and clinical findings.

Clinical Trials. In addition to coordinating internal research at the NDRD, Escolar continues to be involved as principal investigator in several industry-sponsored projects. Current projects include a safety, pharmacokinetics, and pharmacodynamics/efficacy phase I trial of SBC-103, a new treatment for mucopolysaccharide disease (Sanfilippo) type B (MPS IIIB); a phase I/II trial investigating a treatment for pantothenate kinase–associated neurodegeneration; and a retrospective cross-sectional study to evaluate the neurodevelopmental status of patients with severe MPS type II.

MAJOR LECTURESHIPS, SEMINARS, AND TEACHING

- "Neurodevelopment in MPS Syndromes, Natural History, and Treatment Outcomes," International Symposium on MPS and Related Diseases, Bonn, Germany, July 2016
- "Gene Therapy for Rare Diseases," New York Academy of Sciences and the Biochemical Pharmacology Discussion Group, New York, N.Y., April 2017
- MLD Family Conference, Suffolk, Va., July 2017
- 20th Annual Family and Medical Symposium, Hunter's Hope, Ellicottville, N.Y., July 2017
- "Outcomes of First GM3 Synthase Deficiency Unrelated Matched Umbilical Cord Transplant Following Reduced Conditioning Regimen," Translational Medicine in the Plain Populations Conference, Pittsburgh, Pa., August 2017
- "Psychosine, a Marker of Krabbe Phenotype and Treatment," 13th International Congress of Inborn Errors of Metabolism, Rio de Janeiro, Brazil, September 2017
- Keynote speaker, Paul M. Fernhoff Memorial Lecture, Centers for Disease Control and Prevention and Emory University, Atlanta, Ga., September 2017
- "MPS II Early Diagnosis," Shire Human Genetics Advisory Board Meeting, September 2017
- "MPS II," Regenxbio Advisory Board Meeting, Washington, D.C., September 2017
- "Krabbe Disease Natural History Outcomes," 2017 Global Leukodystrophy Initiative Conference, Philadelphia, Pa., November 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Adrenoleukodystrophy Advisory Board, MPS II Advisory Board, Regenxbio
- Zellweger Spectrum Disorders Advisory Board, Retrophin
- Lysosomal Storage Disease Advisory Board, Regenxbio
- Advisory Board, Abeona
- Shire Human Genetics
- Global Leukodystrophy Association
- Global Leukodystrophy Initiative
- Newborn Screening Translational Network Workgroup, ACMG, supported by the National Institute of Child Health and Development
- Medical Advisory Board, Jacob's Cure
- Advisory Committee, Senator Paul D. Wellstone Muscular Dystrophy Cooperative Research Center (U54)
- New York State Krabbe Newborn Screening Consortium
- Medical and Scientific Advisory Board, MLD Foundation
- KTRN

HONORS/AWARDS

- RARE Tribute to Champions of Hope, Medical Care and Treatment Award Recipient, Global Genes Summit (chosen among 300 nominees)

Lina Ghaloul-Gonzalez, MD

RESEARCH

Identification of Novel Genetic Disorders by Exome and Genome Sequencing. Lina Ghaloul-Gonzalez recently transitioned from her position as a fellow in medical genetics to the faculty of the division. Her primary focus is genome and personalized medicine. Current projects include exome and genome sequencing in newborns and genome sequencing in the Western Pennsylvania Plain Communities (Amish and Mennonite).

Characterizing the Burden of Genetic Disease in Old Order Amish. The Old Order Amish communities (Plain People) of North America have altered health risks that stem from unbalanced population sampling of European founders followed by genetic drift in derivative generations. The population effects have resulted in a high prevalence of specific genetic disorders that vary from the general population and from each other. Several characteristics of the communities facilitate genetic analysis. Most isolates keep excellent historical and genealogical records. There is usually little or no migration into the group, and the members of the group exhibit relatively homogeneous lifestyles. Large nuclear families are frequent, which provides adequate numbers of affected and unaffected siblings within a sibship for blood samples. The primary genetic advantage, however, results from the interaction of two overlapping phenomena: the founder effect and inbreeding.

Amish populations are unique in that they represent genetic bottlenecks dating back to the 18th century, distinguishing them from the European population as well genetic drift, which has given rise to variable distributions of pathogenic alleles among North American settlements. The division's mitochondrial studies and other clinical encounters led to the hypothesis that many unrecognized genetic disorders are present in the Mercer County Amish.

The Mercer County Amish are among the least genetically characterized Amish communities in the United States, with no catalog of either genetic disorders or variants seen in the community. The division's researchers have developed a new program to characterize the genetic variability between Amish Mercer County population and other Amish counties by doing whole-exome and mitochondrial DNA sequencing. The project will allow the team to characterize the load of genetic disease in Old Order Amish of Mercer County and identify disorders that can benefit from early treatment.

Mitochondrial DNA mutations have not previously been reported in any Old Order Amish community. The team recently described an Amish family with the MTTL1 mitochondrial gene mutation m.3243A>G. A second patient with an m.13513G>A (D393N) mutation has also been diagnosed. The mutations classically cause mitochondrial encephalomyopathy, lactic acidosis, and stroke-like episodes (otherwise known as MELAS) syndrome. A third patient in that Amish community has been diagnosed with an autosomal recessive respiratory chain disorder due to a homozygous deletion in the NDUFAF2 gene, which was in one of the nine areas of homozygosity detected on single nucleotide polymorphism microarray.

Identification of Reticular Dysgenesis Due to AK2 Mutation: A Novel Genetic Disorder in the Amish Population. Homozygous *AK2* gene mutation [c.622T>C; p.S208P] was identified in an Amish boy for the first time, with immunodeficiency by whole-exome sequencing. *AK2* mutation causes reticular dysgenesis, which is characterized by severe combined immunodeficiency and leukopenia. No Amish patients with AK2 deficiency have been reported in the literature. This highlights the importance of whole-exome sequencing as a diagnostic tool and will add this gene to the already-available Plain People's database of genetic disorders. Functional studies are ongoing to confirm pathogenicity of this mutation.

Human ITCH E3 Ubiquitin Ligase Deficiency in a Non-Amish Girl. Syndromic multisystem autoimmune disease due to human ITCH E3 ubiquitin ligase deficiency was first reported in 10 Old Order Amish children. The children had failure to thrive, with weight and height below the third percentile; relative macrocephaly; developmental delay; delays in gross motor and cognitive skills; dysmorphic features; organomegaly; and autoimmune inflammatory cell infiltration of the lungs, liver, and gut. The team identified a 13-year-old, non-Amish female with a medical history significant for failure to thrive, with height below the third percentile, relative macrocephaly, and mild dysmorphic features with *ITCH* deficiency. Whole-exome sequencing identified a novel, maternally inherited variant in exon 8, c.599 dupC (p.S201fs) that causes frameshift. Deletion studies revealed a *de novo* deletion involving exons 25 and 26. *ITCH* gene mutations in a non-Amish patient provide additional evidence connecting *ITCH* deficiency to human disease. The case also highlights the cost-effectiveness of whole-exome sequencing as a first-tier genetic test in complex patients, which would have reduced the time to diagnosis and saved numerous other tests and procedures that were either non-specific or unrevealing. Functional studies are ongoing to prove pathogeneticy of the mutations.

Characterization of Genetic Factors Contributing to Sepsis in Subjects With Concurrent High Ferritin Levels by Whole-Exome Sequencing. Ghaloul-Gonzalez is co-investigator on this research project with Joseph Carcillo, MD. Organ shutdown in multiple organ dysfunction syndrome (MODS) results when persistent macrophage activation induces inflammation-driven endothelial, epithelial, mitochondrial, and immune cell dysfunction. Ferritin is released into the circulation by activated macrophages. Hyperferritinemic MODS (ferritin > 500 ng/mL) is characterized by reticuloendothelial system activation–induced hepatobiliary dysfunction and disseminated intravascular coagulation. It is variably described by clinicians as sepsis

MODS, macrophage activation syndrome (MAS), and reactive hemophagocytic lymphohistiocytosis. The Histiocytosis Society recommends etoposide and chemotherapy for the condition. In contrast, the researchers studied children with five to six organ failure hyperferritinemic MODS and showed 100% survival with daily plasma exchange, methylprednisone, and intravenous gamma globulin compared to only 50% survival in those treated with the Histiocytosis Society etoposide-based protocol. They also showed that adults with sepsis and features of MAS treated with interleukin 1 receptor antagonist protein had a reduction in mortality to 30%, compared to 60% with placebo. To address this therapeutic controversy, the researchers will test the hypothesis that hyperferritinemic MODS is a common end pathway of inflammation in children, caused by a group of inflammatory conditions that are best treated with precision medicine rather than an etoposide-for-all approach. The researchers are addressing several knowledge gaps in that regard. First, they have determined the ferritin threshold and isotypes related to mortality, which can be used as therapeutic triggers. Second, they have identified the unique "inflammasome" cytokine pattern, which can be targeted with anti-cytokine therapies in patients with ferritin levels above the threshold. Third, they have identified the proportion of patients with gene variants related to (a) inflammasome-driven cryopyrin-associated periodic syndrome and macrophage activation syndrome (IRAP is approved by the U.S. Food and Drug Administration), (b) hypercomplementemia-driven atypical hemolytic uremic syndrome/*aHUS* (C5a antibody is approved by the U.S. Food and Drug Administration), and (c) failed granzyme/perforin or Fas/FasL-mediated activated immune cell death syndromes in hemophagocytic lymphohistiocytosis (etoposide is recommended by the Histiocytosis Society). Fourth, they will explore the feasibility of targeting ferritin heavy-chain potentiation of MODS inflammation with inhibitors of ferritin heavy-chain production and inhibitors of ferritin heavy-chain–induced monocyte/macrophage activation.

STUDY SECTIONS

- Research grant reviewer, Clinical and Translational Science Institute, 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Society of Human GeneticsSociety for Inherited Metabolic Disorders

MAJOR LECTURESHIPS, SEMINARS, AND TEACHING

- "Genetic Variants Associated With Hyperinflammation in Septic Shock, poster/oral presentation, Society of Critical Care Medicine meeting, Hawaii, 2016
- "Translational Medicine in the Plain Communities," grand rounds, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., February 2017
- "Mitochondriopathy in AK2 Deficiency, a Novel Genetic Disorder in the Amish Population," Fifth Annual Plain Populations Translational Medicine Meeting, Pittsburgh, Pa., 2017
- "Diagnostic Mysteries Cases," Fifth Annual Plain Populations Translational Medicine Meeting, Pittsburgh, Pa., 2017
- "Metabolic Diseases in the Plain People: The Path From Genomic Discovery to Therapy," co-chair, International Congress of Inborn Errors of Metabolism, Rio de Janeiro, Brazil, 2017
- "Reticular Dysgenesis and Mitochondriopathy Induced by Adenylate Kinase 2 Deficiency Identified in the Amish Population," International Congress of Inborn Errors of Metabolism, Rio de Janeiro, Brazil, 2017
- "Reticular Dysgenesis and Mitochondriopathy Induced by Adenylate Kinase 2 Deficiency Identified in the Amish Population," poster/oral presentation, International Congress of Inborn Errors of Metabolism, Rio de Janeiro, Brazil, 2017

Eric Goetzman, PhD

RESEARCH

Regulation of Mitochondrial Metabolism by Reversible Lysine Acylation. Eric Goetzman's laboratory studies mitochondrial FAO, the pathway by which fatty acids are broken down for energy. Mutations in the FAO genes are among the most prevalent inborn errors of metabolism. FAO enzymes are heavily modified by post-translational modifications. There are three mitochondrial sirtuin deacylases (SIRT3, SIRT4, and SIRT5), which are believed to reverse some of those modifications. The laboratory's current research focuses on the functional effects of lysine acetylation and succinylation on the FAO pathway and the role the sirtuins play in regulating metabolism. Preliminary data show that SIRT5 also may regulate peroxisomal FAO, important in the kidney and liver. SIRT5 "knockout" mice have reduced mitochondrial and peroxisomal function in the kidney but paradoxically are protected from acute kidney injury. Experiments are under way to investigate the phenomenon.

Lung FAO. Mice "knocked out" for the FAO enzyme LCAD demonstrate reduced lung function. Goetzman's team hypothesizes that LCAD and the FAO pathway are involved in synthesizing and secreting pulmonary surfactant in a specialized lung cell known as the type II pneumocyte. Surfactant is a mixture of phospholipids and proteins that

reduces surface tension in the lung; the effect is necessary to prevent the collapse of the alveoli and promote gas exchange. LCAD knockout mice have reduced amounts of surfactant lipids and an altered phospholipid composition. Preliminary studies have shown an increased susceptibility to infection by influenza. Efforts are under way to determine the molecular mechanisms behind the changes.

Cancer Metabolism. Cancer cells have an unusual reliance on glycolysis for energy. Research has not revealed how and why cancer cells alter their metabolism or what role changes in mitochondrial energy metabolism play in the etiology of cancer or in the ability of cancer cells to escape apoptosis. Answering those questions may reveal cancer's Achilles heel and lead to a cure. Goetzman's team is collaborating with Ed Prochownik to study how the oncogenic transcription factor c-Myc drives cells toward an anabolic metabolic phenotype. It may involve changes in mitochondrial enzyme lysine acetylation, as well as changes in partitioning among glucose, glutamine, and fatty acid metabolism.

MAJOR LECTURESHIPS, SEMINARS, AND TEACHING

- "Regulation of Fatty Acid Oxidation by Reversible Lysine Acylation," Sanford Burnham Prebys Medical Discovery Institute, Lake Nona, Fla., 2016
- "Novel Protein Modifications as Regulators of Fatty Acid Oxidation," INFORM, Boston, Mass., 2016
- "Sirtuin Regulation of Peroxisomes in Cancer," Stimulating Pittsburgh Research in Geroscience Work in Progress Seminar Series, Pittsburgh, Pa., November 2016
- "Fatty Acid Oxidation: Relevance and Regulation," endocrine grand rounds, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., November 2016
- "Mitochondrial Fission/Fusion" and "Mitochondria in Health and Disease," MSCBMP2880: Cellular Biology of Normal and Disease States, University of Pittsburgh, Pittsburgh, Pa., April 2017
- "Gm3 Synthase Deficiency in Mice," Gm3 Synthase Family Day, Clinic for Special Children, Strasburg, Pa., May 2017

STUDY SECTIONS

- Research grant reviewer, Discovery Award, U.S. Department of Defense, August 2016
- Research grant reviewer, Prinses Beatrix Spierfonds, Netherlands, March 2017
- Research grant reviewer, Fondiazone Telathon, Italy, April 2017
- Research grant reviewer, Young Investigator Award Program, Children's Hospital of Pittsburgh Foundation, April 2017
- Research grant reviewer, , Peer-Reviewed Medical Research Program, U.S. Department of Defense, June 2017
- Research grant reviewer/ad hoc member, DDK-B Study Section, NIH, June 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Society for Inherited Metabolic Disorders
- Mitochondria, Aging, and Metabolism Working Group
- Scientific Organizing Committee, INFORM

HONORS/AWARDS

- Team lead, Basic Science, Gm3 Research Summit, Strasburg, Pa., October 2016
- Scientific advisor, International Network for Fatty Acid Oxidation Disorders Research and Management, July 2015 to the present

Uta Lichter-Konecki, MD

RESEARCH

Uta Lichter-Konecki joined the division in August 2016 and has a longtime clinical and research interest in inborn errors of metabolism, beginning with her early clinical and laboratory training. She is an internationally recognized expert in the molecular genetics of PKU. She has published groundbreaking work in the molecular characterization of PKU, including the first description of a mutation in the *PAH* gene causing PKU, and she helped establish the molecular basis for the phenotypic heterogeneity of PKU, a novel genetic property that ultimately became known as genotype-phenotype correlation. In addition to her seminal research in the laboratory, Lichter-Konecki was a member of the multicenter German Collaborative Study on PKU, as well as a member of the international Maternal PKU Study.

Lichter-Konecki's strongest research interest remains the pathophysiology of the brain injury caused by inborn errors of metabolism, whether PKU or urea cycle disorder (UCD). Focusing on an animal model of ornithine transcarbamylase deficiency, a UCD, as well as the patients enrolled in the longitudinal study of UCD, she has examined the role of ammonia and the amino acid glutamine as the toxic agents in UCD. In the culmination of her animal experiments, she was able to show that the primary pathophysiology in acute hyperammonemic encephalopathy was likely a disturbance of astrocyte-mediated water and potassium homeostasis in brain, a major shift in a field previously focused on glutamine-induced hyperosmolality. The findings hold the potential key to developing neuroprotective therapies for acute hyperammonemia in patients with inborn errors of metabolism and hepatic encephalopathy of other causes. Lichter-Konecki has conducted a multicenter pilot study that demonstrated feasi-

bility and safety of hypothermia treatment in acute hyperammonemia, and she has obtained an R34 planning grant from the National Institute of Child Health and Human Development to plan for a large multicenter clinical trial and build consensus regarding treatment during the trial across four different pediatric subspecialties at the 30 sites involved. The planning phase was completed successfully, but the trial was not funded by the NIH. She is now focusing her efforts regarding neuroprotection in hyperammonemia on drugs that can affect water and potassium homeostasis in brain and is collaborating with a researcher that performs drug screens in hyperammonemic zebra fish to take the best candidates and test them in a new mouse model for acute hyperammonemic encephalopathy (from the same collaborator) due to a UCD. On the clinical research side, she is completing analysis of data collected during the longitudinal study for UCD to show the effect of ammonia and glutamine on outcomes in UCD.

She is a co-investigator on the R01 grant for the PHE meter and is working on that clinical research project, too, as well as on the division's industry-sponsored clinical trials regarding inborn errors of metabolism.

Lichter-Konecki studies lysosomal storage diseases. She participated in the hunt for the gene responsible for cystinosis and mapped the locus for an autosomal dominant form of renal Fanconi syndrome (ADRFS) in the human genome. She has an ongoing collaboration with colleagues in the United States and Europe regarding the pathophysiology of the gene defect in ADRFS, and the group is in the process of publishing their work.

MAJOR LECTURESHIPS, SEMINARS, AND TEACHING

- Grand rounds speaker, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., October 2016
- Fellow lecturer, Pediatric Intensive Care Unit (ICU), Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., October 2016
- Fellow lecturer, Neonatal ICU, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., October 2016

STUDY SECTIONS

- Research grant reviewer, Scientific Advisory Board, National PKU Alliance, 2011 to the present
- Research grant reviewer, U.S.–Israel Binational Science Foundation, 2011 to the present
- Research grant reviewer, Raine Medical Research Foundation at the University of Western Australia, 2011 to the present

ADVISORY COMMITTEE MEMBERSHIPS

- Metabolism Work Group, Clinical Genome (ClinGen) Resource Program
- Ad Hoc Review Committee, reviewing staff clinicians of the Medical Genetics Branch of the National Human Genome Research Institute, NIH
- Scientific Advisory Board, National PKU Alliance Therapeutics Committee, ACMG
- Consultant regarding new product development for PKU and UCD, Sanofi

Suneeta Madan-Khetarpal, MD

RESEARCH

Suneeta Madan-Khetarpal is a co-investigator on Vockley's clinical trial projects. She also is a co-investigator on an R01 grant with Zsolt Urban of the Department of Human Genetics in the Graduate School of Public Health, examining genetic disorders of the extracellular matrix. She is a co-investigator on a grant with Judith Yanowitz of the Magee-Womens Research Institute, looking at the effects of the CHD7 genetic mutation. Madan-Khetarpal is collaborating with Cecilia Lo and Ashok Panigraphy on researching the genetic causes of congenital heart defects and brain–cilia defects. She also collaborates with Beth Roman on research regarding hereditary hemorrhagic telangiectasia (HHT).

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Society of Human Genetics
- American Medical Association
- ACMG
- American Cleft Palate–Craniofacial Association
- National Marfan Foundation
- Society for Inherited Metabolic Disorders
- Society for the Study of Inborn Errors of Metabolism
- German Society for Pediatric and Adolescent Medicine
- German Society for Human Genetics
- Society for Neuroscience
- Society for Pediatric Research

MAJOR LECTURESHIPS AND SEMINARS

- "Dysmorphology Assessment for Ear, Nose, and Throat," Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., 2016
- "Introduction to Dysmorphology," Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., October 2016
- "A Day in Clinical Genetics," immunology grand rounds, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., November 2016
- HHT rounds speaker, UPMC Presbyterian Hospital, Pittsburgh, Pa., 2016
- HHT rounds speaker, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., 2016

Al-Walid A. Mohsen, PhD

RESEARCH

Al-Walid A. Mohsen has a dual role. His primary role has long been to provide Vockley's research personnel with experimental design support and research direction to pursue the specific aims of his NIH grants and his new biotech-funded projects. Mohsen has for years been a driver of discovery and innovation. Last year, he introduced a concept with breakthrough potential in drug discovery for biochemical genetic disorders. He demonstrated evidence of its viability in three different pathways in patient cells. One patent application was filed for trimetazidine (TMZ) to treat fatty acid oxidation disorders, and clinical trials are under way. Mohsen is currently focused on optimizing drug efficacy and confirming drug mechanism interaction. Confirming the mechanisms and the pharmacodynamics and characterization of the proposed drug therapy for five fatty acid mitochondrial metabolic disorders, on the molecular level *in vitro* and *in vivo*, will help win a "Breakthrough Therapy" designation by the U.S. Food and Drug Administration.

Development of Chemical Chaperone for Inherited Metabolic Enzyme Deficiencies. Genetic disorders that result from missense mutations can often render the mutated proteins structurally defective, leading to their misfolding or instability. Mutant enzymes that reach the native tertiary or quaternary assembled state may have partial activity, but they are usually thermolabile and vulnerable to proteolysis, making fever, strenuous exercise, or other stress factors life-threatening decompensation triggers. Often protein thermostability and vulnerability to proteolysis improve significantly by ligand binding. Ligands that improve protein stability include the protein/enzyme's own substrate, a substrate analog, or its reaction product(s). Other stabilizing ligands also include ones that bind to allosteric sites or small chaperone molecules that provide stability through mostly undefined mechanisms. Although screening for small chaperone molecules that bind enzymes at sites away from their catalytic sites to stabilize structurally compromised mutants has been a major rationale for drug development, stabilizing molecule(s) such as the enzyme's own reaction product or pathway intermediates of the distal pathway steps may provide such stabilizing effect. Inhibitors of downstream pathway reactions may increase the presence of a defective enzyme's product or other pathway intermediates *in situ* to levels high enough to bind to the defective enzyme/protein. This is the basis for the concept that Mohsen introduced, which has the potential to be a breakthrough in drug discovery for genetic disorders. The validity of his concept, now named inhibitor-induced *in situ* chaperone therapy (I3CT), was tested by investigation of the effect in patient cells with missense mutations of three known drugs that are inhibitors of distal reactions in three different pathways: fatty acid β-oxidation, leucine metabolism, and PHE metabolism pathways.

Development of an I3CT for Fatty Acid β-Oxidation Cycle Enzyme Deficiencies. Mitochondrial fatty acid β-oxidation cycle is a spiral pathway that includes four enzymatic reaction steps. Inherited defects in the *ACADVL* or *HADHA/HADHB* genes coding for the VLCAD and TFP proteins, respectively, are the cause of some of the most serious life-threatening metabolic disorders. Although VLCAD deficiency is the most common among the defects, TFP deficiency shows more life-threatening symptoms. VLCAD deficiency results in a decrease in energy output from long-chain fatty acid β-oxidation, causing the heart, which draws ~ 80% of its energy requirement from long-chain fatty acids, to be at risk of cardiomyopathy. More than 65 of the mutations identified in patients with defects in the ACADVL gene are missense mutations, which are hypothesized to cause instabilities. TMZ is an inhibitor of long-chain ketoacylthiolase (LCKAT) recognized to slow long-chain fatty acid β-oxidation enough, shifting cardiac energy fulfillment from fatty acid oxidation to glucose oxidation, and it is used to treat angina pectoris induced by ischemia of the heart. Compared to conventional therapy, TMZ is reported to have shown significant improvements in non-ischemic and ischemic cardiomyopathy. Because it is an inhibitor of LKCAT, Mohsen examined the effect of TMZ on the presence of β-oxidation enzymes in patients with β-oxidation disorders. Experimental results supported the concept, showing a positive effect of TMZ FAOD cells. In addition, other TMZ analogs have been introduced and have a similar effect on slowing β-oxidation by inhibiting LCKAT. The advantages of the TMZ derivatives are in their added reactive oxygen species (ROS) scavenging activity, a needed feature, as experimental evidence suggests that increased ROS in VLCAD patients may contribute to the pathophysiology of the disease.

Development of Chemical Chaperone for MCAD Deficiency. Deficiency of MCAD rivals PKU as the most common biochemical genetic disorder. Patients with MCAD deficiency are asymptomatic at birth but are at risk for episodes of acute, life-threatening metabolic decompensation. They usually first occur between three and 24 months of age but can occur at any age in association with physiologic stress, such as fasting or infection. The mortality rate during an acute crisis in previously undiagnosed patients pushes 20%. Newborn screening via tandem mass spectrometry now

identifies MCAD deficiency pre-symptomatically, nearly eliminating mortality from the disease. However, treatment requires lifelong dietary monitoring, and significant morbidity still occurs due to hospitalizations for intravenous glucose therapy in the face of reduced oral intake. A single mutation in the *MCAD* gene (a G985A point mutation) has been identified in 90% of the alleles in the *MCAD* gene in deficient patients. The K304E destabilizes the quaternary structure of the enzyme, and the resultant mutant protein is rapidly degraded. *In vivo*, the mutant protein is catalytically active when stabilized, and restoration of only a few percent of normal MCAD activity will restore near-normal metabolic balance in patients. The objective of this current research is to establish the drugability of the MCAD K304E mutant by identifying lead compounds that can stabilize the mutant protein using *in vitro* and *in silico* approaches. Mohsen's current research has led to three different drug-development approaches.

1. Targeting the substrate binding site by using substrate analogs as stabilizing agents. An example that has seen success is the use of phenylbutyrate to stabilize the protein *in vivo* and provide thermal stability. Mohsen's studies led to a clinical trial led by Horizon Pharma to evaluate the effects of the drugs Buphenyl (sodium phenylbutyrate) and Ravicti (glycerol phenylbutyrate) on patients with MCAD K304E and their biochemical acylcarnitine profiles. Mohsen will continue with detailed characterization of Buphenyl's effects in other MCAD-deficient cell lines harboring other missense mutations and use a mouse model with the MCAD K304E mutation to better understand the pharmacokinetics.

2. Targeting remote pharmacophore sites to identify ligands that can bind to known sites on the MCAD tetramer and that have stabilizing pharmacophore characteristics. Using molecular modeling, Mohsen has identified the electron transfer flavoprotein docking site as a drug-development target for MCAD and other ACADs. A mutant 12-mer peptide designed to bind to the docking site increased thermal stability by 2–2.5°C. The proof-of-concept finding was critical in pursuing this site for drug design. Future experiments will focus on this peptide, and its structure will be used to scaffold fragment-based drug design and *in silico* screening.

3. Development of an I3CT to treat MCAD deficiency. Patients' fibroblasts with MCAD K304E mutation respond positively to TMZ treatment. The increase in enzyme activity and protein presence is hypothesized to be due to inhibition of the medium-chain 3-ketoacyl-CoA thiolase in a mechanism similar to the case of the long-chain acyl-CoA mentioned above.

4. Use of chaperone generating agents, namely triphenylbutyrylglycerol or trimetazidine in combination with PPARd agonist, which was shown to increase VLCAD mutant presence. Mohsen found that the combination of trimetazidine and a PPARd agonist tripled the effect of either alone.

Development of Chaperone for Leucine Metabolism Pathway Disorders. Using the I3CT concept, Mohsen identified a potential drug therapy for patients afflicted with Leucine metabolism pathway disorders. Treating patient cells with various Leucine metabolism pathway disorders with epigallocatechin gallate (EGCG), a naturally occurring compound found in green tea, is promising. The intent is to use EGCG, the enzyme that catalyzes the end-product step of the leucine catabolism pathway, to slow pathway intermediates' generation and accumulate ones that can bind back to enzymes and catalyze reactions in the pathway. Several diseases can be targeted with this treatment. Follow-up experiments will be carried out to prepare for clinical trials.

De-Risking the Use of Nitisinone (NTBC) for the Treatment of PKU. Using the same I3CT concept, Mohsen has used NTBC on PKU patients' cells. NTBC is an inhibitor of 4-hydroxyphenylpyruvate dioxygenase and is expected to cause an accumulation of 4-hydroxyphenylpyruvate and tyrosine and bind to tyrosine aminotransferase and PAH, respectively, and increase their stability in case of their deficiencies. Mohsen's studies on PKU patient cells provide a possible mechanistic explanation of the efficacy of NTBC in the PKU mice. The new patient cell data are expected to enable funded clinical trials and further de-risking experiments.

Elucidating the Function of New ACADs and Detailing the Roles of ACAD9 and VLCAD in Physiology.

1. Identification of roles for ACAD10 and mouse *Acad12* in physiology. *ACAD10* has been implicated in diabetes in humans. *Acad10* knockout mice experiments have confirmed a relationship. Although ACAD10 protein has an ACAD domain that is very similar to the ACAD family of flavoenzymes, it also has an extra-large domain that Mohsen hypothesizes is an electron transfer domain that likely binds NAD. Moreover, its active site according to the crystal structure of ACAD11 and modeling seem to contain basic residues, implying possibly more than just an α,β-dehydrogenation biochemical function, but perhaps another function comparable to glutaryl-CoA dehydrogenase, which has an additional decarboxylation function. Another exciting result from the investigations of ACAD10 in mice is the confirmation of the presence of another ACAD protein, ACAD12. This one is surprisingly very similar to a short peptide at the N-terminus, ~ 160 amino acids, plus the ACAD domain of ACAD10 to almost 97% homology. Mohsen is currently pursuing the identification of the function of this version of an ACAD.
2. Differentiation of roles for ACAD9 and VLCAD variant 3 in physiology. A mitochondrial β-oxidation spiral is mostly known to start with VLCAD for long-chain substrates. Whereas ACAD9 seems to utilize mostly unsaturated fatty acids and has been implicated to have a role as an assembly factor interacting with ECSIT, an apparent VLCAD variant, named variant 3, has never been studied for function and role in physiology; only its isoenzyme VLCAD short has been studied. The latter is more specific to very long (> 16 carbon chain length), saturated acyl-CoAs, respectively. Recombinant ACAD9 has activity toward saturated and unsaturated long-chain acyl-CoA substrates, but it is not upregulated in the case of VLCAD deficiency. Mohsen is studying this mechanism.

Characterization and Stability Studies of Isovaleryl-CoA Dehydrogenase (IVD) Naturally Occurring, Disease-Causing Missense Mutations. As many as six naturally occurring missense mutations found in IVA patients have been introduced into recombinant IVD cloned in a prokaryotic expression vector. Four were stable enough to produce, and two failed to produce protein in the cell-free extract. Three of the four had activity. Mohsen intends to characterize the mutants and their thermal stability, then assess them as potential targets for therapy.

Optimization of Anaplerotic Agents for Treating β-Oxidation Disorders. Triheptanoin (UX007) is a synthetic C7 fatty acid triglyceride that is being tested by Ultragenix to treat long-chain fatty acid oxidation disorders and glucose transporter 1 deficiency. Although clinical trials using UX007 have shown positive indicators and met some end points in clinical trials (unpublished), they have missed end points in phase II for the latter indication. Mohsen has proposed to compare the use of other branched chain organic acids to compare their efficacy in alleviating the biochemical phenotypes of many of the energy pathway deficiencies. Mohsen will continue to refine some of the anaplerotic agents and look for metabolic phenotype differences.

MAJOR LECTURESHIPS, SEMINARS, AND TEACHING

- "Mitochondrial Energy Metabolism and Reactive Oxygen Species Level Disruption in ACAD9-Deficient Fibroblasts," abstract, INFORM, Boston, Mass., 2016
- "Assessment of Mitochondrial Bioenergetics in Medium-Chain and Very-Long-Chain Fatty Acid Oxidation–Deficient Fibroblasts," abstract, INFORM, Boston, Mass., 2016
- "Mitochondrial Bioenergetics Disturbance and Increased Superoxide Production in Very-Long-Chain Acyl-CoA Dehydrogenase–Deficient Fibroblasts," abstract, annual

symposium of the Society for the Study of Inborn Errors of Metabolism, Rome, Italy, September 2016
- "Characterization of the Impairment of Mitochondrial Bioenergetics and Dynamics in Fibroblasts From Patients With Complex I Deficiency," abstract, annual symposium of the Society for the Study of Inborn Errors of Metabolism, Rome, Italy, September 2016
- "Newborn Screening for Time-Critical Metabolic Disorders: A New Paradigm to Test at Point of Care," oral presentation, Pediatric Academic Societies meeting, San Francisco, Calif., May 2017
- "Inhibitor-Induced *In Situ* Chaperone Therapy: A Novel Strategy for Treating MCAD and VLCAD Deficiencies," abstract, Flavins and Flavoproteins 19th International Symposium, Groningen, Netherlands, July 2017
- "Inhibiting Long-Chain 3-Ketoacyl-CoA Thiolase: A Novel Strategy for Treating Fatty Acid Oxidation Disorders," abstract, INFORM, Rio de Janeiro, Brazil, September 2017
- "Endoplasmic Reticulum-Mitochondria Crosstalk and Redox Homeostasis Disruption in Very-Long-Chain Acyl-CoA Dehydrogenase–Deficient Fibroblasts," abstract, INFORM, Rio de Janeiro, Brazil, September 2017
- "A Mitochondrial Targeted Antioxidant and a Cardiolipin Binding Peptide Decrease Superoxide Generation and Improve Mitochondrial Respiration in ACAD9-Deficient Fibroblasts," abstract, INFORM, Rio de Janeiro, Brazil, September 2017
- "A Novel Small-Molecule PPARδ Modulator for the Treatment of Fatty Acid Oxidation Disorders," abstract, INFORM, Rio de Janeiro, Brazil, September 2017
- "Elevated Superoxide Levels, Mitochondrial Dysfunction, and Endoplasmic Reticulum-Mitochondria Crosstalk Disruption in ETHE1- and Sulfite Oxidase-Deficient Fibroblasts," abstract, International Congress of Inborn Errors of Metabolism, Rio de Janeiro, Brazil, September 2017
- "Mitochondrial-Targeted Compounds Improve Mitochondrial Bioenergetics Disturbance in Very-Long-Chain Acyl-CoA Dehydrogenase-Deficient Fibroblasts," abstract, International Congress of Inborn Errors of Metabolism, Rio de Janeiro, Brazil, September 2017
- "The Biochemical Basis for Overlap of Clinical Features of LCHAD/TFP Deficiency with Mitochondrial Respiratory Chain Defects: Implications for New Therapeutic Approaches," abstract, International Congress of Inborn Errors of Metabolism, Rio de Janeiro, Brazil, September 2017
- "Developing a Tissue-Specific ACAD9-Deficient Mouse Model Using Cre-lox Recombination," abstract, International Congress of Inborn Errors of Metabolism, Rio de Janeiro, Brazil, September 2017
- "Reticular Dysgenesis and Mitochondriopathy Induced by Adenylate Kinase 2 Deficiency Identified in the Amish Population," abstract, International Congress of Inborn Errors of Metabolism, Rio de Janeiro, Brazil, September 2017
- "Inhibitor-Induced *In Situ* Chaperone Therapy: A Novel Drug Targeting Strategy for Treating Metabolic Disorders," abstract, International Congress of Inborn Errors of Metabolism, Rio de Janeiro, Brazil, September 2017
- "A Novel Drug Therapy Strategy for Treating Fatty Acid β-Oxidation Disorders," abstract, National Organization of Rare Disorders Summit, Washington D.C., October 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Society of Inborn Errors of Metabolism
- Society for the Study of Inborn Errors of Metabolism, European Union

Robert D. Nicholls, DPhil

Robert D. Nicholls is a professor of pediatrics and director of the Birth Defects Laboratories.

RESEARCH

A Pig Model of Prader-Willi Syndrome (PWS). The overarching goals of this work have been to establish a preclinical model for therapeutic testing and to determine the pathophysiological basis of PWS. PWS results from loss of function of a cluster of paternally expressed, imprinted genes and features neonatal failure to thrive, abnormal body composition, childhood-onset hyperphagia, and obesity. In contrast to mouse models, the team hypothesizes that pig models of PWS will display hyperphagia, obesity, and other clinical features of PWS. Using CRISPR/Cas9 genome editing in pig zygotes to generate mutations of the 2.05-kb imprinting control (PWS-IC) region, the team established the first pig models of PWS. Each non-rearranged allele has site-specific mutations ("scarring") at the gRNA target sites, indicating a 100% efficiency of genome editing *in vivo*. Manuscripts describing this work are in preparation. Next, the team will breed gene-edited pigs with PWS-IC mutations to generate cohorts of ~ 4 minipigs each for a PWS group and a control group. Affected animals have the mutation on the paternal allele, and unaffected carriers (mutation on the maternal allele) will allow natural breeding to maintain each stock (via maternal transmission) and continuous production of PWS piglets (via paternal transmission). The team will perform preliminary phenotype analyses for hypoglycemia; hormone deficiencies; body weight; height and length; hyperphagia; and obesity.

A Pig Model of PKU. Nicholls, in collaboration with UPMC pathologist Steven Dobrowolski, PhD, and Randall S. Prather, PhD, and colleagues at the University of Missouri, has generated the first PKU pig and a heterozygous carrier by CRISPR/Cas9 genome editing of the *Pah* gene. The PKU pig presents classic PKU with growth retardation and hypopigmentation; neurological features are currently under study. The *PAH* locus is heterozygous for a deleted allele and has an intact allele having mutations at each gRNA target site, whereas the PKU pig shows compound heterozygosity for deletions of exon 6 and of exons 6-7. The porcine model of PKU is expected to provide a suitable preclinical model for understanding PKU neuropathophysiology and for exploring new therapeutics.

Understanding Multiple Hormone Secretion Deficits in PWS. This project includes collaboration with Peter F. Drain, Department of Cell Biology at the University of Pittsburgh, for his expertise in mechanisms of secretory granule maturation and exocytosis, especially for insulin from β cells. PWS is a multisystem disorder with neonatal failure to thrive; childhood-onset hyperphagia; obesity; and deficits in growth hormone, gonadotropin-releasing hormone, oxytocin, insulin, and glucagon. In a PWS mouse model with failure to thrive, the team previously found severe pancreatic endocrine abnormalities, including early developmental defects and deficient basal and glucose-stimulated insulin secretion. To overcome genetic and physiological complexities in mouse models or humans, the team is using endocrine cell models that express PWS genes to determine the molecular and cellular mechanisms by which PWS genes control hormone secretion, focusing initially on insulin and glucagon (and on growth hormone in a collaboration with Leticia Guida's laboratory in Rio de Janeiro, Brazil). The team demonstrated a deficiency in insulin secretion specifically for the PWS cells, establishing an *in vitro* model. The work is now being written up for publication. Further work is ongoing to: (a) generate sublines with specific deletion of the top PWS candidate gene, (b) assess a small-molecule inhibitor of an enzyme considered to be a therapeutic target for PWS, and (c) generate PWS deletions in the αTC1-6::mCherry model of glucagon secretion.

Activation of Silenced Genes in PWS. The major goal is to develop CRISPR-dCas9 reactivation of imprinted genes from the silenced maternal chromosome for PWS genes. Using standard approaches for epigenetic editing, researchers can activate further the paternal PWS-IC allele encoding the bicistronic *SNURF-SNRPN* locus but not the maternal allele. Therefore, the team is currently assessing a novel approach using the DNA binding domain of a chromatin protein that binds only to the silent, maternal allele. In addition, a novel approach is being tested to increase the frequency of DNA repair by homologous recombination during CRISPR/Cas9 genome editing, because HR-based methods would improve the applications of genome editing for generation of novel cellular and animal models of disease as well as gene therapy approaches.

Transcriptional and miRNA Regulation in the Spastic Paraplegias (SPGs). The major goals of this project are to characterize the transcriptional and miRNA regulatory mechanisms for the 24 cloned SPG loci and to use the regulatory signatures to predict top candidate genes for the ~ 25 uncloned SPG loci. Following their identification of *NIPA1* mutations in SPG type 6 (SPG6), the researchers characterized transcriptional and miRNA regulation in the SPGs, beginning with work published on *SPAST*, the gene most frequently mutated in hereditary SPG, and continuing with identification of regulation of many additional SPG genes by a single miRNA operon (manuscript in preparation).

Molecular Basis for Overexpression of the BMI1 Oncogene in Cancer. The major goal of this project is to determine the molecular basis for overexpression of BMI1 in most cancer types that have been examined, based on epigenetic (DNA hypermethylation) or rare mutation of transcription factor binding sites in a novel "goldilocks enhancer" that normally acts to keep levels of BMI1 mRNA at moderate levels. Funding is being sought to move this exciting project forward.

Other Ongoing PWS-Related Research Projects. The Nicholls laboratory has characterized the PWS imprinted gene domain and the phenotypic characteristics of a brain-derived cell line in ferrets, representative of an epithelial-mesenchymal transition stage. They have also demonstrated efficient genome editing, as a prelude to attempting to generate ferret models of neurobehavioral disease as an alternative mammalian model with a complex, gyrencephalic brain (similar to humans and pigs but unlike rodents, which are lissencephalic). The group has characterized the cat PWS-imprinted domain as a potential mammalian model that is gyrencephalic and useful for reproductive technologies; next steps include collaboration with Leslie Lyons in Missouri to apply genome editing in this species to generate unique animal models. The latter two projects are aimed at ensuring the successful development of a clinically appropriate model of PWS for study of hyperphagia, obesity, and neurobehavioral features of this complex disorder in a species, with lower costs and space requirements for housing, care, and feeding than the current pig models of PWS.

Another ongoing project is assessing the role and translational mechanisms in expression of the SNURF-SmN operon in PWS, and, in collaboration with Vockley, the evolution and function of SNURF, putatively as a ubiquitin-like molecule. New collaborative studies include: (a) a project examining genetic susceptibility to schizophrenia, led by Vishwajit L. Nimgaonkar (Department of Psychiatry, University of Pittsburgh); and (b) collaboration on "A Postmortem Study of von Economo Neurons From Frontal Cortex of Brains of Persons With PWS" with Patrick R. Hof (Mt. Sinai, New York, N.Y.) and Jan Forster (Pittsburgh), funded by a research grant from the Foundation for Prader-Willi Research (FPWR).

STUDY SECTIONS

- Research grant review, FPWR

MAJOR LECTURESHIPS, SEMINARS, AND TEACHING

- "Research Into the Cause (and Cure) of Human Genetic Disease: The Roles of Genetically Modified Animal Models," middle school eighth-grade science, Winchester Thurston School, Pittsburgh, Pa., March 2017
- "Clinical Implications of Genome Editing," Summer Undergraduate Breakfast with Mentors Program, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., July 2017
- "Pancreatic β Cells with CRISPR/Cas9-Engineered 3.16 Mb Deletions Recapitulate the Insulin Secretory Deficits in Prader-Willi Syndrome," FPWR Scientific Day Conference, Indianapolis, Ind., August 2017
- "PWS Hormone-Secreting Cell Lines Generated by CRISPR/Cas9 Genome Editing," FPWR Workshop on Cell-Based Assays for PWS, Indianapolis, Ind., August 2017
- "Porcine Models of Neurobehavioral Genetic Disorders: PKU and PWS," sixth Swine in Biomedical Research Conference, Baltimore, Md., September 2017

ADVISORY COMMITTEE MEMBERSHIPS

- Member, Scientific Advisory Board, PWS Association (United States), includes scientific and medical statements on PWS and grant review, 1992 to the present

Damara Ortiz, MD

RESEARCH

Damara Ortiz is a co-investigator in several studies investigating the natural history of and potential therapeutics for several inborn errors of metabolism, including PKU, FAO defects, and urea cycle disorders. She is the primary investigator for several lysosomal storage disorders, including Fabry disease, Gaucher disease, and select mucopolysaccharidosis. She is a co-investigator for experimental therapies for Hunter disease and Fabry disease.

ADVISORY COMMITTEE MEMBERSHIPS

- Faculty member, Diversity and Inclusion Committee, Children's Hospital of Pittsburgh
- Regional advisor, LAL-D Regional Advisory Board, Alexion Pharmaceuticals

MAJOR LECTURESHIPS AND SEMINARS

- "Introduction to the Dysmorphology Exam," Department of Pediatrics, Division of Neonatology, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., 2016
- "General Principles of Emergency Management of Genetic Disorders," Department of Pediatrics, Division of Emergency Medicine, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., 2016
- "Introduction to the Dysmorphology Exam," Department of Pediatrics Noon Conference, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., 2017
- "The Pennsylvania Newborn Screening Experience for Pompe Disease," abstract, WORLD Symposium of the Lysosomal Disease Network, San Diego, Calif., 2017
- "Newborn Screening for Pompe Disease: The Pennsylvania Experience," abstract, ACMG annual meeting, Charlotte, N.C., April 2017
- "A Novel Derivative Chromosome 5 in a Patient With Multiple Congenital Anomalies: Two-Step Mechanism for Segmental Duplication Mediated Chromosomal Rearrangement," abstract, ACMG annual meeting, Charlotte, N.C., April 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- American Society of Human Genetics
- ACMG

HONORS/AWARDS

- UPMC's Award for Commitment and Excellence in Services (ACES), 2017
- Assistant program director, Medical Genetics, 2016 to the present
- Director, Lysosomal Storage Disorders Program, 2015 to the present

Michele D. Poe, PhD

RESEARCH

Michele D. Poe is the lead statistician and research manager for the Program for the Study of NDRD. Her research interests include finite population statistics and the analysis of longitudinal neurodevelopmental data and neuroimaging. She has been the primary statistician for the NDRD since 2003.

ADVISORY COMMITTEE MEMBERSHIPS

- Early Diagnosis/HGT-HIT-090 MPS II Advisory Board, Shire
- Consensus Conference on Cognitive Endpoints in MPS, National MPS Society, MPS Society of the UK

MAJOR LECTURESHIPS, SEMINARS, AND TEACHING

- "Natural History of Metachromatic Leukodystrophy," WORLD Symposium of the Lysosomal Disease Network, San Diego, Calif., 2016
- "Natural History of Hurler Syndrome," WORLD Symposium of the Lysosomal Disease Network, San Diego, Calif., 2016
- "Psychosine as a Biomarker for Krabbe Disease," WORLD Symposium of the Lysosomal Disease Network, San Diego, Calif., 2016

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- International Society for Pharmacoepidemiology
- Newborn Screening Translational Research Network Workgroup

Yudong Wang, PhD

RESEARCH

Yudong Wang's research is focused on the functional and physical interactions among FAO, electron transfer chain, and tricarboxylic acid cycle. Mitochondria are the site of three of the most important energy-generating pathways in humans: OXPHOS, FAO, and the tricarboxylic acid cycle. The general perception is that the three energy metabolism systems are working independently. Wang's research explores the physical basis of these interactions, hypothesizing that the enzymes together form a functional complex within the mitochondrial matrix. Defining the functional linkages is important to the theoretical understanding of energy metabolism and has potential direct benefit for the clinical diagnosis and treatment of patients with genetic defects in these enzymes.

MAJOR LECTURESHIPS, SEMINARS, AND TEACHING

- "Elucidating the Architecture of Branched Chain Amino Acid Metabolism Enzymes," Seventh Annual Rangos Research Symposium, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., June 2017
- "Elucidating the Architecture of Branched Chain Amino Acid Metabolism Enzymes," Dean's Day, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., April 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Consular, Association of Chinese Scholars and Students of West Virginia University

Audrey Woerner, MD

RESEARCH

Audrey Woerner's primary research interests overlap her clinical efforts to develop telemedicine for medical genetics consultation. Prior to moving to Pittsburgh, she was funded through the Innovation Acceleration Program at Boston Children's Hospital for a pilot project to provide inpatient genetics consultations to Beverly Hospital and South Shore Hospital utilizing telemedicine. The project ultimately moved into clinical practice. Woerner has continued those efforts in Pittsburgh and is launching both an outpatient telemedicine clinic at the Erie Specialty Care center and an inpatient telegenetics consultation service for the UPMC Hamot neonatal ICU in Erie.

ADVISORY COMMITTEE MEMBERSHIPS

- New England Genetics Collaborative, Quality Improvement Workgroup, University System of New Hampshire, 2016

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- ACMG
- American Telemedicine Association
- American Medical Association
- New England Regional Genetics Group

MAJOR LECTURESHIPS, SEMINARS, AND TEACHING

- "A Novel Derivative Chromosome 5 in a Patient With Multiple Congenital Anomalies: Two-Step Mechanism for Segmental Duplication Mediated Chromosomal Rearrangement," abstract, ACMG annual clinical genetics meeting, Phoenix, Ariz., March 2017

TEACHING ACTIVITIES

The division's physicians are actively involved in the training of fellows, medical residents, medical students, genetic counseling students, and nursing students at the University of Pittsburgh. Faculty members lecture in the School of Medicine, School of Nursing, and Graduate School of Public Health. In addition, faculty members give presentations for local support and community groups. They actively teach pediatric residents and medical students in one- to four-week genetics electives or rotations, usually for one month at a time, as well as on routine inpatient rounds. Approximately five to six medical students and residents per year take the genetics elective. Additionally, the division, with extensive involvement of genetic counselors, trains master's-level genetic counseling students. This commitment recently increased from eight to 10 students, each for a one-month rotation. Madan-Khetarpal coordinates the division's teaching activities. The division is one of a handful nationally that have a Medical Biochemical Genetics Fellowship Program.

The division actively participates in the Medical Genetics Residency Training Program recognized by the American Board of Medical Genetics. Ortiz is the program's codirector. Medical genetics residents (as many as four per year) rotate on the clinical genetics service for two months at a time and collaborate on clinical research projects, as time and interest allow. The division has been approved for an ACGME fellowship training program in medical biochemical genetics, with Lichter-Konecki as the program director. Vockley is the founder and director of a national training course given each year by the Society for Inherited Metabolic Disorders for all first-year genetics residents nationally.

Genetic counselors and physicians play an active role in the curriculum of the master's degree program in genetic counseling in the Graduate School of Public Health. This includes lecturing in didactic courses and serving as supervisors for counseling students' rotations through the division. In addition, division counselors provide educational content to a number of patient support groups, including the National Niemann-Pick Disease Foundation, the Fatty Oxidation Disorders Family Support Group, the Organic Acidemia Association, the United Mitochondrial Disease Foundation, and the Pennsylvania chapter of the PKU Family Support Group.

THREE-YEAR BIBLIOGRAPHY

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DIVISION OF NEPHROLOGY

Mission

The mission of the Division of Nephrology is:

- To care for children with renal and urinary tract disorders, directly and/or in consultation with other physicians
- To provide high-quality care, using the highest moral, ethical, and professional conduct
- To promote knowledge of renal topics to primary physicians, residents, medical students, advanced practice providers, and nurses through direct teaching and mentoring
- To extend knowledge of pathophysiology of renal disease through basic, clinical, and translational research
- To train pediatric nephrology fellows to become outstanding clinical nephrologists and to have the fellows engage in high-quality kidney/urinary tract–based research projects

FACULTY

Carlton M. Bates, MD
Professor of Pediatrics
Chief, Division of Nephrology
Codirector, Pediatric Nephrology Fellowship Program
Vice Chair of Basic Research

Michael Moritz, MD
Professor of Pediatrics
Clinical Director, Division of Nephrology
Medical Director, Pediatric Dialysis

Rannar Airik, PhD
Assistant Professor of Pediatrics

Demetrius Ellis, MD
Professor of Pediatrics

Dana Fuhrman, DO
Assistant Professor of Critical Care Medicine and Pediatrics

Jacqueline Ho, MD
Assistant Professor of Pediatrics
Codirector, Pediatric Scientist Development Program
Codirector, Pediatric Nephrology Fellowship Program

Yosuke Miyashita, MD, MPH
Assistant Professor of Pediatrics
Assistant Director, Pediatric Nephrology Fellowship Program

Christina Nguyen, MD
Assistant Professor of Pediatrics
Director, Pediatric Kidney Transplant Program

Sunder Sims-Lucas, PhD
Assistant Professor of Pediatrics

Agnieszka Swiatecka-Urban, MD
Associate Professor of Pediatrics

OVERVIEW OF DIVISION

The faculty consists of eight pediatric nephrologists and two PhD investigators. Among the nephrologists, Demetrius Ellis, Michael Moritz, Yosuke Miyashita, and Christina Nguyen are primarily clinically oriented, whereas Carlton Bates, Agnieszka Swiatecka-Urban, Jacqueline Ho, and Dana Fuhrman (dual appointment to Nephrology and Critical Care) are more focused on research, spanning clinical to basic discovery. Sunder Sims-Lucas, PhD, and Rannar Airik, PhD, are engaged in basic scientific research.

Major accomplishments in the division included new and ongoing research grants, including National Institutes of Heath (NIH) R01 awards (Bates, Ho, Swiatecka-Urban [co-investigator], and Nguyen [co-investigator]), an NIH R56 award (Swiatecka-Urban), NIH P30 pilot and feasibility sub-awards (Airik and Swiatecka-Urban), an NIH cooperative agreement sub-award (Swiatecka-Urban), an NIH U01 (Nguyen [co-investigator]), an NIH R00 Pathway to Independence Award (Airik), an NIH K01 award (Sims-Lucas), an NIH R03 award (Sims-Lucas), an NIH U24 award (Ho and Sims-Lucas [co-investigators]), an NIH GUDMAP award (Sims-Lucas [co-investigator]), an NIH T32 Pediatric Nephrology Fellowship training grant (Bates), an American Heart Association award (Swiatecka-Urban), University of Pittsburgh Vascular Medicine Institute pilot awards (Sims-Lucas), a University of Pittsburgh McGowan Institute for Regenerative Medicine pilot award (Bates and Sims-Lucas), and Children's Hospital of Pittsburgh Research Advisory Committee awards (Airik and Sims-Lucas).

The division engaged in many clinical research programs, quality-improvement projects, and educational initiatives. In association with the Midwest Pediatric Nephrology Consortium, Miyashita led ongoing studies focused on diagnosis and follow-up of white-coat hypertension, which will soon lead to a practice guideline. The kidney transplant program, led by Nguyen, recently joined the Improving Renal Outcomes Collaborative (IROC) Network, which is devoted to eliminating the survival disparity between children with kidney diseases and the general population. In 2017, Sims-Lucas became the director of student research training for the Department of Pediatrics and led another highly successful summer undergraduate student research program with 45 participants (chosen from more than 400 applicants), including three high school students from the Pittsburgh City School District. The division hosted its fourth annual Nephrotic Syndrome Symposium on September 28, 2017 (led by Swiatecka-Urban), which featured local, national, and international experts in nephrotic syndrome research.

The division continues to recruit high-quality trainees into the fellowship program, including Melissa Anslow, Caitlin Peterson, and Cassandra Formeck. Paul Fadakar and Emily Joyce graduated from the program in June 2017 and joined the faculty in July 2017. The division successfully recruited two new fellows to the program to start in fiscal year 2018: Amy Lucier and Christine Crana.

CLINICAL ACTIVITIES

The pediatric nephrology division at Children's Hospital of Pittsburgh of UPMC is the sole provider of specialty nephrology care, including dialysis, for children in the Pittsburgh area and for most of the children within a 100-mile radius of the hospital. The pediatric nephrology program is among the top five largest pediatric kidney transplant programs in the country and has included patients from 18 states outside Pennsylvania. The division continues to see patients at the main hospital campus in Lawrenceville as well as two satellites (Wexford and Monroeville) and four outreach sites (Erie, Johnstown, Chippewa, and Sharon), with plans to add an outreach site in Wheeling, W.Va., in 2018.

The division had nearly 3,000 outpatient visits and saw more than 500 inpatients over the past fiscal year. The division offered clinical nutrition consultations for all its patients in the outreach locations via telemedicine, saving patients extra trips to Lawrenceville. The ambulatory blood pressure monitoring program, led by Miyashita, continued to expand. Nguyen and Moritz also performed renal ultrasound exams, saving patients extra trips to the radiology department. Under the direction of Moritz, the division began using low-density lipoprotein apheresis, a novel therapy to treat patients with steroid-resistant forms of nephrotic syndrome, with some striking successes.

RESEARCH AND OTHER SCHOLARLY ACTIVITIES

Carlton M. Bates, MD

RESEARCH

Carlton Bates conducts NIH-funded studies on the role of fibroblast growth factor receptors (FGFRs) and their signaling adapter proteins in the developing kidney and lower urinary tract using the mouse as a model. The studies are clinically relevant given that congenital abnormalities of the kidney and urinary tract are responsible for most cases of chronic kidney disease in children. Using various gene-targeting approaches, the laboratory reveals how FGFRs and their adapters are critical for temporal patterning of multiple renal and bladder tissue lineages. They generate mouse models of pediatric structural kidney and lower urinary diseases, including obstructive nephropathy, renal aplasia/dysplasia/hypoplasia, vesicoureteral reflux and reflux nephropathy, progressive cystic kidney disease, atonic bladders, and functional bladder obstruction. The laboratory continues to elucidate the molecular pathways through which FGFRs pattern developing bladder urothelium and repair postnatal urothelium after injury. The laboratory recently discovered novel critical links between FGFR signaling and glucose metabolism in developing nephron progenitors. These approaches may lead to new biomarkers and therapeutic targets for congenital kidney and lower urinary tract disease.

STUDY SECTIONS

- Basil O'Conner Research Advisory Committee, March of Dimes
- Urologic and Hematologic Diseases D Subcommittee (reviewing training [T32, T35] and career development [K01, K08, K23, K24, K00, R25, R03] applications), National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK), NIH

Carlton M. Bates, MD
Division Chief, Nephrology

ADVISORY COMMITTEE MEMBERSHIPS

- Council of the International Pediatric Nephrology Association
- Council of the Society for Pediatric Research (Nephrology Section)
- Organizing Committee, 16th Congress of the International Pediatric Nephrology Association
- Training and Certification Committee, American Society of Pediatric Nephrology
- Internal Advisory Board, Center for Critical Care Nephrology, University of Pittsburgh School of Medicine

EDITORSHIPS

- Associate editor (review articles), *Pediatric Nephrology*
- Editorial Board, *Journal of the American Society of Nephrology*
- Editorial Board, *Bladder*

MAJOR LECTURESHIPS AND SEMINARS

- "Renal and Urogenital Development and Their Developmental Abnormalities," Developmental Renal

Malformations, Oligo/Anhydramnios: Pathophysiology and Clinical Aspects, National Institute of Child Health and Human Development workshop, Bethesda, Md., August 2016
- "From Tubes to Cysts: Novel Mechanisms Driving Pathology in Cystic Kidney Disease," 17th Congress of the International Pediatric Nephrology Association, Iguassu Falls, Brazil, September 2016
- "From Tubes to Cysts: Novel Mechanisms Driving Pathology in Cystic Kidney Disease," pediatric nephrology grand rounds, University of California, Los Angeles, October 2016
- "FGFR Signaling in Nephron Progenitors: Links to Metabolism and Cystogenesis," renal grand rounds, Cincinnati Children's Medical Center, Cincinnati, Ohio, March 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS
- American Pediatric Society
- American Physiological Society
- American Society for Clinical Investigation
- American Society of Nephrology
- American Society of Pediatric Nephrology
- International Society of Nephrology
- International Pediatric Nephrology Association
- National Kidney Foundation
- Salt and Water Club
- Society for Pediatric Research

Michael Moritz, MD

RESEARCH
Michael Moritz focuses on the epidemiology and treatment of dysnatremias in children. He introduced the concept of using 0.9% sodium chloride in maintenance parenteral fluids for the prevention of hospital-acquired hyponatremia, and he has been an outspoken advocate for this therapy. This has resulted in a worldwide change in medical practice in both children and adults.

He also introduced the concept of using intermittent 3% sodium chloride boluses for the treatment of hyponatremic encephalopathy, and this is now being accepted as the treatment of choice by consensus guidelines. He is an internationally recognized expert in the controversial topic of salt poisoning in children and has served as a medical expert in high-profile trials in the United Kingdom and United States. He also has conducted seminal work in the epidemiology and prevention of breastfeeding-associated hypernatremia. He was the first to report on bleeding complications from high-concentration heparin to pack hemodialysis catheters in children.

He is clinical director of the Division of Nephrology and the medical director of Children's Hospital of Pittsburgh's Pediatric Dialysis Unit. His specific research interests include disorders in sodium and water metabolism (central pontine myelinolysis, hyponatremic encephalopathy, hypernatremic dehydration, salt poisoning, fluid therapy), hemodialysis access in children, and pediatric hypertension trials.

Moritz is also the chief editor of *Frontiers in Pediatrics*. In that capacity, he oversees 20 journal sections and almost 3,000 editorial board members. In 2016, he was selected for the Chief Editor's Award out of a field of 54 chief editors.

ADVISORY COMMITTEE MEMBERSHIPS
- Content Development Team, American Board of Pediatrics
- Section of Fluid and Electrolyte Therapy in Children, Clinical Practice Guideline Subcommittee, American Academy of Pediatrics
- Clinical Affairs Committee, American Society of Pediatric Nephrology
- Committee for "Making Dialysis Safer," Centers for Disease Control and Prevention
- Medical Advisory Board, National Kidney Foundation Serving the Alleghenies

EDITORSHIPS
- Field chief editor, *Frontiers in Pediatrics*
- Specialty chief editor, Pediatric Nephrology Section, *Frontiers in Pediatrics*
- Associate editor, Nephrology Section, *Frontiers in Medicine*
- Research topic editor, "Recent Advances in Hyponatremia," *Frontiers in Medicine* and *Frontiers in Pediatrics*
- Member, Editorial Board, *Pediatric Nephrology*

MAJOR LECTURESHIPS AND SEMINARS
- "Outpatient Pediatric Nephrology," meeting of the Three Rivers Chapter of the National Association of Pediatric Nurse Practitioners, Pittsburgh, Pa., December 2016

- "What Nurses Need to Know About Hyponatremia," American Nephrology Nurses Association Spring Ahead meeting, Pittsburgh, Pa., May 2017
- "Prevention and Treatment of Hospital-Acquired Hyponatremia," pediatric grand rounds, Loma Linda University Health, Loma Linda, Calif., June 2017
- Prevention and Treatment of Hospital-Acquired Hyponatremia," pediatric grand rounds, New York University School of Medicine, New York, N.Y., June 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- American Society of Nephrology
- American Society of Pediatric Nephrology
- International Pediatric Nephrology Association
- National Kidney Foundation
- Society for Pediatric Research

HONORS

- Field Chief Editor's Award, *Frontiers Journals*, 2016
- Chair Distinction Award for outstanding service, 2016
- *Top Doctors*, Castle Connolly, 2017
- *Best Doctors in America*, Woodward/White, Inc., 2017
- Best Doctors, *Pittsburgh Magazine*, 2017
- University of Pittsburgh Honors Convocation, faculty honoree, 2017
- Publons Peer Review Award, top 1 percent peer reviews in medicine, 2017
- Certificate of Expertise, International Consortium for Clinical Research Excellence, Education, and Ethics, 2017

Rannar Airik, PhD

RESEARCH

Rannar Airik's research focuses on deciphering the mechanisms of chronic kidney disease using mouse models of a genetic form of childhood chronic kidney disease, nephronophthisis (NPHP), an autosomal recessive chronic kidney disease characterized by tubulointerstitial fibrosis, tubular basement membrane disruption, and kidney cysts. Renal fibrosis is the primary determinant of end-stage kidney disease. The pathomechanisms underlying NPHP are only poorly understood. Although NPHP has long been considered a "ciliopathy," caused by a dysfunction in cilia, recent gene identification in humans has linked the pathogenesis of NPHP to defective DNA damage response signaling, resulting in genome instability and cell-cycle defects. To study the disease mechanisms of NPHP and to understand the cellular and molecular mechanisms of DNA damage response in the renal pathology of NPHP, Airik has generated knockout mouse models of the human condition. He has employed primary and secondary cell-culture models of NPHP to interrogate the disease mechanisms. His research is supported by one NIH grant and several intramural funds.

STUDY SECTIONS

- Ad hoc reviewer, Kidney Research UK

MAJOR LECTURESHIPS AND SEMINARS

- "The Nephronophthisis Gene Anks6 Regulates Glomerular Development," internal medicine grand rounds, UPMC Presbyterian Hospital, University of Pittsburgh School of Medicine, June 2017
- Lecturer, Integrative Systems Biology Graduate School course Model Systems (#2035), University of Pittsburgh School of Medicine

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Society of Nephrology
- American Society of Human Genetics

Demetrius Ellis, MD

RESEARCH

Demetrius Ellis's research interests include diabetic nephropathy, pediatric hypertension, and pediatric transplantation. His research is supported, in part, by an NIH grant in collaboration with Trevor Orchard (Epidemiology of Diabetes Complications, phase II, DK34818-20).

ADVISORY COMMITTEE MEMBERSHIPS

- Ad hoc, Promotion Committee, University of Pittsburgh School of Medicine

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Society of Pediatric Nephrology
- Council of Pediatric Nephrology
- International Pediatric Nephrology Association
- National Kidney Foundation Serving the Alleghenies

HONORS

- Excellence in Pediatrics, Academic Pediatrician, Children's Hospital of Pittsburgh of UPMC physicians and Children's Hospital of Pittsburgh of UPMC alumni
- Highest overall teaching grade by medical students, nephrology fellows, and pediatric residents
- *Best Doctors in America*, Woodward/White, Inc.
- Best Doctors, *Pittsburgh Magazine* (33 consecutive years)

Dana Fuhrman, DO, MS

RESEARCH

Dana Fuhrman serves as a pediatric intensivist and nephrologist with an overall research interest in predicting and preventing acute kidney injury (AKI) in children and young adults. Specifically, she works toward establishing a method to predict a kidney's response to stress and decline in glomerular filtration rate over time. She previously studied and published a method to quantify renal functional reserve in healthy young adults. She plans to determine whether renal functional reserve values and differences in baseline tubular biomarkers may serve to identify a kidney with favorable "renal fitness," defined by the ability to respond well to renal stress and, therefore, show a decreased risk of acute kidney injury and chronic kidney disease. She has studied young adult patients with congenital heart disease who are at risk for numerous kidney injuries across a lifetime and the development of chronic kidney disease. She was a co-investigator for the Prospective Pediatric Acute Kidney Injury Research Group through Cincinnati Children's Hospital, an international research consortium dedicated to advancing research in pediatric acute kidney injury.

EDITORSHIP

- Review editor, *Pediatric Critical Care Medicine*, 2015 to the present

MAJOR LECTURESHIPS AND SEMINARS

- "Renal Replacement Therapy on Extracorporeal Membrane Oxygenation (ECMO)," Children's Hospital of Pittsburgh's annual ECMO conference, August 2016

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Society of Nephrology
- American Society of Pediatric Nephrology
- Society of Critical Care Medicine

Jacqueline Ho, MD

RESEARCH

Jacqueline Ho studies requirements for miRNAs in kidney progenitor cells during renal development and in the regulation of podocyte structure and function during proteinuric kidney diseases. miRNAs represent a fundamentally new means of controlling gene expression and have been implicated in the regulation of diverse developmental processes. Her previous work demonstrated a critical role for miRNAs in the regulation of kidney progenitor survival during development and in the maintenance of podocyte structure. Work in her laboratory focuses on the identification of specific miRNAs and their mRNA targets that mediate these effects. Her research is supported by an NIH R01 grant and intramural funds. She is a co-investigator on another R01 and a U24.

STUDY SECTIONS

- Reviewer, Division of Kidney, Urologic, and Hematologic Diseases Fellowship, NIDDK

ADVISORY COMMITTEE MEMBERSHIPS

- Research Committee, American Society of Pediatric Nephrology
- Program Committee, American Society of Pediatric Nephrology

EDITORSHIP

- Associate editor, section on pediatric nephrology, *Frontiers in Pediatrics*

MAJOR LECTURESHIPS AND SEMINARS

- "Correlation of Prenatal Imaging of Kidney Malformations as Basis for Intervention, Including Recent Experience With Pediatric Outcomes," Developmental Renal Malformations, Oligo/Anhydramnios: Pathophysiology and Clinical Aspects, National Institute of Child Health and Human Development workshop, Bethesda, Md., August 2016
- "Small RNAs and Small Kidneys," pediatric nephrology grand rounds, Children's Hospital of Philadelphia, Philadelphia, Pa., December 2016
- "Small RNAs and Small Kidneys," renal grand rounds, Northwestern University, Chicago, Ill., December 2016
- "Small RNAs and Small Kidneys," experimental biology meeting, Chicago, Ill., April 2017
- "Small RNAs and Small Kidneys: A Role for the miR-17~92 Cluster," Senior Vice Chancellor's Seminar Series, University of Pittsburgh School of Medicine, Pittsburgh, Pa., April 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- American Society of Nephrology
- American Society of Pediatric Nephrology
- Canadian Society of Nephrology
- Canadian Association of Pediatric Nephrologists
- International Pediatric Nephrology Association
- Society for Pediatric Research

HONORS

- Renée Habib Young Investigator Award, International Pediatric Nephrology Association, 2013

Yosuke Miyashita, MD, MPH

RESEARCH

Yosuke Miyashita's research interests focus on pediatric hypertension and Shiga toxin–producing *Escherichia coli*, as well as atypical hemolyticuremic syndrome. He was the representative for Children's Hospital of Pittsburgh of UPMC in the Midwest Pediatric Nephrology Consortium (MWPNC). He continues to participate in multicenter clinical trials with this consortium, especially in the field of pediatric hypertension and the use of 24-hour ambulatory blood pressure monitoring. His most recent efforts are aimed at formulating an evidence-based practice guideline for white-coat hypertension in children and adolescents. For this effort, he recently completed and published a survey study of pediatric nephrologists in the MWPNC centers regarding white-coat hypertension. He investigated the changes of 24-hour ambulatory blood pressure monitoring results over time for children and adolescents who were being followed for blood pressure concerns but were not on antihypertensive medication. Those were pilot studies for an eventual multicenter retrospective study to investigate the same issue in a larger MWPNC cohort. Lastly, he served as the faculty mentor for two trainees in their respective projects in the MWPNC.

ADVISORY COMMITTEE MEMBERSHIPS

- Clinical Affairs Committee, American Society of Pediatric Nephrology
- MWPNC

EDITORSHIP

- Review editor, Section on Pediatric Nephrology and Nephrology, *Frontiers in Pediatrics* and *Frontiers in Medicine*

MAJOR LECTURESHIPS AND SEMINARS

- "Evaluation and Management of White-Coat Hypertension," MWPNC meeting, Minneapolis, Minn., October 2016

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Society of Pediatric Nephrology
- International Pediatric Hypertension Association
- International Pediatric Nephrology Association

Christina Nguyen, MD

RESEARCH

Christina Nguyen focuses her research in the areas of transplant outcomes, transition of care between pediatric and adult medicine, and infectious complications in kidney transplantation. She is the director of the Pediatric Kidney Transplant Program. She serves as a site sub-investigator in two multicenter, NIH-funded research projects in the field of transplantation. She is a co-investigator for an internal grant with the Hillman Foundation looking at neurocognitive outcomes in very young recipients of kidney transplants. She serves as the site principal investigator in two pharmaceutical trials and a co-investigator on one other pharmaceutical trial. Active projects include outcomes and epidemiology of donor-specific antibodies in pediatric kidney transplantation, a case series on the use of bortezomib to treat acute antibody-mediated rejection, and stratification of pre-transplant psychosocial risk factors that are associated with high-risk post-transplant behaviors in kidney transplant recipients.

ADVISORY COMMITTEE MEMBERSHIPS

- Pediatric to Adult Transition Subcommittee, American Society of Transplantation
- Education Committee, American Society of Pediatric Nephrology
- Outreach Committee, International Pediatric Transplant Association

EDITORSHIP

- Review editor, *Frontiers in Pediatrics*, 2014 to the present
- Editorial Board for Kidney, *Pediatric Transplantation*, 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Society of Pediatric Nephrology
- American Society of Transplantation
- International Pediatric Transplant Association

HONORS

- Chair Distinction Award, Children's Hospital of Pittsburgh of UPMC, 2016

Sunder Sims-Lucas, PhD

RESEARCH

Sunder Sims-Lucas is a basic research scientist whose focus is on the formation of the developing vasculature in the kidney and the predisposition to adult-onset disease when there is defective development. He is involved in a major initiative by the NIH in the growth of new kidney tissues. His laboratory also studies blood flow and hypoxia and the role they play in patterning the various renal lineages. He recently began exploring the role of the microvasculature in acute kidney injury. He has authored more than 45 publications and, despite being an early-stage investigator, has been supported by many NIH grants, including a K01, an R03, and U24 grants. He has acquired local awards,

including a Children's Hospital of Pittsburgh Research Advisory Committee grant, a University of Pittsburgh Vascular Medicine Institute pilot award, and a University of Pittsburgh McGowan Institute for Regenerative Medicine pilot award.

STUDY SECTIONS

- Reviewer, ad hoc, P30 grants, NIDDK, NIH
- Reviewer, ad hoc, R03 grants, NIDDK, NIH
- Reviewer, (Re)Building a Kidney, Partnership Program grants, NIDDK
- Reviewer, Diabetic Complications Consortium (DiaComp) Pilot and Feasibility Program, NIDDK

ADVISORY COMMITTEE MEMBERSHIPS

- Faculty advisor for summer student interns, Children's Hospital of Pittsburgh
- Co-chair, Organizing Committee, Rangos Research Symposium, Children's Hospital of Pittsburgh
- Graduate Schools Admission Committee

EDUCATIONAL ROLES

- Appointed director of student research training at the Children's Hospital of Pittsburgh
- Member of the educational committee at the Children's Hospital of Pittsburgh
- Designed a new course titled, "Pediatrics: Bench to Bedside," which is run out of the Honors College

EDITORSHIPS

- Editorial Board, *Frontiers in Pediatrics*
- Special guest editor, *Frontiers in Cell and Developmental Biology*, *Organogenesis: From Development to Disease*

MAJOR LECTURESHIPS AND SEMINARS

- "Renal Endothelial Progenitors During Development and Injury," 13th International Workshop on Developmental Nephrology, Snowbird Ski and Summer Resort, Salt Lake City, Utah, July 2015
- "Blood Flow and Oxygenation During Kidney Development and Disease," Molecular Medicine Seminar Series, Department of Pediatrics, Children's Hospital of Pittsburgh, Pa., 2016

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Heart Association
- American Physiological Society
- American Society of Nephrology
- North American Vascular Biology Organization

Agnieszka Swiatecka-Urban, MD

RESEARCH

Agnieszka Swiatecka-Urban studies regulation of cell-surface stability and intracellular trafficking of membrane proteins in epithelial cells. One focus of her research activity has been characterization of mechanisms and pathways that regulate the cystic fibrosis transmembrane conductance regulator (CFTR) in order to improve treatment strategies for cystic fibrosis. Swiatecka-Urban has also used her expertise to address a significant unmet need of patients with nephrotic syndrome, a severe form of kidney disease responsible for 20% of pediatric kidney failure. She has been studying the role of protein-protein interactions in the dynamic regulation of nephrin in the podocyte slit diaphragm—an intercellular junction, which is compromised in nephrotic syndrome. Swiatecka-Urban's work on both nephrin and CFTR has resulted in several successful research awards. Her research is supported by an NIH R56 award, an NIH P30 pilot and feasibility sub-award, an NIH cooperative agreement sub-award, and an American Heart Association grant. Finally, she leads the University of Pittsburgh site for the CureGlomerulopathy (CureGN) network cohort study—a multicenter, prospective cohort observational study for patients with nephrotic syndrome.

STUDY SECTIONS

- Research and Research Training Committee, Cystic Fibrosis Foundation
- Basic Cell MSO1 Study Section, American Heart Association
- Ad hoc reviewer, Digestive, Kidney, and Urological Systems, NIH
- Review Committee, University of Pittsburgh Competitive Medical Research Fund
- Research Council United Kingdom
- University of Pittsburgh Competitive Research Fund
- Research Fellowship Grants Committee, Mallinckrodt Pharmaceuticals
- Innovative Research Grant Basic Science 3 Study Section, American Heart Association

ADVISORY COMMITTEE MEMBERSHIPS

- Competitive Medical Research Fund
- Research Council United Kingdom
- PhD Defense Committee, Center for Biodiversity, Functional, and Integrative Genomics, University of Lisbon, Lisbon, Portugal
- PhD Defense Committee, Geisel School of Medicine, Dartmouth College, Hanover, N.H.
- Mallinckrodt Pharmaceuticals, Inc.
- Alexion Pharmaceuticals, Inc.

- Chair, annual Nephrotic Syndrome Symposium: Evidence-Based Management and Personalized Approach, Children's Hospital of Pittsburgh
- Science Policy and Public Policy committees, Personalized Medicine Coalition
- Member, Program Committee, Pediatric Academic Societies/American Society of Pediatric Nephrology, 2016 to the present
- Workshop champion, The Future of Nephrology: Tissue Regeneration, 3D Printing, and the Wearable Kidney, scheduled for the 2017 Pediatric Academic Societies/American Society of Pediatric Nephrology meeting
- Program Committee, Pediatric Academic Societies/American Society of Pediatric Nephrology

EDITORSHIPS

- Editorial Board, *American Journal of Physiology—Renal Physiology*
- Editorial Board, *Conference Papers in Cell Biology*
- Editorial Board, *Frontiers in Physiology*
- Editorial Board, *Journal of Visual Experimentation*
- *Frontiers in Pediatric Research*
- Associate guest editor: nephrotic syndrome, *Frontiers in Pediatric Research*
- Associate editor, section on *Pediatric Nephrology*
- *British Journal of Pharmacology*

MAJOR LECTURESHIPS AND SEMINARS

- "Role of MicroRNA 145 in TGFβ1-Mediated Repression of CFTR in Primary Human Bronchial Epithelium," 131st Salt and Water Club Meeting, University of Pittsburgh School of Medicine, 2016
- "Incidence of Pediatric Nephrotic Syndrome—Single Center Experience," National Kidney Foundation and Kidney and Urology Foundation of America Nephrotic Syndrome Symposium, New York, N.Y., 2016

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Society of Nephrology
- American Society of Pediatric Nephrology
- International Pediatric Nephrology Association
- International Society of Nephrology
- Salt and Water Club
- Society for Pediatric Research

HONORS

- Fellow, American Society of Nephrology
- *Top Doctors*, Castle Connolly, 2015
- Chair, Third Annual Symposium, Nephrotic Syndrome: Clinical Challenges and Evidence-Based Management, Pittsburgh, Pa., 2016
- Faculty, BioSys-PhD Program in Biological Systems, Functional and Integrative Genomics. BioSys-PhD is a multidisciplinary PhD program sponsored by the Portuguese Foundation for Science and Technology. Students receive 50% of their training in Swiatecka-Urban's lab, 2015.
- Tenured associate professor of pediatrics and cell biology, University of Pittsburgh School of Medicine
- UPMC's Excellence in Patient Experience, Physician and Medical Staff Honor Roll
- Award for Excellence in Patient Experience, Physician and Medical Staff, presented by the Wolff Center at UPMC

THREE-YEAR BIBLIOGRAPHY

2015

Acker SN, Mandell EW, **Sims-Lucas S**, Gien J, Abman SH, Galambos C. Histologic identification of prominent intrapulmonary anastomotic vessels in severe congenital diaphragmatic hernia. *Journal of Pediatrics*. 2015;166:178-83.

Agathis NT, Libman IM, **Moritz ML**. Hyponatremia due to severe primary hypothyroidism in an infant. *Front Pediatr*. 2015; 3(96):1-4.

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Galambos C, **Sims-Lucas S**, Ali N, Gien J, Dishop MK, Abman SH. Intrapulmonary vascular shunt pathways in alveolar capillary dysplasia with misalignment of pulmonary veins. *Thorax*. 2015;70:84-5.

Joyce E, **Ho J**, El-Gharbawy A, Salgado CM, Ranganathan S, Reyes-Mugica M. The value of renal biopsy in diagnosing infantile nephropathic cystinosis associated with secondary nephrogenic diabetes insipidus. *Pediatr Dev Pathol*. 2015; 20(1):72-75.

Junttila S, Saarela U, Halt K, Manninen A, Parssinen H, Lecca MR, Brandli AW, **Sims-Lucas S**, Skovorodkin I, Vainio SJ. Functional genetic targeting of embryonic kidney progenitor cells *ex vivo. J Am Soc Nephrol*. 2015;26:1126-37.

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Moritz ML, Ayus JC. Hyponatraemia: Isotonic fluids prevent hospital-acquired hyponatremia. *Nat Rev Nephrol*. 2015;11(4):202-3.

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Phua YL, **Ho J**. MicroRNAs in the pathogenesis of cystic kidney disease. *Curr Opin in Pediatr.* 2015;27:219-26.

Pastor-Soler NM, Sutton TA, Mang HE, Kinlough CL, Gendler SJ, Madsen CS, Bastacky SI, **Ho J**, Al-Bataineh MM, Hallows KR, Singh S, Monga SP, Kobayashi H, Haase VH, Hughey RP. Muc1 is protective during kidney ischemia-reperfusion injury. *Am J Physiol Renal Physiol.* 2015;308(12):F1452-62.

Phua YL, Chu JYS, Marrone AK, Bodnar AJ, **Sims-Lucas S**, **Ho J**. Renal stromal miRNAs are required for normal nephrogenesis and glomerular mesangial survival. *Physiol Reports.* 2015;3(10).

Rymer CC, **Sims-Lucas S**. *In utero* intra-cardiac tomato-lectin injections on mouse embryos to gauge renal blood flow. *J Vis Exp.* 2015;96.

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2016

Airik R*, Schueler M, Airik M, Cho J, Porath JD, Mukherjee E, Sims-Lucas S, Hildebrandt F*. A FANCD2/FANCI-associated nuclease 1-knockout model develops karyomegalic interstitial nephritis. *J Am Soc Nephrol.* 2016 27(12):3552-9. *Co-corresponding authors

Airik R*, Schueler M, Airik M, Cho J, Ulanowicz KA, Porath JD, Hurd TW, Bekker-Jensen S, Schrøder JM, Andersen JS, Hildebrandt F*. SDCCAG8 interacts with RAB effector proteins RABEP2 and ERC1 and is required for hedgehog signaling. *PLoS One*. 2016;11(5):e0156081. *Co-corresponding authors

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Bebee TW, **Sims-Lucas S**, Park JW, Bushnell D, Cieply B, Xing Y, **Bates CM***, Carstens RP*. Ablation of the epithelial-specific splicing factor Esrp1 results in ureteric branching defects and reduced nephron number. 2016. *Dev Dyn*. 245(10):991-1000. *Co-corresponding authors

Casinelli G, LaRosa J, Sharma M, Cherok E, Banerjee S, Branca M, Edmunds L, Wang Y, **Sims-Lucas** S, et al. N-Myc overexpression increases cisplatin resistance in neuroblastoma via deregulation of mitochondrial dynamics. *Cell Death Discov*. 2016;2:16082.

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Hemker SL, **Sims-Lucas S**, **Ho J**. The role of hypoxia in nephrogensis. *Pediatr Nephrol.* 2016;31(10):1571-7.

Joyce EL, Kane-Gill SL, **Fuhrman, DY**, Kellum JA. Drug-associated acute kidney injury: Who's at risk? *Pediatr Nephrol*. 2016;32:59-69.

Liu X, Edinger RS, Klemens CA, Phua, YL, Bodnar AJ, LaFramboise WA, **Ho J**, Butterworth MB. A microRNA cluster miR-3~24~27 is upregulated by aldosterone in the distal kidney nephron where it alters sodium transport. *J Cell Physiol*. 2016 Sept 16.

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Maringer K, Yarbrough A, **Sims-Lucas S**, Saheb E, Jawed S, Bush J. Dictyostelium discoideum RabS and Rab2 colocalize with the Golgi and contractile vacuole system and regulate osmoregulation. *J Biosci*. 2016;41(2):205-17.

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DIVISION OF
NEWBORN MEDICINE

Mission

The mission of the Division of Newborn Medicine at UPMC is:

- To provide high-quality, state-of-the-art clinical care to high-risk neonates admitted to the Neonatal Intensive Care Units (NICUs) at Magee-Womens Hospital of UPMC, Children's Hospital of Pittsburgh of UPMC, UPMC Hamot, UPMC Mercy, and UPMC Horizon
- To provide educational and clinical service needs to community hospitals in the tristate region with which the division has partnered, such as Wheeling Hospital, Excela Westmoreland Hospital, and Washington Hospital.
- To perform basic and clinical research studies at the leading edge of neonatal research
- To provide first-rate medical education and teaching to all levels of students in the University of Pittsburgh School of Medicine, pediatric house officers, and neonatal-perinatal medicine fellows from the Accreditation Council for Graduate Medical Education (ACGME)-accredited UPMC medical education program, as well as to regional obstetricians, pediatricians, and family-practice physicians who care for high-risk mothers and neonates

FACULTY

Richard A. Saladino, MD
Interim Chief, Division of Newborn Medicine
Director, Neonatal-Perinatal Medicine Fellowship Program

Abeer Azzuqa, MD
Assistant Professor of Pediatrics
Associate Clinical Director, NICU, Magee-Womens Hospital of UPMC
Medical Director, UPMC Advanced Practice Provider Neonatal Residency Program

Arcangela Balest, MD
Assistant Professor of Pediatrics

Michael J. Balsan, MD
Associate Professor of Pediatrics
Clinical Director, NICU, UPMC Hamot

Charles V. Bender, MD, MJ
Associate Professor of Pediatrics
Associate Professor of Obstetrics, Gynecology, and Reproductive Sciences
Assistant Chief, UPMC Newborn Medicine Program

Beverly S. Brozanski, MD
Professor of Pediatrics
Professor of Obstetrics, Gynecology, and Reproductive Sciences
Clinical Director, NICU, Children's Hospital of Pittsburgh of UPMC

Justin R. Buland, DO
Assistant Professor of Pediatrics

Carmela Coppola, MD
Clinical Assistant Professor of Pediatrics, UPMC Mercy

Sean A. Frederick, MD
Assistant Professor of Pediatrics

Carol Gilmour, MD, MPH
Assistant Professor of Pediatrics, Obstetrics, Gynecology, and Reproductive Sciences
Clinical Director, NICU and Newborn Services, UPMC Mercy

Misty Good, MD
Assistant Professor of Pediatrics

C. Miles Harmon, MD
Assistant Professor of Pediatrics

James Kiger, MD
Assistant Professor of Pediatrics

Jennifer Kloesz, MD
Assistant Professor of Pediatrics
Assistant Program Director, Neonatal-Perinatal Medicine Fellowship Program
Clinical Director, NICU, Magee-Womens Hospital of UPMC

Burhan Mahmood, MD
Assistant Professor of Pediatrics
Assistant Professor of Obstetrics, Gynecology, and Reproductive Sciences
Clinical Director, Extracorporeal Membrane Oxygenation (ECMO) Program, NICU, Children's Hospital of Pittsburgh of UPMC

William McCarran, MD
Assistant Professor of Pediatrics

Brock Medsker, MD
Assistant Professor of Pediatrics

Sharmilarani Nanda, MBBS
Clinical Assistant Professor of Pediatrics
Clinical Director, University of Pittsburgh Physicians Special Care Nursery, Excela Westmoreland Hospital

Ifeyinwa Nwankwor, MD
Assistant Professor of Pediatrics

Brighid O'Donnell, MD
Assistant Professor of Pediatrics

Karin P. Potoka, MD
Assistant Professor of Pediatrics

Mohammad Riaz, MD
Assistant Professor of Pediatrics

Melissa Riley, MD
Assistant Professor of Pediatrics
Medical Codirector, Neonatal Transport Services, Children's Hospital of Pittsburgh of UPMC
Associate Clinical Director, NICU, Children's Hospital of Pittsburgh of UPMC

Anthony Rudine, MD, MBA
Assistant Professor of Pediatrics

Richard Telesco, MD
Assistant Professor of Pediatrics
Associate Clinical Director, NICU, UPMC Hamot

Amy M. Urban, DO, MPH
Assistant Professor of Pediatrics
Assistant Professor of Obstetrics, Gynecology, and Reproductive Sciences
Clinical Director, Interoperability

Kalyani Vats, MD
Assistant Professor of Pediatrics
Director, Triage and Antenatal Consult Services

Jon F. Watchko, MD
Professor of Pediatrics
Professor of Obstetrics, Gynecology, and Reproductive Sciences

Toby Debra Yanowitz, MD, MS
Associate Professor of Pediatrics
Assistant Professor of Obstetrics, Gynecology, and Reproductive Sciences
Assistant Investigator

OVERVIEW OF DIVISION

The division's major clinical responsibilities include: (a) availability of around-the-clock consultations for high-risk obstetrical patients, including inpatients and outpatients seen in the Fetal Diagnostic and Treatment Center and more than 9,600 well babies admitted this year to the Well Baby Nurseries at Magee-Womens Hospital of UPMC, as well as delivery-room resuscitative support for high-risk newborns at Magee-Womens Hospital of UPMC and 24-hour-a-day clinical care for approximately 2,000 high-risk neonates admitted this year to the Magee-Womens Hospital of UPMC NICU (average daily census of 70 infants); (b) around-the-clock coverage for the NICU at Children's Hospital of Pittsburgh of UPMC, including clinical care for more than 1,200 neonatal medical and surgical patients per year (average daily census of 50 patients); (c) consultative services for infants in the Cardiac Intensive Care Unit at Children's Hospital of Pittsburgh of UPMC; (d) 24-hour-a-day clinical care, along with consultative and resuscitative services, for both high-risk neonates and well babies admitted to the UPMC Mercy NICU and Newborn Nursery; (e) 24-hour-a-day clinical care for approximately 310 high-risk neonates at UPMC Hamot Women's Hospital this year (average daily census of 14), in addition to delivery-room resuscitative support for high-risk newborns, developmental follow-up services, and a 24/7 ground and air transport team; and (f) management of infants seen for follow-up at the High-Risk Infant Follow-Up Clinic, which provides support for infants on home monitors and for those with chronic lung disease, as well as long-term neurodevelopmental and nutritional follow-up care of NICU graduates. The Developmental Services team saw more than 700 neonates in the hospital this year for developmental assessments prior to discharge. The Division of Newborn Medicine continued to be responsible for neonatal ECMO. This program continues to develop detailed clinical consensus protocols and intensive training for all involved faculty, fellows, and neonatal nurse practitioners.

The community neonatology arm of the division played an active role in outreach education and services, including: (1) around-the-clock care of more than 180 infants transferred this year to the Children's Home of Pittsburgh's level II facility from the NICUs at Magee-Womens Hospital and Children's Hospital (average daily census of about seven infants); (b) monthly educational sessions with the Family Practice Residency Program at Washington Hospital and the placement of a nurse practitioner on site to assist with delivery-room attendance and stabilization, to improve transport readiness, and to provide daily education to staff; (c) bimonthly sessions with the pediatric staff at Excela Health Center for continuous quality improvement (QI) of newborn care and the placement of a neonatologist on site; (d) neonatal educational sessions and neonatal resuscitation courses for healthcare professionals in the community; and (e) telemedicine now offered to referral and partner hospitals for use in their nurseries, providing them with around-the-clock access to neonatologists for consultation and pre-transport management strategies.

Transport by ground or helicopter is available 24 hours a day by a team of nurses, respiratory therapists, and physicians with special expertise in dealing with problems of the newborn. The transport numbers have continued to grow since the inception of this service.

The division also provides an extensive and comprehensive educational program for both families and staff to increase the use of breast milk for the infant population; 81% of babies who are admitted at younger than eight days of life received breast milk within 24 hours of discharge.

RESEARCH AND OTHER SCHOLARLY ACTIVITIES

Richard A. Saladino, MD

Richard Saladino's mentorship efforts blend his past laboratory interests (pathophysiology and prevention and treatment of serious bacterial infections) and current clinical interests (process improvement, biosurveillance, procedural sedation, and evaluation and management of traumatic injuries). Saladino is a co-investigator on two major grants: an NIH R01 grant titled "Novel Approaches to Screening for Inflicted Childhood Neurotrauma" and a Patient-Centered Outcomes Research Institute grant titled "Using the Electronic Medical Record to Improve Outcomes and Decrease Disparities in Screening for Child Physical Abuse." He has mentored numerous students, residents, fellows, MD-PhD students, and junior faculty in their clinical and scholarly projects.

PROFESSIONAL AFFILIATIONS/ SOCIETY MEMBERSHIPS

- Society for Pediatric Research
- American Academy of Pediatrics
- Section on Emergency Medicine, American Academy of Pediatrics
- Academy of Master Educators, University of Pittsburgh School of Medicine

Richard A. Saladino, MD
Interim Division Chief, Pediatric Emergency Medicine

Abeer Azzuqa, MD

RESEARCH

Abeer Azzuqa is the physician liaison for the division's telemedicine program. Telemedicine is used during transports from regional and community hospitals to support and supervise the care of infants, improving outcomes of critically ill and often unstable infants. The telemedicine capacity also serves a valuable educational component, allowing the division to broadcast educational conferences to outside sites.

ADVISORY COMMITTEE MEMBERSHIPS

- Fellowship Committee, UPMC Neonatal-Perinatal Medicine Fellowship Program

EDITORSHIPS

- *Handbook of Neonatal Critical Care*

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Perinatal Section, American Academy of Pediatrics

Arcangela Balest, MD

ADVISORY COMMITTEE MEMBERSHIPS

- Chair, NICU Feeding Task Force, Children's Hospital of Pittsburgh
- Chair, Hyperbilirubinemia Guidelines Committee, Children's Hospital of Pittsburgh
- Chair, Bronchiolitis Guidelines Committee, Children's Hospital of Pittsburgh
- Chair, Modified Barium Swallow Guidelines Committee, Children's Hospital of Pittsburgh
- Resident Mentor, Children's Hospital of Pittsburgh
- Clinical Resource Management Committee, Children's Hospital of Pittsburgh
- Bedside Monitoring Taskforce, Children's Hospital of Pittsburgh

Michael J. Balsan, MD

RESEARCH

Michael Balsan is an active member of the Vermont-Oxford Neonatal Database and participates in multicenter clinical research endeavors related to that group. In 2015, Balsan was instrumental in the successful receipt of a University of Pittsburgh–affiliated Pittsburgh Innovation Challenge grant award for NEATCAP, which is a hearing-protection device for babies in neonatal intensive care units that blocks noxious noise to reduce infant stress, improve sleep, and promote brain development.

ADVISORY COMMITTEE MEMBERSHIPS

- Allied Pediatric Health, Board of Directors, Shriners Hospital for Children–Erie Ambulatory
- Maternal Child Health Committee, Pennsylvania March of Dimes
- Pennsylvania Premature Infant Health Network
- Surgery Center and Outpatient Specialty Care Center
- Blood Transfusion Committee, UPMC Hamot

- Infection Control Committee, UPMC Hamot
- Local board member, March of Dimes
- Medical Education Committee, UPMC Hamot
- Patient Safety and Quality Committee, UPMC Hamot

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics

Charles V. Bender, MD, MJ

ADVISORY COMMITTEE MEMBERSHIPS

- Ethics Committee, Children's Hospital of Pittsburgh of UPMC

EDITORSHIPS

- Contributing editor, *Obstetric Anesthesia Digest*

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Carroll F. Reynolds History of Medicine Society
- Southern Association for the History of Medicine and Science

Beverly S. Brozanski, MD

RESEARCH

Beverly Brozanski remains focused on neonatal outcomes research and cost effectiveness. She has worked with 27 children's hospitals in the United States (Children's Hospital Neonatal Consortium [CHNC]) to develop a database for QI and benchmarking for the care of infants admitted to NICUs in children's hospitals. She was the co-chair of the CHNC QI steering committee to help develop and implement QI projects. The group's first QI project was to develop and evaluate a clinical practice recommendation for the care and use of central lines to prevent central line–associated bloodstream infections. The collaboration resulted in a 22% decrease in such infections, from 1.39 to 1.07 infections per 1,000 line days.

A second QI project was initiated to improve perioperative handoffs and decrease postoperative hypothermia. Preliminary results showed a decrease of about 40% in rates of postoperative hypothermia and an improvement in postoperative handoffs as indicated by a decrease in reported care failures. Brozanski worked with clinicians in CHNC to develop best practices for the care of babies with gastroschisis and congenital diaphragmatic hernia.

ADVISORY COMMITTEE MEMBERSHIPS

- Collaborative Initiative for QI Steering Committee, Child Health Corporation of America
- Children's Hospital Neonatal Consortium Board
- National Neonatal Working Group to develop national scoring for *U.S. News & World Report*
- Pittsburgh Milk Bank Medical Advisory Board

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Society for Pediatric Research
- American College of Physician Executives

HONORS

- Best Doctors, *Pittsburgh Magazine*, 2016, 2017
- America's Best Physicians, National Consumer Advisory Board, 2016, 2017
- Best Doctors in America®, 2017

Justin Buland, MD

ADVISORY COMMITTEE MEMBERSHIPS

- Fellowship Committee, UPMC Newborn Medicine Program

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- American Thoracic Society
- Society of Pediatric Research

Carmela Coppola, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics

Sean A. Frederick, MD

RESEARCH

Sean Frederick is a clinical neonatologist within the UPMC Newborn Medicine Program. He serves as the assistant chief medical information officer for Children's Hospital of Pittsburgh of UPMC. As such, he focuses on advancing and developing the hospital's use of its electronic health record.

ADVISORY COMMITTEE MEMBERSHIPS

- NICU Hypothermia Committee, UPMC Newborn Medicine Program
- Neonatal ECMO Core Team, UPMC Newborn Medicine Program

- Neonatal ECMO Consensus Committee, UPMC Newborn Medicine Program
- Non-Emergent Neonatal Intubation Consensus Committee, UPMC Newborn Medicine
- Femoral Line Placement Committee, UPMC Newborn Medicine Program
- Pediatric Physician Lead, Maternal Infant Health Project, UPMC
- Readmissions Project Team, Children's Hospital of Pittsburgh of UPMC
- CNET Committee, Children's Hospital of Pittsburgh of UPMC
- Critical Care Dose Range Checking Committee, Children's Hospital of Pittsburgh of UPMC
- Pediatric Physician Lead, Maternal Infant Health Project, UPMC
- Chair, Medical Informatics Committee, UPMC Newborn Medicine Program
- eRecord Physician Advisory Council, Children's Hospital of Pittsburgh of UPMC

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Perinatal Section, American Academy of Pediatrics
- American Medical Association

Carol Huntress Gilmour, MD, MPH

ADVISORY COMMITTEE MEMBERSHIPS

- Medical Executive Committee, UPMC Mercy
- Perinatal and Gynecology Morbidity and Mortality Committee, UPMC Mercy Hospital
- Chair, Neonatal Care and Quality Committee, UPMC Mercy Hospital
- Quality Committee, UPMC Mercy Hospital

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Perinatal Section, American Academy of Pediatrics
- Academy of Breastfeeding Medicine

Misty Good, MD

RESEARCH

Misty Good's laboratory focuses on the cellular and molecular mechanisms involved in the development of necrotizing enterocolitis (NEC), a gastrointestinal disease affecting premature infants. Utilizing a mouse model of NEC, she investigates the mechanisms by which breast milk and amniotic fluid prevent the progression of the disease. Good has demonstrated that amniotic fluid inhibits Toll-like receptor 4–mediated pro-inflammatory signaling in the fetal and neonatal intestinal epithelium and prevents NEC via epidermal growth factor (EGF) receptor activation (*PNAS*, 2012). Furthermore, she has discovered that the EGF in breast milk is responsible for the protective effect against NEC (*Mucosal Immunology*, 2015). To pursue translational significance, she has developed a premature piglet model of NEC and has begun testing therapeutics in this model (*AJP-GI* and *Liver*, 2014). Her laboratory continues to study the intestinal mucosal host defense involved in NEC pathogenesis, to gain insights that will lead to the development of novel therapeutics for the disease. She conducts ongoing translational research studies evaluating the differences in the biological signature of premature infants with and without NEC. The long-term goal is to determine which infants are the most susceptible to the disease, which would afford the opportunity to intervene earlier in the disease course and ultimately prevent the development of this devastating disease altogether.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Pediatric Academic Societies

James Kiger, MD

ADVISORY COMMITTEE MEMBERSHIPS

- Medical Director, Neonatal Respiratory Therapy and Care, UPMC Newborn Medicine
- Medical Codirector of Respiratory Care, Children's Hospital of Pittsburgh of UPMC
- Physician chair, Ventilator Adverse Event Committee, Children's Hospital of Pittsburgh

Jennifer Kloesz, MD

ADVISORY COMMITTEE MEMBERSHIPS

- Fellowship Committee, UPMC Neonatal-Perinatal Medicine Fellowship Program
- Medical Executive Committee, Magee-Womens Hospital of UPMC
- Newborn Quality Assurance Committee, Magee-Womens Hospital of UPMC
- Risk Management Committee, Magee-Womens Hospital of UPMC
- Board member, Three Rivers Mothers' Milk Bank
- Premature Infant Health Network
- Transfusion Committee, Magee-Womens Hospital

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Perinatal Section, American Academy of Pediatrics

HONORS

- Jon F. Watchko, MD, Fellowship Mentoring Award, UPMC Neonatal-Perinatal Fellowship Training Program, 2016

William McCarran, MD

ADVISORY COMMITTEE MEMBERSHIPS

- Children's Hospital Neonatal Database Severe Chronic Lung Disease Working Group, Child Health Corporation of America

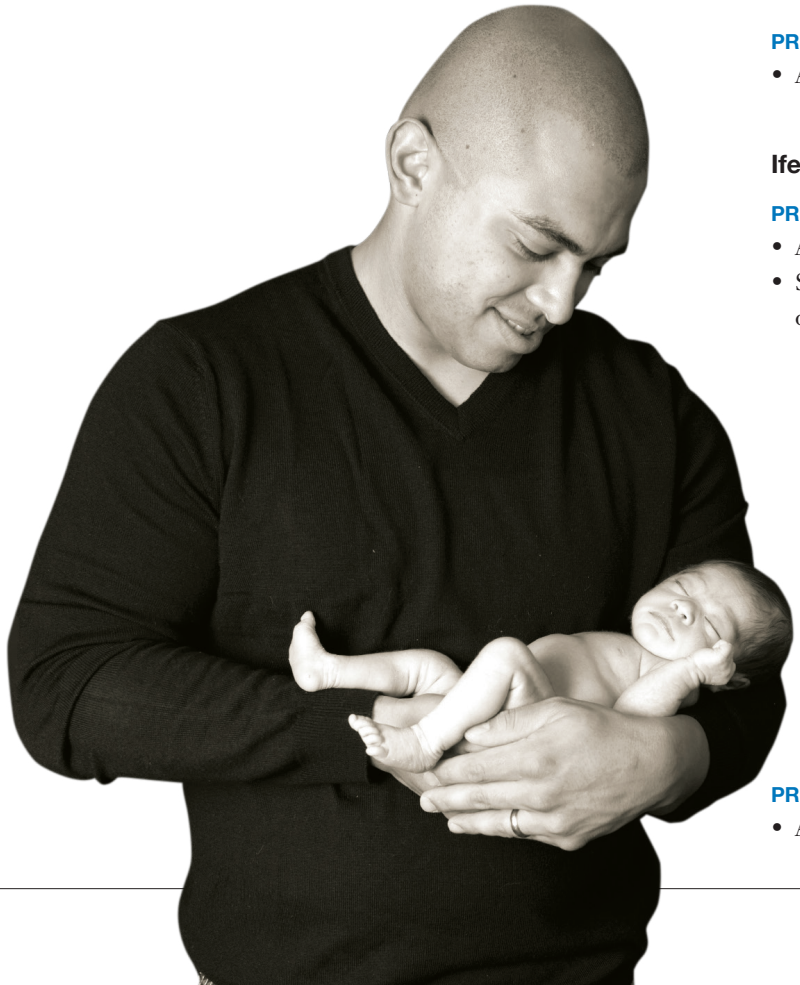
PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Society for Neuroscience
- Pulmonary Hypertension Association

Burhan Mahmood, MD

RESEARCH

Burhan Mahmood is medical director of the neonatal ECMO program. His research focuses on usage and prediction of need for ECMO.



ADVISORY COMMITTEE MEMBERSHIPS

- Fellowship Committee, UPMC Neonatal-Perinatal Medicine Fellowship Program
- Coordinator, Neonatal ECMO Core Team, Division of Newborn Medicine, Children's Hospital of Pittsburgh of UPMC
- Coordinator, Neonatal Congenital Diaphragmatic Hernia Core Team, Division of Newborn Medicine, Children's Hospital of Pittsburgh of UPMC
- Chair, Neonatal Nutritional Support Committee, Magee-Womens Hospital of UPMC
- Chair, Neonatal Medication Committee, Magee-Womens Hospital of UPMC
- Magee-Womens Hospital Pharmacy and Therapeutics Committee, Magee-Womens Hospital of UPMC
- UPMC Pharmacy and Therapeutics Committee, Children's Hospital of Pittsburgh of UPMC

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics

Sharmilarani Nanda, MBBS

ADVISORY COMMITTEE MEMBERSHIPS

- Clinical Director, Special Care Nursery, Westmoreland Hospital

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics

Ifeyinwa Nwankwor, MBBS

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Section on Perinatal Pediatrics, American Academy of Pediatrics

Brighid O'Donnell, MD

RESEARCH

Brighid O'Donnell's research focuses on the role of endoplasmic reticulum–associated degradation in the regulation of ROMK associated with type II Bartter syndrome.

ADVISORY COMMITTEE MEMBERSHIPS

- Fellowship Committee, UPMC Newborn Medicine Program

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics

HONORS

- T32 Institutional National Research Service Award, Children's Hospital of Pittsburgh of UPMC, Mark Lowe, MD (T32DK071492-0), 2014–2016
- K12 Physician Scientist Award Program, Children's Hospital of Pittsburgh of UPMC, "Molecular Basis of Pediatric Disease," Terence S. Dermody, MD (K12HD052892), 2016–2017

Karin Plummer Potoka, MD

RESEARCH

Karin Plummer Potoka's laboratory studies the use of novel nitric oxide–independent targeting of soluble guanylate cyclase (sGC) to improve pulmonary vascular dysfunction in mouse models of sickle cell disease. The laboratory continues testing these sGC targeting agents' abilities to induce fetal hemoglobin in erythroid progenitor cells. The research has the potential to benefit extremely preterm infants who are at risk of developing pulmonary hypertension as a result of their chronic lung disease of prematurity. Oxidative pressure plays a role in their pulmonary hypertension and lack of response to nitric oxide or phosphodiesterase inhibition. sGC modulators may improve pulmonary vascular resistance in this disease process. Additionally, babies who screen positive for sickle cell disease may be enrolled in early treatment with hydroxyurea to increase their fetal hemoglobin levels and decrease the number of complications from their sickle cell disease. The sGC targeting agents also have the potential to increase fetal hemoglobin in "hydroxyurea non-responders" in the future.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Pediatric Academic Societies

HONORS

- Faculty, Eastern SPR Platform Session, Young Investigator Award

Mohammad Riaz, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Section on Perinatal Pediatrics, American Academy of Pediatrics

Melissa Riley, MD

RESEARCH

Melissa Riley is the division's outreach liaison, collaborating with community hospitals in the region. In this position, she provides access to educational and clinical support both before and after births and works to improve medical outcomes for high-risk neonates.

ADVISORY COMMITTEE MEMBERSHIPS

- Gastroschisis Committee, UPMC Division of Newborn Medicine and Pediatric Surgery Medicine Breastmilk Committee, UPMC Newborn Medicine
- Delayed Cord Clamping Committee, UPMC Newborn Medicine
- Extreme Prematurity Working Group, UPMC Newborn Medicine
- Donor Breastmilk Guidelines Committee, UPMC Newborn Medicine
- Division of Newborn Medicine Oral Care Policy, UPMC Newborn Medicine
- Neonatal Perinatal Fellowship Program Evaluation Committee, UPMC Newborn Medicine
- Fellowship Clinical Competency Committee, UPMC Newborn Medicine
- Neonatal ECMO Core Team, UPMC Newborn Medicine
- Extracorporeal Life Support Organization Task Force, Children's Hospital of Pittsburgh
- UPMC Medical Records and Forms Committee
- STAT MedEvac Medical Advisory Committee
- NICU Multidisciplinary Committee, Children's Hospital of Pittsburgh
- Disaster/Emergency Preparedness Committee, Children's Hospital of Pittsburgh
- Magee/Uniontown Hospital Women's Service Line Committee
- Magee-Womens Hospital Woman's Health Outreach Team
- Task Force 2019 for Neonatal Transport, Children's Hospital of Pittsburgh

- STEPP IN (Safe Transitions and Euthermia in the Perioperative Period in Infants and Neonates)
- AAP physician liaison, UPMC Division of Newborn Medicine
- Neonatal consultant, Excela Westmoreland Hospital
- Medical Advisory Board, Three Rivers Mothers' Milk Bank

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Section on Perinatal Pediatrics, American Academy of Pediatrics
- Section on Transport Medicine, American Academy of Pediatrics
- Pediatric Academic Societies

HONORS

- Snee-Reinhardt Charitable Foundation Gant for Tecotherm Device, 2016
- Jon F. Watchko Fellowship Mentoring Award, 2017

Anthony Rudine, MD, MBA

RESEARCH

Anthony Rudine's laboratory focuses on using a murine model to study fetal brain development and the impact of corticosteroids. The laboratory continues working on pharmacologic treatments to modify the response of glucocorticoids in the developing brain to protect more infants from the devastating effects and complications of prematurity. Molecular techniques are used for genome evaluation. Using this model, the laboratory has shown that antenatally administered glucocorticoids can alter brain development in the fetus, leading to lifelong changes in genomic expression and behavior. The laboratory investigates the gender- and race-specific responses to some fetal treatments, and studies are ongoing to evaluate potential pharmacologic co-treatments that would maximize the benefit of glucocorticoids for all infants.

Additionally, Rudine is the primary investigator on multiple Institutional Review Board protocols, and he has funded his studies through the Clinical and Translational Science Institute at the University of Pittsburgh School of Medicine as well as the Magee-Womens Research Institute, in addition to being the recipient of the University of Pittsburgh Medical Center Department of Pediatrics Endowed Instructorship. He has studied the creation of neural stem cells from human tissue and the patterns of genomic expression in steroid-exposed cells. Another study has looked at cells obtained from umbilical cord blood to determine whether a pattern of gene expression exists at the cellular level for infants who are or are not exposed to steroids *in utero*. The goal of these studies was to identify biomarkers for newborn brain injury and to stratify risk for the development of disease later in life.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Fellow, American Academy of Pediatrics, 2016
- Section on Neonatal-Perinatal Medicine, American Academy of Pediatrics
- Representative, Children's Hospital Network Database Health Information Exchange

Richard Telesco, MD

RESEARCH

Richard Telesco continues participating in the NEATCAP trial as a co-investigator studying a decibel-reduction device to improve neurodevelopmental outcomes. Site principal investigator is Michael Balsan.

ADVISORY COMMITTEE MEMBERSHIPS

- Associate clinical director, UPMC Hamot NICU

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics

Amy M. Urban, DO, MPH

RESEARCH

Amy Urban, the course developer for Neonatal Critical Care Communication, focuses on providing neonatal fellows and nurse practitioners with fundamental communication skills to interact with families of very ill neonates. Her work was disseminated in "Neonatal Critical Care Communication (NC3): Training NICU Physicians and Nurse Practitioners," published in the *Journal of Perinatology*. She continues as program developer of the Neonatal Simulation Program. She shares her work in simulation at both the International Meeting for Simulation in Healthcare and the International Pediatric Simulation Symposia and Workshops on the potential roles for simulation with the introduction of the Next Accreditation System in graduate medical education.

She is director of the bereavement team at Magee-Womens Hospital of UPMC and works closely with the Center for Organ Recovery and Education to promote organ donation. She serves on the organizing committee for Schwartz Center Rounds at Children's Hospital. She is a member of the clinical advisory board for the Pennsylvania Preemie Network and is the co-chair for the network's webinar on neonatal abstinence syndrome.

ADVISORY COMMITTEE MEMBERSHIPS

- Advisory Board, PA Preemie Network, American Academy of Pediatrics
- Clinical Competency Committee, UPMC Newborn Medicine
- Improving Patient Handoffs Collaborative, Children's Hospital of Pittsburgh
- Director, Bereavement Committee, UPMC Newborn Medicine
- Physician Champion, Center for Organ Recovery and Education, UPMC Newborn Medicine

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- American Osteopathic Association
- Society for Simulation in Healthcare

Kalyani Vats, MD

ADVISORY COMMITTEE MEMBERSHIPS

- Obstetrician Services Committee, UPMC
- Fellowship Committee, UPMC Neonatal-Perinatal Medicine Fellowship Program
- Consultant Center for Advance Fetal Diagnosis, Magee-Womens Hospital of UPMC
- Medical advisor, Parent Advisory Council, Magee-Womens Hospital of UPMC
- Director, NICU Triage and Antenatal Consult Services, Magee-Womens Hospital of UPMC
- Co-chair, Clinical Consensus, Magee-Womens Hospital and Children's Hospital of Pittsburgh of UPMC
- Co-chair, WCBC-NICU Committee, Magee-Womens Hospital of UPMC
- Co-chair, Extreme Prematurity Task Force Working Group, Magee-Womens Hospital of UPMC
- Clinical research mentor, University of Pittsburgh medical students
- Codirector, Center for Advance Fetal Diagnosis, Magee-Womens Hospital of Pittsburgh

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Indian Academy of Pediatrics

HONORS

- Chairman's Distinction Bonus, Department of Pediatrics, 2016

Jon F. Watchko, MD

RESEARCH

Jon Watchko's research focuses on the pathogenesis of bilirubin-induced brain injury, the mechanisms of brain bilirubin clearance, and novel pharmacologic neuroprotective strategies against kernicterus. He also studies the genetic architecture of severe neonatal hyperbilirubinemia. In collaboration with Ashok Panigrahy, chief of the Department of Pediatric Radiology, Watchko uses advanced magnetic resonance imaging to examine the effects of hazardous bilirubin on subcortical neural circuitry.

EDITORSHIPS

- Guest co-editor, "Neonatal Jaundice," *Seminars in Perinatology*

ADVISORY COMMITTEE MEMBERSHIPS

- Coordinator, Washington Hospital Neonatal Outreach Program
- Neonatal-Perinatal Medicine Fellowship Committee, University of Pittsburgh School of Medicine
- Ethics Committee, Children's Hospital of Pittsburgh of UPMC
- Institutional Review Board, University of Pittsburgh
- Neonatal Jaundice in Low- and Middle-Income Countries Working Group, International Panel of Experts on Neonatal Jaundice
- Mercer County Amish Outreach Education (Rh disease)
- Stakeholder Group on Periviable Counseling Decision Support Tool

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Society for Pediatric Research
- American Physiological Society
- Midwest Society for Pediatric Research
- Perinatal Research Society
- American Pediatric Society

HONORS

- *Top Doctors*, Castle Connolly, 2010–2017

Toby Debra Yanowitz, MD, MS

RESEARCH

Toby Debra Yanowitz engages in clinical and translational research in the areas of perinatal brain injury, cardiac function, regional hemodynamics of preterm infants, neonatal outcomes, and database creation analysis. Yanowitz continues to lead multicenter clinical studies at Magee-Womens Hospital of UPMC and Children's Hospital of Pittsburgh of UPMC. Yanowitz is the co-chair

of the Newborn Research Support Service at Magee-Womens Hospital. She is the site representative to the Children's Hospital Network Database subgroup on outcomes for babies with surgical NEC and was recently elected chair of the committee.

ADVISORY COMMITTEE MEMBERSHIPS

- Chair, Review Committee, Magee-Womens Research Institute Clinical Trainee Research Award, Magee-Research Institute
- Member, Institutional Review Board, University of Pittsburgh School of Medicine
- Member, Neonatal-Perinatal Fellowship Committee Annual Program Evaluation
- Representative, Newborn Medicine Division, Vermont Oxford Network and Newborn Medicine Database Maintenance
- Chair, Clinical Working Group, "Guidelines for Oxygen Saturation Limits for Newborns," UPMC Newborn Medicine
- Chair, Clinical Working Group, "Guidelines for Acute Management of Neonatal Seizures," UPMC Newborn Medicine
- Chair, Clinical Working Group, "Guidelines for Management of the Patent Ductus Arteriosus Among Premature Infants," UPMC Newborn Medicine
- Chair, Clinical Working Group, "Guidelines for Management of Early-Onset Sepsis," UPMC Newborn Medicine
- Member, Clinical Working Group, "Guidelines for Use of Non-Invasive Ventilation in Newborn Infants," UPMC Newborn Medicine
- Member, Clinical Working Group, "Guidelines for Use of Nitric Oxide in Newborn Infants," UPMC Newborn Medicine
- Codirector, UPMC Newborn Medicine Clinical Consensus Committee, UPMC Newborn Medicine
- Neonatology representative, Brain Care Institute, Children's Hospital of Pittsburgh
- Member, Clinical Working Group, "Guidelines for Use of Hemodynamic Support in Newborn Infants," UPMC Newborn Medicine
- Member, Clinical Working Group, "Guidelines for Use of Respiratory Support in the Delivery Room," UPMC Newborn Medicine
- Member, Newborn Quality and Patient Safety Council, Magee-Womens Hospital
- Chair, Clinical Working Group, "UPMC Newborn Medicine, OB/GYN, and Maternal-Fetal Medicine Clinical Consensus Guideline for Delayed Umbilical Cord Clamping in Preterm Infants ≤ 34 6/7 Weeks Gestation," Divisions of UPMC Newborn Medicine, OB/GYN, and Maternal-Fetal Medicine
- Chair, Clinical Working Group, "UPMC Newborn Medicine, OB/GYN, and Maternal-Fetal Medicine Clinical Consensus Guideline for Delayed Umbilical Cord Clamping in Term and Late Preterm Infants ≥ 35 0/7 Weeks Gestation," UPMC Divisions of Newborn Medicine, OB/GYN, and Maternal-Fetal Medicine
- Co-chair, Clinical Working Group, "UPMC Newborn Medicine Clinical Consensus Guideline for Care of the Extremely Low Gestational Age Newborn," UPMC Division of Newborn Medicine
- Neonatal/Perinatal Fellowship Clinical Competency Committee, Department of Pediatrics, University of Pittsburgh School of Medicine
- Director, UPMC Newborn Medicine Neonatal Neuro-Intensive Care Unit, UPMC Newborn Medicine

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Alpha Omega Alpha
- American Academy of Pediatrics
- Perinatal Section, American Academy of Pediatrics
- Midwest Society for Pediatric Research
- Society for Pediatric Research
- Perinatal Research Society
- Association for Clinical and Translational Science

THREE-YEAR BIBLIOGRAPHY

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PHILIPS

DIVISION OF
PEDIATRIC CARDIOLOGY

Mission

The mission of the Division of Pediatric Cardiology is:

- To provide comprehensive, high-quality clinical services for fetuses and children with all forms of heart disease and for adults with congenital heart disease
- To train the next generation of clinical pediatric cardiologists and physician-scientists through a comprehensive fellowship program of superior standards
- To perform high-quality clinical and basic science research in the fields of heart disease and developmental cardiology
- To teach the fundamentals of pediatric cardiology to medical students, pediatric residents, and pediatric and adult cardiology fellows

FACULTY AND STAFF

Vivek Allada, MD
Professor of Pediatrics
Interim Chief, Division of Pediatric Cardiology
Clinical Director, Pediatric Cardiology
Director, Heart Institute

Gaurav Arora, MD
Associate Professor of Pediatrics
Associate Director, Electrophysiology
Associate Vice Chair of Pediatrics for Clinical Affairs, Ambulatory Care

Lee B. Beerman, MD
Professor of Pediatrics
Director, Electrophysiology

Mark DeBrunner, MD
Assistant Professor of Pediatrics
Director, Pediatric Cardiology Fellowship Program

Stacey Drant, MD
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Assistant Professor of Pediatrics

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Associate Professor of Pediatrics and Clinical and Translational Science
Medical Director, Heart Failure and Heart Transplantation

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Assistant Professor of Pediatrics
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Assistant Professor of Pediatrics
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Jacqueline Kreutzer, MD, FACC, FSCAI
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Director, Cardiac Catheterization Laboratory

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Director of Research, Cardiology

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Adolescent and Young Adult Congenital Heart Disease Center

Concetta Lombardo, BSN, MPAS, PA-C
Outpatient Services

OVERVIEW OF DIVISION

The Division of Pediatric Cardiology forms an integral part of the Heart Institute service line at Children's Hospital of Pittsburgh of UPMC and is the leading provider of comprehensive pediatric cardiovascular services in Western Pennsylvania and the tristate area. It is an internationally recognized center of excellence in pediatric heart failure, cardiomyopathy, mechanical circulatory support, and heart and lung transplantation. The Heart Institute integrates the various divisions and programs necessary to provide state-of-the-art cardiovascular services to children and young adults with congenital heart disease and to children with acquired heart disease. The components of the Heart Institute include the Division of Pediatric Cardiology (Interim Chief Vivek Allada), the Division of Cardiothoracic Surgery (Chief Victor Morell), the Cardiac Intensive Care Unit (CICU) (Chief Ricardo Muñoz), and the Division of Cardiac Anesthesia (Chief Patrick Callahan). The CICU is a 12-bed unit and is the only dedicated pediatric CICU in the region. The multidisciplinary team approach has resulted in excellent clinical outcomes.

The Heart Institute actively participates in numerous National Institutes of Health (NIH)-, foundation-, and industry-sponsored clinical and basic research programs. The program has an active pediatric cardiology fellowship program accredited by the Accreditation Council for Graduate Medical Education (ACGME). The program also offers postgraduate training in pediatric cardiac intensive care to fellows who have completed training in pediatric cardiology or pediatric intensive care.

The program continues to expand clinical services to the region, ensuring that children have access to top-quality cardiology services through on-site clinics and telemedicine technology. For the most recent four-year reporting period for the Society of Thoracic Surgeons, the program's surgical mortality was 2.1% (national average = 3.23%). In fact, the Heart Institute's success was the best in the country among medium and large programs. The program's achievements were acknowledged by *U.S. News & World Report* with a #12 ranking among pediatric cardiac centers in the nation.

The cardiology research program thrives under the new leadership of Bernhard Kühn, with continued NIH funding to perform multicenter trials in pediatric heart transplantation, development of novel mechanical circulatory support devices for use in children, and expansion of the fellowship program. Strong research collaborations exist with the Department of Biomedical Engineering in the Swanson School of Engineering at the University of Pittsburgh, the Thomas E. Starzl Transplantation Institute, the Vascular Medicine Institute, and the cardiac genetics research program in the Department of Developmental Biology (Cecilia Lo, chair) of the University of Pittsburgh School of Medicine.

CLINICAL ACTIVITIES

The Division of Pediatric Cardiology provides comprehensive clinical services to children and adults with congenital heart disease, as well as to children with acquired heart disease. The clinical program includes ambulatory diagnostic services at 16 locations: Children's Hospital in the Lawrenceville neighborhood of Pittsburgh; the three satellite facilities in Wexford, Monroeville, and South Fayette; Magee-Womens Hospital of UPMC; UPMC Presbyterian; and 10 other outreach locations throughout the western and central areas of Pennsylvania and the panhandle of West Virginia. With the extensive outreach program, cardiology is taking its services out into the community and has the most extensive outreach effort of any program at Children's Hospital. This year, office visits amounted to nearly 14,000 in all locations, including almost 2,500 patients seen in remote outreach locations beyond the metropolitan Pittsburgh area. This successful model has served as a "best practices" template for other divisions. Ambulatory services include diagnostic outpatient office visits; noninvasive imaging, including echocardiography (more than 18,000 studies); arrhythmia detection, including electrocardiography, 24-hour Holter, and 30-day event recorder monitoring; cardiopulmonary exercise testing; and preventive cardiology services.

The program supports a comprehensive Perinatal Cardiology Program in collaboration with the obstetrics team at Magee-Womens Hospital. The program has three facets: neonatal cardiology, fetal cardiology, and maternal cardiology. This unique and comprehensive program is one of the busiest in North America. The Division of Pediatric Cardiology also provides inpatient and same-day services at Children's Hospital, including diagnostic and state-of-the-art interventional cardiac catheterization with the region's only biplane hybrid catheterization laboratory; diagnostic and therapeutic arrhythmia management, including arrhythmia ablation with radiofrequency or cryoablation (reaching more than 100 electrophysiology patients in fiscal year 2017); and medical management of cardiac dysrhythmias, preventive cardiology, inflammatory diseases of the heart, and heart failure. In fact, the division's world-renowned pediatric heart and heart-lung transplant program has expanded its focus to encompass methods of heart-failure support and recovery.

The cardiology faculty also follows all children receiving surgical management of congenital or acquired cardiovascular conditions. The pediatric CICU is dedicated to the medical and surgical management of critically ill patients with congenital or acquired cardiovascular disease. It is the only such unit in the region.

The improved medical and surgical outcomes of children with congenital heart disease have led to a growing number of survivors flourishing in the community. It is now estimated that there are more adults living with congenital heart disease than children. Adult congenital heart disease comprises approximately 10%–15% of the work of the Heart Institute and continues to grow. The division's Adolescent and Young Adult Congenital Heart Disease Service introduced a transition program to enable seamless transfers of care for adolescents from pediatric specialists to adult congenital heart disease cardiologists.

The interventional cardiac catheterization program, under the leadership of Jacqueline Kreutzer, is one of the premier programs in the country, with incorporation of new procedures and technologies (such as percutaneous valve implantation), performance of hybrid procedures, and extensive participation in multicenter trials. The program has seen a progressive increase in patient and procedure volume in the catheterization laboratory.

Finally, the cardiology program continues to push state-of-the-art technology, using telemedicine technology to improve access, including a telemedicine echocardiography service that supports the reading of fetal and pediatric transthoracic echocardiograms throughout Western Pennsylvania and West Virginia. The program enables hospitals throughout the region to comply with state law and American Academy of Pediatrics (AAP) recommendations to screen newborn babies for critical congenital heart disease. The cardiology division provides expert consultative services, echocardiography training and interpretation, and transport of critically ill children from remote sites to the Children's Hospital facility for advanced cardiac services.

Of note, Gaurav Arora performed the first fluoroless ablation (true zero fluoroscopy) in the history of Children's Hospital in early 2017 and has continued to perform more procedures with zero radiation.

Cardiology at Children's Hospital has always been recognized for its excellence in clinical care, service delivery, and communication with referring physicians. Seven pediatric cardiologists were named to the Best Doctors list by *Pittsburgh Magazine*. The division has continued to focus on those areas throughout the course of the year. As part of the Heart Institute, the division has a robust quality-assurance process-improvement program with a number of quality initiatives, including protocols to reduce catheter infection rates and ongoing participation in national quality initiatives, including the cardiac catheterization IMPACT and C3PO (Congenital Cardiac Catheterization Outcomes Project Quality Improvement) programs. Members of the cardiology faculty routinely receive the highest levels of patient satisfaction from both Children's Hospital and national surveys. This year, three members were recognized for the Department of Pediatrics Chair's Distinction: Libby Lanford, Lee Beerman, and Kreutzer.

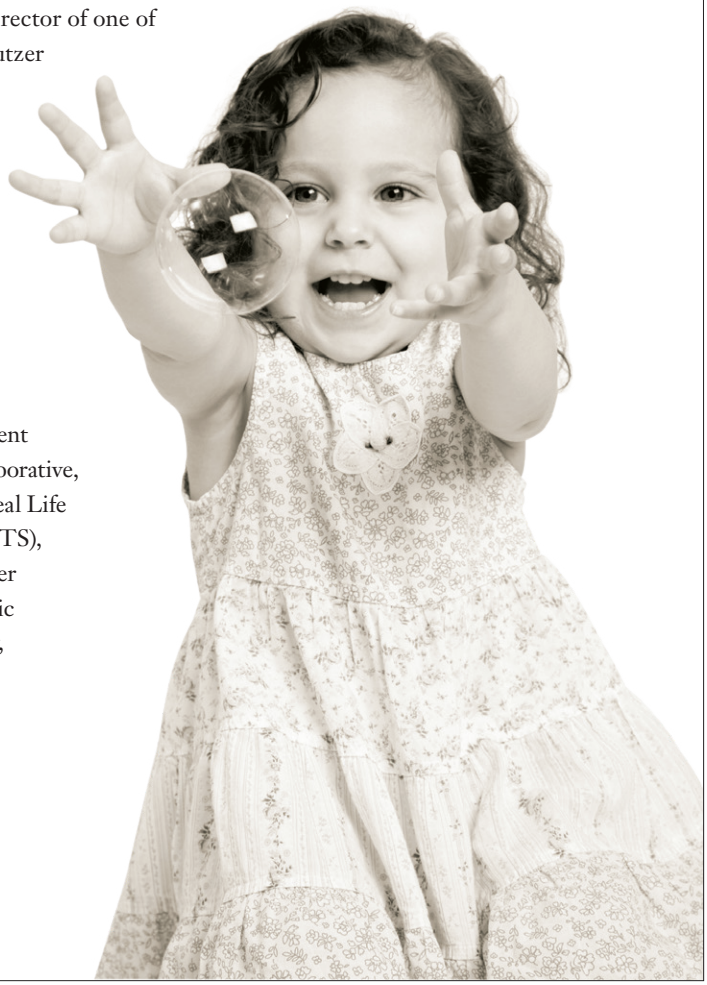
The clinical activities of the division continue to grow, as demonstrated by the spectacular 10-year trends shown in the table below. As a result of this growth, the division has successfully recruited three new faculty members for 2017 from some of the top centers across the country.

KEY INDICATORS	FY07	FY08	FY09	FY10	FY11	FY12	FY13	FY14	FY15	FY16	FY17	10-Year Growth
Outpatient Visits (Total)	10,042	11,516	13,384	13,457	13,863	13,547	14,364	15,099	14,635	13,163	17,706	62%
Outreach Outpatient Visits	908	1,146	1,898	1,958	1,951	2,110	2,138	2,611	2,472	1,934	1,212	33%
Electrocardiograms	11,456	14,371	14,826	14,701	17,134	20,220	22,012	22,988	24,944	26,669	27,063	136%
Echo (Total)	12,895	13,273	13,966	14,585	15,214	16,423	16,889	17,918	18,223	17,959	18,178	62%
Transthoracic Echocardiograms	11,421	11,666	12,338	12,984	13,522	14,421	15,109	16,127	16,629	15,938	17,525	63%
Transesophageal Echocardiograms	441	446	442	436	419	577	516	609	595	610	653	48%
Fetal Echocardiograms	1,033	1,161	1,186	1,165	1,273	1,425	1,264	1,363	1,212	1,411	1,247	20%
Holter Monitors	407	782	1,487	1,277	1,207	1,335	1,562	1,528	1,714	1,758	1,602	105%
Exercise Stress Tests	298	333	358	638	684	763	748	680	736	711	860	189%
Cardiac Catheterization Patients	635	641	648	700	708	743	739	793	783	724	766	21%
Electrophysiology Patients	66	104	86	91	88	99	104	100	99	107	94	42%

RESEARCH AND OTHER SCHOLARLY ACTIVITIES

The faculty leads multiple, active, clinical, translational, and basic research programs in cardiovascular disease. Kühn, as director of research for the division, successfully established his research laboratory. Kühn was granted the inaugural Fund for Genomic Discovery Award for his study titled "Discovering Fibrosis Genes by Gene Expression Analysis in Single Heart Cells," a project to identify the specific genes that cause heart tissue fibrosis in persons with congenital heart disease. Kühn also published his work, titled "Stimulation of Cardiomyocyte Regeneration in Neonatal Mice and in Human Myocardium With Neuregulin," in the prestigious journal *Science Translational Medicine*. The study helped to determine that neuregulin-1 (rNRG1) was most effective at improving myocardial function and reducing the prevalence of scarring when it was administered very early in the lifecycle. Although the work was performed with mice, it provides important insights as Kühn and his team work toward developing clinical trials focused on using rNRG1 to stimulate myocardial regeneration in pediatric heart patients. In acknowledgement of his accomplishments, Kühn was elected to the American Society for Clinical Investigation in 2016. He was one of four new members from the University of Pittsburgh that year and the only one from Children's Hospital.

The clinical research program is multifaceted, with clinical studies and trials in all aspects of pediatric cardiology, cardiac intensive care, and congenital heart surgery. Particular strengths include the research programs focusing on optimizing outcomes following pediatric heart and lung transplantation and the multidisciplinary program developing novel mechanical circulatory support devices for children with failing hearts. The electrophysiology program under the direction of Lee Beerman and Arora has produced nine publications, including information on the use of three-dimensional mapping to reduce radiation exposure during electrophysiology procedures. Cardiac genetics research is being carried out in collaboration with Lo, an internationally recognized expert on the genetics of congenital cardiovascular malformations. Brian Feingold continues his research into factors influencing outcomes for pediatric heart transplant candidates and recipients. In addition, he is the institutional principal investigator for the National Institute of Allergy and Infectious Diseases (NIAID)-funded Clinical Trials in Organ Transplantation in Children (CTOT-C). As director of one of the top interventional pediatric catheterization programs, Kreutzer has been instrumental in implementing novel procedures and technologies as part of multicenter trials, such as percutaneous Melody and Sapien valve implantation and hybrid procedures. Kreutzer is the national principal investigator for the Melody post-approval study and participates in COAST (Coarctation Stent Trials). Highlighting the division's commitment to high-quality care, the program has been a long-standing and active participant in quality-improvement (QI) projects, including C3PO, C3PO-QI, Improving Pediatric and Adult Congenital Treatments (IMPACT Registry), the National Pediatric Cardiology Quality Improvement Collaborative, the Cardiac Neurodevelopmental Outcomes Collaborative, the Pediatric Cardiac Critical Care Consortium, the Extracorporeal Life Support Organization, the Pediatric Heart Transplant Study (PHTS), and the Society of Thoracic Surgeons database. In addition, under the leadership of Stacey Drant and Jennifer Johnson, the pediatric echocardiography laboratory received commendation for quality, receiving reaccreditation from the Intersocietal Accreditation Commission at Children's Hospital and Magee-Womens Hospital echocardiography laboratories.



Finally, the research arm of the cardiology division has continued to develop a comprehensive cardiology database, based on a long-standing record of patients from the 1960s to the present and incorporating all programs within the Heart Institute. This powerful tool has allowed and will continue to enable research studies aimed at improving the lives of children and adults with congenital heart disease. The database, with more than 200,000 patients, highlights some of the program's strengths and now includes special sections for heart-failure transplant patients and adults with congenital heart disease. This year, the division plans to add a section for fetal cardiology.

Vivek Allada, MD

RESEARCH

Echocardiography Physician Productivity. Vivek Allada continues to study physician productivity at academic pediatric cardiology echocardiography laboratories. He serves as the founding member of the Committee on Pediatric Echocardiography Laboratory Productivity. This study, the first of its kind, aims to understand the practice patterns, human resource needs, and benchmarks for productivity. The research culminated in the co-authorship of a book chapter, "Echocardiography Lab Productivity," in *Echocardiography in Pediatric and Congenital Heart Disease: From Fetus to Adult*, published in 2016.

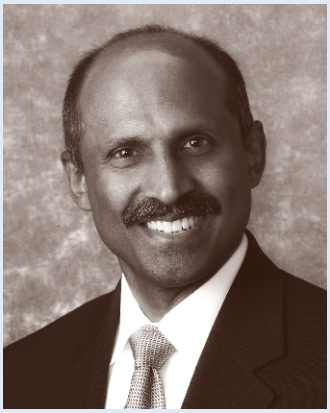
Physician Productivity. Allada has developed and implemented a clinical productivity assessment tool. With it, the division has seen a remarkable growth in volume and efficiency. Allada aims to create a software application to expand its adoption throughout UPMC and beyond.

ADVISORY COMMITTEE MEMBERSHIPS

- Chair, Subcommittee on Critical Congenital Heart Disease, Newborn Screening and Follow-Up Technical Advisory Board, Pennsylvania Department of Health
- Committee on Pediatric Echocardiography Laboratory Productivity, American Society of Echocardiography
- Clinical Practice Working Group, American College of Cardiology (ACC) Adult Congenital and Pediatric Cardiology (ACPC)
- Working Group, ACC ACPC Noninvasive Quality Metrics

EDITORSHIPS

- Guest editorial reviewer, *American Journal of Cardiology*
- Guest editorial reviewer, *Congenital Heart Disease*
- Guest editorial reviewer, *Pediatric Research*



Vivek Allada, MD
Interim Division Chief,
Pediatric Cardiology

MAJOR LECTURESHIPS AND SEMINARS

- Codirector, educational program, Ninth Annual Master Class in Congenital Cardiac Morphology with Professor Robert Anderson, Children's Hospital of Pittsburgh, Pittsburgh, Pa., October 2016

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Fellow, AAP
- Cardiology and Cardiac Surgery Section, AAP
- ACC
- ACPC Section, ACC
- American Society of Echocardiography
- Adult Congenital Heart Association

HONORS

- *Best Doctors in America*, Woodward/White, Inc., 2012–2016
- Best Doctors, *Pittsburgh Magazine*, 2012–2016

Gaurav Arora, MD

RESEARCH

Gaurav Arora's research focuses on management of arrhythmias.

Ablation of Accessory Pathways in Pediatric Patients: Association of Adenosine Sensitivity and Local Electrograms. This study reviews pediatric patients undergoing catheter ablation for Wolff-Parkinson-White syndrome and characteristics of ablation sites based on other accessory pathway characteristics.

Automated QT Analysis on Holter Monitor for Diagnosis of Long QT Syndrome. This study utilizes 24-hour Holter monitors to help identify differences between patients with long QT syndrome and controls. This study was awarded best fellow presentation at both the Pittsburgh American Heart Association (AHA) Fellows' Research Day and the Midwest Pediatric Cardiology Society annual meeting.

Lyme Carditis in Pediatric Patients Across U.S. Children's Hospitals. This study utilizes the Pediatric Health Information Systems database to study Lyme carditis across 44 U.S. children's hospitals and the changes in epidemiology and utilization over time.

Electrocardiograms in Pediatric Patients Presenting With Seizure to the Emergency Department. This study looks at the yield of electrocardiograms performed in pediatric patients who presented to the emergency room for evaluation of seizure.

Cost-Effectiveness of Implantable Cardioverter Defibrillators (ICDs) in Patients With Repair of Tetralogy of Fallot (TOF). This study evaluates the use of implantable cardioverter fefibrillators in patients undergoing repair of tetralogy of Fallot and the cost-effectiveness of this strategy compared with conventional therapy.

Retrospective Study of Exercise Testing and Improvements Due to Implementation of Electronic Reporting. This study evaluated the impact of switching to electronic reporting of exercise tests and showed that it improved the time to finalization for such reports.

ADVISORY COMMITTEE MEMBERSHIPS

- Chair, Fellows Research Day Task Force, AHA, 2015–2016
- Fellows Research Day Task Force, AHA
- Finance Committee, Pediatric and Congenital Electrophysiology Society
- Pediatric Cardiology Scholarly Oversight Committee, Children's Hospital of Pittsburgh
- Physician Advisory Committee, Children's Hospital of Pittsburgh
- eRecord Transformation Committee, Children's Hospital of Pittsburgh
- Pediatric Cardiology Fellowship Committee, Children's Hospital of Pittsburgh
- Electronic Medical Record Enhancement Task Force, Children's Hospital of Pittsburgh
- Advanced Practice Providers Fellowship Leadership Committee, Children's Hospital of Pittsburgh
- Leadership Committee, Department of Pediatrics Ambulatory Advisory Council, Children's Hospital of Pittsburgh
- Task Force 2019–Advanced Practice Providers, Children's Hospital of Pittsburgh

MAJOR LECTURESHIPS AND SEMINARS

- "Pediatric Murmurs: When to Refer," Three Rivers Pediatric Update, Children's Hospital of Pittsburgh, Pittsburgh, Pa., May 2015
- "Pediatric Cardiac Emergencies," emergency medicine grand rounds, Allegheny General Hospital, Pittsburgh, Pa., March 2016
- "Syncope and Chest Pain," Three Rivers Pediatric Update, Children's Hospital of Pittsburgh, Pittsburgh, Pa., May 2016
- "Using a Smartphone to Diagnose Arrhythmias," Heart Rhythm Society annual meeting, Chicago, Ill., May 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Heart Rhythm Society
- Pediatric and Congenital Electrophysiology Society
- Pittsburgh Electrophysiology Society

HONORS

- Best Doctors, *Pittsburgh Magazine*, 2015–2016
- Chairman's Distinction Award, 2017–2018

Lee B. Beerman, MD

RESEARCH

Assessment of Properties of Adenosine-Sensitive Accessory Pathways. Lee Beerman is reviewing data on pathways determined to be sensitive to adenosine, looking for markers of local ventricular-atrial timing and conduction properties. These are vital steps toward mapping of the pathways.

Evaluation of Arrhythmic Events Noted on Pacemaker Interrogations. Beerman is assessing correlations between nonsustained ventricular tachycardia on interrogation and clinical events.

ADVISORY COMMITTEE MEMBERSHIPS

- Rheumatic Fever, Endocarditis, and Kawasaki Disease Committee, Council on Cardiovascular Disease in the Young, AHA
- Writing groups, "Guidelines for the Diagnosis of Rheumatic Fever Using the Jones Criteria," "Update in the Era of Echocardiography," and "Update on Management of Bacterial Endocarditis in Pediatric Population," AHA
- Past president and current Executive Committee member, Children's Hospital of Pittsburgh of UPMC Alumni Association
- President, Children's Hospital of Pittsburgh of UPMC Professional Staff, 2013–2015
- Chair, Credentials Committee, Children's Hospital of Pittsburgh of UPMC, 2013–2015

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- AHA
- Heart Rhythm Society

- Pediatric and Congenital Electrophysiology Society
- Pittsburgh Electrophysiology Society

HONORS

- Best Doctors, *Pittsburgh Magazine*, 2012–2015
- Howard A. Mermelstein Award for Excellence in Pediatrics, Children's Hospital of Pittsburgh of UPMC, 2013
- Peter J. Safar Pulse of Pittsburgh Award, a lifetime achievement award, AHA, 2012

Mark DeBrunner, MD

RESEARCH

Use of the Myocardial Performance Index (MPI) Derived by Echocardiography to Identify Rejection in Patients After Heart Transplant. Standard, noninvasive imaging has not proven to be very effective in identifying patients with rejection following heart transplantation. The MPI, derived through Doppler and tissue Doppler measurements, has been used to measure subtle forms of myocardial dysfunction. This retrospective study utilized a large transplant database and confirmed that the MPI calculated with spectral Doppler predicted cardiac transplant rejection. Ongoing studies will utilize tissue Doppler indices to measure MPI in a prospective fashion.

Additionally, Mark DeBrunner participated in the "Brain Dysplasia Associated With Ciliary Dysfunction in Infants With Congenital Heart Disease" study.

MAJOR LECTURESHIPS AND SEMINARS

- "Atrioventricular Septal Defect in the Setting of Isomerism of the Atrial Appendages," Ninth Annual Master Class in Congenital Cardiac Morphology, Pittsburgh, Pa., September 2016
- "The Pulmonary Valve," Ninth Annual Master Class in Congenital Cardiac Morphology, Pittsburgh, Pa., September 2016

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- AAP
- Society of Pediatric Cardiology Training Program Directors

Stacey Drant, MD

RESEARCH

Aortic Stiffness in Aortic Root Dilatation. A subgroup of patients with bicuspid aortic valve develops aortic dilatation during childhood and is at increased risk for aortic dissection during adulthood. Patients with other forms of aortic dilatation have been found to have abnormal aortic elastic qualities. Using echocardiography to measure aortic distensibility and stiffness, Stacey Drant's team is evaluating normal pediatric patients and those with bicuspid aortic valve to identify patients who will be at increased risk for future aortic dissection and assess response to medical therapy.

National Clinical Trial of Beta Blocker Therapy (Atenolol) Versus Angiotensin II Receptor Blocker Therapy (Losartan) in Individuals With Marfan Syndrome. Drant is the site principal investigator of this study with the primary aim to compare the effect of atenolol therapy to that of losartan therapy on the rate of aortic growth and progression of aortic regurgitation.

Use of the MPI Derived by Echocardiography to Identify Rejection in Patients After Heart Transplant. Standard, noninvasive imaging has not proven to be very effective in identifying patients with rejection following heart transplantation. The MPI, derived through Doppler and tissue Doppler measurements, has been used to measure subtle forms of myocardial dysfunction. This retrospective study used a large transplant database and confirmed that the MPI calculated with spectral Doppler predicts cardiac transplant rejection. Ongoing studies will utilize tissue Doppler indices to measure MPI in a prospective fashion.

Exercise Training on Sedentary Obese Pediatric Patients. Drant is overseeing a study that assesses the effects of exercise training on left ventricular mass in sedentary obese pediatric patients.

ADVISORY COMMITTEE MEMBERSHIPS

- Member, Pediatric Quality Metrics Committee, ACC

EDITORSHIPS

- Guest editorial reviewer, *Journal of the American Society of Echocardiography*
- Guest editorial reviewer, *Pediatric Cardiology*
- Guest editorial reviewer, *Congenital Heart Disease*

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Society of Echocardiography

HONORS

- Patient Satisfaction Award, Children's Hospital of Pittsburgh, 2016
- UPMC Excellence in Patient Experience and Medical Staff Honor Roll, 2016

Johanna L. Drickman, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- AAP
- ACC
- American Physicians Fellowship

David S. Ezon, MD

RESEARCH

The HeartView Collaborative. David Ezon is the founder of the HeartView Collaborative, a multi-institutional effort to employ virtual and augmented reality technologies to improve pre-operative and intra-operative guidance in caring for children with congenital heart disease.

Echocardiographic Correlates to Coarctation of the Aorta Gradients at Catheterization. This study is evaluating the best echocardiographic methods to predict the blood pressure gradient at catheterization caused by coarctation of the aorta. The study will help guide clinicians on the best time to refer patients to the catheterization laboratory for balloon angioplasty of the obstruction.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- ACC
- AAP

Brian D. Feingold, MD, MS, FAHA

RESEARCH

Listing Strategies for Allosensitized Pediatric Heart Transplant Candidates. Brian Feingold was awarded a five-year NIH KL2 grant in July 2010 to determine historical outcomes for children listed for heart transplantation with a requirement for prospective crossmatch. This includes modeling outcomes for two competing wait-list strategies for allosensitized patients. Analyses are being performed with data from the United Network for Organ Sharing and PHTS databases and have resulted in multiple publications.

Chronic Graft Destruction: Interplay of Allo- and Autoantibodies and Nonadherence Role of Alloantibodies in Cardiac Transplantation: Intervention, Outcomes, and Mechanisms. Feingold is the Children's Hospital site primary investigator on this NIH NIAID award. The program (CTOT-C) seeks to enhance understanding of the role of alloantibodies in pediatric heart transplantation. Pediatric heart transplant candidates are frequently allosensitized based on prior exposure to blood products, homografts, and ventricular assist devices, and this has led to increased pre- and post-transplant mortality. This research program brings together a group of seven leading heart transplant centers and leading transplantation scientists to study the impact of preformed and *de novo* alloantibodies on mid-term pediatric heart transplant outcomes. Mechanistic studies are being performed to determine why some children develop graft injury but others appear to "accommodate" their grafts in the presence of donor-specific antibody and a positive donor-specific cross-match.

Epstein-Barr Virus (EBV) Infection in the Immunocompromised Host. Primary EBV infection is a major cause of morbidity and mortality after pediatric thoracic organ transplantation and frequently is associated with the development of post-transplant lymphoproliferative disorder (PTLD). Feingold serves as a co-investigator on Diana Metes' NIH R01, which is examining chronic high EBV load and the risk of PTLD in pediatric thoracic transplant patients.

sST2/IL33 as a Biomarker for Acute Rejection in Pediatric Heart Transplant Recipients. In this collaboration with Heth Turnquist from the Thomas E. Starzl Transplantation Institute, the team is seeking noninvasive markers of acute rejection. The research is supported by the Roche Organ Transplantation Research Foundation.

Cardiac Fibrosis After Pediatric Heart Transplantation. In collaboration with investigators at the cardiac magnetic resonance imaging center at UPMC Presbyterian, Feingold and colleagues are working to quantify fibrosis after pediatric heart transplantation, determine risk factors, and elucidate mechanisms for development. Fibrosis occurs variably after transplantation and is thought to be associated with limitations in late post-transplant survival.

Pediatric Cardiomyopathy—Biomarkers. This NIH-funded consortium of pediatric heart-failure centers is working together to understand the utility of blood and imaging biomarkers in the diagnosis and prognosis of cardiomyopathies. Feingold serves as site primary investigator.

Noninvasive Detection of Acute Rejection. Feingold has partnered with an industry sponsor and the Hillman Transplant Foundation to explore possible clinical biomarkers for the diagnosis of acute heart transplant rejection.

Heart Transplantation in Barth Syndrome. This study, funded in part by the Barth Syndrome Foundation, seeks to quantify the world experience and outcomes of heart transplantation for individuals with Barth Syndrome.

STUDY SECTIONS

- Grant review panelist, NIH/National Heart, Lung, and Blood Institute Pediatric Heart Network Clinical Research Centers
- Grant review panelist, AHA Strategically Focused Research Network Phase One

ADVISORY COMMITTEE MEMBERSHIPS

- Vice chair, Scientific Council on Pediatric Thoracic Transplantation and Heart Failure, International Society for Heart and Lung Transplantation

- Fellow Research Day Task Force, Great Rivers Affiliate, AHA
- Member, Pediatric Heart Failure Committee, Cardiovascular Disease in the Young Council, AHA
- Cardiovascular Disease in the Young Council's liaison to the Council on Clinical Cardiology Heart Failure and Transplantation Committee, AHA
- Chair, AHA Writing Group for Scientific Statement on Cardiac Management of Neuromuscular Diseases
- Medical monitor, Pediatric Heart Network's Fontan Exercise Longitudinal Assessment (FUEL) Trial
- Member, AHA Summer Undergraduate Research Program in Cardiovascular Sciences
- Steering Committee, University of Pittsburgh Vascular Medicine Institute

MAJOR LECTURESHIPS AND SEMINARS

- "Innovations in Pediatric Heart Failure," symposium, San Diego, Calif., December 2015
- Invited faculty, Barth Syndrome Foundation biannual conference, Clearwater Beach, Fla., July 2016
- "Management of the Highly Allosensitized Pediatric Heart Transplant Patient," invited presenter, Optum Conference, Pittsburgh, Pa., May 2016
- Invited faculty, Eighth Annual Neonatal and Pediatric extracorporeal membrane oxygenation (ECMO) Conference, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., August 2016
- Invited faculty, International Society of Heart and Lung Transplantation annual scientific sessions, San Diego, Calif., April 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Society for Pediatric Research
- Fellow, AHA
- Council on Cardiovascular Disease in the Young, AHA
- International Pediatric Transplant Association
- American Society of Transplantation
- International Society for Heart and Lung Transplantation
- PHTS

EDITORSHIPS

- Associate editor, *Pediatric Cardiology*
- Editorial Board, *Pediatric Transplantation*

HONORS

- Best Doctors, *Pittsburgh Magazine*, 2012–2017

Tyler H. Harris, MD

RESEARCH

Tyler Harris' research interests involve the use of innovative technologies and curricula as well as cross-institutional collaboration to improve education in pediatric cardiology.

Pediatric Heart Disease Simulation Curriculum: Educating the Pediatrician. This study created a simulation-based curriculum to train pediatric residents in the evaluation, management, and triage of critical pediatric heart disease.

Evaluation of Boot Camp Practices in Pediatric Cardiology Fellowship. In this study, being conducted with the AAP Section on Cardiology and Cardiac Surgery's Committee for Education and Training, Harris is leading a survey of current practices and a needs assessment for cardiology training programs' "Boot Camp" processes.

Crowdsourcing the Best Literature in Pediatric Cardiology. In a collaborative effort, Harris is working to create a resource of highly important, field-changing research within pediatric cardiology using the technique of crowdsourcing with expert review via a Delphi method. The output will be a continually updated list of the literature considered paramount to the field.

University of Pittsburgh Graduate Medical Education Research Collaborative. Harris is working with education researchers from across the Pitt health system to identify and evaluate high-yield education research projects in a collaborative setting, allowing sharing of expertise and experience.

ADVISORY COMMITTEE MEMBERSHIPS

- Pediatric Cardiology Scholarly Oversight Committee, Children's Hospital of Pittsburgh
- Pediatric Cardiology Fellowship Committee, Children's Hospital of Pittsburgh
- Curriculum coordinator, Pediatric Advanced Practice Providers Fellowship Committee, Children's Hospital of Pittsburgh

EDITORSHIPS

- Guest editorial reviewer, *Cardiology in the Young*

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Fellow, AAP
- AP Section on Cardiology and Cardiac Surgery Committee on Education and Training
- Society of Pediatric Cardiology Training Program Directors
- Association of Pediatric Program Directors
- Pediatric Acute Cardiac Care Collaboration

Jennifer Johnson, DO

RESEARCH

Fetal Echocardiographic Predictors of Postnatal Coarctation in the Setting of Ventricular Discrepancy. Johnson has focused her research on fetal cardiology. She is evaluating the fetal echocardiographic predictors of postnatal coarctation in the setting of ventricular discrepancy. She is determining whether fetuses of mothers with thyroid disease may be at higher risk for developing supraventricular tachycardia.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Society of Echocardiography

Jacqueline Kreutzer, MD, FACC, FSCAI

RESEARCH

Novel Technologies for Transcatheter Intervention, Including Percutaneous Valve Therapy, Device and Stent Development, and Their Clinical Applications. Jacqueline Kreutzer's research interests are in novel technologies for transcatheter intervention, including percutaneous valve therapy, device and stent development, and their clinical applications. Kreutzer has been principal investigator for the following projects.

The Medtronic Melody Transcatheter Pulmonary Valve Post-Approval Study. Kreutzer served as the principal investigator for this multicenter study on post-market surveillance of the Melody pulmonary valve.

Congenital Cardiovascular Interventional Study Consortium Coarctation Study. This is a multicenter study designed to compare outcomes among surgery, balloon angioplasty, and intravascular stent placement for recurrent or native coarctation of the aorta.

Coarctation of the Aorta Stent Trial (COAST I and COASTII) Multicenter Studies. These studies examine the use of Cheatham platinum bare metal stents, as well as covered stents, for coarctation of the aorta.

C3PO-QI. This is a multi-institutional quality-improvement project to monitor outcomes of pediatric cardiac catheterization.

COMPASSION XT Post-Approval Study. This is a multicenter study on post-market surveillance of the Sapien XT pulmonary valve.

EDITORSHIPS

- Editor, *Journal of the American College of Cardiology*

ADVISORY COMMITTEE MEMBERSHIPS

- Congenital Heart Disease Committee, Society for Cardiac Angiography and Intervention
- Congenital Cardiovascular Interventional Study Consortium
- Program chair, Congenital Heart Disease, Society of Cardiac Angiography and Intervention, 2016
- National Cardiovascular Data Registry and IMPACT Registry Research and Publications Committee, ACC
- Vice president, Congenital Cardiovascular Interventional Study Consortium
- Melody® Global Publications Committee
  - Member, internal advisory board for a grant application for the AHA Strategically Focused Children's Research Network
  - Chair, Data Monitoring Committee, Medtronic Harmony™ Transcatheter Pulmonary Valve (TPV) Clinical Study

MAJOR LECTURESHIPS AND SEMINARS

- "Pediatric Interventional Cardiology: State of the Art and Future Perspective," pediatric ground rounds, Children's Hospital of Pittsburgh of UPMC, July 2016
- "Cardiac Catheterization Procedures for ECMO Patients," Eighth Annual Neonatal and Pediatric ECMO Educational Conference, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., August 2016
- "Advances in Pediatric Interventional Cardiology: State of the Art and Future Perspective," Boxer Memorial Lecture Speaker, pediatric grand rounds, Cohen's Children's Hospital, Northwell Health, Long Island, N.Y., September 2016
- "Interventional Cardiac Catheterization for the Aortic Root Disease," Ninth Annual Master Class in Congenital Cardiac Morphology, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., September 2016
- "Transcatheter PVR Using the Edwards and Melody Valves in Patients With Conduits and Bioprosthetic Valves: Technical Considerations," invited faculty, 28th Annual Scientific Symposium of Transcatheter Cardiovascular Therapeutics, Washington, D.C., October 2016
- Invited faculty, live-case presenter, Pediatric and Adult Interventional Cardiac Symposium 2017, January 2017
- "Hybrid Procedure for Neonatal Hypoplastic Left Heart Syndrome," Progress in Perinatal Cardiology: Detection and Management of Fetal Congenital Heart Disease, Tampa Bay, Fla., February 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- ACC
- Congenital Heart Disease and Pediatric Cardiology Section, ACC
- Women in Cardiology Section, ACC
- AHA
- Society for Cardiac Angiography and Interventions
- Sociedad Argentina de Cardiologia
- Sociedad Latina de Cardiologia
- IMPACT Registry Research and Publications Subcommittee, National Cardiovascular Data Registry
- Congenital Cardiovascular Interventional Study Consortium

HONORS

- *Best Doctors in America*, Woodward/White, Inc.
- Best Doctors, *Pittsburgh Magazine*, 2012–2016
- Chair's Distinction Award for performance during fiscal year 2016, Department of Pediatrics

Bernhard Kühn, MD, FACC

RESEARCH

Mechanisms of Myocardial Development and Regeneration. Bernhard Kühn's research is on the mechanisms of myocardial development and regeneration. The long-term goal is to develop a therapeutic strategy for stimulating myocardial regeneration in children.

Kühn is a Mellon Scholar and director for research for the division. He is developing a bench-to-bedside program dovetailing the heart-failure program with the aim of developing novel therapies for children. He advises and mentors fellow researchers and serves on the Fellow Scholarship Oversight Committee. Kühn is also a clinician with one general clinic per week and collaborative participation in the heart-failure clinic.

New grants awarded:

- "Discovering Fibrosis Genes by Gene Expression Analysis in Single Heart Cells," funds for genomic discovery, Children's Hospital of Pittsburgh Foundation, principal investigator, $200,000 total
- "Eliciting Heart Regeneration Through Cardiomyocyte Division," Fondation Leducq, Transatlantic Network of Excellence, about $500,000, 2016–2021
- University of Pittsburgh Physicians Foundation Award, "Discovering New Fibrosis Mechanisms by Gene Expression Analysis of Single Human Heart Cells"

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Diplomat, AAP
- Fellow, ACC
- American Association for the Advancement of Science
- AHA
- Elected member, American Society of Clinical Investigation

HONORS

- American Society of Clinical Investigation, 2016

Lizabeth Lanford, MD

RESEARCH

Change in Occipital-Frontal Head Circumference Percentile in Single Ventricle Patients Undergoing Glenn Anastomosis. Lizabeth Lanford's research interest lies in changes in growth parameters associated with Glenn surgery. This research was presented at the 2016 Eastern Society for Pediatric Research meeting and the 2016 Pediatric Academic Societies meeting.

Lanford also studies cardiovascular abnormalities and pulmonary hypertension in children with sickle cell disease.

MAJOR LECTURESHIPS AND SEMINARS

- "Echocardiographic Features of Normal Anatomy," Master Class in Congenital Cardiac Morphology, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., September 2016
- "Echocardiographic Features of Aortic Root," Master Class in Congenital Cardiac Morphology, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., September 2016
- "Echocardiographic Features of Normal Anatomy," Master Class in Congenital Cardiac Morphology, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., October 2017
- "Echocardiographic Features of Tetralogy of Fallot," Master Class in Congenital Cardiac Morphology, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., October 2017

HONORS

- Chair's Distinction Award for providing outstanding services, 2016
- *Best Doctors in America*, Woodward/White, Inc.

Francis M. McCaffrey, MD

RESEARCH

Clinical Trial of Coenzyme Q10 and Lisinopril in Muscular Dystrophies. This is a multicenter study that enrolls patients with muscular dystrophies early in their disease, before the onset of cardiomyopathy, into a coenzyme Q10/

lisinopril arm versus a traditional treatment control. Echocardiographic monitoring is used to determine whether they qualify for enrollment and to track cardiac function.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Society of Echocardiography

Susan A. Miller, MD

RESEARCH

C3PO. This multi-institutional quality-improvement project monitors outcomes of pediatric cardiac catheterization. Susan Miller's research involves outcomes after pediatric heart transplantation. She is a co-investigator on many active protocols, including the following.

- Pediatric cardiomyopathy—biomarkers: This NIH-funded consortium of pediatric heart-failure centers is working together to understand the utility of blood and imaging biomarkers in the diagnosis and prognosis of cardiomyopathies. Feingold is site primary investigator.
- Chronic graft destruction: This study examines the interplay of allo- and autoantibodies and nonadherence.
- Role of Alloantibodies in Cardiac Transplantation: Intervention, Outcomes, and Mechanisms (NIH/NIAID CTOT-C): This program seeks to enhance understanding of the role of alloantibodies in pediatric heart transplantation.
- sST2/IL33 as a Biomarker for Acute Rejection in Pediatric Heart Transplant Recipients
- INTERMACS (Interagency Registry of Mechanically Assisted Circulatory Support) cohort study
- Cardiac fibrosis after pediatric heart transplantation: In collaboration with investigators at the cardiac magnetic resonance imaging center at UPMC Presbyterian (principal investigator, Feingold), this study is working toward quantifying fibrosis after pediatric heart transplantation, determining risk factors, and elucidating mechanisms for development.
- EBV infection in the immunocompromised host—primary EBV: This study examines chronic high EBV load and the risk of PTLD in pediatric thoracic transplant patients.
- Waitlist and transplant events and outcomes in the United States
- Biomarker analysis after heart transplantation
- Immune cell function and transplant outcomes in lung and heart/lung transplants
- PHTS

ADVISORY COMMITTEE MEMBERSHIPS

- Medical codirector, Bill Neches Heart Camp for Kids

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Pediatric Cardiomyopathy Foundation

Evonne Morell, DO

RESEARCH

Effects of Anesthetics and Hypothermia on Ciliary Function in the Respiratory Epithelia of Mice. This study, which has been submitted for publication, evaluated the effects of multiple anesthetics on the ciliary function of mice.

Preoperative Management of Patients With Single-Ventricle Physiology. This study evaluated current preoperative management, which maximizes equal distribution of cardiac output by afterload reduction using a type III phosphodiesterase inhibitor and increases pulmonary vascular resistance using inhaled nitrogen, as well as the effect it has on multisystem organ dysfunction.

Evolving Approaches in the Management of Hypoplastic Left Heart Syndrome. This study focused on the establishment of a single-ventricle clinic.

Evonne Morell also is examining a hybrid strategy for neonates with ductal-dependent systemic circulation at high risk for Norwood.

MAJOR LECTURESHIPS AND SEMINARS

- "Youth Exams and Murmurs: What's Reasonable?" Pennsylvania Osteopathic Medical Association District VIII 30th Annual Educational Winter Seminar, Farmington, Pa., January 2017

ADVISORY COMMITTEE MEMBERSHIPS

- Pediatric Acute Cardiac Care Collaboration, Quality-Improvement Committee

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- AAP
- AHA
- Pennsylvania Chapter of Osteopathic Physicians
- Pediatric Acute Cardiac Care Collaboration

Lan Nguyen, MD

RESEARCH

Urinary Biomarkers in Young Adults With Congenital Heart Disease. This is an ongoing collaborative research project with the Center for Critical Care Nephrology to compare levels of urinary biomarkers of kidney injury and dysfunction in healthy young adults and young adults with congenital heart disease. The main goal of this study is to

establish an objective method to quantify renal fitness in young adult congenital heart disease patients, a growing population that is at risk for numerous kidney insults across a lifetime.

MAJOR LECTURESHIPS AND SEMINARS

- "Adult Congenital Heart Disease: A Focus on Pulmonary Stenosis and Tetralogy of Fallot," Three Rivers Echo Society, Canonsburg, Pa., May 2016
- "The Adult Patient With Congenital Heart Disease II: The Operated Patient," Three Rivers Echo Society, Canonsburg, Pa., May 2015

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Adult Congenital Heart Association
- American Society of Echocardiography

Sandhya Ramlogan, MD

RESEARCH

Intervendor Reproducibility of Biventricular 2D Speckle Tracking Strain in the Structurally Normal Heart in Infants and Children. Clinical adoption of 2D strain echocardiography by speckle tracking in pediatrics is limited due to the lack of evidence proving its reproducibility despite evidence showing its significant incremental value in early detection of subtle myocardial dysfunction in adult studies. Since the EACVI/ASE industry task force established better standardization of strain software packages, no studies have evaluated intervendor strain reproducibility in children < 6 years of age with the newest strain software, which uses the EACVI/ASE industry task force recommended global peak systolic strain algorithm. Sandhya Ramlogan's research seeks to evaluate the reproducibility of global longitudinal and left ventricular circumferential strain for mid-myocardial and endocardial layers in pediatric subjects on the two commonly utilized software platforms.

Noninvasive Detection of Acute Rejection. This research explores possible biomarkers of acute rejection (or nonrejection) in paired clinical data at the time of cardiac allograft biopsy with serial research serum samples and blinded review of serial clinical echocardiograms. This research is funded by Abbott Diagnostics and the Hillman Foundation for Transplant Research. Ramlogan is the echocardiographer co-investigator on this project with Feingold.

EDITORSHIPS

- Guest editorial reviewer, *Pediatric Cardiology*
- Guest editorial reviewer, *Congenital Heart Disease*

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Society of Echocardiography

Linda Russo, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Society of Echocardiography
- AHA
- Immediate past medical director, Bill Neches Heart Camp for Kids

Sara Trucco, MD

RESEARCH

Sara Trucco's research involves interventional cardiac catheterization and includes the following.

- Acute and Intermediate Follow-Up Results of Hybrid Device Closure of Ventricular Septal Defects: A Multicenter Experience
- Comparison Between Surgical Versus Balloon Angioplasty Versus Intravascular Stent Placement for Recurrent or Native Coarctation of the Aorta
- Covered Cheatham Platinum Stent for Prevention or Treatment of Aorta Wall Injury Associated With Aortic Coarctation (COAST II)
- Amplatzer Atrial Septal Defect Occluder Post-Marketing Study II, a clinical study through AGA Medical Corporation
- Access-related adverse event multicenter project through C3PO (principal investigator)
- C3PO-QI radiation-safety benchmark study (subinvestigator)
- Medtronic Melody valve post-marketing study
- Endovascular therapies in congenital heart disease
- Edwards-Sapien XT transcatheter pulmonary valve post-marketing study (co-investigator)
- CCISC coronary artery fistula study (local principal investigator)
- ALK1 Signaling in Development of Superior Cavopulmonary Anastomosis-Associated Pulmonary Arteriovenous Malformations (principal investigator; co-principal investigator: Beth Roman, PhD)

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- ACC
- AHA
- Society for Cardiac Angiography and Interventions
- Elected as clinical vice president of the Pediatric Interventional Cardiology Early-Career Section, Society for Cardiac Angiography and Interventions

MAJOR LECTURESHIPS AND SEMINARS

- "Interventional Cardiac Catheterization for Diseases of the Pulmonary Root," Ninth Annual Pittsburgh Master Class in Congenital Cardiac Morphology, Pittsburgh Pa., September 2016

- "Hybrid Strategy for Neonates With Ductal-Dependent Systemic Circulation at High Risk for Norwood," "Best of the Best" abstract winner, Pediatric and Adult Interventional Cardiac Symposium, Miami Beach, Fla., 2016
- "Reducing Adult Congenital Heart Disease Cardiac Catheterization Complications in a Pediatric Facility: A Single Institution Experience," award finalist, 20th Annual Update on Pediatric Cardiovascular Disease, Orlando, Fla., 2017
- "Interventional Cardiology Research Projects II," Heart Institute Research Retreat, Pittsburgh, Pa., April 2017

Jacqueline Weinberg, MD

RESEARCH

Jacqueline Weinberg's research interests involve noninvasive cardiac imaging, fetal and perinatal cardiology, and brain development and neurodevelopmental outcomes in congenital heart disease. Her current research projects include the following.

- Predictors of brain injury and neurodevelopmental outcomes in infants with congenital heart disease
- Outcomes after prenatal versus postnatal diagnosis of d-transposition of the great arteries

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- AAP
- Fetal Heart Society
- American Society of Echocardiography

MAJOR LECTURESHIPS AND SEMINARS

- "Diagnosis of Fetal Arrhythmias," Progress in Perinatal Cardiology: Detection and Management of Fetal Congenital Heart Disease, Tampa, Fla., February 2017

Shawn West, MD, MSc

RESEARCH

Shawn West's research interests include cardiac transplantation in children following single-ventricle physiology, cardiomyopathies, acute rejection following heart transplantation, and noninvasive measurements of systolic and diastolic function in children with structural congenital heart disease. West explores outcomes-based research in children with congenital heart disease and heart transplantation, cost analysis of heart transplant, quality improvement in care of single-ventricle patients through third-stage palliation, assessment of myocardial deformation using velocity vector imaging/speckle tracking, and outcomes of fetal cardiomyopathies. His current research projects include the following.

- CTOT, CTOT-09 (site sub-investigator)
- PHTS (site subinvestigator)
- Heart transplant EBV study (site sub-investigator)
- Pediatric cardiomyopathy registry biomarker study (site sub-investigator)
- Use of Advanced Cardiac Therapies for Patients with Duchenne Muscular Dystrophy
- Open-label study for Sanfilippo type B for SBC-103
- Left heart decompression and outcome in patients on veno-arterial ECMO support
- Midterm analysis of the bicuspid polytetrafluoroethylene conduit (Masa valve) for right ventricular outflow tract reconstruction in neonates and infants
- Inert Gas Rebreathing for Predicting Disease Status in Subjects With Pulmonary Hypertension
- Pediatric Heart Failure Observational Study Group: This is a multicenter registry study through the International Society for Heart and Lung Transplantation, examining pediatric acute myocarditis in a contemporary multicenter cohort.
- Fontan-Related Protein-Losing Enteropathy and Transplant Observational Study: This is a multicenter registry study analyzing patients referred for heart transplant evaluation for Fontan-related protein-losing enteropathy.

ADVISORY COMMITTEE MEMBERSHIPS

- American Society of Transplant
- PHTS Practice

EDITORSHIPS

- Guest editor reviewer, *Journal of Heart and Lung Transplantation*
- Guest editor reviewer, *International Journal of Cardiology*
- Guest editor reviewer, *Cardiology in the Young*
- Guest editor reviewer, *Journal of Thoracic Disease*

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- International Pediatric Transplant Association
- International Society for Heart and Lung Transplantation
- PHTS
- Variation Working Group, PHTS, 2017 to the present
- AHA
- American Society of Transplantation
- Patient Transition Committee, American Society of Transplantation, 2015 to the present

MAJOR LECTURESHIPS AND SEMINARS

- Pedimag National Training Conference, Children's Hospital of Pittsburgh, Pittsburgh, Pa., June 2017

HONORS

West serves as the pediatric cardiologist for the UPMC Hereditary Hemorrhagic Telangiectasia Center, which has been approved as a North American Center of Excellence.

Matthew Zinn, DO

RESEARCH

Matthew Zinn's research interests include the associations between surveillance biopsy intensity and outcomes after heart transplantation in children, mechanical circulatory support in children, cardiomyopathies, and antibody-mediated rejection after heart transplantation. Zinn was the site investigator for a recent multinational study evaluating outcomes in pediatric heart failure for patients supported with the HeartWare HVAD. He is part of a study evaluating the cardiac presentation of an Amish patient with propionic acidemia. His current research projects include the following.

- CTOT, CTOT-09 (site sub-investigator)
- PHTS (site sub-investigator)
- Pediatric cardiomyopathy registry biomarker study (site sub-investigator)
- PediMACS (site-investigator)

ADVISORY COMMITTEE MEMBERSHIPS

- Regional Review Board, Region 2, United Network for Organ Sharing, 2016 to the present
- Medical director, Bill Neches Heart Camp for Kids, 2016 to the present

EDITORSHIPS

- Guest editorial reviewer, *Pediatric Transplantation*

MAJOR LECTURESHIPS AND SEMINARS

- "Pediatric Cardiac Extracorporeal Life Support," Eighth Annual Neonatal and Pediatric ECMO Conference at Children's Hospital of Pittsburgh, Pittsburgh, Pa., August 2016
- "Myocardial Failure: Patient Selection and Indications for Mechanical Circulatory Support," Foundations of Pediatric Mechanical Circulatory Support: PediMag National Training Program, Pittsburgh, Pa., June 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- AHA
- International Society for Heart and Lung Transplantation
- AAP
- American Osteopathic Association
- PHTS

TEACHING ACTIVITIES

The pediatric cardiology faculty plays an active role in the teaching of medical students, residents, fellows, nurse practitioners, ultrasound students, and adult cardiology trainees. The program has an ACGME-accredited fellowship training program that has trained more than 60 pediatric cardiologists and currently is training eight categorical fellows. In addition, predoctoral and postdoctoral trainees form part of the cardiovascular research programs of the cardiology division and the Heart Institute.

A team of cardiologists participated in the University of Pittsburgh School of Medicine second-year medical student cardiovascular course on congenital heart disease, giving lectures and providing a hands-on workshop. In addition, the Heart Institute, led by Allada, and the cardiology division continue to host the Master Class in Congenital Cardiac Morphology with world-renowned professor Robert Anderson, now in its 10th year. This highly successful educational program brings attendees from all over the world to hear from experts at Children's Hospital and utilizes the Frank E. Sherman and Cora C. Lenox Heart Museum.

Cardiology fellows presented abstracts at numerous regional and national meetings. To meet the ever-growing need for expertise in adult congenital heart disease, the program has successfully implemented the adult congenital heart disease fellowship program curriculum, with the successful graduation of the inaugural fellow in 2015, Lan Nguyen, who has joined the UPMC adult congenital heart disease program as a faculty member. The graduating fellows continue to achieve national recognition, with advanced fellowship and faculty positions at some of the most prestigious institutions in the country, including Mayo Clinic, University of Michigan, Medical University of South Carolina, and Boston Children's Hospital. The cardiology division continues to train the next generation and impart the Children's Hospital of Pittsburgh's expertise across the country.

Finally, this year marked the 27th anniversary for the Bill Neches Heart Camp for Kids. Under the leadership of the medical directors, Linda Russo and Susan Miller, this highly successful camp gives Heart Institute patients, children, and young adults the opportunity to get together and share in their successes.

THREE-YEAR BIBLIOGRAPHY

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DIVISION OF
PEDIATRIC EMERGENCY MEDICINE

Mission

The division strives to have the **best** pediatric emergency department because it has the **best** people who provide the **best** patient care, keeping in mind that patients and families believe that **best** means timely care accompanied by compassion, communication, and partnered decision making.

Specific elements of the mission of the Division of Pediatric Emergency Medicine include:

- To deliver the highest quality of care to children with acute illnesses and injuries and to do so efficiently and with attention to safety and cost
- To provide excellence in service to patients, families, and referring clinicians
- To educate fellows, residents, students, nurses, advanced practice providers, and prehospital care personnel
- To pursue scholarly clinical and laboratory research questions
- To advance the field of pediatric emergency medicine both clinically and academically

FACULTY AND STAFF

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Chief, Division of Pediatric Emergency Medicine
Clinical Director, Division of Pediatric Emergency Medicine
Medical Director, Emergency Department, Children's Hospital of Pittsburgh of UPMC

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Director of Quality and Safety, Division of Pediatric Emergency Medicine

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Director, Pediatric Emergency Medicine Specialty Care Clerkship, University of Pittsburgh School of Medicine

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Director, Medical Toxicology Fellowship
Medical Director, West Virginia Poison Center

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Associate Director, Pediatric Emergency Medicine Fellowship Program

Joseph H. Yanta, MD
Assistant Professor of Emergency Medicine

Noel S. Zuckerbraun, MD
Associate Professor of Pediatrics
Director, Pediatric Emergency Medicine Fellowship Program

ADVANCED PRACTICE PROVIDERS

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Julie Giuffre, CRNP
Jessica Higgins, PA-C
Kaylynn Hlasnik, PA-C
April D. Hollenbeck, PA-C
Debra A. Hornyak, CRNP
Erin C. Madden, PA-C
Laura Pete, PA-C
Jodee Stevens, PA-C

OVERVIEW OF DIVISION

The objective of the Division of Pediatric Emergency Medicine is to be a "quadruple-threat" division focused on clinical care, education, scholarly pursuit, and quality science. The division has created an environment that provides excellence in clinical care and a dense educational experience at every level, including students, house staff, fellows, faculty, nurses, and advanced practice providers. Scholarly opportunities for division members have their origins in the fertile clinical environment of the emergency department and are a result of a wide collaborative research network.

CLINICAL ACTIVITIES

The division's attending physicians are all board eligible or certified in pediatrics and pediatric emergency medicine and staff the emergency department 24 hours per day. The emergency department at Children's Hospital had an annual patient volume of nearly 82,000 during fiscal year 2017, an increase of more than 51% during the past decade. Approximately 19% of patients who presented to the emergency department were admitted to the hospital.

The emergency department cares for children with both medical and surgical problems, including traumatic injuries. Children's Hospital is the only level 1 pediatric trauma care facility in the tristate region and includes a catchment area spanning all of Western Pennsylvania, northern West Virginia, and northeastern Ohio. As a tertiary-care referral center, the hospital admits approximately 1,700 children with traumatic injuries each year.

RESEARCH AND OTHER SCHOLARLY ACTIVITIES

The Division of Pediatric Emergency Medicine has a robust research infrastructure that includes a 120-square-foot office dedicated to research and located adjacent to the emergency department. Research assistants screen and enroll study patients seven days per week. The research staff includes three registered nurses and two undergraduate students. A partial list of research topics includes: treatment of urinary tract infections (UTIs), computer-based adaptive screening for risk of suicide, non-invasive detection of ventriculoperitoneal shunt malfunction, and prevalence of influenza. The emergency department research group has partnered with other divisions in the Department of Pediatrics, including General Academic Pediatrics, Pediatric Infectious Diseases, Pulmonology, and Hematology/Oncology, to collaborate on several additional studies related to pediatric acute and emergency care.

Richard A. Saladino, MD

Richard A. Saladino is chief of the Division of Pediatric Emergency Medicine.

MENTORSHIP AND RESEARCH

Saladino's mentorship efforts blend his past laboratory interests (pathophysiology and prevention and treatment of serious bacterial infections) and current clinical interests (process improvement, biosurveillance, procedural sedation, and evaluation and management of traumatic injuries). Saladino currently is a co-investigator on two major grants: a National Institutes of Health R01 grant titled "Novel Approaches to Screening for Inflicted Childhood Neurotrauma" and a Patient-Centered Outcomes Research Institute grant titled "Using the Electronic Medical Record to Improve Outcomes and Decrease Disparities in Screening for Child Physical Abuse." He has mentored numerous students, residents, fellows, MD-PhD students, and junior faculty in their clinical and scholarly projects.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Society for Pediatric Research
- American Academy of Pediatrics
- Section on Emergency Medicine, American Academy of Pediatrics
- Academy of Master Educators, University of Pittsburgh School of Medicine

Richard A. Saladino, MD
Division Chief, Pediatric Emergency Medicine

Michael Abesamis, MD, MPH

RESEARCH

Michael Abesamis' research interests include novel antidotes for anticoagulant toxicity and toxicity associated with hydraulic fracturing.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American College of Medical Toxicology
- American Academy of Clinical Toxicology

Mananda S. Bhende, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- American College of Emergency Physicians
- American Association of Physicians of Indian Origin

Kristin M. Farrell, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics

Martin G. Hellman, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- American College of Emergency Physicians

Robert W. Hickey, MD

RESEARCH

Robert W. Hickey is director of research for the division. He has a track record of laboratory-based research related to *in vitro* and *in vivo* models that examine the molecular basis for hypoxic-ischemic brain injury following resuscitation from cardiac arrest.

Hickey's clinical research has encompassed common pediatric emergency medicine conditions (e.g., UTI, bronchiolitis, pneumonia), with a special emphasis on cardiopulmonary resuscitation (CPR). Selected contributions include describing the prevalence of UTI in young febrile children, showing that outpatient therapy is equivalent to inpatient therapy for infants with UTI, defining the role of imaging in young children with UTI, comparing automated with manual urinalysis, demonstrating that blood cultures are unnecessary in management of patients with uncomplicated pneumonia, describing the prevalence of retinal hemorrhages in child-abuse victims presenting with apparent life-threatening events, describing the occurrence and success of bystander CPR in pediatric cardiac arrest, showing that children resuscitated from cardiac arrest frequently develop hypothermia followed by fever, and showing that hyperoxia secondary to supplemental oxygen administration is common (and prolonged) in children resuscitated from cardiac arrest.

Selected ongoing projects include investigating paraspinal, intramuscular injection of ropivacaine for treatment of headache; investigating a non-invasive device for detecting

ventriculoperitoneal shunt malfunction; investigating steroids for prevention of scarring in pyelonephritis; comparing short-course versus traditional-course antimicrobial therapy for UTI; testing a computer-based screening algorithm for detecting adolescents at risk for suicide; and comparing anti-epileptic medications for treatment of children with benzodiazepine-resistant seizures.

Hickey has an established reputation as an expert in pediatric CPR. He has served as the co-chair of the International Liaison Committee on Resuscitation (ILCOR) Pediatric Task Force and as the co-chair for the ILCOR Conference on Emergency Cardiovascular Care and CPR.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Society for Pediatric Research
- American Academy of Pediatrics
- American Heart Association
- Society for Neuroscience
- ILCOR

Janet M. Kinnane, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics

Rhett H. Lieberman, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- American Academy of Pediatric Program Directors

Amanda Lovallo, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics

Michael Lynch, MD

RESEARCH

Michael Lynch's research interests include novel treatments for withdrawal conditions, novel methods of mortality prediction in acetaminophen toxicity, and illicit drug adulterants and their medical consequences.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American College of Medical Toxicology
- American Academy of Clinical Toxicology

Mioara D. Manole, MD

RESEARCH

Mioara D. Manole continues her investigation of the cerebral effects of pediatric asphyxial cardiac arrest in a small-animal model. She is the primary investigator for a National Institutes of Health R01 grant titled "CYP 450-Mediated CBF Dysregulation and Neurotoxicity in Pediatric Cardiac Arrest."

ADVISORY COMMITTEE MEMBERSHIPS

- Chair, Scholarly Oversight Committee, Division of Pediatric Emergency Medicine

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Society for Pediatric Research

Jennifer R. Marin, MD

Jennifer Marin has significant training and experience in ultrasound. She developed the curriculum for training and assessing competency in bedside ultrasound for faculty and fellows in the Division of Pediatric Emergency Medicine. She was appointed quality director for point-of-care ultrasound for Children's Hospital and, as such, oversees the use of bedside ultrasound by other divisions in the hospital. Marin also co-chaired an international multidisciplinary consensus on emergency diagnostic imaging.

RESEARCH

Marin's research focus is on diagnostic imaging, including the use of point-of-care ultrasound and computed tomography utilization and variation. She serves as a research mentor for pediatric emergency medicine fellows.Marin was co-lead author of the "Policy Statement and Technical Report on Point-of-Care Ultrasound," recently published by the American Academy of Pediatrics.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Co-chair, American College of Emergency Physicians
- Member, sections on Emergency Ultrasound, Policy and Government Relations, and Ultrasound, American College of Emergency Physicians
- Section on Emergency Ultrasound, Society for Academic Emergency Medicine
- American Institute of Ultrasound in Medicine
- American Academy of Pediatrics
- Chair, Pediatric Emergency Medicine Point-of-Care Ultrasound (P2)

EDITORSHIPS

- Associate editor, *Pediatric Emergency Care*

Brett L. McAninch, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics

Erin D. Phrampus, MD

Erin Phrampus has established a collaborative relationship with the WISER Center, where she has developed a simulation-based curriculum for the pediatric emergency medicine and pediatric critical care programs. She directs a number of courses held at both the WISER Center and Children's Hospital of Pittsburgh Pediatric Simulation Center.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics

Raymond D. Pitetti, MD

RESEARCH

Raymond D. Pitetti's research interests include the laboratory detection, evaluation, and management of apparent life-threatening events in children. He continues to develop a comprehensive research program designed to evaluate the use of various medications for procedural sedation, such as dexmedetomidine, nitrous oxide, and propofol, as well as evaluate programmatic changes, such as the behavioral aspects of children undergoing sedation and the impact of cancelled and failed sedations.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Section on Emergency Medicine, American Academy of Pediatrics
- Society for Pediatric Research
- Section on Complementary, Holistic, and Integrative Medicine, American Academy of Pediatrics
- Society for Pediatric Sedation

Anthony F. Pizon, MD

RESEARCH

Anthony Pizon's research interests include snake envenomations, novel treatments for withdrawal conditions, novel methods of mortality prediction in acetaminophen toxicity, and illicit drug adulterants and their medical consequences.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Board of Directors, American College of Medical Toxicology
- American Academy of Clinical Toxicology

Johanna R. Rosen, MD

Johanna Rosen is the director of quality and process improvement for the Division of Pediatric Emergency Medicine. She has developed a broad-based program to support initiatives that focus on quality and process improvement as well as patient safety.

RESEARCH

Rosen's research and scholarly interests are focused on quality science. Her projects include initiatives related to early identification and treatment of children with septic shock, timely pain reduction for children with acute fractures, and reduction in time to corticosteroids for children with asthma exacerbation.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics

Melissa A. Vitale, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics

Joseph H. Yanta, MD

RESEARCH

Joseph Yanta's research interests include novel interventions to link those with opioid addiction to outpatient resources and prevent early relapse after discharge from the emergency department, novel methods of mortality prediction in acetaminophen toxicity, and illicit drug adulterants and their medical consequences.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Toxicology Section, American College of Emergency Physicians
- American College of Medical Toxicology
- American Academy of Clinical Toxicology

Noel S. Zuckerbraun, MD

Noel Zuckerbraun is the director of the fellowship program in pediatric emergency medicine. She continues to develop curricula, with a recent focus on developing the research and academic skills curricula, restructuring both into longitudinal courses. She continues to direct and enhance simulation programming for pediatric emergency medicine fellows and emergency medicine and pediatric residents. She directs a number of courses held at both the WISER Center and Children's Hospital of Pittsburgh Pediatric Simulation Center. In addition, Zuckerbraun is one of the creators of the Department of Pediatrics faculty development series

and a member of the Department of Pediatrics Bridges workgroup for faculty development.

RESEARCH

Zuckerbraun's research and scholarly activities continue to focus on clinical pediatric emergency medicine, including injury, medical education, and ongoing quality improvement in the emergency department.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Academic Pediatric Association
- Society for Pediatric Research
- Section on Emergency Medicine, American Academy of Pediatrics

THREE-YEAR BIBLIOGRAPHY

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Steven
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DIVISION OF PEDIATRIC ENDOCRINOLOGY, DIABETES, AND METABOLISM

Mission

The Division of Pediatric Endocrinology, Diabetes, and Metabolism supports the tripartite goals of exceptional teaching, world-acclaimed research, and superb patient care for children with all hormonal disorders, including growth, thyroid, adrenal, gonadal, calcium, diabetes, and carbohydrate-related metabolic disorders. The division's core missions are to:

- Maintain and surpass its record of excellence in patient care
- Teach medical students, residents, and fellows and train the next generation of pediatric endocrinologists
- Conduct state-of-the-art research focused on understanding and treating endocrine disorders and diabetes

FACULTY AND STAFF

Radhika H. Muzumdar, MD
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Mark A. Sperling, MD
Emeritus Professor of Pediatrics

Pushpa Viswanathan, MD
Assistant Professor of Pediatrics

Selma F. Witchel, MD
Professor of Pediatrics
Director, Pediatric Endocrinology Fellowship Program

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Crystal Brown, RN, BSN, CPN

Amy Gilliland, RN, MSN, CDE

Leon Green, RN

Erika McCann, BS, BSN, MS

Shaylyn McDaniel, RN

Cynthia Puchtler, BS

OVERVIEW OF DIVISION

The faculty and fellows continue to be nationally and internationally recognized for their clinical expertise and their research endeavors. Six members of the division were listed again in the survey of "best doctors" in the United States and Pittsburgh. Development of satellite and outreach clinics combined with regional referrals and those from other centers resulted in a steady increase in patient numbers. Members of the faculty and division nurses continue to receive invitations to be speakers and panel members, expert committee members, and journal reviewers. Two new fellows started their clinical and research training in the program this year, and two fellows graduated from the program. Starting July 2018, the number of trainees will increase to three fellows per year.

Radhika Muzumdar is chief of the division and an associate professor of pediatrics and cell biology. Her research focuses on growth hormone (GH); insulin-like growth factors (IGFs) and their binding partners; and the effects of novel

mediators of glucose homeostasis, energy metabolism, and aging. One mediator is humanin, a mitochondria-associated peptide recognized for its effects on cell survival. Muzumdar found that it has a marked effect on insulin sensitivity and that it increases insulin secretion through enhancement of beta-cell glucose metabolism. Her laboratory discovered a role for humanin in regulating hepatic lipid fluxes through the hypothalamus. She continues her studies on humanin, aging, life span, cardiovascular health, and diabetes at Children's Hospital of Pittsburgh of UPMC. She is joined by Zhenwei Gong, a research assistant professor studying the role of humanin in augmenting existing therapies for diabetes and the effects of humanin and analogs on lipid metabolism, cellular processes of autophagy, and oxidative stress. Muzumdar and Gong investigate the role of a novel acyl CoA dehydrogenase enzyme, ACAD10, and an inflammasome-associated protein, Aim 2, on glucose metabolism and energy homeostasis. Muzumdar serves as a standing member on the National Institutes of Health (NIH) Study Section on Aging Systems and Geriatrics, in addition to being a reviewer for international and national granting agencies, including the U.S. Food and Drug Administration.

Dorothy Becker is a grant reviewer and a member of NIH and national diabetes committees. She continues as the principal investigator examining the prediction and prevention of type 1 diabetes in the Research Project Grant (R01) and three multicenter Opportunities for Collaborative Research at the Clinical Center (U01) programs. Becker is the U.S. principal investigator and a member of the International Executive Committee of a large primary prevention diabetes study, "Trial to Prevent Insulin-Dependent Diabetes in the Genetically at Risk: Nutritional Primary Prevention of Type 1 Diabetes (TRIGR)."

Henry Dong is NIH funded to study the role of forkhead box O (FoxO) transcription factors in glucose and lipid homeostasis. He is funded by the American Diabetes Association (ADA) to study the role of hepatic insulin on glucose homeostasis.

Ingrid Libman continues her research of the epidemiology of both type 1 and type 2 diabetes. She explores the complications of type 1 diabetes and the effects of obesity and insulin resistance in children with features of both type 1 and type 2 diabetes. She studies innovative, technology-based approaches to improve care of children with diabetes. Her findings have been the foundation for several awarded grants. She is youth/pediatric diabetes chair, ADA Scientific Sessions Meeting Planning Committee, 2016–2018.

Kara Hughan, through her K23 project, is investigating the mechanisms of macrovascular disease related to insulin resistance. She received the EnVision award from the Cystic Fibrosis Foundation. Becker, Silva Arslanian, Oscar Escobar, Libman, and Selma Witchel were co-investigators on several other NIH- and foundation-supported grants.

Escobar Luigi Garibaldi continues with national GH and gonadotropin-releasing hormone analog studies.

The division has continued its high level of research productivity, with 39 active research studies, sustained by the NIH, foundation grants, and several pharmaceutical grants. The grants have supported the peer-reviewed publications and chapters in textbooks in the appended bibliographic list (2015–2017).

The faculty are recognized for their teaching skills. Mark Sperling has been a very successful editor of the *Pediatric Diabetes Journal*, a journal he established. Sperling, Muzumdar, and Witchel are on the editorial panel of *Frontiers of Pediatric Endocrinology*. In addition, Muzumdar, Becker, Sperling, Dong, Witchel, Libman, and Arslanian have taken part in scientific peer review of NIH grants, have chaired sessions at major scientific meetings, and have been invited to give several national and international lectures. Witchel has been active nationally and internationally on committees related to androgen excess disorders. Muzumdar and Witchel are chairs of the Research Affairs Council and Education Council for the Pediatric Endocrine Society (PES). Hecht-Baldauff and Libman are involved in the Education Council and Diabetes Special Interest Group at the PES.

This year, five trainees participated in the Pediatric Endocrine Fellowship Training Program under the overall directorship of Witchel. The research training portion of the program is supported by a T32 training grant from the NIH and is directed by Muzumdar, who also oversees the David Nicholas Fellowship. Baldauff, Garibaldi, and Escobar assist Witchel with the fellowship clinical education program and teach residents and students. The division's fellows received competitive grants for their research projects and presented their work at national meetings.

CLINICAL ACTIVITIES

Flint, associate clinical director, and Garibaldi have continued to expand the clinical program, adding more satellite and outreach clinics to meet the demands of a growing patient population. They maintain the division's delivery of team care and intensive diabetes therapy in satellite and outreach sites. Libman, as the director of diabetes, and Ismail, as clinical director of diabetes, have continued to expand access to the transition program, which is a vital component of diabetes care for patients who graduate from high school and move on to college or the workforce. The division pioneered and refined the retinopathy screening via fundus photography, which has the potential to become the standard of care.

The Weight Management and Wellness Center has been incorporated into the division of endocrinology. Patients who call for appointments are now triaged more efficiently into regular endocrine clinics, diabetes clinics, or endocrine wellness clinics, which provide intensive nutritional support for patients with weight concerns.

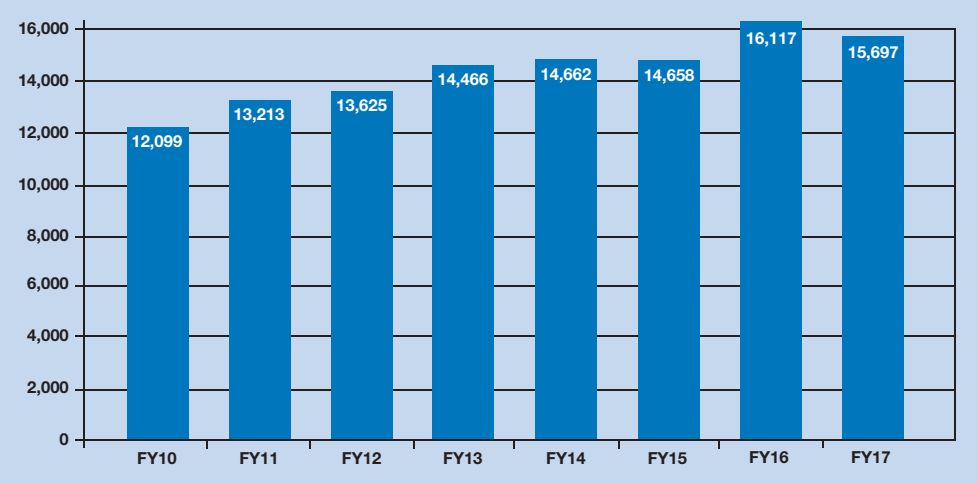
Faculty, fellows, 11 full-time advanced practice providers, and the diabetes team keep pace with the large and ever-increasing outpatient clinical service, as shown in the figure below. The division supports a vibrant inpatient service, including frequent consultations throughout Children's Hospital of Pittsburgh and Magee-Womens Hospital. The number of new diabetes patients, already on the rise over the past decade from about 160 to 250 per year, increased by 20% this year. Patients are educated by the division's superb team of diabetes educators and dietitians. The diabetes educators deliver traditional diabetes teaching for all patients with new-onset type 1 or type 2 diabetes and staff diabetes clinics at all sites. They initiate and provide training with increasingly sophisticated devices, such as insulin infusion pumps and glucose sensors. To meet the needs of patients, the team established a technology clinic, where a diabetes educator with expertise in advances in diabetes technology advises patients and families. The diabetes educators maintain a daytime call-in service for the approximately 2,200 patients with diabetes. The division recently added extended hours of telephone support for the hours from 5 to 11 p.m. This should improve patient care and satisfaction considerably, as evening is a peak time for diabetes-related calls. The division also provides most of the medical staff for the ADA-sponsored diabetes camp each summer.

The endocrine clinical and teaching nurse team has grown to seven full-time nurses to accommodate growing patient numbers and the need for dynamic endocrine testing. The nurses perform more than 1,000 tests per year, by scheduled appointment. Additionally, during the past year, the division introduced same-day testing to offer the advantage of dynamic testing at the time of patient encounter. The nurse team facilitates physician workflow while improving patient satisfaction and quality of care.

The multidisciplinary approach to the care of children with chronic endocrine disorders includes a Diabetes Center led by Libman, Ismail, Muzumdar, and Becker, with the education coordinated by Karen Kelly. In addition to the transition program, the diabetes subdivision offers a specialized diabetes care program, including the recently initiated post-transplant diabetes clinic and program with Garibaldi and Flint, as well as the cystic fibrosis–related program with Hughan, Witchel, and Ismail. The division continues to actively participate in the autologous islet cell transplant program in collaboration with the Transplant Division at Children's Hospital and UPMC Presbyterian Hospital for children and young adults who undergo total pancreatectomy for severe, chronic pancreatitis; this is one of very few such pediatric programs in the United States.

The division offers specialized and disease-focused services as follows: the Growth Center, led by Escobar; the Center for Disorders of Sexual Development, under the direction of Witchel; a multidisciplinary Thyroid Center, under the direction of Pushpa Viswanathan, Witchel, and Gurtunca; a Lipid Clinic, under the direction of Flint; the Pittsburgh Endocrine Gender Center for gender non-conforming youth, under the direction of Witchel; and an endocrine surveillance and follow-up clinic and program for cancer survivors in collaboration with the Division of Oncology/Hematology, under the leadership of Gurtunca.

DIVISION OF ENDOCRINOLOGY, DIABETES, AND METABOLISM ANNUAL OUTPATIENT VOLUME (FY: FISCAL YEAR)



RESEARCH AND OTHER SCHOLARLY ACTIVITIES

Radhika H. Muzumdar, MD

RESEARCH

Muzumdar's research spans the GH/IGF axis in glucose homeostasis, the role of the hypothalamus in regulation of whole-body energy and glucose homeostasis, and the role of a novel mitochondria-associated peptide called humanin in aging and age-related diseases, especially glucose homeostasis and cardiovascular health. Her work has led to important insights on the role of IGFs and IGF binding proteins in insulin sensitivity and regulation of intermediary metabolism by nutrient sensing. Her work on the role of reductions in serum IGF-1 levels on healthspan was published in *Aging Cell* and was selected for F1000Prime as being of special significance in its field. Her work demonstrating that impaired metabolism in states of GH resistance results from direct actions of GH on lipid uptake and *de novo* lipogenesis was published in *Diabetes* and highlighted in the *Year Book of Endocrinology* at the international PES meeting.

Muzumdar has shown that humanin, known for its effects on cell survival in the nervous system, has significant effects on glucose homeostasis; this work is the basis of her NIH R01 funding. Humanin and analogs improve insulin sensitivity through the hypothalamus and increase insulin secretion through enhancement of glucose metabolism in beta cells. She has also won NIH funding to study the role of humanin on beta-cell function in aging. The studies have led her to focus on the synergy between humanin and GLP-1.

Radhika H. Muzumdar, MD
Division Chief, Pediatric Endocrinology, Diabetes, and Metabolism

Muzumdar has found that humanin analog administered prior to or following ischemia protects the myocardium following ischemia-reperfusion injury and improves cardiac function in mice. Muzumdar has tested the cardioprotective effects of humanin analogs in swine models of ischemia and reperfusion. The studies, performed in collaboration with David Lefer, director of the National Heart, Lung, and Blood Institute (NHLBI) Consortium for Preclinical Assessment of Cardioprotective Therapies at Louisiana State University Health Sciences Center at New Orleans, demonstrated that humanin offers cardioprotection in swine models of myocardial ischemia and reperfusion. Muzumdar received a grant from the Pittsburgh Foundation to conduct the preliminary studies, which are the basis of an R01 with multiple principal investigators that was submitted this year. She is also collaborating with Eric Goetzman's laboratory to understand the mechanisms behind humanin-mediated cardioprotection, through studies on substrate flux and metabolism.

Ischemia/reperfusion is associated with significant oxidative stress. Recently, Muzumdar's laboratory found that humanin analog protects against oxidative stress through critical upregulation of key antioxidant enzymes in a tyrosine kinase–dependent manner. Because oxidative stress is intimately linked to the cellular recycling process of autophagy, Muzumdar and Gong studied the effects of humanin on autophagy and, particularly, chaperone-mediated autophagy, in collaboration with Ana Maria Cuervo at AECOM. A manuscript describing the observations was recently accepted for publication in the *Journal of Cellular Biology*.

Endogenous levels of humanin decline with age. Humanin and its potent analogs have been shown to have beneficial effects in many age-related diseases, including Alzheimer's disease, stroke, diabetes, myocardial ischemia and reperfusion, atherosclerosis, amyotrophic lateral sclerosis, and certain types of cancer. An association between humanin levels, the GH/IGF axis, and lifespan was demonstrated in various mouse models with mutations in the GH/IGF axis. Muzumdar is currently collaborating with Ghazi's laboratory to study the effects of humanin on healthy aging and lifespan in a *Caenorhabditis elegans* model.

Muzumdar's laboratory also studies the regulation of energy and glucose homeostasis. A knockdown of a gene called *Absent in Melanoma 2* (*Aim 2*), a tumor suppressor gene and a part of the inflammasome, results in spontaneous obesity and impaired glucose tolerance. The effects seem to be mediated through upregulation of ifi202b, an interferon-related protein. The findings are especially significant, as human obesity is associated with upregulation of ifi202b. The studies were funded through a Cochrane Weber grant and are the focus of an R01 submitted with Gong.

In collaboration with Vockley and Goetzman, Muzumdar is studying the role of ACAD 10, a novel acyl CoA dehydrogenase in glucose homeostasis, and has shown that absence of ACAD10 in certain mouse background leads to a phenotype of obesity and insulin resistance. This is interesting, as polymorphisms in the ACAD 10 gene have been reported in Pima Indians, a population at high risk for obesity and type 2 diabetes. Knockout of the gene also results in a novel phenotype of hypoglycemia on fasting, with no evidence of defects in fatty acid oxidation. This collaboration is ongoing and has resulted in publications and funding from NIH, the Endocrine Fellows Foundation, and a Cochrane Weber grant.

In addition to the NIH, Muzumdar has received grant support from multiple funding agencies, including the PES, American Federation for Aging Research (AFAR), Beck Foundation, and Endocrine Fellows Foundation for her work on the roles of GH/IGF/ IGFBP-3 in glucose homeostasis, as well as the role of humanin in insulin secretion and myocardial infarction.

She is on several national and international study sections, including the NIH, AFAR research council, United States–Israel Binational Science Foundation, Israeli Science Foundation, Binational Science Foundation between Israel and Italy, Marsden Fund (New Zealand), and Wellcome Trust (United Kingdom). She serves as a standing member of the NIH Aging Systems and Geriatrics Study Section. She chairs the Research Advisory Council of the PES and serves on AFAR's National Scientific Advisory Council. She has chaired sessions and been an invited speaker at national and international endocrine meetings. She is a reviewer for numerous reputed journals and national meetings. She continues to be an active member of the PES, with leadership roles.

Muzumdar is the principal investigator of the Research Training in Pediatric Endocrinology Program. The goal of the T32 training grant is to provide state-of-the-art training in the molecular, cellular, physiologic, genetic, and biochemical aspects of pediatric endocrinology to ensure that the physician-scientists who graduate from the program are well prepared for productive academic careers in translational research related to pediatric endocrinology.

ADVISORY COMMITTEE MEMBERSHIPS/ STUDY SECTIONS

- Chair, Clinical and Translational Research Registry Subcommittee, PES
- Chair, Research Affairs Council, PES
- National Mentoring Initiative, PES
- International Research Collaborations Subcommittee, PES
- Invited member, Research Advisory Council, PES
- Invited member, Committee on National Mentoring Initiative, PES
- Moderator, scientific sessions, ADA
- Ad hoc member, Integrative Physiology of Obesity and Diabetes Study Section, National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK), NIH
- Mentor, Endocrine Society
- Standing member, Aging Systems and Geriatrics Study Section, National Institute on Aging, NIH

- Strategic Initiatives Planning Committee, Children's Hospital of Pittsburgh
- Pediatric Physician Scientist Program Committee, Children's Hospital of Pittsburgh
- K-12 Advisory Committee, Children's Hospital of Pittsburgh
- Member, Honors and Awards Committee, PES

EDITORSHIPS

- Editor, *International Journal of Case Reports and Images*
- Associate editor, *Frontiers in Pediatric Endocrinology*

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Endocrine Society
- ADA
- Society for Pediatric Research
- American Pediatric Society
- PES

Natalie Hecht Baldauff, DO

RESEARCH

Natalie Hecht Baldauff's research focuses on the role of adiponectin in new-onset type 1 diabetes and noninvasive maximization of fertility in a peri-pubertal population of patients with Klinefelter syndrome.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- PES
- Endocrine Society
- International Society for Pediatric and Adolescent Diabetes

Dorothy J. Becker, MBBCh, FCP (paed)

RESEARCH

Dorothy J. Becker's major interests are the prediction and prevention of type 1 diabetes and its complications. Her efforts are supported by several grants.

Research Training in Pediatric Endocrinology. The goal of the T32 training grant is to provide state-of-the-art training in the molecular, cellular, physiologic, genetic, and biochemical aspects of pediatric endocrinology to ensure that the physician-scientists who graduate from the program are well prepared for productive academic careers in translational research related to pediatric endocrinology. Becker serves as senior transition consultant.

Juvenile Diabetes Mellitus: Epidemiology and Etiology. This is a 37-year study funded by the NIH until this year that evaluates the immunologic, genetic, and environmental determinants of the risk of insulin-dependent diabetes mellitus in children and families of children with probands with type 1 diabetes. It is a prospective biannual study of T-cell markers of autoimmunity and viral infection and assessments of insulin resistance in genetically at-risk, first-degree relatives with islet cell antibodies and diabetes end points. Becker is principal investigator.

TrialNet (Prediction and Prevention of Type 1 Diabetes). TrialNet is an NIH collaborative study (Pittsburgh being one of the original centers) in the United States and Europe to examine the risks and mechanisms of

autoimmune progression. Data will be used to plan and implement intervention strategies to maintain beta-cell function in patients with new-onset type 1 diabetes and to prevent diabetes in first-degree relatives at high risk.

TRIGR. Pittsburgh is a major center and the coordinating center for the United States for this double-blind, randomized, controlled trial involving subjects with an affected first-degree relative and risk-associated human leukocyte antigen genotypes. This is an international, multicenter consortium involving 73 centers in 15 countries; it is funded by the NIH and Canadian Institutes of Health. The six- to eight-month intervention to compare the effects of either hydrolyzed casein or standard cow milk–based weaning formula has been completed. All subjects are being followed for 10 years for measurements of serological markers of intact cow milk exposure, autoantibodies predictive of diabetes (the end point at age 6 years), and the clinical and/or metabolic indices of diabetes (the end point at age 10 years). A large, cross-linked repository of stored sera, DNA, and cryopreserved peripheral blood mononuclear cells allows independently funded ancillary and mechanistic studies related to the natural history of prediabetes and the hypothesis to be tested. Becker is principal investigator.

The Environmental Determinants of Diabetes in the Young. This is an NIH-funded, multicenter, epidemiologic study of the environmental pathogenesis of autoimmunity in first-degree relatives of subjects with type 1 diabetes. Pittsburgh is a satellite. Becker is co-investigator.

Slow or Nonprogressive Autoimmunity to the Islets of Langerhans (SNAIL Study). This multicenter study in the United Kingdom; Germany; Pittsburgh, Pa.; and Denver, Colo., is exploring factors that slow the progression of diabetes autoimmunity from multiple autoantibodies to clinical diabetes.

Adolescents With and Without Diabetes: Transition to Emerging Adulthood. The goal of this NIH-funded research is to examine the impact of insulin-dependent diabetes on quality of life over the transition from adolescence into adulthood as compared to nondiabetic individuals. This study will concentrate on the follow-up of graduates of the diabetes program for analyses of psychosocial behaviors and risk factors and evaluation of their relationship with metabolic control and diabetes-related complications. Becker is co-investigator.

Epidemiology of Diabetes Complications. Led by Trevor Orchard, this long-term study evaluates the risk factors and trajectory of diabetes complications in a long-term cohort of type 1 diabetes patients who graduated from the program more than 30 years ago.

ADVISORY COMMITTEE MEMBERSHIPS

- Diabetes Advisory Committee, Children's Hospital of Pittsburgh
- National Diabetes Data Group
- Data and Safety Monitoring Board, DirectNet, NIH
- Abstract reviewer, scientific sessions, International Society for Pediatric and Adolescent Diabetes
- Abstract reviewer, scientific sessions, Pediatric Academic Societies

EDITORSHIPS

- Associate editor, *Pediatric Diabetes*
- Associate editor, *Diabetes in America*, third edition
- International Society for Pediatric and Adolescent Diabetes e-learning

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- ADA
- Endocrine Society
- PES
- Society for Pediatric Research
- International Society of Pediatric and Adolescent Diabetes
- European Association for the Study of Diabetes
- American Pediatric Society
- Juvenile Diabetes Research Federation

HONORS

- Best Doctors, *Pittsburgh Magazine*, 2017
- *Best Doctors in America*, Woodward/White, Inc.

H. Henry Dong, PhD

RESEARCH

H. Henry Dong is conducting research focusing on insulin signaling in glucose and lipid metabolism and beta-cell function in obesity and type 2 diabetes.

Mechanism of Beta-Cell Compensation and Beta-Cell Failure. Type 2 diabetes results from beta-cell failure, culminating in the inability of cells to compensate for insulin resistance in at-risk subjects with obesity. Beta-cell compensation is an adaptive mechanism by which cells increase insulin secretion to overcome insulin resistance or oxidative stress for maintaining euglycemia in obesity. Failure of beta cells to compensate for insulin resistance or oxidative stress contributes to insulin insufficiency and overt diabetes. Likewise, beta-cell compensation, accompanied by increased insulin synthesis and secretion, occurs in women during pregnancy. Impaired beta-cell compensation

predisposes at-risk subjects to develop gestational diabetes. Dong's research is positioned to characterize the genetic factor(s) responsible for coupling beta-cell compensation with nutrient signals to elucidate the underlying mechanism of beta-cell failure in diabetes. His goal is to delineate the insulin-Akt/PKB-FoxO pathway in regulating beta-cell function and mass in obesity and type 2 diabetes. The project is funded by NIH grant R01 NIDDK DK098437.

Pathophysiology of Insulin Resistance and Diabetic Dyslipidemia. Hypertriglyceridemia is a hallmark of metabolic syndrome and is characterized by a triad plasma lipid profile (i.e., increased triglyceride and low-density lipoprotein levels and decreased high-density lipoprotein levels). Due to its proatherogenic potential, hypertriglyceridemia is considered an independent risk factor for coronary artery disease. The pathophysiology of hypertriglyceridemia is incompletely understood. Its close association with adiposity and type 2 diabetes implicates insulin resistance as a causative factor in the development of hypertriglyceridemia. However, factors that mechanistically link insulin resistance to the pathogenesis of hypertriglyceridemia are incompletely characterized. Dong's group has identified FoxO1 as a key player in regulating triglyceride metabolism. FoxO1 regulates triglyceride metabolism via apolipoprotein C-III and microsomal triglyceride transfer protein—two rate-limiting steps in very-low-density lipoprotein (VLDL)-triglyceride hydrolysis and VLDL-triglyceride secretion. This research is funded by NIH grants R01 DK066301 and R01 DK087764. For clinical translation, Dong's team has uncovered two lead compounds via screening 540,000 compounds for FoxO1 inhibitors. His laboratory is poised to validate FoxO1 as a potential therapeutic target for improving lipid metabolism and ameliorating hypertriglyceridemia in obesity and diabetes, with the NIH pending grant 1R01DK100312.

Dong has also received the ADA's Career Development Award.

ADVISORY COMMITTEE MEMBERSHIPS

- Standing member, Cellular Aspects of Diabetes and Obesity Study Section, NIDDK, NIH, 2015–2019
- Standing member, Research Administrative Committee of Children's Hospital of Pittsburgh

EDITORSHIPS

- Editorial Board, *Journal of Biological Chemistry*
- Editorial Board, *Journal of Diabetes and Its Complications*
- Editorial Board, *World Journal of Gastrointestinal Pathophysiology*
- Editorial Board, *Frontiers*
- Editorial Board, *Molecular Metabolism*
- Editorial Board, *Journal of Geriatric Cardiology*
- Editorial Board, *Immunology, Endocrinology, and Metabolic Agents*

MAJOR LECTURESHIPS AND SEMINARS

- "FoxO1 in Insulin Action and Lipid Metabolism," University of California, Berkeley, San Francisco, Calif., April 2017
- "FoxO1 in Insulin Resistance and Diabetic Dyslipidemia" A&M University, College Station, Texas, April 2017
- "FoxO1 in Beta-Cell Mass and Function Regulation" Vanderbilt University, Nashville, Tenn., January 2017
- "What Causes Beta-Cell Failure in Type 2 Diabetes?" University of New Mexico, Albuquerque, N.M., September 2016

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Heart Association
- ADA
- New York Academy of Sciences
- American Society of Gene Therapy

Oscar Escobar, MD

RESEARCH

As director of the Growth Center, Oscar Escobar's primary focus is on his clinical research regarding the treatment of growth disorders in children.

National Norditropin Registry. The primary goal of this study is to evaluate the long-term safety and efficacy of Norditropin. The growth-hormone study is based on data collected in an observational setting. The study came to an end in December 2016. Escobar served as local principal investigator.

Central Precocious Puberty Registry: A Multicenter, Observational Study of Pediatric Females With Central Precocious Puberty Receiving Supprelin LA. The primary goal of this study is to categorize the recovery of the hypothalamic-pituitary-gonadal axis after discontinuation of treatment with Supprelin LA, an implantable gonadotropin-releasing hormone analog. The study is based on data collected in an observational setting. Escobar is local principal investigator.

The VELOCITY Clinical Study–14VR4: Versartis Long-Acting Growth Hormone in Children with GH Deficiency. This study is a phase 3, randomized, one-year, open-label, multicenter, noninferiority trial in prepubertal children

with GH deficiency. The primary goal is to assess the efficacy and safety of VRS-317 in prepubertal children with GH deficiency compared to daily GH. It is sponsored by Versartis, Inc.; Escobar is the local principal investigator.

The VISTA Study–13VR3: Vesartris Long-Acting Growth Hormone Somavaratan in Children with GH Deficiency. This is an open-label, long-term safety study of long-acting human growth hormone Somavaratan (VRS-317) in prepubertal children with GH deficiency. It is sponsored by Versartis, Inc.; Escobar is local principal investigator.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Endocrine Society
- ADA
- Colombian Association of Endocrinology
- Colombian Association of Pediatric Endocrinology
- PES

HONORS

- *America's Top Doctors*, Castle Connolly Medical, Ltd., 2017
- Best Doctors, *Pittsburgh Magazine*, 2017

Amanda Flint, MD

RESEARCH

Amanda Flint's research focuses on type 2 diabetes.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Endocrine Society
- PES

Luigi Garibaldi, MD

RESEARCH

Luigi Garibaldi is the local co-principal investigator in the following data-collection studies.

- National Norditropin Registry, sponsored by Novo Nordisk
- Central Precocious Puberty Registry, sponsored by ENDO Pharmaceuticals
- VELOCITY study, sponsored by Versartis Pharmaceuticals
- VISTA study

ADVISORY COMMITTEE MEMBERSHIPS

- Diabetes Advisory Committee, Children's Hospital of Pittsburgh
- Institutional Data and Safety Monitoring Board, University of Pittsburgh
- 9A leadership group, Division of Pediatric Endocrinology, Diabetes, and Metabolism

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Endocrine Society
- PES
- Society for Pediatric Research

HONORS

- *America's Top Doctors*, Castle Connolly Medical, Ltd., 2017
- Best Doctors, *Pittsburgh Magazine*, 2017

Zhenwei Gong, PhD

RESEARCH

Zhenwei Gong's major research interest is the role of humanin in metabolic disorders, with emphasis on the effects of humanin on lipid metabolism and whole-body glucose homeostasis. Humanin improves whole-body glucose homeostasis by regulating insulin sensitivity and increasing glucose-stimulated insulin secretion from the beta cells. Using a high-fat, diet-induced obesity mouse model, he has demonstrated that treatment with humanin decreases weight gain, visceral fat, and hepatic

triglyceride accumulation. The decrease in hepatic triglyceride accumulation is due to induced activity of hepatic microsomal triglyceride transfer protein and increased hepatic triglyceride secretion. In addition, intracerebroventricular infusion of humanin acutely increases triglyceride secretion from the liver, whereas vagotomy blocks the effects of intracerebroventricular humanin on hepatic triglyceride secretion. Furthermore, vagotomy also blocks hepatic triglyceride secretion induced by intravenous administration of humanin. Finally, he has shown that the central effects of humanin on hepatic triglyceride flux are mediated through the melanocortin system. He continues to define the roles of humanin in central regulation of lipid metabolism.

Gong is also interested in studying the effects of humanin in cardiovascular disease. While studying the mechanisms by which humanin protects cardiomyocytes from stress-induced cell death, he found that humanin activates chaperone-mediated autophagy (CMA). He demonstrated that this effect occurs in cardiomyocytes, fibroblasts, and neuronal cells. Treatment with humanin increases long-lived protein turnover and prevents the cells from oxidative stress–induced cell death. Humanin lost its protective effects on these cells with knockdown of lysosome-associated membrane glycoprotein 2A, suggesting that the protective effect is CMA dependent. His current focus is to identify the underlying mechanisms by which humanin activates CMA.

The third ongoing project for Gong is to study the role of a tumor suppressor, Absent in Melanoma (AIM2), in energy homeostasis. Gong has found that knockout of AIM2 in mice induces spontaneous obesity and insulin resistance. This is at least partially due to the increased adipogenesis in WAT and BAT dysfunction–induced decrease in thermogenesis and subsequently reduced energy expenditure. Notably, he also has found that the effects of AIM2 in energy homeostasis are independent of its role in the inflammasome, as caspase1 activity and serum levels of IL-1β are not changed in AIM2-/- mice under physiological conditions when AIM2 inflammasome is inactivated. He is investigating the potential mechanisms by which AIM2 regulates energy metabolism and insulin sensitivity.

EDITORSHIPS

- Review editor, *Frontiers in Pediatric Endocrinology*

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- ADA

LECTURESHIPS AND SEMINARS

- "Role of Humanin in Autophagy," special seminar in the School of Life Science, Northeast Normal University, Changchun, China, October 2016
- "Mitochondria Signaling, Cellular Homeostasis," department seminar, Dalian Polytechnic University, Liaoning, China, November 2016

Nursen Gurtunca, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- South African Medical Society
- American Pediatric Society
- PES
- Endocrine Society

Kara Hughan, MD

RESEARCH

Kara Hughan's translational research is focused on metabolic and hormonal factors associated with cardiometabolic disease.

Nitrite Modulation of Hypertension, Platelet Activation, and Endothelial and Mitochondrial Function. This translational study aims to examine the effects of chronic oral nitrite therapy on blood pressure, endothelial function, and insulin sensitivity and whether it modulates mitochondrial energetics, oxidative stress, and redox signaling in platelets and skeletal muscle. The study is funded by NIH K23 NHLBI grant HL124051-01A1 and the McKamish Family Fund. Hughan is principal investigator.

An Open-Label Study of Oral Nitrite in Adults With Metabolic Syndrome and Hypertension. This study aims to investigate the effects of oral inorganic nitrite on the cardiometabolic and hormonal disturbances in metabolic syndrome and hypertension, targeting a population of overweight/obese adults with metabolic syndrome and hypertension at risk to develop insulin resistance and endothelial dysfunction. The study is supported by a University of Pittsburgh Clinical and Translational Science Institute award; the University of Pittsburgh Vascular Medicine Institute; and McKamish, Inc. Hughan is principal investigator.

Postprandial Glycemia in the Free-Living Condition and Cardiometabolic Risk in Obese Youth. This study aims to assess the relationship between postprandial glycemia in free-living conditions via continuous glucose monitoring and cardiometabolic risk in obese youth. Hughan is principal investigator.

Treatment Options for Type 2 Diabetes in Adolescents and Youth 2 (TODAY2). This is a multicenter, cooperative, NIH-funded trial regarding the treatment of youth with type 2 diabetes. It continues to follow the original TODAY study subjects to understand the persistence of the effects of the different treatment regimens used in TODAY, to track the continued evolution of beta-cell function, and to describe the development of vascular complications and risk factors for complications. Hughan is co-investigator.

Nitrite Benefits to Mediate Fatigability in Older HFpEF Patients. This National Institute on Aging/NIH-funded, randomized, controlled, double-blinded trial of oral nitrite therapy in older (> 70 years) HFpEF patients proposes there are intrinsic physiological components of HFpEF pathophysiology that predispose to fatigability, a concept defined specifically as a deterioration in function over time (performance fatigability) and subjective tiring from an exercise stimulus (perceived fatigability). The study will evaluate the pleiotropic benefits of oral inorganic nitrite, including enhanced performance of skeletal muscle (strength and metabolism) and vasomotor responses (systemic and pulmonary arterial). Hughan is co-investigator.

Cystic Fibrosis (CF) Foundation's EnVision CF: Emerging Leaders in CF Endocrinology Award. This award, funded by the CF Foundation, trains Hughan and 15 other emerging U.S. endocrinologists caring for patients with CF and related endocrine conditions. In addition to Hughan providing designated pediatric endocrine-related patient care within the division's CF Care Center, this proposal includes the opportunity to submit CF and related endocrine disorder research projects. Hughan is principal investigator.

A Randomized, Double-Blind Study With an Open-Label Extension Comparing the Effect of Once-Weekly Dulaglutide With Placebo in Pediatric Patients With Type 2 Diabetes Mellitus (Assessment of Weekly Administration of LY2189265 in Diabetes-PEDiatric Study). This phase III study, sponsored by Eli Lilly and Company, aims to test the hypothesis that dulaglutide (0.75 mg and 1.5 mg, pooled) given subcutaneously once a week for 26 weeks to children and adolescents with type 2 diabetes mellitus who have inadequate glycemic control, despite diet and exercise, with or without metformin or basal insulin, is superior to placebo in the treatment of type 2 diabetes mellitus, as measured by baseline to week 26 change in hemoglobin A1c. Hughan is co-investigator.

An Open-Label, Randomized, Multicenter, Single-Dose, Parallel-Group Trial to Evaluate Pharmacokinetics and Pharmacodynamics of Empagliflozin in Children and Adolescents From 10 to Less Than 18 Years of Age With Type 2 Diabetes Mellitus. In this phase I trial sponsored by Boehringer-Ingelheim Pharmaceuticals, Inc., Hughan completed eight subjects at Children's Hospital out of the 27 subjects completed internationally. Hughan is site principal investigator.

MAJOR LECTURESHIPS AND SEMINARS

- "Nitrite Modulation of Hypertension, Insulin Sensitivity, and Endothelial Function," Ninth Annual Children's Hospital of Pittsburgh Child Health Research Center K12 Retreat, Pittsburgh, Pa., April 2016
- "Pediatric Endocrine Emergencies," Allegheny Health Network/Drexel University College of Medicine, Emergency Medicine Residency Lectureship, Pittsburgh, Pa., August 2016
- "Mitochondrial Function in Cystic Fibrosis," EnVision webinar program broadcast to EnVision CF trainees and mentors, Cystic Fibrosis Foundation, November 2016
- "Diabetes Through the Ages—Special Consideration in the Care of Infants and Toddlers With Diabetes," American Association of Diabetes Educators Western Pennsylvania Local Networking Group, Pittsburgh, Pa., November 2016
- "Mitochondrial Function in Cystic Fibrosis," Pediatric Endocrine Research Conference, Children's Hospital, Pittsburgh, Pa., February 2017
- "Oral Nitrite Modulation of Hypertension, Endothelial Function, and Insulin Sensitivity in Humans," Nutrition Center and Integrated Health Seminar Series, Division of Gastroenterology, Hepatology, and Nutrition, Children's Hospital of Philadelphia, Philadelphia, Pa., March 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- PES
- ADA
- Endocrine Society
- Vascular Medicine Institute, University of Pittsburgh School of Medicine
- Nitric Oxide Society

ADVISORY COMMITTEE MEMBERSHIPS

- Children's Hospital Pediatric Endocrine Fellowship Curriculum Committee, Accreditation Council for Graduate Medical Education
- Children's Hospital Pediatric Endocrine Fellowship Interview Committee, Accreditation Council for Graduate Medical Education
- Comorbidity Assessment Committee, TODAY2

HONORS

- EnVision CF: Emerging Leaders in CF Endocrinology Program, Cystic Fibrosis Foundation

Heba Ismail, MBBCh, MSc, PhD

Heba Ismail joined the division in December 2017 as the new clinical director of the diabetes program.

RESEARCH

Ismail's research focuses on the pathogenesis and treatment of type 1 diabetes mellitus, with emphasis on the use of technology in the management of type 1 diabetes.

Nutrition Education Through Mobile Gaming for Young Patients With Type 1 Diabetes. This is a study to assess the effect of using a neuropsychology-based gaming application on the food choices and consumption of children with type 1 diabetes as compared to conventional, standard diet teaching. Ismail is principal investigator.

Bridges Education Innovation Project Grant: A Day in the Life of a Patient With Diabetes Mellitus: An Experiential Learning. This is a study to assess an innovative method of education using role-playing as a form of experiential learning. The goal is to familiarize pediatric trainees with diabetes technology as well as enhance a culture of empathy. Ismail is principal investigator.

TrialNet (Prediction and Prevention of Type 1 Diabetes). This is a collaborative study of 14 centers in the United States and five in Europe to plan and implement intervention strategies to maintain beta-cell function in patients with new-onset type 1 diabetes and prevent diabetes in first-degree relatives at high risk. Ismail is sub-investigator.

MAJOR LECTURESHIPS AND SEMINARS

- "Glucose and C-Peptide Secretion Patterns," endocrine grand rounds, Indiana University, October 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- ADA
- Endocrine Society
- PES

HONORS

- Young Investigator Award, TrialNet award in recognition of research efforts

Ingrid Libman, MD, PhD

RESEARCH

Ingrid Libman's research has focused on the etiology, prevention, and treatment of type 1 diabetes mellitus, with emphasis on the effect of obesity in type 1 diabetes. She directs the Diabetes Transition Program, which aims to provide the best care and to research the best ways to support and guide successful transition for young patients with diabetes mellitus. She is involved in the development and testing of age-appropriate shared decision-making tools to educate about the importance of prevention and screening for complications of diabetes. She works on numerous multicenter studies of type 1 and type 2 diabetes.

Transition to Adulthood for Adolescent Patients With Type 1 Diabetes Mellitus. This three-year project funded by the Children's Hospital Foundation David Paul Diabetes Transition Care Research Initiative will develop and test a series of educational modules for patients and families as the patient transitions from pediatric to adult care and management of type 1 diabetes. Libman is principal investigator.

Availability of a Program to Screen for Retinopathy in Children With Type 1 Diabetes. Does It Improve Compliance? The overall goal of this project, funded by the Beckwith Foundation, is to design, integrate, and evaluate a shared decision-making approach, including appropriate tools, to engage children with type 1 diabetes in understanding the importance of yearly screening for diabetic retinopathy and what can be done to prevent or delay the complication. Libman is co-principal investigator.

Are Current Screening Guidelines for Complications in Youth With Type 1 Diabetes Appropriate? This study is funded by the University Partnership Program Academic Foundation. Libman is principal investigator.

Novel Biomarkers for Early Diabetic Nephropathy in Children With Type 1 Diabetes. This study is funded by the Cochrane Weber Endowment. Libman is co-investigator.

A Shared Decision-Making Approach to Engage Youth With Type 1 Diabetes in Cardiovascular Disease Prevention. This study, funded by the Cochrane Weber Endowment, aims to design, integrate, and evaluate a shared decision-making approach, including appropriate tools, to engage children with type 1 diabetes in understanding the importance of yearly screening for cardiovascular disease risk factors and what can be done to prevent or delay this complication. Libman is co-principal investigator.

A Day in the Life of a Patient With Diabetes Mellitus: An Experiential Learning. The objective of this proposal, funded by the Bridges Education Innovation Award, is to have residents and endocrine fellows "experience" living with diabetes, using insulin pumps and glucose testing to familiarize themselves with the different clinical scenarios and diabetes technology and ensure a culture of empathy. Libman is co-investigator.

Type 1 Diabetes Exchange Project. This multicenter study funded by the Leona M. and Harry B. Helmsley Charitable Trust aims to create a registry and biobank of individuals with type 1 diabetes mellitus to address pertinent clinical issues and conduct exploratory and hypothesis-generating analyses. Libman is principal investigator at the Pittsburgh center.

Research on Emerging Adults Changing Health. The goal of this NIH-funded research in collaboration with Carnegie Mellon University is to examine the impact of insulin-dependent diabetes on quality of life over the transition from adolescence into adulthood as compared to nondiabetic individuals. The study will concentrate on the follow-up of graduates of the diabetes program for the analyses of psychosocial behaviors and risk factors and the evaluation of their relationship with metabolic control and diabetes-related complications. Libman is co-investigator.

TrialNet (Prediction and Prevention of Type 1 Diabetes). This is a collaborative study of 14 centers in the United States and five in Europe to plan and implement intervention strategies to maintain beta-cell function in patients with new-onset type 1 diabetes and prevent diabetes in first-degree relatives at high risk. Libman is co-investigator.

SNAIL Study. The aim of this multicenter study, funded by JDRF, is to identify and characterize "slow progressors" from several longitudinal studies of type 1 diabetes. Libman is co-investigator.

TODAY2. This is a multicenter, cooperative, NIH-funded trial regarding treatment of youth with type 2 diabetes. It continues to follow the original TODAY study subjects to understand the persistence of the effects of the different treatment regimens used in TODAY, to track the continued evolution of beta-cell function, and to describe the development of vascular complications and risk factors for complications. Libman is co-investigator.

ADVISORY COMMITTEE MEMBERSHIPS

- Youth/pediatric chair, Scientific Sessions Planning Committee, ADA
- Chair, Diabetes in Youth Interest Group, ADA
- Member, Organizing Committee, International Meeting of Pediatric Endocrinology, to be held in Buenos Aires, Argentina, 2021
- Member, Training Council, PES
- Member, Diabetes Special Interest Group, PES
- Invited member, Eunice Kennedy Shriver National Institute of Child Health and Human Development's Initiative to Advance Pediatric Therapeutics, NIH
- Comorbidity Assessment Committee, TODAY study
- Ancillary Studies Committee, TODAY study
- Member and reviewer, Institutional Review Board, University of Pittsburgh
- Member, Diversity Committee, Institute of Clinical Research Education, Clinical and Translational Science Institute, University of Pittsburgh

EDITORSHIPS

- Editorial Board (international), *Latin ADA Journal*
- International Editorial Board, *Revista Medica de Rosario*

MAJOR LECTURESHIPS AND SEMINARS

- "Double Diabetes or Type 1.5: How Frequent Is It and How to Treat?" invited speaker, International Society for Diabetes in Adolescents and Youth 2016 Congress, Valencia, Spain, October 2016
- "Type 2 Diabetes Mellitus in Children and Youth: Myth or Reality?" invited speaker, Latin ADA Congress, Bogota, Colombia, November 2016
- "Prevention and Treatment of Type 1 Diabetes in Youth: What Is New?" invited speaker, Latin ADA Congress, Bogota, Colombia, November 2016

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- ADA
- Latin ADA
- European Association for the Study of Diabetes
- International Society for Pediatric and Adolescent Diabetes
- Society for Pediatric Research
- PES

HONORS

- Best Doctors, *Pittsburgh Magazine*, 2017
- *Best Doctors in America*, Woodward/White, Inc., 2017
- Outstanding Patient Award, Children's Hospital, 2016
- *America's Top Doctors*, Castle Connolly Medical, Ltd., 2017

Pushpa Viswanathan, MD

RESEARCH

As codirector of the Thyroid Center, Pushpa Viswanathan's primary clinical research focus is treatment of thyroid disorders and thyroid cancers in children. Viswanathan's current research involves clinical and prognostic evaluation of pediatric thyroid disease.

ADVISORY COMMITTEE MEMBERSHIPS

- Co-chair, Multidisciplinary Thyroid Center, Children's Hospital of Pittsburgh
- Patient Experience Outpatient Committee, Children's Hospital of Pittsburgh

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Endocrine Society
- American Academy of Pediatrics
- ADA

Selma F. Witchel, MD

RESEARCH

Selma Witchel's research projects address androgen excess, glucocorticoid receptors, and gender questioning in children and adolescents.

Persistent Symptoms of Hyperandrogenism in Girls With a History of Premature Adrenarche or Adolescent Hyperandrogenism. The goal of this project is to ascertain outcome data for girls with a history of premature adrenarche or adolescent hyperandrogenism to test the hypothesis that these girls have an increased risk to develop polycystic ovary syndrome (PCOS). The project has been expanded to include investigation of the novel bioactive adrenal steroid hormones in normal adrenarche, premature adrenarche, adolescent hyperandrogenism, and congenital adrenal hyperplasia.

Substance Use Disparities Among Transgender Youth. The overarching goal of this study is to systematically examine substance use and associated health disparities among transgender and gender-questioning youth.

Noninvasive Maximization of Fertility in a Peripubertal Population of Patients With Klinefelter Syndrome. The goal of this pilot project funded by the Clinical and Translational Science Institute is to determine whether sperm can be detected in the urine or in semen samples of adolescent or young adult patients, as well as whether there is a correlation between the presence of sperm and the physical/endocrine end points routinely assessed in these patients.

Role of Glucocorticoids in Prenatal Brain Development. The goal of this project is to investigate the developmental consequences of premature glucocorticoid exposure on the developing brain. Initial studies have found that dexamethasone exposure alters the development trajectory of the brain.

ADVISORY COMMITTEE MEMBERSHIPS

- Research Advisory Committee, Children's Hospital of Pittsburgh
- Chair, Disorders of Sexual Differentiation Committee, Children's Hospital of Pittsburgh
- Co-chair, Multidisciplinary Thyroid Center, Children's Hospital of Pittsburgh
- CARES Foundation Advisory Board
- Intern Selection Committee, Children's Hospital of Pittsburgh
- Co-chair, Publications Committee, Androgen Excess and PCOS Society
- Chair, Education Committee, PES
- Program chair, Androgen Excess–PCOS Society annual meeting, Lorne, Australia, November 2016

EDITORSHIPS

- Editorial Board, *Journal of Clinical Endocrinology and Metabolism*

MAJOR LECTURESHIPS AND SEMINARS

- "Biology of Gender," Midwest PES meeting, Chicago, Ill., March 2016
- "Sex Steroids and Gender Identity—The Endocrinologists' Perspective," Pittsburgh TransPride Conference, Pittsburgh, Pa., September 2016
- "Biology of Gender," pediatric endocrinology grand rounds, University of Michigan, Ann Arbor, Mich., December 2016
- "Premature Adrenarche and PCOS," Endocrinology Research Conference, University of Michigan, Ann Arbor, Mich., December 2016

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Endocrine Society
- PES
- Society for Pediatric Research
- American Academy of Pediatrics
- ADA
- Androgen Excess and PCOS Society
- European Society for Pediatric Endocrinology

HONORS

- Best Doctors, *Pittsburgh Magazine*, 2017
- *America's Top Doctors*, Castle Connolly Medical, Ltd.

2015

Forno E, Han YY, **Muzumdar R**, Celedon JC. Insulin resistance, metabolic syndrome, and lung function in U.S. adolescents with and without asthma. *J Allergy Clin Immunol*. 2015;136:304-11.

Katari S, Wood-Trageser MA, Jiang H, Kalynchuk E, **Muzumdar R**, Yatsenko SA, Rajkovic A. Novel inactivating mutation of the FSH receptor in two siblings of Indian origin with premature ovarian failure. *J Clin Endocrinol Metab*. 2015;100(6):2154-7.

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DIVISION OF PULMONARY MEDICINE, ALLERGY, AND IMMUNOLOGY

Mission

The Division of Pulmonary Medicine, Allergy, and Immunology is committed to serving the community through excellence in patient care, teaching, and research. The division provides outstanding comprehensive care to children with acute or chronic pulmonary disorders, allergic diseases, cystic fibrosis, immune deficiencies, airway lesions, and sleep disorders. The division is dedicated to improving patients' health through research, including basic science, and translational and clinical investigations related to pulmonary and allergic diseases. Educational goals include teaching patients, their families and caregivers, medical students, residents, fellows, physicians, and other health care professionals about the physiology, pathophysiology, and treatment of lung disorders and allergic diseases.

FACULTY AND STAFF

Juan C. Celedón, MD, DrPH
Niels K. Jerne Professor of Pediatrics
Professor of Internal Medicine, Epidemiology, and Human Genetics
Division Chief, Pediatric Pulmonary Medicine, Allergy, and Immunology
Director, Pediatric Asthma Center, Children's Hospital of Pittsburgh of UPMC

John F. Alcorn, PhD
Associate Professor of Pediatrics

Nadia Boutaoui, PhD
Assistant Professor of Pediatrics
Laboratory Director

Sangeeta S. Chakravorty, MD
Associate Professor of Pediatrics
Director, Pediatric Sleep Evaluation Center

Wei Chen, PhD
Associate Professor of Pediatrics, Biostatistics, and Human Genetics

Hey Chong, MD, PhD
Assistant Professor of Pediatrics
Clinical Director, Allergy and Immunology

Mark Dovey, MD
Associate Professor of Pediatrics

Jonathan D. Finder, MD
Professor of Pediatrics
Director, Outreach Pulmonary Clinics

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Program Director, Allergy and Immunology
Fellowship Director, Food Allergy Research and Education (FARE) Center of Excellence

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Professor of Pediatrics
Director, Pediatric Flexible Bronchoscopy Service
Director, Pediatric Pulmonary Transplantation Program

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Program Director, Allergy and Immunology Fellowship

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Codirector, Pediatric Sleep Program

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Director Emeritus, Antonio J. and Janet Palumbo Cystic Fibrosis Center

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Associate Professor of Pediatrics
Director, Antonio J. and Janet Palumbo Cystic Fibrosis Center
Medical Director, Pulmonary Function Laboratory
Medical Director, Exercise Laboratory

John Broyles, CRNP

Lori Holt, CRNP

OVERVIEW OF DIVISION

The Division of Pulmonary Medicine, Allergy, and Immunology at the Children's Hospital of Pittsburgh was ranked fifth in the country in the annual listing prepared by *U.S. News & World Report*. The division faculty was pleased to welcome Mark Dovey as an associate professor of pediatrics in March 2017. Hey Chong was appointed clinical director of allergy and immunology in July 2016. Wei Chen was promoted to associate professor of pediatrics, biostatistics, and human genetics in February 2017.

The Pediatric Pulmonary Medicine Fellowship Program trained six fellows during fiscal year 2017 (FY17). In June 2017, Sylvia Szentpetery and Sandeep Puranik graduated from the program. Upon graduation, Szentpetery accepted a position as an assistant professor of pediatrics at the Medical University of South Carolina in Charleston, S.C., and Puranik accepted a position as an assistant professor of pediatrics at the Indiana University School of Medicine in Indianapolis, Ind. In July 2017, two new fellows, Jeremy Landeo and Erica Stevens, began training in the division.

The Allergy and Immunology Fellowship Program trained four fellows during FY17. June 2017 saw the graduations of Shari Montandon and Stacy Rosenberg. Montandon joined a private practice in Coeur d'Alene, Idaho. Rosenberg joined UPMC as a clinical instructor in pediatrics. In July 2017, two new fellows, Leanna-Maire Sims and Kara Coffey, began training in the division.

CLINICAL ACTIVITIES

The division provides comprehensive services to children with a variety of respiratory tract and allergic diseases. The division's Cystic Fibrosis Center has been honored by the Cystic Fibrosis Foundation as a benchmark center. The division actively participates in the pediatric lung transplantation program and provides consultative services for patients with asthma, recurrent pneumonia, bronchopulmonary dysplasia, neuromuscular disorders, airway lesions, food allergies, allergic rhinitis, atopic dermatitis, immune deficiencies, and other respiratory and allergic diseases. Under the direction of Sangeeta Chakravorty and Hiren Muzumdar, the division has developed a comprehensive sleep program for children. The infant pulmonary function laboratory is the only program of its kind in Western Pennsylvania.

RESEARCH AND OTHER SCHOLARLY ACTIVITIES

Over the past fiscal year, the division received additional funding from the National Institutes of Health (NIH), including an R01 grant to study the effects of psychosocial stress on response to inhaled corticosteroids in Puerto Rican children, an R01 to study the mechanisms of secondary *Staphylococcus Aureus* pneumonia after influenza infection, and a T32 training grant in pediatric pulmonary medicine. Moreover, the division has added new study sites (at Boston Children's Hospital and Washington University in Saint Louis) to the ongoing U01-funded clinical trial of vitamin D supplementation to prevent severe asthma exacerbations in children.

Juan C. Celedón, MD, DrPH

RESEARCH

Juan C. Celedón's research is focused on identifying genetic and epigenetic factors and early-life environmental exposures that influence the pathogenesis of asthma and chronic obstructive pulmonary disease (COPD) in general and among ethnic minorities in particular.

STUDY SECTIONS

- Board of Scientific Counselors, National Institute of Environmental Health Sciences

ADVISORY COMMITTEE MEMBERSHIPS

- Chair, Planning Committee, Environmental and Occupational Health Assembly, American Thoracic Society (ATS)
- Chair, ATS and European Respiratory Society Workshop/Task Force on Respiratory Health in Migrant and Refugee Populations
- Elected member (currently secretary-treasurer), Executive Committee, ATS

EDITORSHIPS

- Editorial Board, *American Journal of Respiratory and Critical Care Medicine*
- Editorial Board, *Thorax*
- Associate editor, *American Journal of Respiratory and Critical Care Medicine*

Juan C. Celedón, MD, DrPH
Division Chief, Pulmonary Medicine, Allergy, and Immunology

MAJOR LECTURESHIPS AND SEMINARS

- "Can We Predict Severe Asthma Exacerbations?" Congress of the Latin American Thoracic Association, Santiago, Chile, 2016
- "Genetics and Epigenetics of Asthma in Latino Americans," Congress of the Latin American Thoracic Association, Santiago, Chile, 2016
- "Genetics and Epigenetics of Stress and Asthma," World Allergy Organization international scientific conference, Jerusalem, Israel, 2016
- "Solving the Puzzle of Asthma Disparities," annual symposium of the Saban Research Institute of Children's Hospital of Los Angeles, Los Angeles, Calif., 2017

- "Asthma in Hispanics: An Update," Partners Asthma grand rounds, Brigham and Women's Hospital and Harvard Medical School, Boston, Mass., 2017
- "Epigenetics of Asthma," annual Pneumo Trieste scientific conference, Trieste, Italy, 2017
- "COPD in Hispanics," ATS/European Respiratory Society Workshop on Respiratory Health in Migrant and Refugee Populations, ATS international conference, Washington, D.C., May 2017
- "Genetics and Health Equality," ATS workshop on "High-Throughput Sequencing in Respiratory, Critical Care Medicine, and Sleep Research," ATS international conference, Washington, D.C., May 2017
- "Con: Vitamin D Supplementation Should Be Used in Asthma Management," symposium on "Pro/Con Debate in Pediatric Asthma," ATS international conference, Washington, D.C., May 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Fellow, American College of Physicians
- Fellow, American College of Chest Physicians
- ATS
- Fellow, American Academy of Allergy, Asthma, and Immunology
- American Society of Human Genetics
- Federation of American Societies for Experimental Biology

HONORS

- Elected member, American Society for Clinical Investigation, 2010 to the present
- Elected member, American Pediatric Society, 2013 to the present
- Elected member, Association of American Physicians, 2015 to the present
- John M. Peters Award for outstanding contributions to environmental and population health, ATS Environmental, Occupational, and Population Health Assembly, 2017
- Elected to the ATS Executive Committee (first faculty member of the University of Pittsburgh and first Latino American ever elected to this position since the inception of ATS in 1905), 2017–2022

John F. Alcorn, PhD

RESEARCH

John F. Alcorn studies the role of type 17 immunity in viral and bacterial pneumonia. His studies have focused on influenza A infection and suppression of type 17 immunity against secondary staphylococcal infection. He has studied the mechanisms of asthma and allergic airway disease, with an emphasis on the role of type 17 cellular immunology. His studies have suggested that disease induced by Th17 cells is steroid resistant and may represent a model for steroid-insensitive asthma.

STUDY SECTIONS

- Immunity and Host Defense Study Section, NIH
- Chair, FP-Flu Panel, Department of Defense
- P01 Special Emphasis Panel, National Institute of Allergy and Infectious Diseases, NIH
- F13 Microbiology F Fellowship Study Section, NIH
- Immunity and Host Defense Overflow Viral Immunity Topics Study Section, NIH

EDITORSHIPS

- Associate editor, *Journal of Immunology*
- Associate editor, *American Journal of Respiratory Cell and Molecular Biology*

MAJOR LECTURESHIPS AND SEMINARS

- "Influenza Infection and Susceptibility to Secondary Bacterial Pneumonia," Montana State University, Bozeman, Mont., March 2015
- "Influenza Infection and Susceptibility to Secondary Bacterial Pneumonia," University of Georgia, Athens, Ga., April 2015
- "Th17 Cells in Lung Immunity and Allergy," ATS international meeting, Denver, Colo., May 2015
- "Influenza Infection and Susceptibility to Secondary Bacterial Pneumonia," MedImmune, Gaithersburg, Md., October 2015

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- ATS
- American Association of Immunologists

HONORS

- Parker B. Francis Jo Rae Wright Award for Scientific Excellence, Parker B. Francis Fellowship Program and the Francis Family Foundation, May 2015
- Elected member, Society for Pediatric Research, 2016 to the present

Nadia Boutaoui, PhD

RESEARCH

Nadia Boutaoui directs the division's genetics and epigenetics laboratory, which studies the impact of heredity and environmental exposures in early life on asthma, particularly in minorities. She trains junior faculty and fellows on genetic and epigenetic laboratory methods to study asthma.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- ATS
- American Society of Human Genetics

Sangeeta S. Chakravorty, MD

RESEARCH

Sangeeta Chakravorty has established a Sleep Disorders Registry and has enrolled children with sleep disorders from those referred to the pediatric sleep evaluation clinic. She has collaborated with Jeffrey Simons and Allison Tobey (otolaryngologist) on INSPIRE, a proposed hypoglossal nerve stimulator implant in adolescents with trisomy 21.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- National Sleep Subcommittee, Autism Treatment Network, 2008 to the present
- American Academy of Sleep Medicine, 1999 to the present

MAJOR LECTURESHIPS AND SEMINARS

- "Pharmacological Management of Narcolepsy," invited speaker, Annual Respiratory Care Conference, Cranberry, Pa., September 2016
- "Polysomnography 101," Combined ENT/ Pulmonology Airway Conference, August 2017

HONORS

- Division chief, sleep medicine, Professional Medical Staff Society, Children's Hospital of Pittsburgh, June 2017 to the present

Wei Chen, PhD

RESEARCH

Wei Chen's research focuses on statistical methods and analysis for genetic and genomic studies of complex diseases. He has developed novel methods for genotype calling in next-generation sequencing and has studied genetic risk factors for COPD, asthma, and age-related macular degeneration. In addition, he has studied epigenetics and its interaction with environment on asthma. He has two active R01s from NIH and several collaborative grants from NIH and multiple foundations.

EDITORSHIPS

- Ad hoc reviewer, *Nature Genetics*
- Ad hoc reviewer, *Nature Communications*
- Ad hoc reviewer, *American Journal of Human Genetics*
- Ad hoc reviewer, *PLOS Genetics*
- Ad hoc reviewer, *Bioinformatics*
- Ad hoc reviewer, *BMC Genetics*
- Ad hoc reviewer, *PLOS ONE*
- Ad hoc reviewer, *BMC Medical Genetics*
- Ad hoc reviewer, *Molecular Vision*
- Ad hoc reviewer, *Acta Ophthalmologica*
- Ad hoc reviewer, *Human Heredity*
- Ad hoc reviewer, *Methods*
- Ad hoc reviewer, *Briefings in Bioinformatics*
- Ad hoc reviewer, *BMC Bioinformatics*
- Statistical Advisory Board, *PLOS ONE*
- Guest editor, *PLOS Genetics*

STUDY SECTION

- Children's Health Exposure Analysis Resource: Data Repository, Analysis, and Science Center (U2C), National Institute of Environmental Health Sciences, Research Triangle Park, N.C.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Society of Human Genetics
- American Statistical Association
- International Biometric Society

MAJOR LECTURESHIPS AND SEMINARS

- "Statistical Issues in Genome-Wide Association Studies of Age-Related Macular Degeneration Progression," Third Taihu International Statistics Forum, Shanghai, China, July 2016
- "AMD Genetics: Association and Prediction," annual meeting of Eastern North American Region Biometric Society, Austin, Texas, March 2016
- "Statistical Issues in Genome-Wide Association Studies of Age-Related Macular Degeneration Progression," Center for Quantitative Science, Vanderbilt University, Nashville, Tenn., May 2016
- "The Power of Statistics in Making Sense of Omics Data," Department of Pediatrics, Molecular Medicine Research Seminar, University of Pittsburgh, Pittsburgh, Pa., September 2016
- "A Novel Method for Clustering Droplet-Based Single-Cell Data," Recomb-seq, Hong Kong, China, 2017
- "DIMMS-SC: Methods for Single-Cell RNA-Seq Data," Joint Statistical Meeting, Baltimore, Md., 2017

Hey J. Chong, MD, PhD

RESEARCH

Hey J. Chong is the site principal investigator for two multicenter studies through the Primary Immunodeficiency Treatment Consortium. Study 6903 continues looking at patients treated for chronic granulomatous disease with or without transplant. Study 6904 continues looking at Wiskott-Aldrich syndrome and the role of transplantation. She is also the site principal investigator for six industry-sponsored food-tolerance clinical trials through Aimmune and DBV Technologies.

ADVISORY COMMITTEE MEMBERSHIPS

- Continuing Medical Education Committee, Clinical Immunology Society, 2014 to the present
- Fellowship Research Committee, Children's Hospital of Pittsburgh of UPMC

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Clinical Immunology Society
- American Academy of Allergy, Asthma, and Immunology

MAJOR LECTURESHIPS AND SEMINARS

- "Primary Immunodeficiency in Your Office" Children's Hospital of Pittsburgh grand rounds, Pittsburgh, Pa., September 2017
- Panel member, Seventh Annual Update in Allergy and Sino-Nasal Disorders, University of Pittsburgh School of Medicine, Pittsburgh, Pa., May 2017
- "Primary Immunodeficiency for the Intensivist," invited speaker, grand rounds, Children's Hospital Intensive Care Unit, Pittsburgh, Pa., November 2016
- "How the Clinical Laboratory Helps Identify, Diagnose, and Manage Patients With Immunodeficiency Diseases," invited speaker, Association of Medical Laboratory Immunologists meeting, Pittsburgh, Pa., August 2016
- "Immunology in Health and Disease," first-year medical course, University of Pittsburgh School of Medicine, Pittsburgh, Pa., 2013–2016

Mark Dovey, MD

RESEARCH

Mark Dovey is the site principal investigator of two multicenter clinical trials of investigational agents to treat cystic fibrosis lung disease: (1) a phase III, randomized, double-blind, placebo-controlled study of inhaled vancomycin for the treatment of persistent methicillin-resistant *Staphylococcus aureus* lung infection in cystic fibrosis patients and (2) a phase III, open-label study to evaluate the pharmacokinetics, safety, and tolerability of VX661 in combination with Ivacaftor in subjects 6 through 11 years of age with cystic fibrosis, homozygous or heterozygous for the F508delCFTR mutation.

EDITORSHIPS

- Ad hoc reviewer, *Blood (Journal of the American Society of Hematology)*
- Ad hoc reviewer, *American Journal of Respiratory and Critical Care Medicine*
- Ad hoc reviewer, *Pediatric Pulmonology*
- Ad hoc reviewer, *Journal of Pediatrics*
- Ad hoc reviewer, *American Journal of Perinatology*
- Ad hoc reviewer, *Journal of Developmental and Behavioral Pediatrics*

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- ATS

HONORS

- Top Doctor, *U.S. News & World Report*, ranked top 1% in nation, 2013–2016
- Top Doctor in Pediatric Pulmonology, *Boston Magazine*, 2015–2016

Jonathan D. Finder, MD

ADVISORY COMMITTEE MEMBERSHIPS

- Pharmacy and Therapeutics Committee, Children's Hospital of Pittsburgh, 2000 to the present
- Codirector, Medical Staff Campaign, Children's Hospital of Pittsburgh Foundation, 2001 to the present
- Pharmacy Innovative Use Committee, Children's Hospital of Pittsburgh, 2007 to the present
- Ambulatory e-Record Optimization Steering Committee, Children's Hospital of Pittsburgh, 2011 to the present
- Clinical Transformation Steering Committee, Children's Hospital of Pittsburgh, 2014 to the present
- Task Force 2019, Children's Hospital of Pittsburgh, April 2016 to the present

EDITORSHIPS

- Editorial Advisory Board, *RT Magazine*, 2004 to the present

MAJOR LECTURESHIPS AND SEMINARS

- "Respiratory Management of Patients With Neuromuscular Disorders," grand rounds, University of Pittsburgh, Department of Pediatrics, Children's Hospital of Pittsburgh, February 2016
- Creator and organizer, Respiratory Outcomes in Muscular Dystrophy, meeting of Parent Project Muscular Dystrophy, U.S. Food and Drug Administration, NIH, international research community, and industry to result in a consensus statement, April 2016

- "Respiratory Management of Muscular Dystrophy," Parent Project Muscular Dystrophy annual meeting, Orlando, Fla., June 2016
- "Respiratory Management of Duchenne Muscular Dystrophy," Muscular Dystrophy Foundation of South Africa regional workshop, Hope School, Johannesburg, South Africa, August 2016
- "Respiratory Management of Duchenne Muscular Dystrophy," Muscular Dystrophy Foundation of South Africa regional workshop, Albert Lethuli Hospital, Durban, South Africa, August 2016
- "Respiratory Management of Duchenne Muscular Dystrophy," Muscular Dystrophy Foundation of South Africa regional workshop, Red Cross Hospital, Cape Town, South Africa, September 2016
- "Respiratory Management of Duchenne Muscular Dystrophy," South African Paediatric Association Second Biennial Congress, Durban, South Africa, September 2016
- "Respiratory Management of Patients with Neuromuscular Disorders," Pennsylvania Society for Respiratory Care Western Regional Conference, Cranberry, Pa., September 2016
- "Respiratory Management of Patients with Neuromuscular Disorders," grand rounds, Department of Pediatrics, Nationwide Children's Hospital, University of Ohio, December 2016
- "Pulmonary Secrets for Pediatricians," grand rounds, Children's Hospital of Pittsburgh, October 2017
- "Standards of Care," Parent Project Muscular Dystrophy, Boston regional conference, October 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Advisor to board, Parent Project Muscular Dystrophy, 1999 to the present
- ATS, 1991 to the present

HONORS

- Best Doctors, *Pittsburgh Magazine*, 2012–2016
- *Best Doctors in America*, Woodward/White, Inc., 2003–2017
- 2013 Care Center Champion Award, Cystic Fibrosis Foundation, 2014
- University of Pittsburgh School of Medicine's Sheldon Adler Innovation in Medical Education Award, 2017

Erick Forno, MD, MPH

RESEARCH

Erick Forno studies the environmental and genetic risk factors for asthma and asthma severity in children. His primary focus is on the effect of overweight and obesity on childhood asthma. He has recruited for a study looking at the epigenetics and genomics of adipose tissue and childhood asthma, and he has participated in ongoing studies of epigenetics of asthma in Puerto Rico and of vitamin D supplementation for persistent asthma.

EDITORSHIPS

- Review Board, *Journal of Allergy and Clinical Immunology*
- Ad hoc reviewer, *American Journal of Respiratory and Critical Care Medicine*
- Ad hoc reviewer, *Journal of Allergy and Clinical Immunology–In Practice*
- Ad hoc reviewer, *The Lancet Respiratory Medicine*
- Ad hoc reviewer, *Annals of the American Thoracic Society*
- Ad hoc reviewer, *European Respiratory Journal*
- Ad hoc reviewer, *Pediatrics*
- Ad hoc reviewer, *Chest*
- Ad hoc reviewer, *Pharmacogenomics Journal*
- Ad hoc reviewer, *PLOS ONE*
- Ad hoc reviewer, *Pediatric Pulmonology*
- Ad hoc reviewer, *Annals of Allergy, Asthma, and Immunology*
- Ad hoc reviewer, *Thorax*
- Ad hoc reviewer, *Respirology*
- Ad hoc reviewer, *Journal of Pediatrics*
- Ad hoc reviewer, *Obesity Reviews*
- Ad hoc reviewer, *Journal of Asthma*
- Ad hoc reviewer, *Jornal de Pediatria (Brasil)*

MAJOR LECTURESHIPS AND SEMINARS

- "Multi-Omics Approach to Identify Genes Associated With Childhood Asthma Risk and Morbidity," mini-symposium, ATS international conference, San Francisco, Calif., May 2016
- "Omics of Environmental Lung Disease," co-chair, mini-symposium, ATS international conference, San Francisco, Calif., May 2016
- "Obesity and Asthma in Children," guest speaker, Chronic Diseases in Low- and Middle-Income Countries course, Johns Hopkins Bloomberg School of Public Health, Baltimore, Md., August 2016
- "Obesity, Stress, and Asthma," guest speaker, Societal Change, Asthma, and Allergy in Latin America international meeting, Salvador da Bahía, Brazil, December 2016
- "Childhood Obese Asthma," guest speaker, Pulmonary Division, University of Wisconsin School of Medicine and Public Health, Madison, Wis., January 2017
- "Obesity and Asthma in Children" and "Severe Phenotypes of Childhood Asthma," guest speaker, XXX Central American and Caribbean Congress of Pulmonology and Thoracic Surgery, San Jose, Costa Rica, March 2017

- "Obese Asthma in Pediatrics," guest speaker, 39th Conference of the German Pediatric Pulmonology Association, Essen, Germany, March 2017
- "Obesity and Asthma" and "Asthma Management Beyond Monotherapy," invited lectures, Panama Respiratory Symposium, Panama City, Panama, April 2017
- "A Pro/Con Debate in Pediatric Asthma," co-chair, Scientific Symposium, ATS international conference, Washington, D.C., May 2017
- "Obesity and Chronic Respiratory Diseases in Children: Recent Advances and Clinical Implications," co-chair, scientific symposium, ATS international conference, Washington, D.C., May 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- ATS
- American Society of Human Genetics
- Society for Pediatric Research

HONORS

- Accepted to the Society for Pediatric Research, 2013
- Member, Program Committee, ATS Assembly of Pediatrics, 2015–2018
- Mentored Career Development Award (K08), NIH, 2015–2019
- Research Advisory Committee Award, Children's Hospital of Pittsburgh, 2015–2016
- Klostenfrau International Award for Research of Airway Diseases in Childhood, German Society of Pediatric Pulmonology, 2017
- Robert B. Mellins Achievement Award for Outstanding Research, Assembly on Pediatrics, ATS, 2017

Todd D. Green, MD

RESEARCH

Todd D. Green serves as director for the FARE Center of Excellence at Children's Hospital of Pittsburgh of UPMC. In that position, he serves as principal investigator for multiple clinical trials looking at novel potential therapies for food allergy, a condition for which there is currently no treatment. He continues to serve as principal investigator in studies examining quality-of-life issues in children with food allergy, as well as characterizing outcomes of oral food challenges. Green was a co-investigator (collaborating with William Doyle, principal investigator) on a pilot study of the effect of intranasal ragweed challenge in adult allergy rhinitis subjects on middle-ear pressure regulation.

EDITORSHIPS

- Section editor (Food Allergy), *Current Allergy and Asthma Reports*
- Ad hoc reviewer, *Allergy (European Journal of Allergy and Clinical Immunology)*
- Ad hoc reviewer, *Annals of Allergy, Asthma, and Immunology*
- Ad hoc reviewer, *Immunotherapy*
- Ad hoc reviewer, *Journal of Allergy and Clinical Immunology*
- Ad hoc reviewer, *Journal of Allergy and Clinical Immunology–In Practice*
- Ad hoc reviewer, *Journal of Asthma*
- Ad hoc reviewer, *Journal of Pediatric Gastroenterology and Nutrition*
- Ad hoc reviewer, *New England Journal of Medicine*
- Ad hoc reviewer, *Pediatrics*

ADVISORY COMMITTEE MEMBERSHIPS

- Outcomes Research Advisory Board, Patient-Centric Food Allergy Research Program, FARE, supported by the Patient-Centered Outcomes Research Institute through the Eugene Washington Engagement Award
- Medical Advisory Board, International FPIES (food protein–induced enterocolitis syndrome) Association
- UPMC Patient Experience Physician Advisory Group
- Clinical Advisory Board, FARE
- Medical-Scientific Council, Asthma and Allergy Foundation of America
- Medical Advisory Team, Kids With Food Allergies
- Medical advisor, Epi-Life, Food Allergy Parent Support Group
- Residency Education Committee, Children's Hospital of Pittsburgh of UPMC
- Pediatric Residency Selection Committee, Children's Hospital of Pittsburgh of UPMC

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Fellow, American Academy of Allergy, Asthma, and Immunology
- Representative to the Council of Pediatric Subspecialties from the American Academy of Allergy, Asthma, and Immunology
- Governor, Region 2, Federation of the Regional, State, and Local American Association of Immunologists Societies Assembly
- Pediatrics Councilor, Program Directors Assembly
- Executive Committee, American Academy of Allergy, Asthma, and Immunology
- Clinical Immunology Society
- Past president, Board of Regents, Pennsylvania Allergy and Asthma Association

MAJOR LECTURESHIPS AND SEMINARS

- "Primary Prevention of Food Allergy," Children's Community Pediatrics, "Lunch 'N Learn" teleconference, Pittsburgh, Pa., June 2016

- "Food Allergy Potpourri: FPIES, FIEP, FPE, LEAP ...," faculty, Three Rivers Pediatric Update, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., May 2016
- "Pediatric Allergy Cases," faculty, 11th Annual Update in Sino-Nasal Disorders and Allergy, University of Pittsburgh School of Medicine, Pittsburgh, Pa., May 2016
- "Participating in a Research Study," webinar offered by FARE, April 2016

Yueh-Ying Han, PhD, MS

RESEARCH

Using epidemiological methodologies and quantitative analysis, Yueh-Ying Han studies etiology and risk factors for asthma and asthma severity in children. Her primary study is on dietary and nutritional intake in association with childhood asthma. She works closely with fellows and colleagues in various pulmonary studies. She also serves as the overall data manager for Celedón's research group, participating in several study projects at UPMC and Children's Hospital of Pittsburgh of UPMC.

EDITORSHIPS

- Ad hoc reviewer, *Pediatric Allergy and Immunology*
- Ad hoc reviewer, *American Journal of Public Health*

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Public Health Association
- Society for Epidemiology Research

TEACHING ACTIVITIES

- "Environmental Epidemiology," guest lecturer, University of Pittsburgh Graduate School of Public Health, Pittsburgh, Pa., January–April 2016

Geoffrey Kurland, MD

EDITORSHIPS

- Associate editor, lung transplantation, *Journal of Pediatric Transplantation*

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Pediatric Pulmonary Sub-Board, American Academy of Pediatrics
- Pediatric Planning Committee, ATS
- Pediatric Interstitial Lung Disease Consortium
- Medical volunteer, Western States 100-Mile Endurance Run, Sierra Mountains, Calif.
- Alpha Omega Alpha
- American Academy of Pediatrics
- American Pediatric Society
- American Society of Transplant Physicians
- ATS
- European Respiratory Society
- International Pediatric Transplantation Association
- International Society of Heart and Lung Transplantation
- Pediatric Pulmonary Training Directors Association

Allyson Larkin, MD

RESEARCH

Allyson Larkin is involved in multiple asthma-related projects. She continues work on developing an asthma game that will enhance understanding of treatment and disease processes. She sees patients in the Severe Asthma Research Program and works to develop an active asthma registry. She participates in the following studies.

- Best African American Response to Asthma Drugs
- Step-Up Yellow Zone Inhaled Corticosteroids to Prevent Exacerbations
- Peanut Allergy Oral Immunotherapy Study of AR101 for Desensitization in Children and Adults (Palisade)
- A Double-Blind, Placebo-Controlled, Randomized Phase III Pivotal Trial to Assess the Efficacy and Safety of Peanut Epicutaneous Immunotherapy With Viaskin® Peanut in Peanut-Allergic Children
- A Double-Blind, Placebo-Controlled Randomized Trial to Study Viaskin® Milk Efficacy and Safety for Treating IgE-Mediated Cow's Milk Allergy in Children

ADVISORY COMMITTEE MEMBERSHIPS

- Pennsylvania Allergy and Asthma Association Board of Regents, June 2013
- Children's Community Pediatrics Asthma Care Program
- Accreditation Council for Graduate Medical Education
- Clinical Competency Committee, Allergy/Immunology Fellowship Program
- Curriculum and Program Evaluation Committee, Allergy/Immunology Fellowship Program
- Research Committee, Allergy/Immunology Fellowship Program

MAJOR LECTURESHIPS AND SEMINARS

- "Review of Primary Immunodeficiencies Related to B-Cell Defects and Phagocytic Defects," Immunology in Health and Disease, University of Pittsburgh School of Medicine, Pittsburgh, Pa., February 2016
- "Food Allergy Awareness," St. Edmond's Academy, Pittsburgh, Pa., May 2016
- "Use of Anti-IL4/IL5 Therapies in Allergic Disease," grand rounds, Allergy and Immunology, Children's Hospital of Pittsburgh, Pittsburgh, Pa., May 2016
- "Review of Primary Immunodeficiencies Related to B-Cell Defects and Phagocytic Defects," Immunology in Health and Disease, University of Pittsburgh Medical School, Pittsburgh, Pa., March 2017
- "Gateway Asthma Webinar," Pittsburgh, Pa., August 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Pennsylvania Allergy and Asthma Association
- American Academy of Allergy, Asthma, and Immunology

Hiren Muzumdar, MD

RESEARCH

Hiren Muzumdar is an investigator in a study on sleep disturbances in recent survivors of childhood cancer.

ADVISORY COMMITTEE MEMBERSHIPS

- Telehealth for Technology Dependent Children Program, Children's Hospital of Pittsburgh, 2014 to the present

EDITORSHIPS

- Reviewer, *American Journal of Respiratory and Critical Care Medicine*
- Reviewer, *Chest*
- Reviewer, *Pediatric Pulmonology*
- Reviewer, *Sleep*
- Reviewer, *Sleep Medicine*
- Reviewer, *International Journal of Pediatric Otolaryngology*
- Reviewer, *Annals of the American Thoracic Society*
- Reviewer, *Journal of Clinical Sleep Medicine*

MAJOR LECTURESHIPS AND SEMINARS

- "Pediatric Obstructive Sleep Apnea," annual Pennsylvania Society for Respiratory Care Western Regional Conference on Respiratory and Sleep Medicine, October 2015
- "Sleep Update," Three Rivers Pediatric Update, Children's Hospital of Pittsburgh, Pittsburgh, Pa., May 2016

HONORS

- Best Doctors, *Pittsburgh Magazine*, 2014 to the present

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- ATS
- American Academy of Sleep Medicine

David M. Orenstein, MD, MA

RESEARCH

David Orenstein's 37-year research career has focused on various aspects of exercise in patients with cystic fibrosis and other lung disorders. Orenstein continues as the principal investigator of the Pittsburgh Translational Center for the Cystic Fibrosis Foundation's National Therapeutics Development Network and as a driving force in many multicenter clinical studies.

ADVISORY COMMITTEE MEMBERSHIPS

- Board of Trustees, Western Pennsylvania Chapter, Cystic Fibrosis Foundation
- Chair, Ethics Committee, Children's Hospital of Pittsburgh of UPMC
- Data Safety Monitoring Committee, Cystic Fibrosis Foundation Therapeutics Development Network
- Pediatric Pulmonary Fellowship Programs, Appeals Panel for the Accreditation Council for Graduate Medical Education
- Professional Education and Training Committee, Cystic Fibrosis Foundation (national)

- Chair, Publications and Presentations Committee, Cystic Fibrosis Foundation Therapeutics Development Network

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- American College of Sports Medicine
- American Pediatric Society
- American Society for Bioethics and Humanities
- Cystic Fibrosis Foundation
- Society for Pediatric Research
- Center for Bioethics and Health Law

HONORS

- *Best Doctors in America*, Woodward/White, Inc., 1989 to the present
- Antonio J. and Janet Palumbo Professor of Cystic Fibrosis, Children's Hospital of Pittsburgh of UPMC
- Health Care Heroes Lifetime Achievement Award, *Pittsburgh Business Times*, 2015

Daniel J. Weiner, MD

RESEARCH

Daniel Weiner is the local principal investigator for a number of multicenter cystic fibrosis clinical trials, including studies of novel pharmaceuticals to enhance ion transport, pancreatic enzyme–replacement therapies, and hydrating therapies (hypertonic saline). He continues to investigate measurements from multiple-breath washout studies as an outcome for clinical trials in cystic fibrosis.

COMMITTEE MEMBERSHIPS

- Center Committee, Cystic Fibrosis Foundation
- Proficiency Standards for Pulmonary Function Laboratories Committee, ATS
- Board of Directors, Western Pennsylvania Chapter of the Cystic Fibrosis Foundation
- Proficiency Standards for Pulmonary Function Laboratories Committee, ATS
- Accreditation for Pulmonary Function Laboratories Committee, ATS
- Sub-Board of Pediatric Pulmonology, American Board of Pediatrics
- PENTEC (Pediatric Normal Tissue Effects in the Clinic) Working Group
- Pulmonary Working Group, Children's Oncology Group
- Pulmonary Dysfunction Guidelines International Harmonisation Group

EDITORSHIPS

- Ad hoc reviewer, *CHEST*
- Ad hoc reviewer, *Pediatric Emergency Care*
- Ad hoc reviewer, *European Respiratory Journal*
- Ad hoc reviewer, *American Journal of Respiratory and Critical Care Medicine*
- Ad hoc reviewer, *Pediatric Pulmonology*
- Ad hoc reviewer, *Anesthesia and Analgesia*
- Ad hoc reviewer, *Journal of Cystic Fibrosis*
- Ad hoc reviewer, *Acta Pathologica, Microbiologica, et Immunologica Scandinavica*
- Ad hoc reviewer, *Journal of Pediatrics*
- Ad hoc reviewer, *Journal of Asthma*
- Ad hoc reviewer, *Respiratory Care*
- Ad hoc reviewer, *International Journal of Radiation Oncology, Biology, Physics*
- Review editor, *Frontiers in Pediatric Pulmonology*

MAJOR LECTURESHIPS AND SEMINARS

- "Cystic Fibrosis," Pennsylvania Association of Physician Assistants, Greentree, Pa., October 2016
- "Spirometry," lecture for pulmonary research coordinators, Division of Pulmonary Medicine, Children's Hospital of Pittsburgh, October 2016
- "Spirometry and Beyond," grand rounds, Section of Allergy and Immunology, Children's Hospital of Pittsburgh, November 2016
- "What About the Kids? Special Considerations for Pediatric Pulmonary Function Measurement and Interpretation" and "PG-21 Pulmonary Function and Cardiopulmonary Exercise Testing: Moving From Theory to Clinical Practice," ATS international conference, Washington, D.C., May 2017
- "To Spirometry and Beyond: Advanced Pulmonary Function Testing," grand rounds, Section of Allergy and Immunology, Children's Hospital of Pittsburgh, October 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Alpha Omega Alpha
- American College of Chest Physicians
- ATS
- European Respiratory Society

HONORS

- Outstanding Cystic Fibrosis Advocate Award, Cystic Fibrosis Foundation, 2016
- Outstanding Mini-Elective, University of Pittsburgh School of Medicine, October 2017

TEACHING ACTIVITIES

The division has two training programs accredited by the Accreditation Council for Graduate Medical Education (Pediatric Pulmonary Medicine and Allergy/Immunology). Six fellows currently are in training in the Pulmonary Medicine Program, and four fellows currently are in training in the Allergy/Immunology Program. The division's clinics serve as a popular elective rotation for medical students and residents.

The faculty teaches classes in molecular medicine and integrated life science; directs journal clubs and research conferences; bolsters training in other departments (e.g., the School of Nursing, Otolaryngology, Pediatric Critical Care); and participates in the Unified Fellows Course. The division's educational efforts included three predoctoral students, one of whom was in the Medical Scientist Training Program; two summer students; one summer undergraduate student; and two medical student researchers.

THREE-YEAR BIBLIOGRAPHY

2015

Oczypok EA, Milutinovic PS, **Alcorn JF**, Khare A, Crum LT, **Manni ML**, Epperly MW, Pawluk AM, Ray A, Oury TD. Pulmonary receptor for advanced glycation end-products promotes asthma pathogenesis through IL-33 and accumulation of group 2 innate lymphoid cells. *J Allergy Clin Immunol*. 2015;136(3):747-56.

Otsubo C, Bharathi S, Uppala R, Ilkayeva OR, Wang D, McHugh K, Zou Y, Wang J, **Alcorn JF**, Zuo YY, Hirschey MD, ES Goetzman. Long-chain acylcarnitines reduce lung function by inhibiting pulmonary surfactant. *J Biol Chem*. 2015;290:23897-904.

Alcorn JF, Sarkar SN. What is the oligoadenylate synthetases-like protein and does it have therapeutic potential for influenza? *Expert Rev Respir Med*. 2015;9(1):1-3.

Alcorn JF, **Kolls JK**. Physiology. Killer fat. *Science*. 2015;347(6217):26-7.

Rynda-Apple A, Robinson KM, **Alcorn JF**. Influenza and bacterial superinfection: Illuminating the immunologic mechanisms of disease. *Infect Immun*. 2015;83(10):3764-70.

Brehm JM, **Ramratnam SK**, Tse SM, Croteau-Chonka DC, Pino-Yanes M, **Rosas-Salazar C**, Litonjua AA, Raby BA, **Boutaoui N**, **Han YY**, **Chen W**, **Forno E**, Marsland AL, Nugent NR, Eng C, Colon-Semidey A, Alvarez M, Acosta-Perez E, Spear ML, Martinez FD, Avila L, Weiss ST, Soto-Quiros M, Ober C, Nicolae DL, Barnes KC, Lemanske RF Jr., Strunk RC, Liu A, London SJ, Gilliland F, Sleiman P, March M, Hakonarson H, Duan QL, Kolls JK, Fritz GK, Hu D, Fani N, Stevens JS, Almli LM, Burchard EG, Shin J, McQuaid EL, Ressler K, Canino G, **Celedón JC**. Stress and bronchodilator response in children with asthma. *Am J Respir Crit Care Med*. 2015;192(1):47-56.

Wang T, Guan W, Lin J, **Boutaoui N**, Canino G, Luo J, **Celedón JC**, **Chen W**. A systematic study of normalization methods for Infinium 450K methylation data using whole-genome bisulfite sequencing data. *Epigenetics*. 2015;10(7):662-9.

Sordillo JE, Kelly R, Bunyavanich S, McGeachie M, Qiu W, Croteau-Chonka DC, Soto-Quiros M, Avila L, **Celedón JC**, **Brehm JM**, Weiss ST, Gold DR, Litonjua AA. Genome-wide expression profiles identify potential targets for gene-environment interactions in asthma severity. *J Allergy Clin Immunol*. 2015 Oct;136(4):885-92.e2.

Nguyen NLH, Pilewski JM, **Celedón JC**, Mandalapu S, Blanchrd ML, DeRicco A, Hartigan E, **Alcorn JF**, Kolls JK. Vitamin D supplementation decreases *Aspergillus fumigatus* specific Th2 responses in CF patients with *Aspergillus* sensitization: A phase 1 open-label study. *Asthma Res Practice*. 2015;1:3.

Chen W, **Brehm JM**, Lin J, Wang T, **Forno E**, Acosta-Perez E, **Boutaoui N**, Canino G, **Celedón JC**. Expression quantitative trait loci (eQTL) mapping in Puerto Rican children. *PLOS One*. 2015;10(3):e0122464.

Li B, Wei Q, Zhan X, Zhong X, **Chen W**, Li C, Haines J. Leveraging identity-by-descent for accurate genotype inference in family sequencing data. *PLOS Genet*. 2015;11(6):e1005271.

Chen W, **Brehm JM**, Manichaikul A, Cho MH, **Boutaoui N**, Yan Q, Burkart KM, Enright PL, Rotter JI, Petersen H, Leng S, Obeidat M, Bosse Y, Brandsma CA, Hao K, Rich SS, Powell R, Avila L, Soto-Quiros M, Silverman EK, Tesfaigzi Y, Barr RG, **Celedón JC**. A genome-wide association study of chronic obstructive pulmonary disease in Hispanics. *Ann Am Thorac Soc*. 2015;12(3):340-8.

Chen R, Wei Q, Zhan X, Zhong X, Sutcliffe JS, Cox NJ, Cook EH, Li C, **Chen W**, Li B. A haplotype-based framework for group-wise transmission/disequilibrium tests for rare variant association analysis. *Bioinformatics*. 2015;31(9):1452-9.

Wei Q, Zhan X, Zhong X, Liu Y, Han Y, **Chen W**, Li B. A Bayesian framework for *de novo* mutation calling in parents-offspring trios. *Bioinformatics*. 2015;31(9):1375-81.

Fan R, Wang Y, Boehnke M, **Chen W**, Li Y, Ren H, Lobach I, Xiong M. Gene-level meta-analysis of quantitative traits by functional linear models. *Genetics*. 2015;200(4):1089-104.

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Ray M, Horne W, McAleer JP, Ricks DM, Kreindler JL, Fitzsimons MS, Chan PP, Trevejo-Nunez G, Chen K, Fajt M, **Chen W**, Ray A, Wenzel S, Kolls JK. RNA-seq in pulmonary medicine: How much is enough? *Am J Respir Crit Care Med*. 2015;192(3):389-91.

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Finder JD. The natural history of pulmonary function in Duchenne muscular dystrophy. *Pediatric Pulmonology*. 2015;50:421-2.

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DIVISION OF RHEUMATOLOGY

Mission

The mission of the Division of Rheumatology is to ensure outstanding care for children with rheumatologic diseases now and in the future. To accomplish this mission, the division is dedicated to the following goals:

- To provide state-of-the-art, compassionate care to children with rheumatologic disorders.
- To generate new knowledge by clinical and basic research that will lead to improved care and cure.
- To provide high-quality education and training in the field of pediatric rheumatology to students, trainees, and other health professionals.

FACULTY

A. Kim Ritchey, MD
Professor of Pediatrics
Vice Chair for International Affairs
Interim Chief, Division of Rheumatology

Scott Canna, MD
Assistant Professor of Pediatrics and Immunology
Scholar, Mellon Institute for Pediatric Research

Elaine A. Cassidy, MD
Assistant Professor of Pediatrics
Associate Director, Fellowship Program

Abbe N. de Vallejo, PhD
Associate Professor of Pediatrics and Immunology
Director, Flow Core Facility
Faculty, University of Pittsburgh Cancer Institute
Faculty, McGowan Institute for Regenerative Medicine
Investigator, Pittsburgh Claude Pepper Older Americans Independence Center

Daniel A. Kietz, MD, PhD
Professor of Pediatrics and Internal Medicine
Director, Fellowship Program
Clinical Director, Rheumatology

Margalit E. Rosenkranz, MD
Assistant Professor of Pediatrics

Kathryn S. Torok, MD
Assistant Professor of Pediatrics
Assistant Professor of Clinical and Translational Science

OVERVIEW OF DIVISION

The Pediatric Rheumatology Division is composed of three full-time clinicians, one clinician/investigator, and two basic scientists. Members of the division are highly regarded as outstanding clinicians, investigators, and educators. Clinicians routinely receive the highest patient satisfaction scores in the hospital. Investigators are actively and successfully competing for research support in times of limited funding. A very highly regarded new investigator, Scott Canna, MD, joined the division, and his expertise broadens the basic research portfolio of the division. An additional basic scientist, Amanda Poholek, PhD, will be joining the division in the next fiscal year. The fellowship program for training pediatric rheumatologists is highly regarded nationally, and every year the program accepts one outstanding fellow. One challenge for the division has been recruitment of a permanent division chief. Nonetheless, because of outstanding faculty and excellent support staff, the division remains highly effective and productive at all levels.

CLINICAL ACTIVITIES

The clinical service continues to be a model of efficiency in delivery of care. Most clinical activity is in the outpatient arena. Within the past fiscal year, the division began to offer same-day clinic appointments. All patients who called for an appointment were offered an appointment that day, and 68% of the same-day appointments were filled. Clinic volume was 4,889 visits, of which 33% were new patients and 67% return visits. This represents a 1.3% increase in outpatient volume and 2.85% increase in charges.

Many patients with rheumatologic disease require prolonged infusions of medication. Activity in the Infusion Center has increased over the past decade as new medications have become available. In this fiscal year, the division supervised 751 infusions of medications in the Infusion Center. Increasingly, ultrasound is being used in the clinic to document joint disease as well as response to treatment to enhance patient care. Injection of medication directly into a joint is an important part of overall treatment management of patients with rheumatologic disorders and joint disease. The division performed 134 joint injections during the past year.

The division primarily provides a consultative service for hospitalized patients. Approximately 90 consultations were performed last year.

Daniel Kietz, MD, PhD, continues to be the clinical director of the division. Because he is trained in both medical and pediatric rheumatology, he attends a satellite clinic for adolescents, young adults, and adult patients within the UPMC Lupus Center of Excellence in the Pittsburgh neighborhood of Oakland.

Kathryn Torok is a national leader in the area of pediatric scleroderma and the clinical director of one of the few pediatric scleroderma clinics in the nation. The center at Children's Hospital of Pittsburgh is part of the University of Pittsburgh Scleroderma Center. Patients are seen for clinical care and are also enrolled into numerous studies regarding pediatric scleroderma.

RESEARCH AND OTHER SCHOLARLY ACTIVITIES

The division is actively involved in clinical, translational, and basic research. The clinical faculty participate in CARRA—the Childhood Arthritis and Rheumatology Research Alliance—the main national research group for collaborative research at a national level. They are involved in registry activity and several prospective, observational outcome trials. With Margalit Rosenkranz's leadership, the division is also involved in pharmaceutical trials of novel agents for the treatment of rheumatology conditions in children. Torok's research is focused on the inflammatory and profibrotic cytokines involved in pediatric scleroderma. She uses a translational approach by comparing these proteins in serum and skin biopsy specimens with clinical parameters of disease activity and damage in her pediatric scleroderma cohort. Abbe De Vallejo leads a basic research program on the immunobiology of aging and inflammatory syndromes. Canna uses genetic and functional insights from patients and model systems to find ways to subtype patients with systemic inflammatory response syndrome (SIRS)/sepsis in diagnostically and therapeutically meaningful ways. Grant dollars to the division totaled $1,083,556, of which $985,381 were direct dollars. Total grant dollars increased by 75% when compared to the prior year.

A. Kim Ritchey, MD

RESEARCH

Children's Oncology Group (COG). A. Kim Ritchey is an active clinical investigator and local principal investigator (PI) for a number of COG trials at Children's Hospital of Pittsburgh of UPMC. The local PI of a COG trial is responsible for overseeing the research activity of that protocol at the institution. One such trial is testing the efficacy of the tyrosine kinase inhibitor dasatinib when it is added to standard chemotherapy for children with Philadelphia chromosome–positive acute lymphoblastic leukemia. This trial is partially funded by Bristol-Myers Squibb. Ritchey is involved with oversight of the development and performance of pediatric cancer clinical trials at the national level. He is a member of the National Cancer Institute (NCI) Pediatric Leukemia/Lymphoma Steering Committee, which reviews all COG proposals for clinical research in the areas of leukemia and lymphoma. As chair of the COG Data Safety Monitoring Committee, he leads the group which has oversight of all phase II and III clinical trials.

Ritchey served as local PI for the Pediatric Brain Tumor Consortium, a collaboration of 12 institutions funded by the NCI to offer potentially practice-changing phase I research for children with brain tumors.

Ritchey served as the local PI for a study of the thrombopoietin agonist romiplostim for children with chronic idiopathic thrombocytopenic purpura.

ADVISORY COMMITTEE MEMBERSHIPS

National:

- Chair, Data Safety Monitoring Committee A, COG
- Pediatric Leukemia/Lymphoma Review Committee, NCI, National Institutes of Health (NIH)

Departmental:

- Center for Rare Diseases Committee
- Leadership Council
- Leadership Team
- Executive Committee
- Promotion and Tenure Committee

Divisional (hematology/oncology):

- Fellowship Oversight Committee
- Chair, Clinical Competency Committee
- Chair, Advanced Practice Provider Committee

Hospital-based:

- Global Health Service Committee
- Medical Executive Committee

A. Kim Ritchey, MD
Interim Division Chief, Rheumatology

Regional:
- Board of Directors, Hemophilia Center of Western Pennsylvania
- Board of Directors, Make-a-Wish Foundation

EDITORSHIPS
- Editor-in-chief, Pediatric Treatment Board, Physician Data Query, NCI, NIH

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS
- American Society of Clinical Oncology
- American Society of Hematology
- American Society of Pediatric Hematology/Oncology
- COG
- Thrombosis and Hemostasis Research Society

HONORS
- *Best Doctors in America*, Woodward/White, Inc.
- Best Doctors, *Pittsburgh Magazine*
- UPMC Honor Roll: Excellence in Patient Care (top three of 70)
- Patient Satisfaction Award, Children's Hospital of Pittsburgh of UPMC
- Top 10 Press Ganey faculty scores

Scott W. Canna, MD

RESEARCH
Inflammation is a core pathogenic mechanism in virtually every disease process. Systemically, this culminates in SIRS, identified since ancient times as sepsis. Blocking inflammation in SIRS has been largely disappointing, conferring neither broad benefit nor harm. This research uses genetic and functional insights from patients and model systems to find ways to subtype patients with SIRS/sepsis in diagnostically and therapeutically meaningful ways.

Patients found to have monogenic defects causing excessive innate immune responses have been particularly helpful. Most "autoinflammatory" patients have chronic organ-specific or systemic inflammation but not typically SIRS. The dramatic response of many "autoinflammatory" patients to inhibition of the inflammasome-activated cytokine interleukin (IL)-1 has reinvigorated the quest for anti-inflammatory targets in SIRS and reinforced the therapeutic potential of targeting the inflammasome and related innate immune pathways.

This group studies the intersections of hyper- and auto-inflammation, specifically two related disorders that typify the concept of hyperinflammatory SIRS: hemophagocytic lymphohistiocytosis and macrophage activation syndrome. The researchers combine clinical insights from rheumatology and innate immunity with basic models of overwhelming systemic inflammation to define new disease subtypes and disease activity biomarkers, to flesh out mechanisms of inflammatory disease, and to test promising therapeutic strategies.

REGULATORY EXPERIENCE
Human:
- Associate investigator and site PI, "Therapeutic Use of Tadekinig Alfa in NLRC4 Mutation and XIAP Deficiency," NCT03113760, 2017 to the present
- PI, University of Pittsburgh Human Subjects Protocol PRO16120025, "Natural History of Autoinflammatory Disease," 2017 to the present
- Associate investigator, National Institute of Allergy and Infectious Diseases protocol 17-I-0016, "Natural History, Pathogenesis, and Outcome of Autoinflammatory Diseases (NOMID/CAPS, DIRA, CANDLE, SAVI, NLRC4-MAS, Still's-like Diseases, and Other Undifferentiated Autoinflammatory Diseases)," NCT02974595, 2016 to the present
- Sub-investigator, CARRA Legacy Registry, 2013 to the present

Animal:
- PI, Animal Protocol 16129653, 2016 to the present

STUDY SECTIONS
- CARRA Scientific Review Committee, 2017 to the present
- Peer reviewer, Rheumatology Research Foundation Innovative Research Award, Basic Science Section, 2016 to the present
- Abstract reviewer, American College of Rheumatology annual scientific meeting, 2016 to the present

ADVISORY COMMITTEE MEMBERSHIPS
- Medical Advisory Board, AB2Bio Ltd., Geneva, Switzerland, 2015 to the present

EDITORSHIPS
- *Arthritis and Rheumatology*
- *Frontiers in Immunology*

MAJOR LECTURESHIPS

- "IL-18 at the Intersection of Auto- and Hyperinflammation," invited presentation, KFO 249 Symposium: Defects of the Innate Immune System in Autoinflammation and Autoimmunity, Dresden, Germany, August 2017
- "IL-18 at the Intersection of Auto- and Hyperinflammation," invited presentation, FOCIS annual meeting, NIH Immunology Interest Group Symposium, Chicago, Ill., June 2017
- "Chronic IL-18 of Diverse Origins Defines and Drives the Hyperinflammatory Macrophage Activation Syndrome," oral abstract presentation, Keystone Pattern Recognition Signaling Symposium, Banff, Alberta, Canada, March 2017
- "CNS Manifestations of Autoinflammatory Disease," Children's Hospital of Pittsburgh Neuroimmunology Conference, Pittsburgh, Pa., March 2017
- "Cytokines in Systemic Inflammation: Following the Monogenic Breadcrumb Path," Children's Hospital of Pittsburgh Molecular Medicine Research Seminar, Pittsburgh, Pa., January 2017
- "IL-18 at the Intersection of Auto- and Hyper-Inflammation," University of Pittsburgh Department of Immunology Seminar Series, Pittsburgh, Pa., December 2016

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics, 2006 to the present
- American College of Rheumatology, 2009 to the present
- CARRA, 2010 to the present
- Histiocyte Society, 2013 to the present

Elaine A. Cassidy, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- American College of Rheumatology

MAJOR LECTURESHIPS

- "Juvenile Idiopathic Arthritis: Classification, Diagnosis, and Management," Dubois Regional Medical Center continuing medical education course
- "Pediatric Rheumatology: Diagnoses, Testing, and Treatment," Arthritis Foundation 2016 Family Day, Pittsburgh, Pa.
- Longitudinal Alliance Project, University of Pittsburgh School of Medicine

Abbe N. de Vallejo, PhD

RESEARCH

Abbe N. de Vallejo leads a research program examining the immunobiology of aging and inflammatory syndromes. His research on inflammatory syndromes focuses on rheumatic diseases. The three primary diseases of interest are adult rheumatoid arthritis, juvenile idiopathic arthritis, and juvenile dermatomyositis. Studies are aimed at examining functional subsets of T cells that either alleviate or aggravate disease manifestation and how such cell subsets are affected by medications or are themselves effectors of favorable outcomes of medications. The ultimate goal of these studies is to develop alternative cell-targeted therapies.

De Vallejo's research on aging takes a developmental view of aging rather than focusing on terminal stages of adult life. Projects examine the aging process across the life span, including mechanisms of lymphocyte senescence, immune/endocrine interactions in the regulation of life span, and immunologic predictors of health outcomes of aging and longevity. The studies include analyses of tissue specimens from human subjects from two large cohorts of elderly persons and from a novel long-lived mouse model. The goal of the projects is to develop age-targeted strategies to enhance immune function.

STUDY SECTIONS

- National Scientific Advisory Council, American Federation of Aging Research
- Hypersensitivity, Allergy, and Immune-Mediated Diseases Study Section, Center for Scientific Review, NIH
- Special Emphasis Panel: ZRG1 BDCN-M (02) M, Disorders in Brain Development and in Aging, Center for Scientific Review, NIH

ADVISORY COMMITTEE MEMBERSHIPS

National:

- Abstract Selection Committee, Gerontological Society of America

Departmental/interdepartmental:

- Immunology Seminar Series Steering Committee, University of Pittsburgh School of Medicine

University level:

- Healthy Brain Aging Workgroup, University of Pittsburgh Institute on Aging
- Immunology Graduate Program Steering Committee, University of Pittsburgh School of Medicine
- Tenure and Academic Freedom Committee (re-elected), Faculty Senate, University of Pittsburgh
- Faculty Assembly (elected), University Senate, University of Pittsburgh

EDITORSHIPS

- Editorial Board, *Aging and Disease*
- Editorial Board, *Rheumatology Current Research*

MAJOR LECTURESHIPS

- "Wait-to-Height Ratio, Vascular Health, and Cardiometabolic Risk in Vitamin D–Deficient Overweight and Obese Children," 2017 Annual Meeting of the Pediatric Academic Societies, San Francisco, Calif., May 2017
- "*In Vivo* Imaging of Venous Side Microcirculation in Older Adults at 7T," Conference on Alzheimer's Disease as a Neurovascular Inflammatory Disorder, New York Academy of Sciences, New York, N.Y., December 2016
- "Deletion of Pregnancy-Associated Plasma Protein A (PAPPA) Protects Long-Lived Mice from LPS Endotoxicosis," IGF Basic Science Symposium, Eighth International Congress of the IGF and Growth Hormone Research Societies, Tel Aviv, Israel, November 2016
- "Synovitis in Juvenile Idiopathic Arthritis Is Mediated by TCR-Independent Axis of Inflammation," Autoimmunity Workshop, 2016 International Congress of Immunology, Melbourne, Australia, August 2016

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Association of Immunologists
- American College of Rheumatology
- Gerontological Society of America

HONORS

- Julie Martin Career Award on Aging, American Federation for Aging Research
- Fellow, Royal Society of Medicine, London, England
- Elected fellow, Global Healthspan Policy Institute, Washington, D.C.
- Medical Student Research Mentoring Merit Award, University of Pittsburgh School of Medicine, 2016
- Lifetime Membership, Growth Hormone Research Society
- Travel Award, Eighth International Congress of the IGF Growth Hormone Research Societies, GRS/IGF Secretariat, 2016
- Faculty Travel Award, International Congress of Immunology, American Association of Immunologists, 2016

Daniel A. Kietz, MD, PhD

RESEARCH

Daniel A. Kietz is engaged in various collaborative, industry-sponsored, clinical translation projects on pediatric rheumatic diseases pertaining to the longitudinal perspective of disease courses, treatment responses, and outcomes. He is PI of a phase IV study on safety and efficacy of abatacept in juvenile idiopathic arthritis, a prevalent autoimmune disease of childhood for which the underlying immune mechanisms are not well understood. He is also co-investigator on a project examining the basic immunology of juvenile idiopathic arthritis.

Kietz is interested in transition of pediatric/adolescent patients into adulthood. Given his qualification as a rheumatologist for children and adults, he can continue providing care to his patients after they reach adulthood. Kietz has interest in investigating the influence of this continuity of care with comprehensive anticipatory guidance on long-term outcomes (medical, social, and professional realms, as well as quality of life).

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- American College of Rheumatology
- American Psychoanalytic Association
- Southeastern Child Analytic Consortium

HONORS

- Patient Satisfaction Award for Outstanding Achievements in Patient Care, 2016

Margalit E. Rosenkranz, MD

RESEARCH

Margalit E. Rosenkranz has been involved in overseeing clinical research studies and is the Pittsburgh site PI for multiple industry-sponsored studies and pediatric rheumatology research group registries and studies (CARRA). She is currently working on bringing in more studies sponsored by industry and the NIH.

ADVISORY COMMITTEE MEMBERSHIPS

- CARRA
- Pediatric Rheumatology Collaborative Study Group

MAJOR LECTURESHIPS AND SEMINARS

- "Immunopathogenesis of Juvenile Arthritis," Molecular Pathobiology (MSCMP2740), University of Pittsburgh graduate school course, July 2014, 2015, and 2016
- "Joint Pain in Pediatrics," physical therapy DPM lecture, University of Pittsburgh, August 2014, 2015, and 2016
- "Evaluation of a Dosing Regimen for Tocilizumab in Patients Younger Than Two Years of Age With Systemic Juvenile Idiopathic Arthritis," European League Against Rheumatism conference, Madrid, Spain, June 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- American College of Rheumatology
- Childhood Arthritis and Rheumatology Research Alliance
- Pediatric Rheumatology Collaborative Study Group

Kathryn S. Torok, MD

RESEARCH

Kathryn S. Torok's research is focused on the inflammatory and profibrotic cytokines involved in pediatric scleroderma. She uses a translational approach by comparing these proteins in serum and skin biopsy specimens with clinical parameters of disease activity and damage in her pediatric scleroderma cohort. Understanding which key inflammatory mediators are present during the active phase of disease may foster the development of more effective therapies in localized scleroderma to prevent disease damage, such as limb-length discrepancy and skin and muscle atrophy.

Torok serves as the PI for the National Registry for Childhood Onset Scleroderma and as the director of the Pittsburgh Childhood Scleroderma Clinic. Combining these two resources, the University of Pittsburgh hosts the largest single-center collection of clinical and serologic data on pediatric patients with scleroderma in the nation. In addition, Torok is a leader of the Pediatric Scleroderma Steering Committee (localized scleroderma and systemic sclerosis) in CARRA, overseeing the involvement of scleroderma subjects in the CARRA registry and CARRA-related studies. The Torok laboratory serves as the North American biorepository for CARRA's pediatric scleroderma subjects.

ADVISORY COMMITTEE MEMBERSHIPS

- Chair, Governing Board, Research and Mission committees, Great Lakes Region and Western Pennsylvania Chapter, Arthritis Foundation
- Lead member, CARRA-Rx Juvenile Localized Scleroderma Steering Committee, CARRA
- Lead member, Juvenile Systemic Sclerosis Committee, CARRA
- Pediatric team, Meeting Planning Committee, Annual American College of Rheumatology

EDITORSHIPS

- Member, Editorial Board, *Journal of Scleroderma and Related Disorders*

MAJOR LECTURESHIPS AND SEMINARS

- "Health-Related Quality of Life in Pediatric Scleroderma" and "Updates in Research in Pediatric Scleroderma," National Scleroderma Patient Education Conference, Unmask the Cure, New Orleans, La., July 2016
- "Updates in Localized Scleroderma," adult rheumatology grand rounds, University of Pittsburgh Arthritis Institute

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Society for Pediatric Research
- American Academy of Pediatrics
- American College of Rheumatology
- American Medical Association
- Childhood Arthritis and Rheumatology Research Alliance
- Pennsylvania Medical Society

HONORS

- Doctor of the Year, Scleroderma Foundation
- America's Top Pediatricians, Consumers' Research Council of America

EDUCATIONAL ACTIVITIES

The division has a training program in pediatric rheumatology that is accredited by the Accreditation Council for Graduate Medical Education. It is led by Director Kietz and Associate Director Cassidy. Four fellows are currently in training. All fellows receive extensive clinical training in the field and pursue scholarly activity. This year, fellows are learning the technique of joint ultrasound in patient management. The rheumatology clinic is a popular elective that is available for both medical students and residents. Cassidy is the course director for the elective. Faculty members continue to educate pediatric residents, pediatric rheumatology fellows, adult rheumatology fellows, and medical students in the outpatient clinic. Torok maintains a localized scleroderma clinical rotation for pediatric dermatology fellows at UPMC. De Vallejo is actively involved in teaching medical students and students in the Graduate School of Public Health. He also serves on several dissertation committees for graduate students in immunology and molecular genetics. He has served as one of the chairs of the comprehensive examination committees graduate students in immunology and cellular and molecular pathology. Finally, Canna and Cassidy have developed an immunology curriculum for all pediatric fellows, which will begin in fiscal year 2018.

THREE-YEAR BIBLIOGRAPHY

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Children's Hospital of Pittsburgh

RICHARD KING MELLON FOUNDATION INSTITUTE FOR PEDIATRIC RESEARCH

Mission

The Richard King Mellon Foundation Institute for Pediatric Research fosters an environment of scientific excellence in which multidisciplinary teams of physicians and scientists work together on cutting-edge solutions in medical science.

The core goals of the institute are:

- To support a state-of-the-art laboratory unit for research in molecular and cellular biology
- To enable the recruitment of the most innovative and capable pediatric scientists by supporting their most promising work
- To develop a competitive edge in recruiting and retaining scarce clinical and research talent
- To strengthen the educational program for future pediatric leaders

FACULTY AND STAFF

Jay K. Kolls, MD
Director, Richard King Mellon Foundation Institute for Pediatric Research
Professor of Pediatrics and Immunology
Vice Chair for Translational Research
Interim Director, T32 Fellowship Training Award, Division of Rheumatology

Scott Canna, MD
Mellon Scholar, Richard King Mellon Foundation Institute for Pediatric Research
Assistant Professor of Pediatrics

Kong Chen, PhD
Assistant Professor of Pediatrics

Timothy W. Hand, PhD
Mellon Scholar, Richard King Mellon Foundation Institute for Pediatric Research
Assistant Professor of Pediatrics

Bernhard Kühn, MD, FACC
Mellon Scholar, Richard King Mellon Foundation Institute for Pediatric Research
Associate Professor of Pediatrics
Director of Research in Cardiology, Department of Pediatrics

Pawan Kumar, PhD
Research Instructor

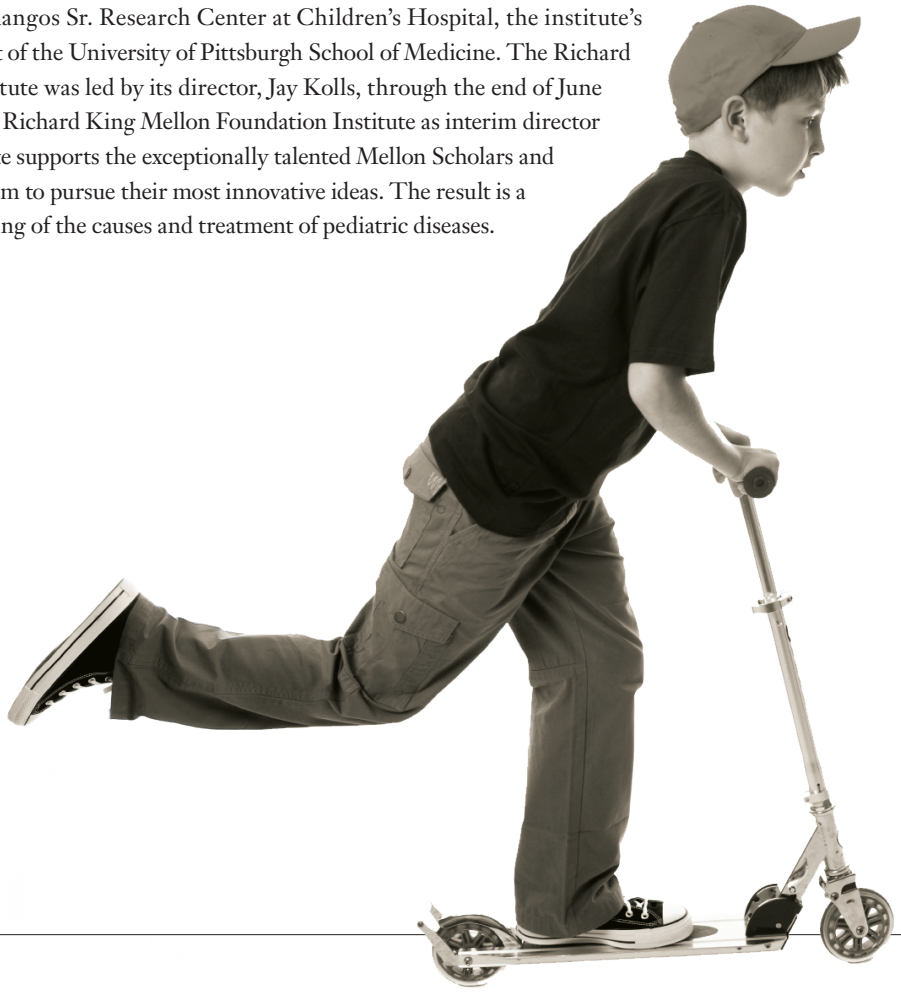
Amanda C. Poholek, PhD
Assistant Professor of Pediatrics

OVERVIEW

The pursuit of bold new advances for the treatment of some of the world's most debilitating childhood illnesses defines the work of the Richard King Mellon Foundation Institute for Pediatric Research.

Established through a groundbreaking gift from the Richard King Mellon Foundation, the institute is an incubator for research that challenges conventional wisdom, leading to paradigm shifts in pediatric medicine. This kind of high-risk, high-impact investigation is not typically funded through government or conventional sources, placing Children's Hospital of Pittsburgh of UPMC in a unique realm of pediatric research centers.

Located within the John G. Rangos Sr. Research Center at Children's Hospital, the institute's faculty and programs are a part of the University of Pittsburgh School of Medicine. The Richard King Mellon Foundation Institute was led by its director, Jay Kolls, through the end of June 2017. Bernhard Kühn leads the Richard King Mellon Foundation Institute as interim director effective July 2017. The institute supports the exceptionally talented Mellon Scholars and their teams by encouraging them to pursue their most innovative ideas. The result is a profound effect on understanding of the causes and treatment of pediatric diseases.



RESEARCH AND OTHER SCHOLARLY ACTIVITIES

Jay K. Kolls, MD

RESEARCH

The major goal of Jay Kolls' research is to investigate mechanisms of lung host defenses in normal and immunocompromised hosts. Kolls' team is investigating how interleukin (IL)-23 and IL-17 regulate neutrophil recruitment in response to infectious stimuli in the lung. Team researchers study cellular sources of IL-17A, IL-17F, and IL-22 in the lung and liver, as well as their signaling in response to pulmonary infection or hepatitis. The team also has long-standing interests both in determining whether Th17 cells and their cytokine products contribute to airway destruction in cystic fibrosis (CF) and in understanding cytokine biology in the lung. The lung immunology work focuses on CD4+ T cells and their subsets in regulating mucosal immunity to extracellular pathogens in the lung.

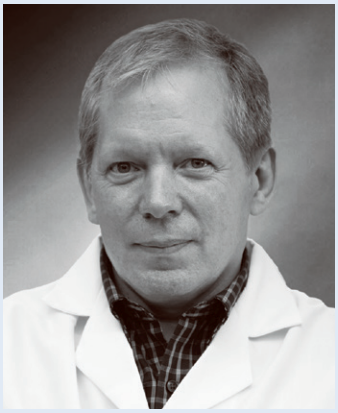
Improved Therapeutics and Diagnostics for Pneumocystis Pneumonia. The long-term goal of this project is to use the recently released pneumocystis genomes to advance treatment and diagnostics of this critical fungal infection.

T Cells and Pneumocystis Carinii *Pneumonia.* The aims of this project are to understand mechanisms by which CD4+ T cells provide host defense against *Pneumocystis* infection.

Th17 Cytokines and Lung Immunity. The aims of this project are to understand the role of Th17 cytokines in mucosal immunity against *Klebsiella pneumoniae* and *Staphylococcus aureus* in the lung.

Immune Airway-Epithelial Interactions in Steroid-Refractory Severe Asthma. The purpose of this proposal is to establish a new paradigm for severe asthma based on which novel therapeutics could be developed in the future using cutting-edge immunological, cellular, and RNA sequencing techniques.

Generation of Novel Human Monoclonal Antibodies for Lung Disease. This project aims to clone memory B cells from the lungs of patients with CF to characterize the B-cell response and generate a panel of human monoclonal antibodies from the CF lung.



Jay Kolls, MD
Director, Richard King Mellon Foundation Institute for Pediatric Research

EDITORSHIPS

- Deputy editor, *Journal of Immunology*
- Consulting editor, *Journal of Clinical Investigation*
- Associate editor, *American Journal of Respiratory and Critical Care Medicine*
- Advisory editor, *Journal of Experimental Medicine*

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Southern Medical Association
- Associate, American College of Physicians
- American College of Chest Physicians
- American Thoracic Society
- American Society for Microbiology
- American Association for the Advancement of Science
- American Society of Gene and Cell Therapy
- Society for Immunotherapy of Cancer
- Southern Society for Clinical Investigation
- American Society for Clinical Investigation
- Association of American Physicians
- Member, F1000 Immunology

Scott W. Canna, MD

RESEARCH

Inflammation is a core pathogenic mechanism in virtually every disease process. Systemically, this culminates in the systemic inflammatory response syndrome (SIRS), identified since ancient times as sepsis. Blocking inflammation in SIRS has been largely disappointing, conferring neither broad benefit nor harm. Scott Canna's research uses genetic and functional insights from patients and model systems to find ways to subtype patients with SIRS/sepsis in diagnostically and therapeutically meaningful ways.

Patients found to have monogenic defects causing excessive innate immune responses have been particularly helpful. Most "autoinflammatory" patients have chronic organ-specific or systemic inflammation but not typically SIRS. The dramatic response of many "autoinflammatory" patients

to inhibition of the inflammasome-activated cytokine IL-1 has reinvigorated the quest for anti-inflammatory targets in SIRS and reinforced the therapeutic potential of targeting the inflammasome and related innate immune pathways.

Canna's group studies the intersections of hyper- and auto-inflammation. In particular, the group studies two related disorders, hemophagocytic lymphohistiocytosis (HLH) and macrophage activation syndrome (MAS), because they typify the concept of hyperinflammatory SIRS. Whereas the pathogenesis of HLH clearly includes the inflammatory effects of defects in granule-mediated cytotoxicity, the mechanisms at work in MAS are less clear. The researchers combine clinical insights from rheumatology and innate immunity with basic models of overwhelming systemic inflammation to define new disease subtypes and disease activity biomarkers, to flesh out mechanisms of inflammatory disease, and to test promising therapeutic strategies.

STUDY SECTIONS

- Abstract/grant review, Scientific Review Committee, Childhood Arthritis and Rheumatology Research Alliance
- Peer reviewer, Basic Science Section, Rheumatology Research Foundation Innovative Research Award
- Abstract reviewer, annual scientific meeting, American College of Rheumatology
- Peer reviewer, Fondation Innovations en Infectiologie

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- American College of Rheumatology
- Childhood Arthritis and Rheumatology Research Alliance Histiocyte Society

EDITORSHIPS

- Editorial Board, *Arthritis and Rheumatology*
- Editorial Board, *Frontiers in Immunology*

MAJOR LECTURESHIPS AND SEMINARS

- "IL-18 at the Intersection of Auto- and Hyper-inflammation," University of Pittsburgh Department of Immunology Seminar Series, Pittsburgh, Pa., December 2016
- "Monogenic Inflammation: From Inflammasomes to SIRS," Pediatric Academic Society annual meeting, Baltimore, Md., May 2016
- "IL-18 at the Intersection of Auto- and Hyper-inflammation," invited presentation, KFO 249 Symposium: Defects of the Innate Immune System in Autoinflammation and Autoimmunity, Dresden, Germany, August 2017
- "IL-18 at the Intersection of Auto- and Hyper-inflammation," invited presentation, Federation of Clinical Immunology Societies annual meeting, National Institutes of Health (NIH) Immunology Interest Group Symposium, Chicago, Ill., June 2017
- "Chronic IL-18 of Diverse Origins Defines and Drives the Hyperinflammatory Macrophage Activation Syndrome," Keystone Pattern Recognition Signaling Symposium, Banff, Alberta, Canada, March 2017
- "Cytokines in Systemic Inflammation: Following the Monogenic Breadcrumb Path," Children's Hospital of Pittsburgh Molecular Medicine Research Seminar, Pittsburgh, Pa., January 2017

Kong Chen, PhD

RESEARCH

Kong Chen is studying memory Th17 responses using mouse models of *Klebsiella pneumoniae*. Chen's previous work (*Immunity*, 2011;35(6):997-1009) demonstrated that immunization-induced Th17 cells provide serotype/antibody-independent protection against a variety of strains of *K. pneumoniae*, including the recently described multidrug-resistant New Delhi metallo-beta lactamase strain. These Th17 cells recognized conserved *Klebsiella* outer-membrane proteins (OMPs), and immunization with OMPs also conferred serotype/antibody-independent protection. Current work includes understanding the mechanism of generation of these memory Th17 cells, cloning the highly conserved OMPs from *K. pneumoniae*, and testing their immunogenicity as Th17-based vaccine candidates. Successful vaccine strategy can be used not only in prevention of multidrug-resistant bacteria outbreaks, but also in prophylactic treatment for immunocompromised patients.

Additionally, Chen is studying memory Th17 responses using macaque models of simian immunodeficiency virus (SIV) and *Streptococcus pneumoniae*. Both *K. pneumoniae* and *S. pneumoniae* are extracellular pathogens with polysaccharide capsules. The data from a mouse model with *K. pneumoniae* suggest that Th17 cells can provide clade-specific immune protection regardless of capsular serotypes. Chen found that Th17 responses are critical for pulmonary host defense against *S. pneumoniae* in rhesus macaques. Th17 cell frequency is increased in the antiretroviral therapy–treated SIV-infected macaques, which is associated with improved antibacterial immune responses after pulmonary *S. pneumoniae*. Ongoing research using next-generation sequencing will improve the understanding of the impacts of SIV, antiretroviral therapy, and bacterial infection on the lung transcriptome and identify biomarkers of mucosal immunity in the lung that may assist in disease management of individuals infected with human immunodeficiency virus.

Chen also studies pathogenic Th17 cells, especially their inflammatory roles leading to emphysema and lung cancer. He found that cigarette smoke is a strong Th17 adjuvant in the lung and that the Th17 pathway is involved in smoke-induced emphysema in mouse models. Th17 cell frequency is increased in lung cancer animal models. Carefully phenotyping the T cells in this model will increase the understanding of Th17 pathways in cancer and provide new therapeutic targets in lung cancer.

Chen is highly interested in cutting-edge, next-generation sequencing technology; he has developed several applications and is seeking NIH and CF Foundation support. Based on the preliminary data that the production of proinflammatory chemokines and cytokines from CF epithelial cells was elevated and that this elevated production can be abolished by BET inhibitor, a small molecule that inhibits epigenetic enhancement of gene transcription, Chen hypothesizes that the enhanced production of proinflammatory mediators is, in part, controlled by an epigenetic regulatory program that will be revealed in the chromatin accessibility landscape analysis by ATAC-seq, a newly established assay for transposase-accessible chromatin using next-generation sequencing, which provides information on nucleosome positioning, chromatin accessibility, and transcription factor binding simultaneously. This study is currently supported by the CF Foundation.

STUDY SECTIONS

- Reviewer for grants, Biotechnology and Biological Sciences Research Council

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Association of Immunologists
- American Thoracic Society

EDITORSHIPS

- Lead guest editor, *Mediators of Inflammation*

HONORS AND AWARDS

- Outstanding Research Award, Pittsburgh-Munich International Lung Conference, 2016
- American Thoracic Society Abstract Scholarship, 2016

MAJOR LECTURESHIPS AND SEMINARS

- "Epithelial IL-17R Is Required for Pulmonary Host Defense Against *K. Pneumoniae*," Gordon Research Conference on Biology of Acute Respiratory Infection, 2016
- "Anti-Inflammatory Effects of Bromodomain and Extra-Terminal Domain Inhibition in Cystic Fibrosis Lung Inflammation," American Thoracic Society international conference, 2016
- "Updates on Research Projects Supported by the CF Foundation," CF Center Seminar, University of Pittsburgh, Pittsburgh, Pa., January 2017

Timothy W. Hand, PhD

RESEARCH

The major goal of Timothy W. Hand's research is to describe the role of immune response in intestinal disease. The laboratory is focused on the following projects.

The Role of Antibodies in Preventing the Development of Necrotizing Enterocolitis and Pediatric Crohn's Disease. In collaboration with Mike Morowitz and Kevin Mollen, Hand is looking at whether monoclonal antibodies can be used to block the development of these diseases. In addition, he is investigating whether deficits in secreted antibody contribute to the development of disease.

Mucosal T-Cell Memory. Hand is looking at the factors necessary for the development of an effective memory T-cell response in the gastrointestinal tract and how these may be affected by chronic gastrointestinal conditions.

T-Cell Regulation and Inflammatory Bowel Disease. Hand is looking at the cellular requirements to prevent immunopathology in the gastrointestinal tract driven by T-cell responses against the commensal microbiota.

Immune Dysfunction in the Intestine of CF Patients. Using intestine-specific CF transmembrane conductance regulator knockout mice, the Hand laboratory is investigating whether loss of control over the microbiota by a dysfunctional immune response contributes to distal intestinal obstruction syndrome and cirrhosis.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Association of Immunologists
- Society for Mucosal Immunology
- Faculty of 1000

STUDY SECTIONS

- Grant reviewer, Wellcome Trust (United Kingdom)
- Grant reviewer, Deutsche Forschungsgemeinschaft (Germany)

MAJOR LECTURESHIPS AND SEMINARS

- "Establishing a New Gnotobiotic Facility: Education, Missions, and Accommodating Success," Institute

for Laboratory Animal Research Workshop on Gnotobiotics, National Academy of Sciences, Washington, D.C., 2016
- "Long-Term Immune Consequences of Gastrointestinal Infection," Molecular Medicine Research Seminar, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., 2016
- "A Holistic Approach to Immune-Bacterial Interactions in the Gastrointestinal Tract," joint immunology/microbiology departmental retreat, Pittsburgh, Pa., 2016
- "Chronic Effects of Acute Gastrointestinal Infection," Senior Vice Chancellor Seminar Series, Pittsburgh, Pa., 2016
- "Staying Frenemies—The Love/Hate Relationship Between the Microbiota and the Immune System," Science 2016 Game Changers, Pittsburgh, Pa., 2016
- "Immunity to Intestinal Bacterial Colonization," Louisiana State University Veterinary College, Baton Rouge, La., 2017
- "Maternal Antibodies, the Neonatal Microbiota, and Necrotizing Enterocolitis," American Society for Investigative Pathology, Pittsburgh Pa., 2017
- "Maintenance of the Host/Microbiome Relationship in the Intestine," Microbiology and Molecular Genetics Seminar, University of Pittsburgh, Pittsburgh Pa., 2017
- "Maintenance of the Host/Microbiome Relationship in the Intestine," Molecular Medicine Research Seminar, Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., 2017
- "Maintenance of the Host/Microbiome Relationship in the Gastrointestinal Tract," plenary speaker, Cold Spring Harbor Laboratory meeting: Fundamental Immunology and Its Therapeutic Potential, Cold Spring Harbor, N.Y., 2017

Bernhard Kühn, MD, FACC

RESEARCH

The research laboratory that Bernhard Kühn directs has three interconnected goals: to understand the mechanisms of growth and regeneration in the heart; to provide mechanistic explanations for the huge differences in regenerative activity that exist in biology; and, drawing on the answers to these two fundamental questions, to conduct translational research for the diagnosis and treatment of heart muscle diseases. Prior to Kühn's work, it was commonly thought that heart muscle cells, cardiomyocytes, are in irreversible proliferative arrest after birth and that myocardial regeneration cannot be increased in mammals. Physicians and scientists were skeptical that it would be possible to stimulate cardiomyocyte proliferation after birth, let alone that this mechanism would regenerate myocardium. Kühn is credited with demonstrating that the postnatal mammalian heart has cardiomyocytes that can be stimulated to divide and that this process gives rise to myocardial regeneration. Kühn has developed an approach of using extracellular factors to stimulate cardiomyocyte proliferation. A peptide of periostin, a component of the extracellular matrix, stimulates cardiomyocyte proliferation and myocardial regeneration in a rat model of myocardial infarction. This work was published in *Nature Medicine* in 2007. Kühn followed up this seminal study with large-animal experiments published in 2012.

Kühn has shown that administration of neuregulin, a growth factor produced by endothelial cells in the heart muscle, stimulates cardiomyocyte proliferation and myocardial regeneration in animals. This paper was published in 2009 in *Cell*, the most prestigious journal for basic laboratory investigations. The work is especially significant because it raises the possibility of using subcutaneous administration of recombinant neuregulin in human patients.

Kühn has shown that both factors activate the same cellular mechanism, which is proliferation of a subpopulation of mononucleated cardiomyocytes. He has identified the receptors and intracellular pathways by which periostin peptide and neuregulin act on cardiomyocytes.

Kühn has developed a cellular growth chart of the human heart. His model shows that cardiomyocyte proliferation and enlargement contribute to developmental myocardial growth between birth and adulthood. This growth model significantly advances the conventional model, which had been the basis for medical textbooks since the 1950s. Kühn published this paper in *Proceedings of the National Academy of Sciences* in 2013. Kühn's growth model of the human heart led him to make three important predictions. First, young humans may be able to regenerate heart muscle. Second, cardiomyocyte proliferation may be a mechanism that is altered in myocardial diseases. Third, it raises the possibility to stimulate cardiomyocyte proliferation therapeutically in children with the goal of promoting myocardial regeneration. Kühn's current research efforts are based on these three advances.

STUDY SECTIONS

- Exploratory Grant Review Committee, Maryland Stem Cell Program
- Special Emphasis Panel, Pathway to Independence Awards, NIH
- Special Emphasis Panel, Program Project Parent Review Committee, National Heart, Lung, and Blood Institute, NIH
- Ad hoc reviewer, French Muscular Dystrophy Association
- Cardiac Contractility, Hypertrophy, and Failure Study Section, NIH
- Grant Review Committee, Translational Research Program, Boston Children's Hospital
- Biomaterials and Biointerfaces Study Section, NIH
- Special Emphasis Panel ZRG1 CVRS E, NIH
- Ad hoc reviewer, Research Foundation Flanders (Belgium)
- Ad hoc reviewer, Telethon Italy
- Ad hoc reviewer, Israel Science Foundation
- Basic Cell GE2 Study Section, American Heart Association

HONORS

- American Society of Clinical Investigation, 2016

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- American College of Cardiology
- American Association for the Advancement of Science
- Elected member, American Society of Clinical Investigation

MAJOR LECTURESHIPS AND SEMINARS

- "Postnatal Mammalian Cardiomyocyte Proliferation—When and How Much?" invited speaker and panelist, Weinstein Cardiovascular Development and Regeneration Conference, 2016
- "Growth Factor Injection for the Treatment of Heart Failure Cardiovascular Outcomes," McGowan Institute for Regenerative Medicine Retreat, University of Pittsburgh, 2016
- "From Current Understanding to New Regenerative Concepts," invited plenary speaker, Third Munich Conference on Cardiac Development, 2016
- "Genetic and Genomic Models of Polyploidy," invited speaker and workshop, Allied Genetics Conference, Genetics Society of America, 2016
- Invited plenary speaker, Victor Chang Cardiac Research Institute 17th International Symposium, Sydney, Australia, 2016
- "Neuregulin Stimulation of Human Myocardium Reveals a Therapeutic Window," invited plenary speaker, American Heart Association annual scientific sessions, 2016
- "Targeting Cardiomyocyte Proliferation for Heart Regeneration," research seminar, John A. Burns School of Medicine, University of Hawaii at Manoa, 2016
- "Generation of Heart Muscle Cells in Humans," research seminar, Pediatric Cardiology, Children's Hospital of Philadelphia, 2017
- "Heart Muscle Regeneration in Mammals," Developmental and Stem Cell Biology Colloquium, Duke University, 2017
- "Heart Muscle Regeneration in Mammals," research seminar, McAllister Heart Institute and Integrative Program for Biological and Genome Sciences, University of North Carolina Chapel Hill, 2017
- "A Transcriptional Map of Human Heart Muscle Cell Differentiation at the Single Cell Level," Single Cell User Group Meeting, University of Pittsburgh Medical School, 2017

Pawan Kumar, PhD

RESEARCH

Pawan Kumar is studying the role of IL-17 in regulating commensal microbiota colonization. Kumar found that enteric IL-17R signaling is critical for regulating colonization of segmented filamentous bacteria. His recent work suggests that enteric IL-17A/F signaling regulates the gut microbiota by regulating the levels of α-defensins, Nox1, and pIgR expression and that abrogation of this signaling pathway leads to gut dysbiosis, dysregulated expansion of Th17 cells, and an increased predisposition to autoimmunity. Kumar studies the role of IL-17R signaling in secretory and paneth cell development and function. Kumar's research will provide insights into IL-17-dependent regulation of host defense pathways and thus the interaction among the microbiome, intestinal immune cells, and enteric and autoimmune inflammation.

Kumar studies the role of enteric IL-17R signaling in regulating autoimmune hepatitis. IL-17R knockout mice are resistant to autoimmune hepatitis; Kumar's group found that IL-17RA knockout mice have expanded IL-22 responses in the serum. IL-22 has a protective role in the liver. Furthermore, intestinal IL-17R signaling regulates commensal microbiota-dependent IL-22 generation. Kumar discovered that intestinal IL-17R knockout mice have expanded IL-22 generation. Thus, IL-17R-dependent, gut-derived IL-22 could play a dominant role in hepatocyte regeneration and protection. A concanavalin-mediated liver-injury model will be used to investigate intestinal

IL-17R-signaling-dependent regulation of IL-22 in hepatocyte regeneration.

Kumar is also interested in studying the metabolic pathway in regulating Th17 cells, especially the role of Aryl hydrocarbon receptor (Ahr), Nrf2, and SOD3 axis in modulating metabolic programming of T cells. He found that Ahr-dependent regulation of Nrf2 and SOD3 regulates Th17 differentiation *in vitro*.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Association of Immunologists

Amanda C. Poholek, PhD

RESEARCH

The goal of the research program in Amanda C. Poholek's laboratory is to understand how transcription factor networks are regulated in a cell-type-specific manner to control the differentiation and function of immune cells. The Poholek laboratory is focused on the regulation and function of the transcriptional repressor Blimp-1 across multiple immune cell types. Blimp-1 is associated with constraining T-cell-mediated autoimmune disease and is highly expressed in exhausted T cells present in chronic viral infection and the tumor microenvironment. In addition, Blimp-1 is the master regulator of plasma cell formation in B cells. Therefore, Blimp-1 has wide-ranging functions depending on cell type, suggesting that its role in disease is context-dependent. Currently, the team is focused on T cells to understand the factors that regulate expression of Blimp-1 in CD4 and CD8 T cells, as well as to identify the non-coding genetic regulatory elements such as enhancers that are critical for cell-type-specific expression of Blimp-1.

The Poholek laboratory is also exploring the genes regulated by Blimp-1 in CD4 T-cell subsets using next-generation sequencing technologies such as ChIP-seq, RNA-seq, and ATAC-seq to understand the function of Blimp-1 in T cells. In addition, the team is extending *in vitro* observations to clinically relevant diseases such as allergic asthma to understand the functional consequences of Blimp-1 in disease settings.

Regulation of Blimp-1 in Immune Cells. The long-term goal is to identify the extracellular signals and genetic elements that control Blimp-1 expression in various immune cell types to understand how the regulation of Blimp-1 expression constrains T-cell-mediated autoimmunity or promotes allergic asthma.

Function of Blimp-1 in Immune Cells. The aim of this project is to identify the genes regulated by Blimp-1 both directly and indirectly in various immune cell types to understand the context-dependent and cell-type-specific functions of Blimp-1 in various disease settings.

Identify Blimp-1-Mediated Allergic Asthma Disease. The aim of this project is to understand why the selective absence of Blimp-1 in T cells results in reduced lung inflammation and development of allergic airway disease.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Association of Immunology

MAJOR LECTURESHIPS AND SEMINARS

- "IL-10 Induces an Autoregulatory Loop in T Cells That Promotes Blimp-1 Restriction of Cell Expansion," platform presentation, Cold Spring Harbor Laboratory meeting: Gene Expression and Signaling in the Immune System, Cold Spring Harbor, N.Y., April 2016

THREE-YEAR BIBLIOGRAPHY

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PAUL C. GAFFNEY

DIAGNOSTIC REFERRAL SERVICE

Mission

The mission of the Paul C. Gaffney Diagnostic Referral Service is:

- To provide the most comprehensive and coordinated health care for children
- To provide medical education of the highest quality to students, residents, practicing physicians, and other health care workers
- To contribute to the pediatric body of knowledge and care of patients by participating in research projects and writing case reports, reviews, books, and book chapters that will provide guidance to other health care workers, as well as participating in continuing education conferences that enhance learning and improve the care of patients

FACULTY

Basil J. Zitelli, MD
Professor of Pediatrics
Chief, Paul C. Gaffney Diagnostic Referral Service

Lauren Alessi, MD
Assistant Professor of Pediatrics

Andrew Buchert, MD
Assistant Professor of Pediatrics

Carol Lynn Cabral, MD
Assistant Professor of Pediatrics

Sylvia S. Choi, MD
Associate Professor of Pediatrics

Michael Decker, MD
Assistant Professor of Pediatrics

Stephanie B. Dewar, MD
Associate Professor of Pediatrics

Leigh Anne DiCicco, MD
Assistant Professor of Pediatrics

Allison Fleischer, MD
Assistant Professor of Pediatrics

Jessica Garrison, MD
Assistant Professor of Pediatrics

Catalina Hoyos, MD
Assistant Professor of Pediatrics

Joyce Leifer, MD
Clinical Associate Professor of Pediatrics

Andrew McCormick, MD
Assistant Professor of Pediatrics

Sara C. McIntire, MD
Professor of Pediatrics

Benjamin Miller, MD
Assistant Professor of Pediatrics

Laura Navarro-Borelly, MD
Assistant Professor of Pediatrics

Laura Panko, MD
Assistant Professor of Pediatrics

Catherine Polak, MD
Assistant Professor of Pediatrics

Allison Rometo, MD
Assistant Professor of Pediatrics

Erin Schaffner, MD
Assistant Professor of Pediatrics

Elizabeth Sensenig, MD
Assistant Professor of Pediatrics

Tony Tarchichi, MD
Assistant Professor of Pediatrics

Andrew H. Urbach, MD
Professor of Pediatrics

Kishore Vellody, MD
Associate Professor of Pediatrics

OVERVIEW OF DIVISION

The division has a long history of clinical service, dating back to the early days of the Department of Pediatrics under Edmund R. McCluskey in 1948. McCluskey was a preeminent consultant pediatrician who tapped Paul C. Gaffney to take up the mantle he had carried for more than 25 years. The Diagnostic Referral Service has 24 physicians, with more than 16 clinical full-time equivalents. Activities include serving as hospitalists and providing inpatient care and consultation for many acutely ill and chronically ill children. Hospitalist activity has increased yearly, such that the division now provides attending physician services for 100% of children admitted under inpatient or observation status to the general pediatrics service. In addition to this main role as inpatient hospitalists, duties and services provided by the division include an emphasis on close communication and referral back to local physicians for ongoing care; general pediatrics inpatient consultation services for medical and surgical subspecialties; follow-up of complex patients; rounding on pediatric patients in the pediatric transitional care program at the Children's Home of Pittsburgh and Lemieux Family Center; physician support for inpatient and ambulatory Condition HELP calls; and staffing of the ambulatory consultation clinic, the Down Syndrome Clinic, the Feeding and Swallowing Clinic, and the Vascular Anomalies Clinic. The Diagnostic Referral Service directs and manages the Down Syndrome Center of Western Pennsylvania, and Down Syndrome Clinic sites have been added to include Wexford, Hermitage, Erie, and Chippewa, Pa.

The division's major strengths lie in the clinician-educator role. Faculty members have major clinical responsibilities to see patients referred to Children's Hospital of Pittsburgh of UPMC by physicians throughout Western Pennsylvania and surrounding states, as well as from various national and international facilities. The patients in the faculty's care frequently are among the most medically challenging and complex. Many of them have multisystem diseases that do not neatly fit into one subspecialty category, so the division aids in the coordination of care. Many patients are technologically dependent, including ventilator-dependent children. Each of the members of the division has excelled in his or her clinical training program and has been recognized as a superb clinician. The division

has achieved a high degree of respect among subspecialists and community physicians. The Diagnostic Referral Service physicians are also involved in education at all levels: medical students, including preclinical and clinical levels; residents in pediatrics, medicine-pediatrics, triple-board, and family practice; and practicing physicians via continuing medical education courses at the regional, national, and international levels.

The evening hospitalist program provides in-house attending coverage and education for students and residents. The evening hospitalist has three main functions: (a) provide for patient safety by being available to house staff, nurses, and families to address immediate concerns, including being a part of the Condition HELP rapid response team in the evening; (b) work up and evaluate Diagnostic Referral Service admissions; and (c) provide attending-level education to the night house-staff team. The team is also available to answer phone calls from families and physicians. Teaching effort is substantial to residents and students, as evening hospitalists can directly observe histories and physical examinations. This program has been extremely successful, and admissions have risen steadily for the evening team to more than 1,500, with informal house-staff consultations averaging two per evening, over and above any formal consultations from other services.

The division's referral activities enhance systemwide efficiency, quality of care, and the patient experience. The services of the division attract Children's Community Pediatrics physicians and other community physicians to refer inpatients to the Diagnostic Referral Service and to refer outpatients to the Diagnostic Referral Service Ambulatory Program. The resultant increase in ambulatory activity is accommodated by decreased wait times for patients who require evaluation. The average time to an appointment in the Diagnostic Referral Service remains less than three calendar days and is often less than 24 hours. Beginning in May 2017, the division implemented same-day ambulatory access, wherein patients can be seen on the same day of referral. Patients are triaged to some degree by the Diagnostic Referral Service and, if necessary, referred to subspecialty clinics. This substantially decreases wait times for subspecialty evaluations, increases patient satisfaction, increases physician satisfaction, and ultimately increases ambulatory referrals.

The division runs a limited teaching service, in which faculty members care for patients with limited involvement from house staff. Daytime activities include having a senior resident work directly with the hospitalist to learn the basics of how to be a hospitalist while another hospitalist covers that service at night without resident support. This service provides around-the-clock, in-house coverage for that team's patients. This has decreased the overall patient census for the general pediatric house-staff teams, better allowing the residency program to comply with the resident duty-hour regulations.

The Diagnostic Referral Service provides medical leadership and staffing for the Feeding and Swallowing Center of Children's Hospital, which is an interdisciplinary team consisting of speech pathology, occupational therapy, behavioral health, nutrition, and nursing. The team supports an inpatient feeding program at the Children's Home of Pittsburgh and Lemieux Family Center.

CLINICAL ACTIVITIES

INPATIENT ACTIVITY

In addition to evaluating and managing patients admitted to the service, consulting regarding subspecialty services, providing post-hospital follow-up when necessary, and coordinating care for medically complex patients, the division helps create a medical home for each patient in a joint effort with the patient's primary care physician. Inpatient hospitalist activity falls under the following five major categories.

- Serving as hospitalist for all general medical patients admitted to the inpatient units (under inpatient or observation status)
- Serving as attending physician in the observation unit
- Serving, in conjunction with Behavioral Health, as medical attending physician for patients with eating disorders
- Serving, in conjunction with the Division of Genetic and Metabolic Disorders, as medical attending physician for patients with genetic and/or metabolic disorders
- Serving patients at the Children's Home of Pittsburgh and Lemieux Family Center

The Diagnostic Referral Service provides 100% of attending activity on the inpatient ward. The division supervises the observation unit, where the attending physician conducts intense one-on-one teaching of residents and students. For fiscal year 2016, the Diagnostic Referral Service cared for more than 5,000 inpatients and observation patients on its service, representing 25% of Children's Hospital's acute-care and observation discharges. The average length of stay for acute-care patients in the Diagnostic Referral Service was 4.0 days, which compares favorably to Children's Hospital's average length of stay of 6.2 days.

The Diagnostic Referral Service increasingly is caring for subspecialty patients whose diagnoses fall into the realm of general pediatrics. Patients with certain neurological or gastrointestinal disorders are admitted to the Diagnostic Referral Service. In addition, the Diagnostic Referral Service cares for all medical adolescent, allergy, ophthalmology, dermatology, dental, and genetic metabolic patients, as well as all ear-nose-throat patients without acute hemorrhage or airway compromise. The Diagnostic Referral Service has been designated as the pediatric service for the Center for Cleft Palate and Craniofacial Deformities, and it works closely with Plastic Surgery and Dermatology for inpatient management of patients with hemangiomas and vascular anomalies.

AMBULATORY ACTIVITY

The Diagnostic Referral Service continues to meet and exceed service-standard guidelines by seeing ambulatory patients within a three-day waiting period. Frequently, patients can be seen either the same day or within 24 hours, if necessary. The volume of ambulatory activity in the Diagnostic Clinic, the Down Syndrome Clinic, the Feeding Clinic, and the Vascular Anomalies Clinic remains on the rise.

DOWN SYNDROME CENTER OF WESTERN PENNSYLVANIA

The Diagnostic Referral Service directs and manages the Down Syndrome Center of Western Pennsylvania, which is under the leadership of Kishore Vellody. Patient volume has steadily increased from 361 prior to his assumption of leadership to more than 500 patients this past year. Moreover, new patients' wait times have decreased from up to five months to three days. He has also created the only podcast in the world focused on medical issues in Down syndrome. Since its inception in 2013, it has been downloaded more than 21,000 times in more than 60 countries. In addition, he created and maintains a Down syndrome blog, which has had 6,000+ page views in 40 countries and counting.

RESEARCH AND OTHER SCHOLARLY ACTIVITIES

Basil J. Zitelli, MD

RESEARCH

The Diagnostic Referral Service participates in clinical research activities, such as supporting resident clinical research projects. Faculty members have published and presented several case reports. Many members are involved in quality-improvement projects that have been accepted for publication and presentation through peer-review processes. In addition, several members' primary scholarly work focuses on medical education, which has included presentations at national conferences.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Hospitalist Medicine Section, American Academy of Pediatrics
- Alpha Omega Alpha
- Society of Hospital Medicine

MAJOR LECTURESHIPS AND SEMINARS

- "Visual Diagnosis Marathon," 20th Annual General Pediatrics Review and Self-Assessment, Miami, Fla., May 2017

HONORS

- Academy of Master Educators, University of Pittsburgh School of Medicine
- Best Doctors, *Pittsburgh Magazine*
- *Best Doctors in America*, Woodward/White, Inc.

Basil J. Zitelli, MD
Division Chief, Paul C. Gaffney Diagnostic Referral Service

Lauren Alessi, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Society of Critical Care Medicine
- Alpha Omega Alpha
- Gold Humanism Society

MAJOR LECTURESHIPS AND SEMINARS

- "Multi-Drug-Resistant Organism Sepsis in Pediatric Liver Transplant Recipients—A Burgeoning Problem," 46th Annual Critical Care Congress, Honolulu, Hawaii, January 2017

ADVISORY COMMITTEE MEMBERSHIPS

- Hospitalist representative, Improved Pediatric Sepsis Outcomes (IPSO) Committee
- Facilitator, end-of-life care education sessions for faculty and residents
- Faculty advisor, Pediatric Residency Global Health Interest Group, Children's Hospital of Pittsburgh
- Co-champion, Quality-Improvement Analysis of Clinical Effectiveness Guideline for Abnormal Uterine Bleeding, Children's Hospital of Pittsburgh

HONORS

- A. Vincent Londino Jr., MD, Pediatric Senior Resident Award
- Glasgow-Rubin Certificate of Commendation, American Medical Women's Association

Andrew Buchert, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- American College of Physician Executives

ADVISORY COMMITTEE MEMBERSHIPS

- Medical director, Education Outreach and Clinical Resource Management, Children's Hospital
- Chair, Clinical Resource Management Committee, Children's Hospital
- Hand Hygiene Committee, Children's Hospital
- Co-chair, Patient Safety and Quality-Improvement Committee, UPMC Medical Education

Lynn Cabral, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics

HONORS

- *Best Doctors in America*, Woodward/White, Inc.
- Best Doctors, *Pittsburgh Magazine*

Sylvia S. Choi, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Ambulatory Pediatric Association
- Alpha Omega Alpha
- Academy of Breastfeeding Medicine

MAJOR LECTURESHIPS AND SEMINARS

- "A Pediatrician's Approach to Feeding Problems in Early Childhood," Practice and Patience: Strategies to Address Feeding Problems in Early Childhood, Pittsburgh, Pa., September 2016
- "Death and Dying: A One-Day Course to Enhance Pediatric Resident Education," 21st International Congress on Palliative Care, Montreal, Canada, October 2016
- "Teaching Residents to Mitigate Prejudice: Role Play With Simulated Parents to Address Prejudice in the Workplace," workshop, Association of Pediatric Program Directors (APPD) annual spring meeting, Anaheim, Calif., April 2017

HONORS

- Best Doctors, *Pittsburgh Magazine*
- *Best Doctors in America*, Woodward/White, Inc.

ADVISORY COMMITTEE MEMBERSHIPS

- Codirector, Three Rivers Pediatric Update
- Codirector, Feeding and Swallowing Center conference
- Facilitator and member of the planning committee, Schwartz Center rounds

Michael Decker, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Society of Pediatric Hospital Medicine
- Council on Medical Student Education in Pediatrics

ADVISORY COMMITTEE MEMBERSHIPS

- Physician leader, Condition HELP
- Codirector, Three Rivers Pediatric Update

HONORS

- *Best Doctors in America*, Woodward/White, Inc.
- Best Doctors, *Pittsburgh Magazine*

Stephanie B. Dewar, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Alpha Omega Alpha
- APPD

MAJOR LECTURESHIPS AND SEMINARS

- "Faculty Development and Engagement," invited lecture, Association of University Professors of Ophthalmology: Educating the Educators, San Diego, Calif., January 2017
- "Tense Tummies in Kids," 44nd Refresher Course in Family Medicine, Pittsburgh, Pa., March 2017
- "Promoting Diversity in the Pipeline of Physicians: Recruiting and Mentoring of Under-Represented Minority Physicians," APPD, 2017 annual meeting, Anaheim, Calif., April 2017
- "Teaching Residents to Mitigate Prejudice: Role Play With Simulated Parents to Address Prejudice in the Workplace," workshop, APPD 2017 annual spring meeting, Anaheim, Calif., April 2017

HONORS

- Academy of Master Educators, University of Pittsburgh School of Medicine

Leigh Anne DiCicco, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Alpha Omega Alpha

Allison Fleischer, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics

ADVISORY COMMITTEE MEMBERSHIPS

- Emergency Preparedness Leadership Committee, Children's Hospital of Pittsburgh
- Physician co-lead, Falls PI Team, Children's Hospital of Pittsburgh

Jessica Garrison, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Alpha Omega Alpha

ADVISORY COMMITTEE MEMBERSHIPS

- Co-chair, Booksburgh, project to promote early literacy, Children's Hospital
- Insurance Denials Committee, Children's Hospital

Catalina Hoyos, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Global Health Educator Group, APPD

ADVISORY COMMITTEE MEMBERSHIPS

- Diversity and Inclusion Committee, Children's Hospital
- Co-chair, Booksburgh, project to promote early literacy, Children's Hospital
- Faculty advisor, pediatric residency Global Health Interest Group, Children's Hospital of Pittsburgh
- Director, pediatric residency Global Health Curriculum, Children's Hospital of Pittsburgh

MAJOR LECTURESHIPS AND SEMINARS

- "Common Pediatric Problems in Tropical Settings," Global Health Month and Parasitology Course for UPMC Internal Medicine Global Health Track, Pittsburgh, Pa., October 2017

Joyce Leifer, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Alpha Omega Alpha

HONORS

- *Best Doctors in America*, Woodward/White, Inc.
- Best Doctors, *Pittsburgh Magazine*

Andrew McCormick, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Alpha Omega Alpha
- Down Syndrome Medical Interest Group
- International Society for the Study of Vascular Anomalies

MAJOR LECTURESHIPS AND SEMINARS

- "Patient Information on the Internet: Readability and Quality of Websites Regarding Hemangioma and Vascular Anomalies," International Society for Study of Vascular Anomalies, 21th International Workshop on Vascular Anomalies, Buenos Aires, Argentina, 2016
- "Infantile Hemangiomas Are Often Present at Birth," International Society for Study of Vascular Anomalies, 21th International Workshop on Vascular Anomalies, Buenos Aires, Argentina, 2016
- "Healthy Transitions: Pathway to Adulthood (Quality-Improvement Project)," Council on Quality Improvement and Patient Safety Program, American Academy of Pediatrics national conference and exhibition, San Francisco, Calif., 2016
- "Infantile Hemangioma: An Evolution in Care," National Healthcare Quality Week and International Infection Prevention Week, Pittsburgh, Pa., 2017
- "Vascular Tumors of the Head and Neck," pediatric ear, nose, and throat grand rounds, Pittsburgh, Pa., 2016
- "Healthy Transitions: Pathway to Adulthood," Pennsylvania Community on Transition conference, Hershey, Pa., 2017
- "Have a Question? Ask the Doctor!" National Down Syndrome Congress, Orlando, Fla., 2016
- "Healthy Transitions: Pathway to Adulthood," National Down Syndrome Congress, Sacramento, Calif., 2017
- "Have a Question? Ask the Doctor!" National Down Syndrome Congress, Sacramento, Calif., 2017

HONORS

- Honor Roll Award for Patient Experience, 2016
- Clerkship Preceptor of the Year Award, Pediatrics, 2017

ADVISORY COMMITTEE MEMBERSHIPS

- Chair, Med/Peds Clinical Competency Committee, Children's Hospital of Pittsburgh
- Physician leader, Condition HELP, Children's Hospital of Pittsburgh
- Member, Wellness Task Force, Children's Hospital of Pittsburgh
- Member, Transitional Care Task Force, Children's Hospital of Pittsburgh

Sara C. McIntire, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics
- Alpha Omega Alpha

HONORS

- Academy of Master Educators
- *Best Doctors in America*, Woodward/White, Inc.
- Best Doctors, *Pittsburgh Magazine*

ADVISORY COMMITTEE MEMBERSHIPS

- Advisory dean, University of Pittsburgh School of Medicine
- Medical Executive Committee, Children's Hospital
- Codirector, Three Rivers Pediatric Update
- Faculty Wellness Committee, Children's Hospital
- Medical Staff Campaign, Children's Hospital of Pittsburgh of UPMC Foundation

Benjamin Miller, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- Alpha Omega Alpha
- American Academy of Pediatrics
- APPD

MAJOR LECTURESHIPS AND SEMINARS

- "Pediatric Residents Interested in Medical Education: Inaugural Years and Impact," abstract platform presentation, APPD annual spring meeting, Anaheim, Calif., April 2017

ADVISORY COMMITTEE MEMBERSHIPS

- Medical Executive Committee, Children's Hospital
- Medical Executive Committee, Children's Home of Pittsburgh and Lemieux Family Center
- Medical staff president, Specialty Care Hospital, Children's Home of Pittsburgh and Lemieux Family Center
- Intern Selection Committee, Children's Hospital

HONORS

- Selected as preceptor for American Academy of Pediatrics PedsConnect, section for medical students to communicate with pediatric attendings
- *Best Doctors in America*, Woodward/White, Inc.
- Best Doctors, *Pittsburgh Magazine*

Laura Panko, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Academy of Pediatrics

MAJOR LECTURESHIPS AND SEMINARS

- "Attainment of Pediatric Developmental Milestones and Feeding Progression in Young Children: A Multidisciplinary Discussion—A Pediatrician's

Viewpoint," Children's Hospital of Pittsburgh of UPMC Feeding and Swallowing Center conference, Pittsburgh, Pa., September 2016
- "Clinical Update 2016: Practice and Patience: Strategies to Address Feeding Problems in Early Childhood," co-director report, Feeding and Swallowing Center of Children's Hospital of Pittsburgh of UPMC, Pittsburgh, Pa., September 2016

HONORS
- *Best Doctors in America*, Woodward/White, Inc.
- Best Doctors, *Pittsburgh Magazine*

ADVISORY COMMITTEE MEMBERSHIPS
- Vascular Access Clinical Effectiveness Committee and Peripheral IV Infiltration and Extravasation Subcommittee, Children's Hospital
- Nutrition Advisory Committee, Children's Hospital

Catherine Polak, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS
- American Academy of Pediatrics
- Alpha Omega Alpha

ADVISORY COMMITTEE MEMBERSHIPS
- Emergency Preparedness Leadership Committee, Children's Hospital of Pittsburgh
- Intern Selection Committee, Children's Hospital

Allison Rometo, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS
- American Academy of Pediatrics

Erin Schaffner, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS
- American Academy of Pediatrics
- Alpha Omega Alpha

ADVISORY COMMITTEE MEMBERSHIPS
- IPSO, Children's Hospital
- Empyema/Parapneumonic Effusion Clinical Effectiveness, Children's Hospital

Elizabeth Sensenig, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS
- American Academy of Pediatrics

Tony Tarchichi, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS
- Section of Hospitalist Medicine, American Academy of Pediatrics
- Section on Medicine and Pediatrics, American Academy of Pediatrics
- American College of Physicians

ADVISORY COMMITTEE MEMBERSHIPS
- Clinical Resource Management Committee, Children's Hospital
- Insurance Denial Committee, Children's Hospital
- Combined Internal Medicine and Pediatrics Residency Curriculum Committee, Children's Hospital

HONORS
- William I. Cohen Teacher of the Year Award, University of Pittsburgh School of Medicine, June 2017

Andrew H. Urbach, MD

Andrew H. Urbach is the associate chief medical officer at Children's Hospital.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS
- American Academy of Pediatrics
- American College of Physician Executives

HONORS
- Best Doctors, *Pittsburgh Magazine*
- Inclusion Award, Family-Centered Care Champion, 2013 Initiative, Parent Education and Advocacy Leadership Center
- Coulter Translational Research Partners II/System for Hospital Adaptive Readmission Prevention

ADVISORY COMMITTEE MEMBERSHIPS
- Children's Hospital Art Program
- Board member, Associated Artists of Pittsburgh
- Chief Medical Officer Advisory Board, Children's Hospital Association
- Board member, Mario Lemieux Foundation
- Austin's Playroom
- Robert Wood Johnson Foundation

Kishore Vellody, MD

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS
- President, Executive Committee, National Down Syndrome Congress

- Chair, Professional Advisory Council, National Down Syndrome Congress
- Down Syndrome Medical Interest Group

MAJOR LECTURESHIPS AND SEMINARS

- "Medical Issues in Down Syndrome," Canadian Down Syndrome Society, Banff, Alberta, Canada, May 2017
- "Have a Question? Ask the Doctor!" Canadian Down Syndrome Society, Banff, Alberta, Canada, May 2017
- "Practice-Changing Articles in Down Syndrome," Down Syndrome Medical Interest Group, Orlando, Fla., July 2016
- "Medical Issues in Down Syndrome," National Down Syndrome Congress, Orlando, Fla., May 2016
- "Sibling Issues in Down Syndrome," National Down Syndrome Congress, Orlando, Fla., May 2016
- "Have a Question? Ask the Doctor!" National Down Syndrome Congress, Orlando, Fla., May 2016
- "Navigating the Complex Health Care System," The Arc of Erie County, The Epilepsy Project, April 2017
- Podcast host, Down Syndrome Center of Western Pennsylvania
- *Dear Self*, blog about Down syndrome

HONORS

- Academy of Master Educators, University of Pittsburgh School of Medicine
- President, Executive Committee, National Down Syndrome Congress
- Co-chair, Professional Advisory Council, National Down Syndrome Congress

TEACHING ACTIVITIES

Members of the Diagnostic Referral Service participate in the following teaching activities.

UNIVERSITY OF PITTSBURGH SCHOOL OF MEDICINE

- Advisory dean (McIntire)
- Chair, Committee for Affiliated Faculty Promotions and Appointments (Zitelli)
- Director, Medical Student Pediatric Education Program (Cabral)
- Directors, Medical Student Third-Year Pediatric Inpatient Rotation (Cabral and Decker)
- Weekly case conference with third-year medical students (Cabral and Decker)
- Medical Student Fourth-Year Program (Panko and Rometo)
- Codirectors, Pediatric Advanced Physical Exam course (Vellody and McCormick)
- Pediatric and Adolescent Interest Group (Vellody)
- Codirectors, Advanced Pediatric Medical Interviewing course (Rometo and Garrison)
- MS1 Introduction to Medical Interviewing course (Leifer and Dewar)
- Facilitator, MS1 Behavioral Medicine Small Group (Schaffner)
- Committee member, UPMC Graduate Medical Education Quality Improvement (Dewar)
- Advisors, Faculty and Students Together
- Committee member, University of Pittsburgh School of Medicine Promotions Committee, Retention Committee (Cabral)
- Pediatric Career Advising Group
- MS III Case Conference
- MS IV Conference
- Cofacilitator, Pediatric Intake Medicine Clerkship
- Ethics Conferences (Schaffner, Tarchichi, Rometo, Fleischer, and Miller)
- Premedical Academic Enrichment Program and Prematriculation Program
- School of Medicine Introduction to Patient Care Block Committee
- Medical School Graduate Honors Committee
- University of Pittsburgh Council on Instructional Excellence Committee
- Student Leadership Committee
- Medical Student Fourth-Year Advisor Program
- Annual lecture/workshop on Down syndrome, Human Genetics course (Vellody)
- Annual lecture on Down syndrome, University of Pittsburgh Physical Therapy School (Vellody)
- Annual lecture on Down syndrome, University of Pittsburgh Principles of Genetic Counseling course (Vellody)
- Annual lecture on Down syndrome, Introduction to Being a Physician course (Vellody)
- Consultant, Children's Hospital Ethics Consultative Service (Miller)
- Director, Patient Safety and Quality-Improvement Area of Concentration (Buchert)
- Facilitator, MS2,3 Population Health course, Patient Safety Session (Buchert)
- Director, MS1, MS2 Clinical Experiences course (McCormick)

RESIDENTS

- Codirector, Residency Program (Dewar)
- Associate director, Residency Program (Miller)
- Chair, Internship Selection Committee (Zitelli)
- Chair, Internal Medicine/Pediatrics Competency Committee (McCormick)
- Codirector, Death and Dying course (Choi)
- "To Err is Human," resident sessions (Buchert, Polak, Choi, and Dewar)
- Codirector, Resident Communication Course (Choi)
- Resident clinical pathological case presentation/case report coordination (Decker and Panko)
- Director, Pediatric Residency Quality-Improvement Curriculum (Polak)
- Director/preceptor, resident ethics educational sessions (Miller)
- Director, pediatric residents interested in medical education (Miller)
- Codirectors, Evening Conference Curriculum (Garrison and Schaffner)
- Inpatient resident team attending
- Limited-stay unit faculty attending
- Resident morning report
- Resident mentor
- Residency Advisory Committee
- Resident Curriculum Committee
- Resident Performance Committee
- Resident scholarly project mentor
- Resident noon conferences
- Morbidity and mortality conference
- Teaching Residents to Teach

HOSPITAL

- Family Forum
- Condition HELP Steering Committee
- Grievance Committee
- Board member, Children's Community Pediatrics (Urbach)
- Member, Quality Oversight Committee (Choi)
- Coleader, Patient Experience Oversight Committee (Urbach)
- Member and governmental affairs physician liaison (Urbach)
- Children's Hospital Marketing Committee
- Codirectors, Three Rivers Pediatric Update (Choi, Decker, and McIntire)
- Codirectors, annual Feeding and Swallowing Clinic conference (Panko and Choi)
- Children's Hospital safety rounds (Buchert)
- Children's Hospital Ethics Committee (Buchert, Miller, and Balest)
- Medical director, Feeding and Swallowing Clinic (Choi)
- Children's Hospital Chief Medical Officer Group (Urbach)
- Executive Patient Safety Rounds Oversight Committee (Buchert)
- Patient and Family Education Committee (Decker)
- Pharmacy and Therapeutics Committee, Health Information Management Committee (Choi)
- RN/MD leadership meeting (Panko, Miller, Dewar, and Choi)
- Cerner Physician Advisory Committee (Urbach and Choi)
- Credentials Committee (Zitelli and Choi)
- Medical director, Down Syndrome Center of Western Pennsylvania (Vellody)
- Care Coordination Enhancement Work Group (Urbach)
- Fellows and residents orientation project (Urbach)
- Readmissions Reduction Task Force (Urbach)
- Medical director, nurse practitioner observation unit (Choi)
- Children's Hospital Medical Staff Executive Committee (McIntire, Buchert, and Miller)
- System for Hospital Adaptive Readmission Prediction and Management
- IPSO

THREE-YEAR BIBLIOGRAPHY

2015

Dewar JC, **Dewar SB**. Failure to thrive. In: South-Paul JE, Matheny SC, Lewis EL, eds. *Current Diagnosis and Treatment in Family Medicine.* 4th Edition. New York, NY: McGraw-Hill; 2015.

Dewar JC, **Dewar SB**. Common acute infections in children. In: South-Paul JE, Matheny SC, Lewis EL, eds. *Current Diagnosis and Treatment in Family Medicine.* 4th Edition. New York, NY: McGraw-Hill; 2015.

Lavigne J, Sharr C, Ozonoff A, Prock LA, Baumer N, Brasington C, Cannon S, Crissman B, Davidson E, Florez JC, Kishnani P, Lombardo A, Lyerly J, McCannon JB, McDonough ME, Schwartz A, Berrier KL, Sparks S,

Stock-Guild K, Toler TL, **Vellody K**, Voelz L, Skotko BG. National Down Syndrome Patient Database: Insights from the development of a multi-center registry study. *Am J Med Genet*. 2015; Part A 9999A:1-7.

Butteris SM, Schubert CJ, Batra M, Coller RJ, Garfunkel LC, Monticalvo D, Moore N, Arora G, Moore MA, Condurache T, Sweet LR, **Hoyos C**, Suchdev PS. Global Health Education in Pediatric Residency Programs. *Pediatrics*. 2015; 136(3): 458-465.

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Lawrence K, Lee B, **Dewar S**, Shah J, Dohar J, Nowak AJ, Martin J. Bacteroides bacteremia complicating otogenic Lemierre's syndrome. *Int J Pediatr Otorhinolaryngol Extra.* 2016;13:53-6.

McCormick A, Rosenberg S, Trier K, Balest A. A Case of a Central Conducting Lymphatic Anomaly Responsive to Sirolimus. *Pediatrics*. 2016;137(1).

Foster KA, Ares WJ, Tempel ZJ, **McCormick AA**, Panigrahy A, Grunwaldt LJ, Greene S. PHACE syndrome is associated with intracranial cavernous malformations. *Childs Nerv Syst*. 2016;32(8):1463-9.

McCormick AA, Sebastian J, Madan-Khetarpal S. Case Presentation: HHT with spinal AVM. Respiratory Reader. Spring 2016.

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Zitelli BJ, **McIntire SC**, Nowalk AJ (Eds.). *Zitelli and Davis' Atlas of Pediatric Physical Diagnosis* (7th ed.) Philadelphia, PA: Elsevier; 2017.

Davis KS, **McCormick AA**, Jabbour N. What might parents read: Sorting webs of online information on vascular anomalies. *Intern J Ped Otorhinolaryngology*. 2017;93:63-7.

McCormick A, Grunwaldt L. Vascular anomalies. **In: Zitelli BJ**, **McIntire SC**, Nowalk AJ (Eds.), *Zitelli and Davis' Atlas of Pediatric Physical Diagnosis* (7th ed.) Philadelphia, PA: Elsevier; 2017.

McGhee W, **Panko LM**. Pediatric gastroenteritis. In: Schwinghammer TL, Koehler JM (Eds.), *Pharmacotherapy Casebook: A Patient-Focused Approach* (10th ed.). New York: McGraw-Hill; 2017.

Schaffner E, Hazen B. Index of suspicion case: Sternal mass in adolescent boy. Xiphoid MRSA osteomyelitis. *Pediatr Rev*. 2017;38(9):437-9.

WEIGHT MANAGEMENT AND WELLNESS CENTER

Mission

The Weight Management and Wellness Center (WMWC) was founded in the fall of 2004 as a multidisciplinary center devoted to the treatment and prevention of overweight/obesity in children, as well as treatment of its comorbidities, including prediabetes, type 2 diabetes, polycystic ovary syndrome, nonalcoholic fatty liver disease, hypertension, dyslipidemia, sleep apnea, metabolic syndrome, and more. The center has a three-pronged approach, including clinical care, research, and community outreach, with research being a major component in all, to address the full spectrum of the childhood obesity problem from prevention to early intervention and evaluation of treatment strategies.

FACULTY AND STAFF

Silva Arslanian, MD
Founding Director and Chief, WMWC
UPMC Richard L. Day Endowed Chair in Pediatrics
Director, Pediatric Clinical and Translational Research Center
Professor of Pediatrics

Lesli Dahl, PhD
Clinical Behavioral Psychologist

Heba Ismail-Roberts, MB BCh, MSc, PhD
Assistant Professor of Pediatrics, Pediatric Endocrinology, Metabolism, and Diabetes Mellitus
Clinical Director, WMWC

SoJung Lee, PhD
Associate Professor of Pediatrics

John Weidinger, MPAS, PA-C
Physician Assistant

Haley Sager, PA-C
Physician Assistant

Rose Cloherty
Practice Manager

Joon Young Kim, PhD
Postdoctoral Fellow

OVERVIEW OF CENTER

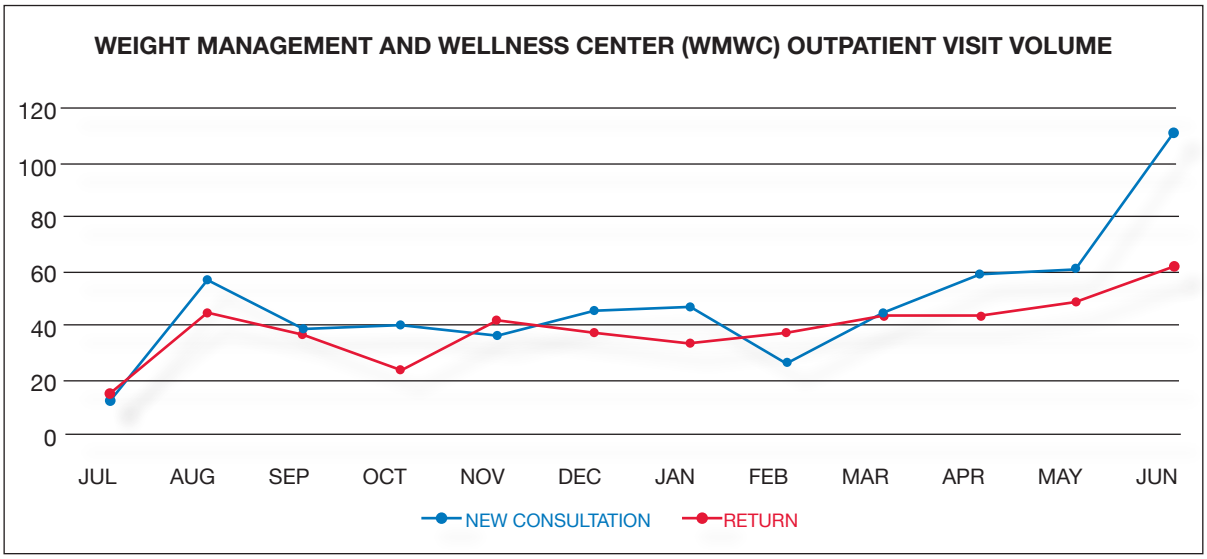
The WMWC was conceived late in 2003 based on a tremendous need by community-referring physicians and by the Children's Hospital of Pittsburgh to centralize the care of obese children and their families. Because of Silva Arslanian's accomplished research in childhood obesity, insulin resistance, and type 2 diabetes, she was asked to found and direct the center and the division with funding from the Department of Defense (DOD). Within a very short time, she recruited the needed personnel and the center reached full operational capacity. The DOD grant was instrumental in establishing and funding the WMWC. Significant divisional downsizing resulted when the DOD grant expired. Ultimately, in January 2017, clinical activities and personnel transitioned to the Division of Endocrinology. The Center for Pediatric Research in Obesity and Metabolism was established to continue research activities.

The division's successes were three-pronged. First, it provided state-of-the-art clinical care for children and their families afflicted with obesity with or without its comorbidities. Second, state-of-the-art research advanced scientific knowledge through extramural funding, including several National Institutes of Health (NIH) grants and several major protocols in addition to other ongoing projects. Third, the division provided community support for physicians, community centers, and parents to identify obesity and provide measures for prevention and treatment. The center made major contributions to various community health fairs and health-related activities in schools, with nurses, and with community physician offices, tackling the problem of obesity on a community level. The team supplied physicians with education and training to integrate the weight-management program into their community-based practices. The center partnered with Children's Community Pediatrics (CCP) practices to develop a model of health care delivery that promotes obesity counseling in the primary care setting, referred to as Healthy Habits 4 Life (HH4Life), with weight-management services offered in 27 CCP offices.

In December 2016, the clinical director was repositioned to the Division of Endocrinology. In January 2017, the WMWC was transitioned to the Division of Endocrinology.

CLINICAL ACTIVITIES

The scope of services provided by the WMWC between July 2016 and June 2017 included 570 new patients and 460 return patients. All visits were accomplished in two-day-per-week clinic sessions. The WMWC provided services to its satellite clinics. The numbers remained comparable to other pediatric obesity programs in the country despite the time and efforts devoted to transitioning to the Division of Endocrinology.



The WMWC was a leader in providing high-quality care to children. Quality initiatives included improving the identification of degree of obesity as measured by body mass index (BMI) and BMI percentile, its documentation in the electronic health record, and identification and management of hypertension among the center's patients, as well as improving rates of screening for lipid disorders. Because of its international reputation, the center continued to receive numerous requests from international physicians, residents and fellows in training, medical students, and scholars to observe the program.

RESEARCH AND OTHER SCHOLARLY ACTIVITIES

The faculty and postdoctoral students of the WMWC were extremely prolific. They presented their results at several national and international scientific meetings and published in high-impact, peer-reviewed journals. Arslanian and the junior faculty from the WMWC continue to be major contributors to the clinical and research productivity, publication record, and grant funding of the Division of Endocrinology, Diabetes, and Metabolism.

- Soon after its inception, the WMWC established a patient registry approved by the Institutional Review Board. The center obtained consent from 6,957 patients for the registry. The registry generates tremendous amounts of information about clinical characteristics of referred pediatric patients and provides longitudinal data on outcomes. Recruitment of participants from the registry has enabled many research programs.
- Arslanian pioneered the investigation of the pathophysiology of youth-onset type 2 diabetes. She was the first to describe the rapid deterioration in pancreatic β-cell function in youth type 2 diabetes. This finding was later evaluated in 699 youth with type 2 diabetes in the multi-center TODAY (Treatment Options for Type 2 Diabetes in Adolescents and Youth) trial, confirming her initial results that deterioration in β-cell function in youth is three to four times faster than what has been observed in adult type 2 diabetes.
- Arslanian continues her funded research, which includes:
    - The 15-year-long TODAY study with its successful five-year competitive renewal and a supplemental grant, "Behavioral Adherence in Emerging Adults With Type 2 Diabetes"
    - The National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK)-funded RISE grant (Restoring Insulin Secretion)

- A multicenter grant titled "DO-IT" (Dyslipidemia of Obesity Intervention in Teens) funded by the National Heart, Blood, and Lung Institute (NHBLI)
- An NIH grant, "Vitamin D and Vascular Function in Obese Youth," as a co-investigator with Kumaravel Rajakumar
- An NIH grant, "Resistance and Cardiorespiratory Time-Matched Exercise in Youth: A Randomized, Controlled Trial," as a co-investigator with SoJung Lee
- An NIH grant, "Pre- and Postnatal Exposure Periods for Child Health: Common Risks and Shared Mechanisms," as a co-investigator with Thomas O'Conner of the University of Rochester and Hyagriv Simhan of Magee-Womens Hospital of UPMC
- A pharmaceutical clinical study, "Liraglutide Obesity Trial: A 56-Week Double-Blind, Randomized, Parallel-Group, Placebo-Controlled Trial," as primary investigator
- A pharmaceutical clinical study, "Dulaglutide in Youth Type 2 Diabetes: A 26-Week Randomized, Double-Blind Trial," as primary investigator
- A pharmaceutical clinical study, "Lixisenatide in Youth Type 2 Diabetes: A 10-Week Randomized, Double-Blind, Placebo-Controlled, Dose-Escalation Study," as primary investigator

With the transition of the clinical activities to the Division of Endocrinology in January 2017, Arslanian has been focusing on establishing the Center for Pediatric Research in Obesity and Metabolism. The objective of the center is to bring together a cadre of investigators to advance scientific discovery in childhood obesity and its comorbidities. This will be accomplished through recruitment of mid-level, R01-funded physician-scientists or PhD-scientists or junior faculty with K awards with high academic promise and potential for excellence in research.

Heba Ismail-Roberts joined the WMWC in September 2014 as the clinical director. Together with Arslanian, she developed evidence-based manuals for the management of dyslipidemia, nonalcoholic fatty liver disease, and hypertension of obesity in youth. In December 2016, she transitioned to the Division of Endocrinology.

Joon Young Kim received a T32 training award in endocrinology, diabetes, and metabolism in the University of Pittsburgh School of Medicine and has been successful in publishing under Arslanian's mentorship.

Silva Arslanian, MD

RESEARCH

Silva Arslanian has been the director of the Pediatric Clinical and Translational Research Center (PCTRC) since 1999. The PCTRC is a component of the Clinical and Translational Science Award to the University of Pittsburgh. The goal of the PCTRC is to provide inpatient and outpatient facilities and laboratory investigations related to disorders of infancy, childhood, and adolescence. The PCTRC provides a valuable resource for the training of physicians in clinical investigation and becomes the stimulus to direct outstanding young physicians toward careers in patient-oriented research.

TODAY2. This is an NIDDK-funded grant, a large, longitudinal study in its 16th year. It continues to follow the TODAY cohort with youth-onset type 2 diabetes, now young adults, to track disease progression and diabetes complications. Arslanian is principal investigator.

Brief Intensive Glycemic Control and Beta-Cell Function in Pediatric Type 2 Diabetes/RISE. This is an NIH-funded grant to study desensitization of beta cells to changes in glucose levels (glucotoxicity), which may contribute to alteration in dynamics of insulin secretion. Arslanian is principal investigator.

Silva Arslanian, MD
Silva Arslanian was division chief for the WMWC.

Vitamin D and Vascular Function in Obese Children. Kumaravel Rajakumar's NIH-funded project investigates the effects of vitamin D supplementation on cardiovascular disease/dysmetabolic syndrome in obese youth. Arslanian is co-investigator.

Resistance and Cardiorespiratory Time-Matched Exercise in Youth: A Randomized, Controlled Trial. SoJung Lee's NIH-funded project aims to investigate the effects of exercise without weight loss on risk markers of type 2 diabetes and cardiovascular disease in obese youth. Arslanian is co-investigator.

DO-IT. This study is funded by the NHLBI and is a double-blind, placebo-controlled, two-year trial to determine whether treatment of combined dyslipidemia of obesity in adolescents with oral pitavastatin will improve vascular biomarkers of early atherosclerosis (carotid artery intima-media thickness, carotid artery stiffness, and pulse wave velocity) and potentially achieve primary prevention of adult cardiovascular disease. Arslanian is principal investigator.

Pre- and Postnatal Exposure Periods for Child Health: Common Risks and Shared Mechanisms. This is an NIH-funded study in response to Environmental Influences on Child Health Outcomes, Request for Applications. Arslanian will provide expertise in analysis of body composition and biochemical data related to offspring obesity. Principal investigators: Thomas O'Conner of the University of Rochester and Hyagriv Simhan of Magee-Womens Hospital of UPMC. Arslanian is co-investigator.

A Randomized, Double-Blind, Placebo-Controlled Trial to Assess Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of Liraglutide in Obese Children Aged 7 to 11 Years. This study funded by Novo Nordisk intends to assess the safety and tolerability of multiple once-daily doses of liraglutide at doses up to 3.0 mg in obese children aged 7–11 years and at Tanner stage 1. Arslanian is principal investigator.

Dulaglutide in Youth Type 2 Diabetes. This is a study funded by Eli Lilly and Company that is a 26-week, randomized, double-blind trial comparing the efficacy of once-weekly subcutaneous dulaglutide (glucagon-like peptide-1, GLP1 receptor agonist) to placebo in youth with type 2 diabetes, measured by change in HbA1c. Arslanian is principal investigator.

Lixisenatide in Youth Type 2 Diabetes. This study funded by Sanofi is a 10-week, randomized, double-blind, placebo-controlled, dose-escalation study of the safety, pharmacokinetics, and pharmacodynamics of subcutaneous lixisenatide, a GLP-1 receptor agonist, in adolescents with type 2 diabetes. Arslanian is principal investigator.

ADVISORY COMMITTEE MEMBERSHIPS

- Scientific Advisory Board, Institut d'Investigacions Biomediques de Girona, Generalitat de Catalunya
- Scientific Advisory Board, European Commission, EU-FP7, Beta-JUDO Grant 279153 (Beta Cell Function in Juvenile Diabetes and Obesity)
- Special Programs Committee, Endocrine Society
- Data and Safety Monitoring Board, Boehringer Ingelheim Pharmaceuticals, Inc.
- Data Monitoring Committee, Astra Zeneca
- Advisory Board, Lilly USA, LLC
- Advisory Board, Novo Nordisk
- Diabetes Advisory Committee, Children's Hospital of Pittsburgh of UPMC
- Expert Obesity Work Group, UPMC Health Plan
- Obesity Advisory Board, Pediatric Liraglutide
- Obesity Task Force, Allegheny County Medical Society
- Review Board for Disease-Related Competence Network on Obesity, German Federal Ministry of Education and Research
- Chair of working group, Studies of Pathophysiology in Youth-Onset Type 2 Diabetes (SPYonT2D), Common Physiological Outcomes
- CORE member, SPYonT2D
- Co-chair, type 2 diabetes position statement, American Diabetes Association
- Writing group, type 2 diabetes guidelines, International Society for Pediatric and Adolescent Diabetes (ISPAD)

EDITORSHIPS

- Associate editor, *Pediatric Diabetes*
- Editorial Board, *Treatments in Endocrinology*
- Editorial Board, *Journal of Diabetes and Its Complications*
- Editorial Board, *U.S. Endocrinology*
- Editorial Board, *Diabetes Care*

MAJOR LECTURESHIPS AND SEMINARS

- "Childhood Obesity and Comorbidities: Assessment and Management," annual scientific meeting of the Chinese Society of Pediatric Endocrinology and Metabolism and Chinese Medical Association, Guilin, China, July 2016
- "Novel Approaches to Treating Type 1 Diabetes," Diabetes Excellence Summit, Tehran, Iran, August 2016
- "Insulin Glulisine: Basal-Bolus Insulin in the Management of Childhood Type 1 Diabetes," Diabetes Excellence Summit, Tehran, Iran, August 2016
- "Polycystic Ovary Syndrome: Diagnosis and Treatment," Sociedad Mexicana De Endocrinologia Pediatrica, XVII Congreso Annual, Centro De Convenciones, Campeche, Mexico, August 2016

- "Early Identification and Treatment of Cardiovascular Risk Factors Associated With Type 2 Diabetes," Sociedad Mexicana De Endocrinologia Pediatrica, XVII Congreso Annual, Centro De Convenciones, Campeche, Mexico, August 2016
- "Childhood Obesity and Comorbidities," pediatric residents' noon conference, Children's Hospital of Pittsburgh, September 2016
- "Metabolic Markers of β-Cell Failure in Youth Insulin Resistance: Do They Conform to Standards?" 42nd Annual Conference of ISPAD, Valencia, Spain, October 2016
- "Youth Type 2 Diabetes: Tribulations of an Epic Love Story," Third Annual Dr. Heather Dean Lecture, Fifth Annual Diabetes Research Symposium, Children's Hospital Research Institute, University of Manitoba, Winnipeg, Canada, November 2016
- "Youth Type 2 Diabetes: A Looming Crisis," pediatric grand rounds, Children's Hospital of Pittsburgh of UPMC, December 2016
- "The Changing Face of Diabetes in Youth: Lessons Leaned From Adolescents in Contrast to Adult Type 2 Diabetes," NIH/National Institute of Child Health and Human Development training program in pediatric and adult endocrinology, endocrinology grand rounds, Bethesda, Md., December 2016
- "The Changing Face of Diabetes in Youth," Third Annual Endocrine Fellows Foundation Research Meeting, Dallas, Texas, January 2017
- "Treating Type 2 Diabetes in Youth: What Options Do We Have?" affiliate study training and initial site study training for "A Study of Dulaglutide in Children and Adolescents With Type 2 Diabetes (AWARD-PEDS)" (NCT02963766 sponsored by Eli Lilly), Rome, Italy, February 2017
- "Finding, Enrolling, and Retaining Patients in Clinical Trials," affiliate study training and initial site study training for "A Study of Dulaglutide in Children and Adolescents With Type 2 Diabetes (AWARD-PEDS)" (NCT02963766 sponsored by Eli Lilly), Rome, Italy, February 2017
- "Prediabetes and Type 2 Diabetes in Youth: Metabolic Aging of the Obese Adolescent," International Obesity Conference, Jeddah, Saudi Arabia, February 2017
- "Childhood Obesity and Comorbidities: Can We Stop the Tsunami?" International Obesity Conference, Jeddah, Saudi Arabia, February 2017
- "Pediatric Diabetes: Meet the Professor Session," ENDO 2017, Diabetes Diagnosis and Management, Endocrine Society, Orlando, Fla., March 2017
- "Type 2 Diabetes in Youth: How Does the Pathogenesis Compare to Adult Disease?" International Society of Nephrology World Congress of Nephrology, Mexico City, Mexico, April 2017
- "The Changing Face of Diabetes in Youth: Lessons Learned From Adolescents with Type 2 Diabetes," University-Wide Endocrine Conference, University of Pittsburgh School of Medicine, May 2017
- "Childhood Obesity Prevention and Management," First International Symposium on Diabesity, St. Luke's Medical Center–Global City, Manila, Philippines, October 2017
- "Management of Type 2 Diabetes in Children and Adolescents," First International Symposium on Diabesity, St. Luke's Medical Center–Global City, Manila, Philippines, October 2017

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Diabetes Association
- American Pediatric Society Diabetes Stakeholders Group, Pennsylvania Department of Health
- Endocrine Fellows Foundation
- Endocrine Society
- ISPAD
- Pediatric Endocrine Society

HONORS

- Scientific Advisory Board, European Commission, EU-FP7, Beta-JUDO Grant 279153 (Beta Cell Function in Juvenile Diabetes and Obesity)
- Scientific Advisory Board, Institut d'Investigacions Biomediques de Girona, Generalitat de Catalunya
- Chair, Rapid Lipidology Symposium, Pediatric Academic Societies annual meeting
- Chair, Type 2 Diabetes in Youth: Pathophysiology and Complications, World Diabetes Congress 2015, International Diabetes Federation, Vancouver, Canada
- America's Most Honored Professionals (top 5%), American Registry
- *Top Doctors*, Castle Connolly, 2016
- Chair, poster discussion session: "Type 2 Diabetes From Pathophysiology to Treatment," American Diabetes Association, 77th Scientific Sessions, San Diego, Calif.
- Chair, poster tour: "Type 2 Diabetes," ISPAD annual conference, Innsbruck, Austria

Heba Ismail-Roberts, MB BCh, MSc, PhD

RESEARCH

Open-Label, Multicenter, Multiple Oral Dose Study to Evaluate the Pharmacokinetics, Pharmacodynamics, and Safety of Canagliflozin in Older Children and Adolescents > 10 to < 18 Years of Age With Type 2 Diabetes Mellitus and Currently on a Stable Dose of Metformin. This was a phase I clinical trial sponsored by Janssen Pharmaceuticals. The open-label, sequential, multiple-dose, multicenter study to evaluate the pharmacokinetics of canagliflozin is completed. Ismail-Roberts was a sub-investigator.

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Diabetes Association
- Pediatric Endocrine Society
- Endocrine Society
- Member, Board of Directors, Western Pennsylvania chapter of the American Diabetes Association

SoJung Lee, PhD

RESEARCH

Resistance and Cardiorespiratory Time-Matched Exercise in Youth: A Randomized, Controlled Trial. This five-year R01 grant funded by the NHBLI in August 2013 examines the effects of various exercise modalities on insulin sensitivity, abdominal obesity, ectopic fat, and cardiovascular risk factors in overweight and obese adolescents. In July 2017, Lee returned to Korea to continue her academic activities there.

EDITORSHIPS

- Associate editor, *Applied Physiology, Nutrition, and Metabolism*

MAJOR LECTURESHIPS AND SEMINARS

- "Body Composition in Children and Adolescents: Influence of Obesity and Ethnicity," International Congress on Obesity and Metabolic Syndrome in conjunction with the 45th Annual Scientific Meeting of the Korean Society for the Study of Obesity, Seoul, Korea, 2016
- "Obesity and Physical Activity in Youth: Does the Type of Exercise Matter for Health?" international symposium: Evidence-Based Exercise Medicine for the Promotion of Lifelong Health, Yonsei University, Seoul, Korea, 2016
- "What Type of Exercise Is Most Optimal for the Treatment of Childhood Obesity?" Department of Sport and Leisure Studies, Yonsei University, Seoul, Korea, 2016

PROFESSIONAL AFFILIATIONS/SOCIETY MEMBERSHIPS

- American Diabetes Association
- Obesity Society
- Pediatric Academic Societies

TEACHING ACTIVITIES

The WMWC trained medical students and pediatric residents. The overall goal was to familiarize students with identification, assessment, and management of obesity and obesity-related problems in children and adolescents. Students saw patients independently in the center under the supervision of Ismail-Roberts and Arslanian. Evaluations completed by the students rated the program positively.

The WMWC hosted visiting international scholars and observers who spent anywhere from a month to a year observing the clinical program and attending the educational and research activities. The WMWC laid the groundwork for community interventions through networking meetings with key professionals, opinion leaders, and community members in at-risk communities. The center developed an education-oriented handbook for referring physicians, which offers guidelines for preventing and treating childhood obesity in primary care.

Additionally, the faculty members of the WMWC taught fellows in the Division of Pediatric Endocrinology, Diabetes, and Metabolism and residents on elective rotations in the center. This included outpatient clinics and inpatient rotations. The faculty taught a course on pediatric advanced physical exam for the second-year medical students.

THREE-YEAR BIBLIOGRAPHY

2015

Baldauff HN, **Arslanian S**. Optimal management of polycystic ovary syndrome in adolescence. *Arch Dis Child.* 2015;100:1076-83.

Brown RE, Kuk JL, **Lee S**. Measurement site influences abdominal subcutaneous and visceral adipose tissue in obese adolescents before and after exercise. *Pediatr Obes.* 2015;10(2):98-104.

Brown RE, Kuk JL, Libman I, Rivera-Vega M, **Lee S**. Associations between visceral fat and liver fat with insulin sensitivity and metabolic risk in obese adolescents. *Biochem Cell Biol*. 2015;93(5):466-71.

Chernausek SD, **Arslanian S**, Caprio S, Copeland KC, El ghormli L, Kelsey MM, Koontz MB, Orsi CM, Wilfley D. Relationship between parental diabetes and presentation of metabolic and glycemic function in youth with type 2 diabetes: Baseline findings from the TODAY trial. *Diabetes Care*. 2015; Nov 17.

de las Heras Monteroa J, Rajakumarby K, **Arslanian S**. Vitamin D and type 2 diabetes mellitus: Are they really related? [Letter to the editor]. *Ann Pediatr (Barc).* 2015;82(2):118-9.

Diesel JC, Eckhardt CL, Day NL, Brooks MM, **Arslanian S**, Bodnar LM. Is gestational weight gain associated with offspring obesity at 36 months? *Pediatr Obes.* 2015;10(4):305-10.

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EDUCATIONAL PROGRAMS

Pediatric Residency Program 2016–17

CHIEF RESIDENTS

Left to right: Regina Toto, MD, Christine March, MD and Lorne Walker, MD

HOUSE STAFF

PL-1 RESIDENTS	MEDICAL SCHOOL
Sherwat Abdelrahman, MBBCH	Ain Shams University Faculty of Medicine
Chelsey Bayer, MD	Mercer University School of Medicine
Kate Belser, MD	Pennsylvania State University College of Medicine
Kristen Bennett, MD	University at Buffalo State University of New York School of Medicine and Biomedical Sciences
James Bohnoff, MD	University of Pittsburgh School of Medicine
Danielle Browning, DO	West Virginia School of Osteopathic Medicine
Michael Certo, MD	Columbia University College of Physicians and Surgeons
Erin Cummings, MD	University of Pittsburgh School of Medicine
Andrea Davis, MD	University of Toledo College of Medicine
Benjamin Edmonds, MD	University of Central Florida College of Medicine
Megan Freeman, MD	Vanderbilt University School of Medicine
Kristina Gaietto, MD	University of Cincinnati College of Medicine
Shireen Ganapathi, MD	University of Toledo College of Medicine
Amita Ghuman, MD	Howard University College of Medicine
Katherine Gitz, MD	University of Cincinnati College of Medicine
Anna-Elisa Heipertz, MD	Universitatsmedizin der Johannes Gutenberg-Universitat Mainz
Aram Kim, MD	New York University School of Medicine
Katherine Kurzinski, MD	Drexel University College of Medicine
Elizabeth Landzberg, MD	Columbia University College of Physicians and Surgeons
Alison Mols, MD	West Virginia School of Osteopathic Medicine
Michelle Perry, MD	University of Pittsburgh School of Medicine
Lisa Plattenberger, MD	Baylor College of Medicine
Catherine Poholek, MD, PhD	University of Alabama School of Medicine
Anthony Pompa, MD	University of Pittsburgh School of Medicine
Erica Prochaska, MD	University of Michigan Medical School

(continued)

Jacquelin Rankine, MD	Icahn School of Medicine at Mount Sinai
Glenn Rapsinski, MD, PhD	Temple University School of Medicine
Kelsey Schweiberger, MD	Florida International University Herbert Wertheim College of Medicine
Dana Tlais, MD	American University of Beirut Faculty of Medicine
Allison Wise, MD	Georgetown University School of Medicine
Tiffany Yang, MD	Vanderbilt University School of Medicine

PL-2 RESIDENTS	MEDICAL SCHOOL
Caroline Albert, MD	Yale School of Medicine
Heather Bernard, MD	University of Pittsburgh School of Medicine
Alanna Boyajian, MD	Columbia University College of Physicians and Surgeons
Katherine Cobb-Pitstick, MD	University of Cincinnati College of Medicine
Elisabeth Cole, MD	Tulane University School of Medicine
Abbye Degan, MD (Floystad)	University of Miami Leonard M. Miller School of Medicine
Alexandra Dreyzin, MS, MD	University of Pittsburgh School of Medicine
Jeffrey Eugene, MD	Morehouse School of Medicine
Dailia Francis, MD, PhD	Icahn School of Medicine at Mount Sinai
Daniel Guillen, MD	Universidad Peruana Cayetano Heredia Facultad de Medicina
Joseph Langham, MD	Morehouse School of Medicine
Elise Lu, MD, PhD	Washington University in St. Louis School of Medicine
Benjamin Macadangdang, MD, PhD	Duke University School of Medicine
Jillian Mador, MD	George Washington University School of Medicine and Health Sciences
Maanasi Mistry, MD	University of Virginia School of Medicine
Onome Oghifobibi, MBBS	University of Benin School of Medicine
Katrina O'Halloran, MBChB	University College Dublin School of Medicine and Medical Science
Aidan Porter, MD	Warren Alpert Medical School of Brown University
Ronald Seese, MD, PhD	University of California, Irvine
Sara Serbin, MD	University of Cincinnati College of Medicine
Neema Shah, MD	University of Virginia School of Medicine
Emily Trauernicht, MD	University of Virginia School of Medicine
Mira Trivedi, MD	Wright State University Boonshoft School of Medicine
Desiree Wagner, MD	University of Rochester School of Medicine and Dentistry
Maria Widmann, MD	Ohio State University College of Medicine
Allison Williams (Holder), MD	Florida International University Herbert Wertheim College of Medicine
Jessie Yester, MD, PhD	Virginia Commonwealth University School of Medicine

HOUSE STAFF *(continued)*

PL-3 RESIDENTS	MEDICAL SCHOOL
Anna Ahn, MD	Vanderbilt University School of Medicine
Elaine L. Chiang, MD	George Washington University School of Medicine and Health Sciences
Dara I. Cohn, MD	Northwestern University Feinberg School of Medicine
Amy E. Collins, MD	Tulane University School of Medicine
Christine M. Crana, MD	State University of New York Upstate Medical University
Valerie L. Curren, MD	Royal College of Surgeons in Ireland School of Medicine
Megan M. Elbon, MD	Baylor College of Medicine
Rebecca A. Epperly, MD	University of Iowa Roy J. and Lucille A. Carver College of Medicine
Casey T. Goldman, MD	University of Florida College of Medicine
Lauren P. Jacobs, MD	University of North Carolina at Chapel Hill School of Medicine
Liny V. John, MD	University of Pittsburgh School of Medicine
Lorraine A. Kiger, MD	Wright State University Boonshoft School of Medicine
Peter D. LaRossa, MD	University of Pittsburgh School of Medicine
Alison S. Levine, MD	University of Pittsburgh School of Medicine
Meghan C. McCormick, MD	Rutgers New Jersey Medical School
Francesca O. Okolie, DO	Nova Southeastern University College of Osteopathic Medicine
Elissa K. Ortolani, MD	University at Buffalo State University of New York School of Medicine and Biomedical Sciences
Andrew D. Prigge, MD	University of Rochester School of Medicine and Dentistry
Christian D. Pulcini, MD	Tufts University School of Medicine
Vikram K. Raghu, MD	University of Pittsburgh School of Medicine
Anthony J. Ross, MD	University of Pittsburgh School of Medicine
Kinsey M. Roth, MD	University of Colorado School of Medicine
Carly Safier, MD	University of Toledo College of Medicine
Eva K. Schwartz, MD	Chicago Medical School at Rosalind Franklin University of Medicine and Science
Gina M. Sequeira, MD	Tulane University School of Medicine
Matthew E. Valente, MD	State University of New York Upstate Medical University
Michael C. Verre, MD	Washington University in St. Louis School of Medicine
Ashleigh M. Welko, MD	Wright State University Boonshoft School of Medicine
Laura J. West, MD	University of Pittsburgh School of Medicine
Tashya J. Whitehead, MD	University of Michigan Medical School

HOUSE STAFF *(continued)*

GRADUATING RESIDENTS	FELLOWSHIP / POSITION
Anna Ahn, MD	Hospitalist, Children's Hospital of Pittsburgh of UPMC
Elaine L. Chiang, MD	Hospitalist, Children's Hospital of Pittsburgh of UPMC
Katherine Cobb-Pistick, MD	Child Neurology Residency, Children's Hospital of Pittsburgh of UPMC
Dara I. Cohn, MD	Fellowship, Allergy/Immunology, Rush University
Amy E. Collins, MD	Fellowship, Adolescent Medicine, Children's Hospital of Pittsburgh of UPMC
Christine M. Crana, MD	Fellowship, Nephrology, Children's Hospital of Pittsburgh of UPMC
Valerie L. Curren, MD	Hospitalist, New York Presbyterian University Hospital of Columbia and Cornell
Megan M. Elbon, MD	Primary Care, PCP Peds (Georgia)
Rebecca A. Epperly, MD	Fellowship, Hematology/Oncology, St. Jude Children's Hospital
Casey T. Goldman, MD	Pediatrician, CHOA Urgent Care (Georgia)
Daniel Guillen, MD	Child Neurology Residency, Children's Hospital of Pittsburgh of UPMC
Lauren P. Jacobs, MD	TBD
Liny V. John, MD	Chief Resident, Children's Hospital of Pittsburgh of UPMC
Lorraine A. Kiger, MD	Primary Care, CCP South Hills
Peter D. LaRossa, MD	Fellowship, Cardiology, University of Michigan
Alison S. Levine, MD	Primary Care, CCP Bass Wolfson
Meghan C. McCormick, MD	Fellowship, Hematology/Oncology, Children's Hospital of Pittsburgh of UPMC
Onome Oghifobibi, MBBS	Fellowship, Neonatal-Perinatal Medicine, Children's Hospital of Pittsburgh of UPMC
Francesca O. Okolie, DO	Fellowship, Neonatal-Perinatal Medicine, Columbia University Medical Center
Elissa K. Ortolani, MD	Child Neurology Residency, Children's Hospital of Pittsburgh of UPMC
Andrew D. Prigge, MD	Chief Resident, Children's Hospital of Pittsburgh of UPMC
Christian D. Pulcini, MD	Fellowship, Emergency Medicine, Children's Hospital of Philadelphia
Vikram K. Raghu, MD	Fellowship, Gastroenterology, Children's Hospital of Pittsburgh of UPMC
Anthony J. Ross, MD	Fellowship, Hematology/Oncology, Emory University
Kinsey M. Roth, MD	Fellowship, Neonatal-Perinatal Medicine, Children's Hospital of Pittsburgh of UPMC
Carly Safier, MD	Hospitalist, Akron Children's Hospital
Eva K. Schwartz, MD	Hospitalist, St. Clair Hospital
Ronald Seese, MD, PhD	Neurodevelopmental Disabilities Resident, Children's Hospital of Pittsburgh of UPMC
Gina M. Sequeira, MD	Fellowship, Adolescent Medicine, Children's Hospital of Pittsburgh of UPMC
Matthew E. Valente, MD	Chief Resident, Children's Hospital of Pittsburgh of UPMC
Michael C. Verre, MD	Hospitalist, Lurie Children's Hospital
Ashleigh M. Welko, MD	Attending Physician, IPC Health Peds East (Arizona)
Laura J. West, MD	Chief Resident, Children's Hospital of Pittsburgh of UPMC
Tashya J. Whitehead, MD	TBD
Jesse Yester, MD, PhD	Fellowship, Cardiology, Children's Hospital of Pittsburgh of UPMC

Resident Accomplishments 2016–17

NAME	PROJECT
Nicholas Stygles **Med-Peds, PGY4**	Stygles N et al. Response to the article: Palliative care interventions for patients with heart failure: A systematic review and meta-analysis. *Journal of Palliative Medicine*. 2017;20(6):582-3.
Lorrie Kiger **Pediatric Residency Program, PGY3**	Conference presentation: **"**Should Residency Programs Intervene to Promote Resident Health Maintenance?" Annual UPMC Graduate Medical Education (GME) Leadership Conference, Pittsburgh, Pa., February 2017
Laura West **Pediatric Residency Program, PGY3**	Conference presentation: "Infant and Young Child Peanut Challenges: A Clinical Application of the Learning Early About Peanut Allergy Study," American Academy of Allergy, Asthma, and Immunology (AAAAI) meeting, Atlanta, Ga., March 2017
Christian Pulcini **Pediatric Residency Program, PGY3**	Pulcini CD et al. Poverty and trends in three common chronic disorders. *Pediatrics*. 2017;139(3). Pulcini CD et al. Refeeding syndrome. *Pediatrics in Review*. 2016;37(12). Conference presentation: "Impact of an Institution-Wide Mentoring Program on a Single-Class Cohort of Pediatric Residents," Pediatric Academic Societies meeting, San Francisco, Calif., May 2017
Liny John **Pediatric Residency Program, PGY3**	Conference presentation: "PRIME: Inaugural Years and Impact," University of Pittsburgh GME Leadership Conference, Pittsburgh, Pa., April 2017
Amy Collins **Pediatric Residency Program, PGY3**	Conference presentations: "Promoting Stakeholder Engagement in Health Surveillance Research of Marginalized Youth," Children's Hospital of Pittsburgh Adolescent and Young Adult Health Research Symposium, Pittsburgh, Pa., May 2017 "Promoting Stakeholder Engagement in Health Surveillance Research of Marginalized Youth," Society of Adolescent Health and Medicine annual meeting, New Orleans, La., March 2017
Gina Sequeira **Pediatric Residency Program, PGY3**	Conference presentation: "Impact of Gender Expression on Disordered Eating, Body Dissatisfaction, and BMI in a Cohort of Transgender Youth," Society for Adolescent Health and Medicine meeting, New Orleans, La., March 2017
Meghan McCormick **Pediatric Residency Program, PGY3**	Conference presentation: "Response to Intranasal Versus Intravenous Desmopressin for DDAVP Challenge Testing," ROAR Research Day, Pittsburgh, Pa., May 2017
Casey Sommerfeld **Pediatric Residency Program, PGY3**	Conference presentation: **"**Predictors of Oral Food Challenge Outcomes in Children With a Diagnosis of Food Allergy," AAAAI meeting, Atlanta, Ga., March 2017
Rebecca Epperly **Pediatric Residency Program, PGY3**	Conference presentation: "Treatment of Pediatric Plasma Cell Myeloma Type Post-Transplant Lymphoproliferative Disorder With Modern Risk-Directed Therapy Including Autologous Stem Cell Transplant," American Society of Pediatric Hematology and Oncology meeting, Montreal, Quebec, Canada, April 2017
Dara Cohn **Pediatric Residency Program, PGY3**	Conference presentation: "Eosinophilia in the Pediatric Allergy Clinic: Atopy, or Parasitic Infection?" 2016 Annual Scientific Meeting of the American College of Allergy, Asthma, and Immunology, San Francisco, Calif., October 2016
Jessie Yester **PedSDP, PGY2**	Yester JW, Kuhn B. Mechanisms of cardiomyocyte proliferation and differentiation in development and regeneration. *Current Cardiology Reports*. 2017;19(2).
Maanasi Mistry **Pediatric Residency Program, PGY2**	Conference presentations: "Predictors of Poor Outcomes in Pediatric Patients Requiring VA ECMO," American Heart Association 26th Annual Fellows Research Day, Pittsburgh, Pa., January 2017 "Predictors of Poor Outcomes in Pediatric Patients Requiring VA ECMO," Cardiology 2017: 20th Annual Update on Pediatric and Congenital Cardiovascular Disease–Care at the Cutting Edge in a Cost-Conscious World, Orlando, Fla., February 2017
Abbye Degan, **Pediatric Residency Program, PGY2**	Conference presentation: "Transportation Characteristics Associated With Nonarrival to Scheduled Pediatric Clinic Appointments," Pediatric Academic Societies meeting, San Francisco, Calif., May 2017

Resident Accomplishments 2016–17 *(continued)*

NAME	PROJECT
Jeffrey Eugene **PALS, PGY2**	Conference presentation: "Using Community-Based Asset Mapping and Respondent-Driven Sampling to Support Recruitment and Retention for a Community-Based Sexual Violence Prevention Program," Society of Adolescent Health and Medicine annual meeting, New Orleans, La., March 2017
Elizabeth Cole **Pediatric Residency Program, PGY2**	Khan N et al. Segmental resection is a safe oncologic alternative to total proctocolectomy in elderly patients with ulcerative colitis and malignancy. *Colorectal Disease*. 2017. doi:10.1111/codi.13721
Alanna Boyajian **Pediatric Residency Program, PGY2**	Conference presentation: "The Proof Is in the Pudding," Pediatric Hospitalist Conference, Nashville, Tenn., July 2017
Onome Oghifobibi **PedSDP, PGY2**	Conference presentation: **"**Both Expressed and Donor Breast Milk Enhance the Proliferation of Human Intestinal Stem Cells," Eastern Society for Pediatric Research meeting, Philadelphia, Pa., March 2017
James Bohnhoff **Pediatric Residency Program, PGY1**	Bohnhoff JC et al. Treatment and follow-up of venous thrombosis in the neonatal intensive care unit: A retrospective study. 2017;37(3):306-10.
Jacquelin Rankine **Pediatric Residency Program, PGY1**	Rankine J et al. Language environment analysis (LENA) in Phelan-McDermid syndrome: Validity and suggestions for use in minimally verbal children with autism spectrum disorder. *Journal of Autism and Developmental Disorders.* 2017;47(6):1605-17.
Elizabeth Landzberg **Pediatric Residency Program, PGY1**	Opotowsky AR et al. Design and implementation of a prospective adult congenital heart disease biobank. *World Journal for Pediatric and Congenital Heart Surgery*. 2016;7(6): 734-43. Opotowsky AR et al. Estimated glomerular filtration rate and urine biomarkers in patients with single-ventricle Fontan circulation. *Heart*. 2016;103(6):434-42.
Kristina Gaietto **Pediatric Residency Program, PGY1**	Shaffer RC et al. Brief report: Diminished gaze preference for dynamic social interaction scenes in youth with autism spectrum disorders. *Journal of Autism and Developmental Disorders*. 2016;47(2):506-13. doi:10.1007/s10803-016-2975-2
Glenn Rapsinski **PedSDP, PGY1**	Rapsinski GJ et al. *Pseudomonas mendocina* native valve infective endocarditis: A case report. *Journal of Medical Case Reports*. 2016;10(1).

Pediatric Fellowship Programs 2016–17

NAME	SPECIALTY	MEDICAL SCHOOL	RESIDENCY LOCATION (noted if not pediatrics)
Alicia Boykin, MD	Adolescent medicine	University of Cincinnati College of Medicine	Cincinnati Children's Hospital Medical Center
Jessica MacCormac, DO	Adolescent medicine	New York College of Osteopathic Medicine	University of Rochester
Merrian Brooks, DO	Adolescent medicine	Ohio University College of Osteopathic Medicine	Cooper University Hospitals
Orquidia Torres, MD	Adolescent medicine	State University of New York at Downstate	Stony Brook University
Jennifer Boeckman, DO	Allergy and immunology	Chicago College of Osteopathic Medicine	University of Illinois (internal medicine)
Supriya Jain, MD	Allergy and immunology	Howard University College of Medicine	North Shore Long Island Jewish
Schweta Arakali, MD	Allergy and immunology	Thomas Jefferson University	Robert Wood Johnson Medical School (internal medicine)
Shaylar Padgett, MD	Allergy and immunology	Indiana University School of Medicine	Indiana University School of Medicine
Jessica Bivens, DO	Cardiology	Arizona College of Osteopathic Medicine	Nationwide Children's Hospital
Elizabeth Caris, MD	Cardiology	Ohio State University College of Medicine	Nationwide Children's Hospital
Defne Magnetta, MD	Cardiology	Case Western Reserve University	Children's Hospital of Pittsburgh of UPMC
Adam Christopher, MD	Cardiology	University of Pittsburgh School of Medicine	Yale New Haven Hospital
Rukmalee Vithana, MD	Cardiology	University of Arizona College of Medicine	Children's National Medical Center
Chris Follansbee, MD	Cardiology	University of Pittsburgh School of Medicine	Children's Hospital of Pittsburgh of UPMC
Christopher Mercer, MD	Cardiology	West Virginia University School of Medicine	West Virginia University
Ugonna Nwankwo, MD	Cardiology	Wake Forest School of Medicine	Children's Hospital of Pittsburgh of UPMC
Wilson Heredia, MD	Child neurology	Pontifica University Catolica Del Ecuador	Bronx Lebanon Hospital
Elissa Ortolani, MD	Child neurology	State University of New York at Buffalo	Children's Hospital of Pittsburgh of UPMC
Vinod Valentine, MBBS	Child neurology	Malankara Orthodox Syrian Church Medical College, India	Texas Tech University
Stacey Elkhatib, MD	Child neurology	University of Virginia School of Medicine	Children's Hospital of Pittsburgh of UPMC
Matthew Ginsberg, MD	Child neurology	Case Western Reserve University	Children's Hospital of Pittsburgh of UPMC
William Welch, MD	Child neurology	University of Oklahoma College of Medicine	Children's Hospital of Pittsburgh of UPMC
Adam Kney, MD	Child neurology	University of Illinois College of Medicine	Children's Hospital of Pittsburgh of UPMC
Neil Munjal, MD	Child neurology	Washington University School of Medicine	Children's Hospital of Pittsburgh of UPMC

Pediatric Fellowship Programs 2016–17 *(continued)*

NAME	SPECIALTY	MEDICAL SCHOOL	RESIDENCY LOCATION (noted if not pediatrics)
Levi Shelton, MD	Child neurology	University of Oklahoma College of Medicine	Children's Hospital of Pittsburgh of UPMC
Katherine Anetakis, MD	Clinical neurophysiology	University of Pittsburgh School of Medicine	Children's Hospital of Pittsburgh of UPMC
Alexis Franks, MD	Neurodevelopmental disabilities	University of Colorado School of Medicine	Children's Hospital of Pittsburgh of UPMC
Jenna Gaesser, MD	Neurodevelopmental disabilities	State University of New York Upstate Medical University	Children's Hospital of Pittsburgh of UPMC
Kathleen Noorbakhsh, MD	Emergency medicine	Eastern Virginia Medical School	Children's Hospital of the King's Daughter
Sriram Ramgopal, MD	Emergency medicine	Sri Ramachandra Medical College, India	Children's Hospital of Pittsburgh of UPMC
Jane Soung, MD	Emergency medicine	Medical College of Wisconsin	Children's Hospital of Pittsburgh of UPMC
Devora Azhdam, MD	Emergency medicine	Sackler School of Medicine, Israel	Northwell Health
Ashley Keilman, MD	Emergency medicine	Case Western Reserve University	Seattle Children's Hospital
Yesha Patel, MD	Emergency medicine	Marshall University	St. Christopher's Hospital for Children
Kim Horner, MD	Emergency medicine	Emory University School of Medicine	Children's Hospital of Pittsburgh of UPMC
Jennifer Melvin, MD	Emergency medicine	State University of New York at Buffalo	Duke University
Nate Weberding, MD	Emergency medicine	Kansas City University School of Medicine	Children's Mercy Hospital and Clinic
Mohamed Saleh, MBBS	Endocrinology	Ain Shams University, Egypt	Flushing Hospital Medical Center
Marissa Avolio, MD	Endocrinology	Robert Wood Johnson Medical School	Baylor College of Medicine
Amr Morsi, MD	Endocrinology	University of Cairo Faculty of Medicine	Texas Tech University
Amy Kakkanatt, MD	Endocrinology	Albany Medical College of Union University	William Beaumont Hospital (medicine/pediatrics)
Pedro Pagan Banchs, MD	Endocrinology	University Central Del Caribe Escuela De Medicina	Miami Children's Hospital
Hilary Michel, MD	Gastroenterology	University of Pittsburgh School of Medicine	Children's Hospital of Pittsburgh of UPMC
Angela Sandell, MD	Gastroenterology	State University of New York at Buffalo	Women and Children's Hospital of Buffalo
Kristen Critelli, MD	Gastroenterology	State University of New York Upstate Medical University	New York Presbyterian Hospital
Ellen Mitchell, MD	Gastroenterology	Robert Wood Johnson Medical School	Thomas Jefferson University
Lauren Mullinax, MD	Gastroenterology	University of South Florida College of Medicine	Miami Children's Hospital
Fateema Turay Rose, MD	Gastroenterology	Pennsylvania State University	University of South Florida

Pediatric Fellowship Programs 2016–17 *(continued)*

NAME	SPECIALTY	MEDICAL SCHOOL	RESIDENCY LOCATION (noted if not pediatrics)
Whitney Sunseri, MD	Gastroenterology	University of Illinois College of Medicine	Children's Hospital of Pittsburgh of UPMC
Ahmad Alhariri, MD	Medical biochemical genetics	University of Damascus, Syria	University of Missouri (internal medicine)
Allison Close, MD	Hematology/oncology	Michigan State University College of Human Medicine	Children's Hospital of Pittsburgh of UPMC
Lisa Maurer, MD	Hematology/oncology	University of Wisconsin	University of California, San Francisco
Deirdre Nolfi-Donegan, MD	Hematology/oncology	Robert Wood Johnson Medical School	Northwell Health
Kaitlin Stanley, MD	Hematology/oncology	State University of New York at Buffalo	New York University
Danielle Bell, MD	Hematology/oncology	Michigan State University College of Human Medicine	Children's Hospital of Pittsburgh of UPMC
Brittani Seynnaeve, MD	Hematology/oncology	West Virginia University School of Medicine	Children's Hospital of Pittsburgh of UPMC
Michael Winstead, MD	Blood and marrow transplantation	University of Illinois College of Medicine	University of New Mexico
Timothy Onarecker, MD	Infectious diseases	University of Oklahoma College of Medicine	Arkansas Children's Hospital
Santiago Lopez, MD	Infectious diseases	University Del Salvadore, Argentina	Nationwide Children's Hospital
Masaki Yamada, MD	Infectious diseases	Kanazawa University, Japan	University of Iowa
Corinne Balint, DO	Neonatal/perinatal	Kirksville College of Osteopathic Medicine	Penn State Medical Center
Erin Bargerstock, MD	Neonatal/perinatal	Case Western Reserve University	Children's Hospital of Pittsburgh of UPMC
Hannah Chalal, MD	Neonatal/perinatal	George Washington University School of Medicine	Children's Hospital of Pittsburgh of UPMC
Anna Sedney, MD	Neonatal/perinatal	University of Virginia School of Medicine	Inova Children's Hospital
Nitin Arora, MD	Neonatal/perinatal	B.R. Ambedkar Medical College, India	West Virginia University Hospital
Kathleen Schwabenbauer, MD	Neonatal/perinatal	Pennsylvania State University	Thomas Jefferson University Hospital
Christine Yankowski, DO	Neonatal/perinatal	Philadelphia College of Osteopathic Medicine	St. Christopher's Hospital for Children
Danielle Alfano, MD	Neonatal/perinatal	Washington University School of Medicine	Medical College of Wisconsin
Laura Jackson, MD	Neonatal/perinatal	Pennsylvania State University	Children's Hospital of Pittsburgh of UPMC
Karena Lawrence, MD	Neonatal/perinatal	University of Connecticut School of Medicine	Children's Hospital of Pittsburgh of UPMC
Courtney Kiser McLean, MD	Neonatal/perinatal	Marshall University	Jefferson Medical College
Jenny Zank, MD	Neonatal/perinatal	Loyola University of Chicago, Stritch School of Medicine	University of Michigan

Pediatric Fellowship Programs 2016–17 *(continued)*

NAME	SPECIALTY	MEDICAL SCHOOL	RESIDENCY LOCATION (noted if not pediatrics)
Cassandra Formeck, MD	Nephrology	Ohio State University College of Medicine	Nationwide Children's Hospital
Melissa Anslow, MD	Nephrology	Temple University School of Medicine	Cleveland Clinic Foundation
Caitlin Peterson, DO	Nephrology	Arizona College of Osteopathic Medicine	Riley Children's Hospital
Paul Fadakar, MD	Nephrology	University of Connecticut School of Medicine	University of Connecticut
Emily Joyce, MD	Nephrology	Case Western Reserve University School of Medicine	Children's Hospital of Pittsburgh of UPMC
Saif Al Qatarneh, MD	Pulmonology	University of Jordan Faculty of Medicine	St. Joseph Children's Hospital
Venkata Chamarthi, MBBS	Pulmonology	Andhra Medical College, India	Texas Tech University Health Science Center
Robert Abood, MD	Pulmonology	Drexel University School of Medicine	Riley Children's Hospital
Anjani Ravindra, MD	Pulmonology	University of Pittsburgh School of Medicine	Hershey Medical Center
Sylvia Szentpetery, MD	Pulmonology	Tulane University School of Medicine	University of Virginia
Sandeep Puranik, MD	Pulmonology	Karnataka Institute Medical Sciences, India	Flushing Hospital Medical Center
Paul Tsoukas, MBBch	Rheumatology	Royal College of Surgeons, Ireland	Tufts University School of Medicine
Laura Tasan, MD	Rheumatology	Ross University School of Medicine	University of Kentucky
Emily Brunner, DO	Rheumatology	Philadelphia College of Osteopathic Medicine	Geisinger Medical Center (medicine/pediatrics)
Brandi Eastman Stevens, MD	Rheumatology	Medical University of South Carolina	Eastern Virginia Medical School
Laura Tasan, MD	Rheumatology	Ross University School of Medicine	University of Kentucky
Emily Brunner, DO	Rheumatology	Philadelphia College of Osteopathic Medicine	Geisinger Medical Center (medicine/pediatrics)
Deepika Singh, MD	Rheumatology	Maulana Azad Medical College	Albert Einstein Medical Center

Graduating Fellows 2016–17

NAME	SPECIALTY	CURRENT POSITION
Merrian Brooks, DO	Adolescent medicine	Global Health Fellow, Children's Hospital of Philadelphia
Orquidia Torres, MD	Adolescent medicine	Attending, Children's Hospital at Montefiore, Bronx, N.Y.
Schweta Arakali, MD	Allergy and immunology	Private practice, Bergenfield, N.J.
Shaylar Padgett, MD	Allergy and immunology	Private practice, High Point, N.C.
Michael Winstead, MD	Blood marrow transplantation	Clinical Assistant Professor of Pediatrics, University of North Carolina
Christopher Follansbee, MD	Cardiology	Assistant Professor of Pediatrics, University of Pittsburgh
Christopher Mercer, MD	Cardiology	Assistant Professor of Pediatrics, West Virginia University
Ugonna Nwankwo, MD	Cardiology	Interventional Cardiology Fellow, Emory University
Adam Kney, MD	Child neurology	Clinical Neurophysiology Fellow, UPMC
Neil Munjal, MD	Child neurology	Pediatric Critical Care Fellow, UPMC
Levin Shelton, MD	Child neurology	Assistant Professor of Pediatrics, University of Pittsburgh
Jenna Gaesser, MD	Neurodevelopmental disabilities	Assistant Professor of Pediatrics, University of Pittsburgh
Kimberly Horner, MD	Emergency medicine	Assistant Professor of Pediatrics, Orlando Children's Hospital
Jennifer Melvin, MD	Emergency medicine	Assistant Professor of Pediatrics, Ohio State University
Nathaniel Weberding, DO	Emergency medicine	Assistant Professor of Pediatrics, University of Pittsburgh
Amy Kakkanatt, MD	Endocrinology	Taking year off for child care
Pedro Pagan Banchs, MD	Endocrinology	Attending, Nicklaus Children's Hospital, Miami, Fla.
Fateema Rose, MD	Gastroenterology	Assistant Professor of Pediatrics, University of Washington
Whitney Sunseri, MD	Gastroenterology	Assistant Professor of Pediatrics, University of Pittsburgh
Danielle Bell, MD	Hematology/oncology	Assistant Professor of Pediatrics, University of Michigan
Brittani Seynnaeve, MD	Hematology/oncology	Assistant Professor of Pediatrics, University of Pittsburgh
Masaki Yamada, MD	Infectious diseases	Advanced Transplant Infectious Diseases Fellow, UPMC
Ahmad Alhariri, MD	Medical biochemical genetics	Locum Tenens, UPMC Altoona
Danielle Alfano, MD	Neonatal-perinatal medicine	Assistant Professor of Pediatrics, Washington University
Laura Jackson, MD	Neonatal-perinatal medicine	Assistant Professor of Pediatrics, University of Pittsburgh
Karena Lawrence, MD	Neonatal-perinatal medicine	Assistant Professor of Pediatrics, University of Pittsburgh
Courtney McLean, MD	Neonatal-perinatal medicine	Assistant Professor of Pediatrics, University of Nebraska
Jennifer Zank, MD	Neonatal-perinatal medicine	Assistant Professor of Pediatrics, University of Pittsburgh
Paul Fadakar, MD	Nephrology	Assistant Professor of Pediatrics, University of Pittsburgh
Emily Joyce, MD	Nephrology	Assistant Professor of Pediatrics, University of Pittsburgh
Sandeep Puranik, MD	Pulmonology	Assistant Professor of Pediatrics, Indiana University (Riley Children's)
Sylvia Szentpetery, MD	Pulmonology	Assistant Professor of Pediatrics, Medical University of South Carolina
Brandi Stevens, MD	Rheumatology	Assistant Professor of Pediatrics, Indiana University (Riley Children's)

Lee W. Bass Community Preceptors 2016–17

MISSION

- Provide mentorship and role modeling for residents in the practice of primary care pediatrics in community settings
- Expose residents to a broad range of pediatric topics as well as aspects of practice management
- Enhance the connection between the community pediatricians and the residents, based on mutual understanding and respect

Lee W. Bass Community Preceptors

Alicia Hartung
Art Kovel
Barbara Ayers
Brandon Reily
Brian Czervionke
Britta Kocak
Elizabeth Masella
Eric Friedlander
Greg Anderson
Hilary Garbon
Jacqueline Saladino
James Shaver
James Tucker
Jerome Gloster
Kate Williams
Katie Cass
Keith Pirl
Kelly Heidenreich
Kim Gordon
Kochikar Pai
Kochikar Pai
Kristin Frederick
Kristin Hanley
Laura Voigt
Leon Brostoff
Lucas Godinez
Marc Yester
Marc Yester
Mary Pagnotto
Meredith Kursmark
Paul Rowland
Rachel Pokorney
Robert Breit
Robert Breit
Sarah Springer
Stephanie Sterrett
Stewart Schott
Thomas Lynch
William Varley
Wilmarie Garcia

Pitt Medical Students Selecting Pediatrics

YEAR	PEDS	TOTAL	PERCENTAGE
FY06	18	131	13.7
FY07	13	143	9.1
FY08	18	139	12.9
FY09	10	140	7.1
FY10	10	121	8.3
FY11	21	149	14.1
FY12	18	152	11.8
FY13	22	140	15.7
FY14	14	148	9.5
FY15	19	132	14.4
FY16	23	132	17.4
FY17	19	158	12

Continuing Medical Education 2016–17

GRAND ROUNDS PROGRAM

Thursday, June 29, 2017

"From Wet Nurses to HMBANA: A History of Donor Breast Milk"
Jennifer L. Kloesz, MD
Associate Professor of Pediatrics, University of Pittsburgh
Medical Director, Neonatal Intensive Care Unit, Magee-Womens Hospital of UPMC
Director, Neonatal-Perinatal Medicine Fellowship Program, UPMC

Thursday, June 22, 2017

"Emerging Challenges to Child Health in America: RX—Transformation"
Neal Halfon, MD, MPH
Professor of Pediatrics, Public Health, and Public Policy, University of California, Los Angeles
Director, Centers for Healthier Children, Families, and Communities

Thursday, June 15, 2017

"Recent Advances in Pediatric Pulmonary Hypertension"
Dunbar Ivy, MD
Selby's Chair in Pediatric Cardiology, Children's Hospital Heart Institute, Children's Hospital of Colorado, University of Colorado School of Medicine

Thursday, June 8, 2017

Howard A. Mermelstein Memorial Lecture: "Pediatrics and PKU: It's All in the Genes: A 40-Year Perspective"
Jane M. Breck, MD, FAAP
Clinical Director of Pediatrics, University of Pittsburgh School of Medicine
Director, PKU Program and Medical Genetics Program, Children's Hospital of Pittsburgh of UPMC

Thursday, June 1, 2017

"Healthcare Transformations of the 21st Century"
Terence S. Dermody, MD
Vira I. Heinz Professor and Chair, Department of Pediatrics
Professor of Microbiology and Molecular Genetics, University of Pittsburgh School of Medicine
Physician-in-Chief and Scientific Director, Children's Hospital of Pittsburgh of UPMC

Thursday, May 25, 2017

"Our Kids Are NOT Broken: Fostering Resilience in Marginalized and Traumatized Youth"
Kenneth R. Ginsburg, MD, MSEd
Professor of Pediatrics, Division of Adolescent Medicine, Children's Hospital of Philadelphia
Professor of Pediatrics, Perelman School of Medicine, University of Pennsylvania, Philadelphia

Thursday, May 18, 2017

Annual Fetterman Lecture: "Placental Pathology and Its Implications for the Neonate"
Ona M. Faye-Petersen, MD
Immediate Past President, Society for Pediatric Pathology
Professor of Pathology and Obstetrics and Gynecology, Division of Anatomic Pathology/Department of Pathology, University of Alabama at Birmingham

Thursday, May 11, 2017

"Social Media Use in Depressed and Anxious Adolescents: A Two-Way Street"
Ana Radovic, MD, MSc
Assistant Professor of Pediatrics
PCOR K12 Scholar
Associate Fellowship Director, Division of Adolescent and Young Adult Medicine, Children's Hospital of Pittsburgh

Thursday, May 4, 2017

"Pediatric Obesity: Consequences and Causes"
Jack A Yanovski, MD, PhD
Chief, Section on Growth and Obesity, Division of Intramural Research, Eunice Kennedy Shriver National Institute of Child Health and Human Development, National Institutes of Health, Hatfield Clinical Research Center, Bethesda, Md.

Thursday, April 27, 2017

"Bruising, History, and Psychosocial Characteristics Discriminate Abusive from Accidental Trauma in Young Children"
Mary Clyde-Pierce, MD
Professor of Pediatrics, Northwestern University, Lurie Children's Hospital of Chicago, Chicago, Ill.

Thursday, April 13, 2017

"Targeting Leukemia-Associated Minor Histocompatibility Antigens With TCR-Transduced Memory CD8+ and CD4+ T Cells for Prevention of Post-Transplantation Relapse"
Marie Bleakley, MD, PhD, M.Msc
Associate Professor of Pediatrics, University of Washington
Associate Member, Fred Hutchinson Cancer Research Center
Attending Physician, Pediatric BMT Service, Seattle Children's Hospital, University of Washington

Thursday, April 6, 2017

Kenny-Drash Lecture: "Surprises Screening for Congenital Hypothyroidism"
Stephen H. LaFranchi, MD
Professor of Pediatrics, Oregon Health and Science University, Portland

Thursday, March 30, 2017

Demetrius Ellis Lecture: "Rebuilding a Kidney from Stem Cells: Are We Close?"
Samir S. El-Dahr, MD
Jane B. Aron Professor and Chair, Department of Pediatrics, Tulane University School of Medicine, New Orleans, La.

Thursday, March 23, 2017

James A. Kangos Memorial Lecture: "Treating Inflammatory Bowel Disease: The Expanding World of Biologics"
Maria Oliva-Hemker, MD
Stermer Family Professor of Pediatric IBD, Johns Hopkins University School of Medicine, Baltimore, Md.
Director, Division of Pediatric Gastroenterology, Hepatology, and Nutrition
Vice Chair for Faculty Development, Diversity, and Promotion

Thursday, March 16, 2017

Moira J. Whitehead Memorial Lecture: "Pennsylvania's Response to the Opioid Crisis"
Rachel L. Levine, MD
Physician General of the Commonwealth of Pennsylvania
Professor of Pediatrics and Psychiatry, Penn State College of Medicine, Harrisburg, Pa.

Thursday, March 9, 2017

"Gender Non-Conforming/Transgender Youth: Current Concepts, Management, and Barriers to Care"
Stephen M. Rosenthal, MD
Professor of Pediatrics, Division of Pediatric Endocrinology
Medical Director, Child and Adolescent Gender Center, University of California, San Francisco

Thursday, March 2, 2017

"Vitamin D: Desirable Concentrations and Dietary Requirements in Children"
Kumaravel Rajakumar, MD, MS
Associate Professor of Pediatrics, University of Pittsburgh School of Medicine, Children's Hospital of Pittsburgh of UPMC

Thursday, February 23, 2017

"Big Data and Genomics in Precision Cancer Medicine"
Adrian V. Lee, PhD
Professor, Department of Pharmacology and Chemical Biology
Director, Women's Cancer Research Center, University of Pittsburgh Cancer Institute, Magee-Womens Research Institute

Thursday, February 9, 2017

"The Plain Communities Translation Medicine Program"
Gerald Vockley, MD, PhD
Cleveland Family Professor of Pediatric Research, University of Pittsburgh
Professor of Human Genetics, Children's Hospital of Pittsburgh of UPMC
Chief of Medical Genetics
Director, Center for Rare Disease Therapy

Thursday, February 2, 2017

"Acute Ataxia in Childhood: Autoimmune and Other Etiologies"
Kavita Thakkar, MD
Assistant Professor of Pediatrics, University of Pittsburgh School of Medicine

Thursday, January 26, 2017

Donald N. Medearis Jr., MD, Memorial Lecture: "Language Barriers in Health Care: How to Eliminate Them, and Key Ethical and Policy Issues"
Glenn Flores, MD, FAAP
Distinguished Chair of Health Policy Research, Media Research Institute
Affiliate Professor of Health Policy and Management, University of Minnesota School of Public Health

Thursday, January 19, 2017
"Center of Excellence for Pediatric Thyroid Disease"
Pushpa Viswanathan, MD
Assistant Professor, Children's Hospital of Pittsburgh of UPMC

Thursday, January 12, 2017
"The 'Other' Scleroderma: Localized Scleroderma (AKA Morphea) Recognition and Review of Clinical Features for the Clinician"
Kathryn Torok, MD
Assistant Professor, Children's Hospital of Pittsburgh of UPMC, University of Pittsburgh School of Medicine
Assistant Professor of Clinical and Translational Science

Thursday, January 5, 2017
"Beginning at the Beginning: Addressing the Epidemic of Chronic Disease Through Lifestyle Medicine"
Michael D. Parkinson, MD, MPH, FACPM
Senior Medical Director, Health and Productivity, UPMC Health Plan and Work Partners

Thursday, December 15, 2016
John Caffey Endowed Lectureship: "Use of US Contrast Agent in Children"
Kassa Darge, MD, PhD
Chair, Department of Radiology
Radiologist-in-Chief, Children's Hospital of Philadelphia
Professor of Radiology, Perelman School of Medicine, University of Pennsylvania

Thursday, December 1, 2016
"Youth Type 2 Diabetes: A Looming Crisis"
Silva Arslanian, MD
Director, Weight Management and Wellness Center
UPMC Richard L. Day Endowed Chair and Professor of Pediatrics, University of Pittsburgh School of Medicine, Children's Hospital of Pittsburgh of UPMC
Director, Pediatric Clinical and Translational Research Center

Thursday, November 17, 2016
"Outpatient Antimicrobial Stewardship"
Jeffrey S. Gerber, MD, PhD
Assistant Professor of Pediatrics, University of Pennsylvania School of Medicine, Division of Infectious Diseases, Children's Hospital of Philadelphia

Thursday, November 10, 2016
"Phenylketonuria (PKU): A Paradigm of Translational Medicine—What Will Be the Next Breakthrough?"
Uta Lichter-Konecki, MD, PhD
Clinical Professor of Pediatrics, University of Pittsburgh School of Medicine
Director, Inborn Errors of Metabolism Clinic, Children's Hospital of Pittsburgh of UPMC

Thursday, November 3, 2016
"Finding Goldilocks: How Do We Screen Enough Children for Physical Abuse Without Screening Too Many?"
Rachel Berger, MD, MPH
Division Chief, Child Advocacy Center, Children's Hospital of Pittsburgh of UPMC
Associate Professor of Pediatrics and Clinical and Translational Medicine, University of Pittsburgh School of Medicine
Associate Director, Safar Center for Resuscitation Medicine, University of Pittsburgh

Thursday, October 27, 2016
"Cybernetics in the ICU: Will Computers Take Over Mechanical Ventilation?"
Robert Chatburn, MHHS, RRT-NPS
Adjunct Professor, Department of Medicine, Lerner College of Medicine, Case Western Reserve University
Fellow, American Association for Respiratory Care
Clinical Research Manager, Section of Respiratory Therapy, Cleveland Clinic
Editorial Board, *Respiratory Care*

Thursday, October 20, 2016
"The Journey Toward Zero Preventable Harm"
Richard E. McClead Jr., MD, MHA
Associate Chief Medical Officer, Nationwide Children's Hospital
Professor Emeritus, Pediatrics, Ohio State University College of Medicine, Columbus

Thursday, October 13, 2016
33rd Annual Paul C. Gaffney Memorial Lecture: "Precision Pediatric Oncology: Early Lessons and Future Challenges"
Rajen Mody, MD, MS
David G. Dickinson Collegiate Professor of Pediatrics, Division of Pediatric Hematology/Oncology, University of Michigan, Ann Arbor

Thursday, October 06, 2016
"Emergency, Surgical, and Trauma Care for Children: The State of the Union"
Mary E. Fallat, MD
Hirikati S. Nagaraj Professor of Surgery, Division Director of Pediatric Surgery, Hiram C. Polk Jr. Department of Surgery, University of Louisville
Surgeon-in-Chief, Kosair Children's Hospital, Louisville, Ky.

Thursday, September 29, 2016
"Obesity in Congenital Heart Disease"
Meryl S. Cohen, MD
Associate Professor of Pediatrics, Perelman School of Medicine, University of Pennsylvania, Children's Hospital of Philadelphia

Thursday, September 22, 2016
"The Role of the Pediatrician: Preventing Bullying Through Science, Policy, and Practice"
Matthew Masiello, MD, MPH, FAAP
Chief Medical Officer
Medical Director, Care Coordination, Children's Institute of Pittsburgh

Thursday, September 15, 2016
"The State of the Department of Pediatrics at Children's Hospital of Pittsburgh of UPMC"
Terence S. Dermody, MD
Vira I. Heinz Professor and Chair, Department of Pediatrics
Professor of Microbiology and Molecular Genetics, University of Pittsburgh School of Medicine
Physician-in-Chief and Scientific Director, Children's Hospital of Pittsburgh of UPMC

Thursday, September 8, 2016
"Evaluation and Management of Aerodigestive Tract Foreign Bodies"
Jeffrey Simmons, MD
Associate Professor, Department of Otolaryngology, Children's Hospital of Pittsburgh of UPMC, University of Pittsburgh School of Medicine

Thursday, July 28, 2016
"What We Have Learned From Registries: The Pediatric Cardiomyopathy Registry"
Steven E. Lipshultz, MD, FAAP, FAHA
Department of Pediatrics, Wayne State University School of Medicine, Children's Hospital of Michigan

Thursday, July 21, 2016
"A Vision for Pediatrics at Children's Hospital of Pittsburgh"
Terence S. Dermody, MD
Vira I. Heinz Professor and Chair, Department of Pediatrics
Professor of Microbiology and Molecular Genetics, University of Pittsburgh School of Medicine
Physician-in-Chief and Scientific Director, Children's Hospital of Pittsburgh of UPMC

Thursday, July 7, 2016
"Metabolic Liver Disease"
Patrick J. McKiernan, MD
Professor of Pediatrics, University of Pittsburgh School of Medicine
Director of Hepatology, Division of Gastroenterology, Hepatology, and Nutrition, Children's Hospital of Pittsburgh of UPMC

Thursday, June 30, 2016
"Diversity and Inclusion at CHP: Past, Present, and Future"
Stephanie B. Dewar, MD
Associate Professor of Pediatrics, Children's Hospital of Pittsburgh of UPMC, University of Pittsburgh School of Medicine

Thursday, June 23, 2016
"HOt Topics in Pediatric Hematology/Oncology"
Kim Ritchey, MD
Professor and Vice Chair for Clinical Affairs, Department of Pediatrics, University of Pittsburgh School of Medicine, Division of Hematology/Oncology, Children's Hospital of Pittsburgh of UPMC

Thursday, June 2, 2016
"Tourette Syndrome: Case Dissection"
Robyn A. Filipink, MD
Medical Director, Fragile X Center, Children's Hospital of Pittsburgh of UPMC
Director, Movement Disorders Clinic, Children's Hospital of Pittsburgh of UPMC
Clinical Associate Professor of Pediatrics, University of Pittsburgh School of Medicine

MOLECULAR MEDICINE RESEARCH SEMINARS 2016–17

September 6, 2016
"Bacterial Adaptations During Chronic Infections Revealed by Evolutionary Genomics"
Vaughn Cooper, PhD
Associate Professor, Microbiology and Molecular Genetics, University of Pittsburgh School of Medicine

September 13, 2016
"Immunophenotyping Pediatric Scleroderma: A Focus on Active Disease"
Kathryn Torok, MD
Assistant Professor, Department of Pediatric Rheumatology, University of Pittsburgh School of Medicine

September 20, 2016
"MKRN3: A New Player in the Regulation of Human Puberty and Reproduction"
Ursula Kaiser, MD
Professor of Medicine and Chief, Division of Endocrinology, Diabetes, and Hypertension, Harvard Medical School and Brigham and Women's Hospital

September 27, 2016
"The Power of Statistics in Making Sense of Biomedical Omics Data"
Wei Chen, PhD
Assistant Professor, Division of Pulmonary Medicine, Allergy, and Immunology, Department of Pediatrics, Children's Hospital of Pittsburgh of UPMC

October 4, 2016
"Control of Nephron Progenitor Cell Renewal: How Can We Obtain Enough Cells to Build New Kidney Tissue?"
Leif H. Oxburgh, DVM, PhD
Associate Professor, Cell, Molecular, and Developmental Biology, Tufts University School of Medicine, Maine Medical Center Research Institute

October 11, 2016
"The Nuclear Lamina in Disease and Development"
Quasar S. Padiath, MBBS, PhD
Assistant Professor, Department of Human Genetics, Graduate School of Public Health, University of Pittsburgh

October 18, 2016
"Type 1 Diabetes and the Developing Brain"
Tamara Hershey, PhD
Professor, Deputy Director, Neuroimaging Labs, Psychiatry, Neurology, Radiology, Psychological and Brain Sciences, Washington University School of Medicine

October 25, 2016
"Probing and Enhancing Tissue Regeneration"
Kenneth D. Poss, PhD
James B. Duke Professor, Department of Cell Biology, Duke University School of Medicine

November 1, 2016
"Growth Factor Signaling Pathways in Mouse Models of Lung Development and Cancer"
David Ornitz, MD, PhD
Alumni Endowed Professor, Department of Developmental Biology, Washington University School of Medicine

November 8, 2016
"The Need for Biomedical Research in Diverse Populations"
Esteban Burchard, MD, MPH
Professor, Department of Bioengineering and Therapeutic Sciences and Medicine; Vice Chair, Department of Bioengineering and Therapeutic Sciences; Director, Center for Genes, Environments, and Health, University of California, San Francisco

November 15, 2016
"Mitochondrial Dysfunction Contributes to the Pathogenesis of Inflammatory Bowel Disease"
Kevin Mollen, MD
Division of Pediatric General and Thoracic Surgery, Samuel P. Harbison Assistant Professor of Surgery, Department of Surgery, University of Pittsburgh School of Medicine

November 29, 2016
"GH/IGF-1/Insulin Axis and Cancer"
Derek LeRoith, MD, PhD
Professor of Medicine and Chief of Endocrinology, Diabetes, and Bone Disease; Director, Metabolism Institute; Director, Metabolic Mechanisms of Diabetes and Cancer Laboratory, Icahn School of Medicine at Mount Sinai, New York

December 6, 2016
"Role of Memory T Cells in Transplant Rejection"
Fadi Lakkis, MD
Frank and Athena Sarris Chair in Transplantation Biology; Professor of Surgery, Immunology and Medicine; Scientific Director, Thomas E. Starzl Transplantation Institute

December 13, 2016
"Renalase: A Novel Survival Factor—Roles in Health and Disease"
Fred Gorelick, MD
Professor of Medicine (Digestive Diseases) and of Cell Biology; Deputy Director, Yale MD, PhD Program, Yale University

December 20, 2016
"Predicting Pandemic Influenza Viruses"
Seema S. Lakdawala, PhD
Assistant Professor, Department of Microbiology and Molecular Genetics, University of Pittsburgh School of Medicine

January 3, 2017
"Cytokines in Systemic Inflammation: Following the Monogenic Breadcrumb Path"
Scott W. Canna, MD
Scholar, Richard King Mellon Foundation Institute for Pediatric Research, University of Pittsburgh School of Medicine

January 10, 2017
"What Causes Pancreatic Beta-Cell Failure in Type 2 Diabetes?"
Henry Dong, PhD
Assistant Professor of Pediatrics, Division of Rheumatology, Children's Hospital of Pittsburgh of UPMC

January 17, 2017
"Host Impairments in MDR *K. pneumoniae* Killing"
Janet Lee, MD
Associate Professor of Medicine, Division of Pulmonary, Allergy, and Critical Care Medicine, UPMC Montefiore Hospital

January 24, 2017
"Protecting Potassium at All Costs: Insights into the Molecular Basis of Potassium Balance"
Paul A. Welling, MD
Professor of Physiology, Graduate Program Director, University of Maryland School of Medicine

January 31, 2017
"Host Factors Required for Chikungunya Virus Infection"
Terence Dermody, MD
Vira I. Heinz Professor and Chair, Department of Pediatrics
Professor of Microbiology and Molecular Genetics, University of Pittsburgh School of Medicine
Physician-in-Chief and Scientific Director, Children's Hospital of Pittsburgh of UPMC

February 7, 2017
"Maintenance of the Host/Microbiome Relationship in the Intestine"
Tim Hand, PhD
Assistant Professor, R.K. Mellon Institute for Pediatric Research, Children's Hospital of Pittsburgh of UPMC, University of Pittsburgh School of Medicine

February 14, 2017
"Moving Novel Therapies for Fatty Acid Oxidation Disorders From the Lab to the Clinic"
Gerard Vockley, MD, PhD
Chief, Division of Medical Genetics; Director, Center for Rare Disease Therapy; Professor of Pediatrics, Professor of Human Genetics, University of Pittsburgh School of Medicine; Children's Hospital of Pittsburgh of UPMC

February 21, 2017
"Genomic Insights Into the Genetic Basis of Aging"
Andreas R. Pfenning, PhD
Assistant Professor, Department of Computational Biology, School of Computer Science; Department of Biological Sciences, Mellon College of Science; Center for the Neural Basis of Cognition, Carnegie Mellon University

February 28, 2017
"Lung Vaso-Occlusion in Sickle Cell Disease Mediated by Arteriolar Neutrophil-Platelet Micro-Emboli"
Prithu Sundd, PhD
Assistant Professor of Medicine and Bioengineering; Principal Investigator, Pittsburgh Heart, Lung, and Blood Vascular Medicine Institute; Division of Pulmonary, Allergy, and Critical Care Medicine, University of Pittsburgh School of Medicine

March 7, 2017
"Factors Modulating the Therapeutic Efficacy of Thioredoxin Reductase Inhibitors to Prevent Lung Injury"
Trent E. Tipple, MD
Associate Professor, Division of Neonatology, Redox Biology Laboratory, Department of Pediatrics, University of Alabama at Birmingham

March 14, 2017
"Mechanisms of Graft-Versus-Host Disease and Graft-Versus-Leukemia"
Warren D. Shlomchik, MD
Visiting Professor of Medicine, Department of Hematology-Oncology; Director, Hematopoietic Stem Cell Transplant and Cell Therapy, University of Pittsburgh School of Medicine

March 21, 2017
"Amino Acid Control of Gene Expression in Health and Disease"
Michael Kilberg, PhD
Department of Biochemistry and Molecular Biology, University of Florida College of Medicine

March 28, 2017
"How Working on Metabolism Brought Us to the Mechanisms of Learning"
Kaveh Ashrafi, PhD
Associate Professor, Department of Physiology, University of California, San Francisco

April 4, 2017
"Airway Host Defense Defects in Cystic Fibrosis: Lessons From Animal Models"
Paul B. McCray Jr., MD
Departments of Pediatrics and Microbiology, University of Iowa

April 11, 2017
"Genes for Type 2 Diabetes From Genetic and Genomic Studies in Mice"
Alan D. Attie, PhD
Jack Gorski Professor of Biochemistry, University of Wisconsin–Madison

April 18, 2017
"The Role of the Paneth Cell in the Defense and Homeostasis of the Developing Small Intestinal Tract"
Steven McElroy, MD
Associate Professor, Division of Neonatology, Stead Family Department of Pediatrics, University of Iowa

April 25, 2017
"Sex Differences in UTI Pathogenesis"
David A. Hunstad, MD
Associate Professor, Pediatrics; Chief, Division of Infectious Diseases; Associate Professor, Molecular Microbiology, Washington University School of Medicine, St. Louis

May 2, 2017
"Understanding CFTR: From Macromolecular Complexes to Personalized Medicine"
Anjaparavanda P. Naren, PhD
Professor, Department of Pediatrics; Codirector, Cystic Fibrosis Research Center; Thomas Boat Chair in Cystic Fibrosis Research, Cincinnati Children's Hospital Medical Center

May 9,2017
"Germinal Center–Derived IgM B Cells Are Rapid Responders to Infection"
Marion Pepper, PhD
Assistant Professor, Department of Immunology, University of Washington

May 16, 2017
"The Liver, Nutrient Excess, and Metabolic Dysregulation"
Robert M. O'Doherty, PhD
Professor of Medicine; Professor of Molecular Genetics and Biochemistry; Codirector, Center for Metabolism and Mitochondrial Medicine; Director, T32 Training Program in Diabetes, Endocrinology, and Metabolism, University of Pittsburgh

May 23, 2017

"Imaging Exit of Single Herpesvirus Particles From Living Cells"
Lynn W. Enquist, PhD
Henry L. Hillman Professor in Molecular Biology, Professor of Molecular Biology and the Princeton Neuroscience Institute, Princeton University

June 6, 2017

"Mechanisms of Tolerance Following HLA-Mismatched Cord Blood Grafts: A Journey Toward Tandem Cadaveric Organ + Marrow Transplantation"
Paul Szabolcs, MD
Professor of Pediatrics and Immunology, University of Pittsburgh School of Medicine; Chief, Division of Blood and Marrow Transplantation, Children's Hospital of Pittsburgh of UPMC

June 13, 2017

"Noonan Syndrome and Related Disorders: RAS-pY Voices Heard Along the Pathway"
Bruce Gelb, MD
Professor, Pediatric Cardiology, Mount Sinai Hospital

June 20, 2017

"Cancer as a Ribosomopathy"
Edward Prochownik, MD, PhD
Paul C. Gaffney Professor of Pediatrics and Professor of Microbiology and Molecular Genetics, Hematology/Oncology, Children's Hospital of Pittsburgh of UPMC

June 27, 2017

"Allergic Asthma and Unfolded Protein Response (UPR)"
Vikas Anathy, PhD
Assistant Professor, Department of Pathology and Laboratory Medicine, University of Vermont College of Medicine

June 11, 2017

"Mitochondrial Dysfunction and Phospholipid Signaling in Acute Brain Injury"
Hulya Bayir, MD
Professor, Department of Critical Care Medicine; Department of Environmental and Occupational Health; Director of Research, Pediatric Critical Care Medicine; Associate Director, Center for Free Radical and Antioxidant Health, Safar Center for Resuscitation Research

June 18, 2017

"Repurposing Pharmacologic Adjuvants to Overcome Brain Barriers"
Robert S. Clark, MD
Chief, Division of Pediatric Critical Care Medicine; Associate Director, Safar Center for Resuscitation Research, University of Pittsburgh Department of Critical Care Medicine

June 25, 2017

"Understanding the Underlying Mechanisms Involved in Energy Defects Associated With Overlapping Features"
Areeg El-Gharbawy, MD, DSc
Assistant Professor of Pediatrics, Division of Medical Genetics, Children's Hospital of Pittsburgh of UPMC

FACULTY

Department of Pediatrics Faculty 2016–17

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Noel Spears Zuckerbraun, MD	Emergency Medicine

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Joyce Leifer, MD	Paul C. Gaffney Diagnostic Referral Service
Loreta Matheo Kass, MD	Adolescent and Young Adult Medicine

RESEARCH ASSOCIATE PROFESSORS

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Al-Walid Mohsen, PhD	Genetics
Michelle Poe, PhD	Genetics

ASSISTANT PROFESSORS

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Rannar Airik, PhD	Nephrology
Lauren Alessi, MD	Paul C. Gaffney Diagnostic Referral Service
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Abeer Azzuqa, MD	Newborn Medicine
Natalie Baldauff, DO	Endocrinology, Diabetes, and Metabolism
Arcangela Balest, MD	Newborn Medicine
Jessie Barnum, MD	Blood and Marrow Transplantation and Cellular Therapies
Riha Bhatt, MD	Gastroenterology, Hepatology, and Nutrition
Aimee Biller, MD	General Academic Pediatrics
Andrew Buchert, MD	Paul C. Gaffney Diagnostic Referral Service
Andrew Bukowinski, MD	Hematology/Oncology
Sean Button, MD	Emergency Medicine
Craig Byersdorfer, MD, PhD	Blood and Marrow Transplantation and Cellular Therapies
Carol Lynn Cabral, MD	Paul C. Gaffney Diagnostic Referral Service
Brian Campfield, MD	Infectious Diseases
Scott Canna, MD	Richard King Mellon Foundation Institute for Pediatric Research/ Rheumatology

ASSISTANT PROFESSORS (continued)

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Beth Carella, DO	Blood and Marrow Transplantation and Cellular Therapies
Elaine Cassidy, MD	Rheumatology
Wen-I Chang, MD	Hematology/Oncology
Diego Chaves-Gnecco, MD, MPH	General Academic Pediatrics
Kong Chen, PhD	Richard King Mellon Foundation Institute for Pediatric Research
Wei Chen, PhD	Pulmonology
Hey Jin Chong, MD, PhD	Pulmonary Medicine, Allergy, and Immunology
Jennifer Clarke, MD	Child Advocacy
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Dana Cummings, MD, PhD	Neurology
Mark Debrunner, MD	Cardiology
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Areeg El-Gharbawy, MD	Genetics
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Allison Fliescher, MD	Paul C. Gaffney Diagnostic Referral Service
Amanda Flint, MD	Endocrinology, Diabetes, and Metabolism
Erick Forno, MD, MPH	Pulmonology

ASSISTANT PROFESSORS (continued)

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Jessica Garrison, MD	Paul C. Gaffney Diagnostic Referral Service
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Carol Gilmour, MD	Newborn Medicine
Elissa B. Gittes, MD	Adolescent and Young Adult Medicine
Amy Goldstein, MD	Neurology
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Heba Ismail, MBBCh, MSc, PhD	Endocrinology, Diabetes, and Metabolism
Anuja Jindal, MD, MPH	Neurology/Child Development Unit
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Randy Windreich, MD	Blood and Marrow Transplantation and Cellular Therapies
Audrey Woerner, MD	Genetics
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Frederico Xavier, MD, MS	Hematology/Oncology
Jennifer Zarit, MD	General Academic Pediatrics
Matthew Zinn, DO	Cardiology

RESEARCH ASSISTANT PROFESSORS

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Kristy Boggs, PhD	Gastroenterology, Hepatology, and Nutrition
Nadia Boutaoui, PhD	Pulmonology
Xiaohua Chen, PhD	Blood and Marrow Transplantation and Cellular Therapies
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Yanhua Gao, MD, PhD	Neurology
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Lina Gonzalez, MD	Genetics
Yueh Ying Han, PhD	Pulmonology
Michelle Manni, PhD	Pulmonology
Sanjay Mishra, PhD	Cystic Fibrosis Research Center

RESEARCH ASSISTANT PROFESSORS (continued)

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Dianna Ploof, EdD	General Academic Pediatrics
Krishnan Raghunathan, PhD	Infectious Diseases
Laurie Silva, PhD	Infectious Diseases
Yudong Wang, PhD	Genetics
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David Nash, MD	Pulmonary Medicine, Allergy, and Immunology
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Mohammad Riaz, MD	Newborn Medicine, UPMC Hamot
Sapana Shah, MD	Gastroenterology, Hepatology, and Nutrition
Amina Smajlovic, MD	Gastroenterology, Hepatology, and Nutrition
Tahniat Syed, MD	Adolescent and Young Adult Medicine

INSTRUCTORS

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Kelly Bailey, MD, PhD	Hematology/Oncology
Zahida Khan, MD, PhD	Gastroenterology, Hepatology, and Nutrition
Gerald Montano, DO, MS	Adolescent and Young Adult Medicine

RESEARCH INSTRUCTORS

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Janet Fromkin, PhD	Child Advocacy
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Lauren Severs, MD	Newborn Medicine
Michael Winstead, MD	Blood and Marrow Transplantation and Cellular Therapies

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RESEARCH AND FUNDING

Grants for the Department of Pediatrics 2016–17

NATIONAL INSTITUTES OF HEALTH

DIVISION	NAME	AGENCY TITLE	GRANT TITLE	ANNUAL DIRECT COST	ANNUAL INDIRECT COST	ANNUAL TOTAL COST	GRANT BEGIN DATE	GRANT END DATE
Adolescent Medicine	Miller, Elizabeth	National Institutes of Health	Tailoring Clinical Interventions for Adolescent Relationship Abuse	$161,504.00	$12,920.00	$174,424.00	14-Apr-14	31-Mar-19
Adolescent Medicine	Miller, Elizabeth	National Institute of Health	College Health Center-Based Alcohol and Sexual Violence Intervention	$413,215.00	$208,390.00	$621,605.00	20-Aug-14	31-Jul-19
Adolescent Medicine	Miller, Elizabeth	National Institutes of Health	Adolescent Research in Community Postdoctoral Training Program	$301,974.00	$14,778.00	$316,752.00	01-May-16	30-Apr-21
Adolescent Medicine	Radovic, Ana	National Institutes of Health	Social Media Intervention for Adolescents with Depression and Anxiety and Their Parents to Increase Use of Mental Health Services	$164,760.00	$13,139.00	$177,899.00	01-Sep-16	31-Aug-20
Bone Marrow Transplant/Cell Therapy	Byersdorfer, Craig	National Institutes of Health	Changes in T-Cell Metabolism during Graft-Versus-Host Disease	$144,544.00	$11,564.00	$156,108.00	01-May-16	30-Apr-20
Bone Marrow Transplant/Cell Therapy	Szabolcs, Paul	National Institutes of Health	Cadaveric Donor Lung and Bone Marrow Transplantation in Immunodeficiency Diseases	$629,031.00	$330,065.00	$959,096.00	06-Jul-16	30-Jun-21
Cystic Fibrosis Research Center	Frizzell, Raymond	National Institutes of Health	Chaperone Actions in CFTR Biogenesis	$222,500.00	$120,150.00	$342,650.00	01-May-16	30-Apr-18
Cystic Fibrosis Research Center	Frizzell, Raymond	National Institutes of Health	Basic and Translational Studies of Cystic Fibrosis, Administrative Core	$677,185.00	$360,820.00	$1,038,005.00	01-Jun-16	31-May-18
Emergency Medicine	Manole, Mioara	National Institutes of Health	CYP 450-Mediated CBF Dysregulation and Neurotoxicity in Pediatric Cardiac Arrest	$207,500.00	$107,000.00	$314,500.00	10-Sep-13	31-May-18
Endocrinology	Becker, Dorothy	National Institutes of Health	Trial to Reduce IDDM in the Genetically at Risk: A Nutritional Primary Prevention	$0.00	$0.00	$0.00	01-Jul-11	30-Jun-17
Endocrinology	Becker, Dorothy	National Institutes of Health	Prediction and Prevention of Type 1 Diabetes (TrialNet)	$340,068.00	$161,497.00	$501,565.00	01-Aug-14	30-Apr-19
Endocrinology	Dong, Hengjiang	National Institutes of Health	Fox01 in Beta-Cell Compensation	$211,992.00	$93,055.00	$305,047.00	15-Jan-14	30-Nov-17
Endocrinology	Muzumdar, Radhika	National Institutes of Health	Novel Mitochondria-Associated Peptide and Beta-Cell Function in Aging	$0.00	$0.00	$0.00	15-Sep-15	31-Aug-17
Endocrinology	Muzumdar, Radhika	National Institutes of Health	Research Training in Pediatric Endocrinology	$179,084.00	$15,307.00	$194,391.00	01-Jul-16	30-Jun-21
General Academic Pediatrics	Bogen, Debra	Health Resources and Services Administration	University of Pittsburgh HRSA NRSA T32 Primary Care Research Training Program	$356,030.00	$23,447.00	$379,477.00	01-Jul-16	30-Jun-22
General Academic Pediatrics	Hoberman, Alejandro	National Institutes of Health	Efficacy of Tympanostomy Tubes for Children with Recurrent Acute Otitis Media	$753,106.00	$189,823.00	$942,929.00	14-Jul-15	30-Jun-20
General Academic Pediatrics	Rajakumar, Kumaravel	National Institutes of Health	Vitamin D and Vascular Function in Obese Children	$478,236.00	$188,268.00	$666,504.00	01-Feb-13	31-Jan-18
General Academic Pediatrics	Rajakumar, Kumaravel	National Institutes of Health	Administrative Supplements for Research on Dietary Supplements	$64,935.00	$35,065.00	$100,000.00	01-Jul-16	31-Jan-17
General Academic Pediatrics	Ray, Kristin	National Institutes of Health	Optimizing Pediatric Subspecialty Care through Telemedicine and e-Consultations	$144,545.00	$11,502.00	$156,047.00	01-Apr-17	31-Mar-22
General Academic Pediatrics	Shaikh, Nader	National Institutes of Health	Corticosteroids for Children with Febrile Urinary Tract Infections	$0.00	$0.00	$0.00	01-Apr-11	31-Mar-18
General Academic Pediatrics	Shaikh, Nader	National Institutes of Health	Efficacy of Antibiotics in Children with Sinusitis: Which Subgroups Benefit?	$778,191.00	$197,179.00	$975,370.00	01-Jun-15	31-May-20
Gastroenterology	Husain, Sohail	National Institutes of Health	HDACs in Pancreatic Recovery after Injury	$264,300.00	$135,080.00	$399,380.00	01-Jul-14	30-Jun-18
Gastroenterology	Husain, Sohail	National Institutes of Health	Calcineurin in Pancreatitis	$276,697.00	$126,159.00	$402,856.00	01-Jul-16	30-Jun-21
Gastroenterology	Lowe, Mark	National Institutes of Health	Institutional Research Training in Pediatrics Gastroenterology	$0.00	$0.00	$0.00	01-Aug-11	31-Jul-17
Gastroenterology	Squires, Robert	National Institutes of Health	A Multicenter Group to Study Acute Liver Failure in Children	$0.00	$0.00	$0.00	30-Sep-10	31-Aug-17

NATIONAL INSTITUTES OF HEALTH (continued)

DIVISION	NAME	AGENCY TITLE	GRANT TITLE	ANNUAL DIRECT COST	ANNUAL INDIRECT COST	ANNUAL TOTAL COST	GRANT BEGIN DATE	GRANT END DATE
Gastroenterology	Squires, Robert	National Institutes of Health	Pittsburgh Cholestatic Liver Disease Consortium	$198,832.00	$105,480.00	$304,312.00	10-Aug-14	31-May-19
Genetics	Goetzman, Eric	National Institutes of Health	Regulation of Mitochondrial Metabolism by Lysine Acylation	$264,200.00	$131,050.00	$395,250.00	01-Jun-16	31-May-21
Genetics	Nicholls, Robert	National Institutes of Health	Gene Imprinting and Obesity, a New Pig Model	$0.00	$0.00	$0.00	08-Aug-14	31-Jul-17
Genetics	Vockley, Gerard	National Institutes of Health	Inborn Errors of Long Chain Fat Metabolism	$238,670.00	$115,003.00	$353,673.00	01-Apr-16	31-Mar-20
Genetics	Vockley, Gerard	National Institutes of Health	Branched Chain acyl-CoA Metabolism and Disease	$306,779.00	$154,861.00	$461,640.00	01-Jul-16	30-Jun-20
Hematology/ Oncology	Lucas, Peter	National Institutes of Health	GPCR Signaling through a Novel NF-kB Pathway	$254,498.00	$130,502.00	$385,000.00	01-Feb-13	31-Jan-18
Hematology/ Oncology	Prochownik, Edward	National Institutes of Health	Control of Metabolism and Energy-Sensing Pathways by c-Myc	$232,489.00	$92,633.00	$325,122.00	20-Jun-14	31-May-19
Infectious Diseases	Brown, Judy	National Institutes of Health	Viral Triggers of Celiac Disease	$28,240.00	$0.00	$28,240.00	14-Sep-16	13-Sep-19
Infectious Diseases	Campfield, Brian	National Institutes of Health	A Critical Role of Follistatin-Like Protein-1 in Lung Homeostasis	$125,350.00	$10,028.00	$135,378.00	01-Sep-15	31-May-20
Infectious Diseases	Dermody, Terence	National Institutes of Health	Molecular Basis of Reovirus Pathogenesis	$297,779.00	$142,989.00	$440,768.00	08-Aug-16	31-Jul-20
Infectious Diseases	Dermody, Terence	National Institutes of Health	Molecular Basis of Pediatric Diseases	$0.00	$0.00	$0.00	01-Dec-11	30-Nov-17
Infectious Diseases	Dermody, Terence	National Institutes of Health	Cell Biology of Reovirus Infection	$378,762.00	$86,686.00	$465,448.00	01-Jun-16	31-Mar-19
Infectious Diseases	Dermody, Terence	National Institutes of Health	Reovirus Attachment Mechanisms	$308,761.00	$105,683.00	$414,444.00	01-Jun-16	30-Apr-20
Infectious Diseases	Dermody, Terence	National Institutes of Health	Chikungunya Virus Replication and Pathogenesis	$662,218.00	$104,131.00	$766,349.00	01-Jun-16	28-Feb-21
Infectious Diseases	Dermody, Terence	National Institutes of Health	Molecular Basis of Pediatric Diseases, Supplement #1	$261,887.00	$20,951.00	$282,838.00	01-Dec-15	30-Nov-17
Infectious Diseases	Dermody, Terence	National Institutes of Health	Basic/Translational Research Training for CHP Pediatric Fellows	$369,781.00	$25,952.00	$395,733.00	01-May-13	30-Apr-18
Infectious Diseases	Knowlton, Jonathan	National Institutes of Health	Mechanisms of Noncytolytic Reovirus Egress	$28,240.00	$0.00	$28,240.00	01-Jul-16	30-Jun-20
Infectious Diseases	Lennemann, Nicholas	National Institutes of Health	Characterizing the Role of ER-Shaping Proteins during RNA Virus Infection	$27,885.00	$0.00	$27,885.00	01-Mar-17	31-Jul-17
Infectious Diseases	Lin, Philana Ling	National Institutes of Health	HIV-TB Co-Infection: Tracking TB Emergence after Asymptomatic (Latent) Infection	$471,766.00	$204,989.00	$676,755.00	15-Mar-14	28-Feb-19
Infectious Diseases	Lin, Philana Ling	National Institutes of Health	Host and Pathogen Diversity in Mtb and SIV Infection	$123,275.00	$66,568.00	$189,843.00	01-Jul-16	30-Jun-18
Infectious Diseases	Williams, John	National Institutes of Health	Host Determinants of Human Metapneumovirus and Pathogenesis	$255,816.00	$124,777.00	$380,593.00	02-Jun-16	31-May-21
Neonatology	Ghazi, Arjumand	National Institutes of Health	The Coordination of Lipid Synthesis and Breakdown in Metabolism and Aging	$180,000.00	$99,450.00	$279,450.00	01-Aug-16	30-Apr-20
Neonatology	Ghazi, Arjumand	National Institutes of Health	The Coordination of Lipid Synthesis and Breakdown in Metabolism and Aging	$206,641.00	$101,359.00	$308,000.00	01-Aug-16	30-Apr-20
Neonatology	Good, Misty	National Institutes of Health	Novel Anti-Inflammatory Properties of Breast Milk in Necrotizing Enterocolitis	$14,836.00	$1,116.00	$15,952.00	05-Sep-14	30-Sep-16
Neonatology	Lamitina, Samuel T.	National Institutes of Health	Bipartite Regulation of Cellular Osmosensing in *C. elegans*	$173,574.00	$89,766.00	$263,340.00	01-Jul-14	30-Nov-17
Neonatology	Lamitina, Samuel T.	National Institutes of Health	Mechanisms of C9orf72-Associated Dipeptide Toxicity	$128,559.00	$63,941.00	$192,500.00	15-Aug-15	31-Jul-17
Neonatology	Lamitina, Samuel T.	National Institutes of Health	Modifiers of C9orf72-Associated Dipeptide Protein Toxicity in a *C. elegans* Model	$112,614.00	$54,796.00	$167,410.00	01-Apr-16	31-Mar-18

NATIONAL INSTITUTES OF HEALTH (continued)

DIVISION	NAME	AGENCY TITLE	GRANT TITLE	ANNUAL DIRECT COST	ANNUAL INDIRECT COST	ANNUAL TOTAL COST	GRANT BEGIN DATE	GRANT END DATE
Nephrology	Airik, Rannar	National Institutes of Health	The Role of DNA Damage Response Signaling in Chronic Kidney Disease	$161,137.00	$83,234.00	$244,371.00	01-Aug-15	31-Jul-18
Nephrology	Bates, Carl	National Institutes of Health	Critical Roles for Fibroblast Growth Factor Receptors in Bladder Development	$225,000.00	$121,500.00	$346,500.00	01-Jul-15	30-Jun-19
Nephrology	Bates, Carl	National Institutes of Health	Research Training in Pediatric Nephrology	$279,973.00	$19,837.00	$299,810.00	01-Jul-16	30-Jun-21
Nephrology	Ho, Jacqueline	National Institutes of Health	The Role of miR-17~92 in Nephron Progenitors	$228,331.00	$110,879.00	$339,210.00	19-Sep-14	31-Aug-19
Nephrology	Maringer, Katherine	National Institutes of Health	The Role of Oxygen Tension in Regulating the Differentiation of Nephron Progenitors	$60,618.00	$0.00	$60,618.00	01-Jan-17	31-Dec-18
Nephrology	Sims-Lucas, Sunder	National Institutes of Health	Renal Stroma-Derived Endothelial Precursors Are Critical for Renal Development	$103,183.00	$8,175.00	$111,358.00	15-Jul-13	31-May-18
Nephrology	Sims-Lucas, Sunder	National Institutes of Health	Kidney-Derived Endothelial Progenitors Play a Critical Role during Kidney Injury	$50,000.00	$27,563.00	$77,563.00	01-Apr-17	31-Mar-18
Nephrology	Swiatecka-Urban, Agnieszka	National Institutes of Health	Novel Pathways in TGF Beta Signaling	$232,484.00	$119,070.00	$351,554.00	15-Sep-16	31-Aug-17
Neurology	Fyffe-Maricich, Sharyl	National Institutes of Health	The Role of ERK1/2 MAP Kinase Signaling in CNS Myelination	$218,750.00	$113,400.00	$332,150.00	01-Jul-15	30-Jun-20
Neurology	Pandey, Udai	National Institutes of Health	Cellular and Molecular Mechanisms of FUS-Related Amyotrophic Lateral Sclerosis	$0.00	$0.00	$0.00	01-Jan-17	28-Feb-18
Neurology	Pandey, Udai	National Institutes of Health	Cellular and Molecular Mechanisms of FUS-Related Amyotrophic Lateral Sclerosis	$8,300.00	$4,482.00	$12,782.00	01-Jan-17	28-Feb-18
Neurology	Pandey, Udai	National Institutes of Health	Cellular and Molecular Mechanisms of FUS-Related Amyotrophic Lateral Sclerosis	$249,267.00	$137,089.00	$386,356.00	01-Mar-14	28-Feb-18
Neurology	Pandey, Udai	National Institutes of Health	Molecular Mechanisms of C9orf72 and Matrin 3 Interaction in ALS	$150,000.00	$82,313.00	$232,313.00	01-Feb-17	31-Jan-19
Pulmonology	Alcorn, John	National Institutes of Health	Influenza A Inhibits TH17 Host Defense against Bacterial Pneumonia	$250,000.00	$137,813.00	$387,813.00	01-Apr-17	31-Mar-21
Pulmonology	Brehm, John	National Institutes of Health	Vitamin D, Genomics, and Asthma Morbidity in Puerto Rican children	$0.00	$0.00	$0.00	01-Aug-13	30-Jun-16
Pulmonology	Celedon, Juan C	National Institutes of Health	Pittsburgh Training Grant in Pediatric Pulmonary Medicine	$141,325.00	$9,819.00	$151,144.00	01-Jul-16	30-Jun-21
Pulmonology	Celedon, Juan C	National Institutes of Health	Vitamin D to Prevent Severe Asthma Exacerbations in High-Risk Children	$630,661.00	$111,781.00	$742,442.00	15-Jun-15	31-May-20
Pulmonology	Celedon, Juan C	National Institutes of Health	Epigenetic Variation and Childhood Asthma in Puerto Ricans	$0.00	$0.00	$0.00	01-Aug-13	31-Oct-17
Pulmonology	Chen, Wei	National Institutes of Health	AMD Genetics: Methods and Analysis for Progression, Prediction, and Association	$0.00	$0.00	$0.00	01-Apr-14	31-Mar-18
Pulmonology	Chen, Wei	National Institutes of Health	Statistical Methods for Population and Family-Based Whole-Genome Sequence Data	$216,216.00	$90,039.00	$306,255.00	25-Apr-14	31-Jan-19
Pulmonology	Forno, Erick	National Institutes of Health	Obesity and Asthma Subphenotypes and Underlying Genetic and Genomic Pathways	$139,339.00	$10,987.00	$150,326.00	01-Jul-15	30-Jun-19
Pulmonology	Wang, Jieru	National Institutes of Health	Regulation of Alveolar Epithelial Homeostasis in Acute Lung Injury	$0.00	$0.00	$0.00	01-Mar-14	28-Feb-18
Richard King Mellon Foundation Institute	Canna, Scott	National Institutes of Health	Mechanisms of NLRC4 Inflammasome-Associated Hyperinflammation	$150,000.00	$12,000.00	$162,000.00	01-Mar-17	28-Feb-19
Richard King Mellon Foundation Institute	Hand, Timothy	National Institutes of Health	Basic and Translational Studies of Cystic Fibrosis	$82,560.00	$44,582.00	$127,142.00	01-Jun-16	31-May-17
Richard King Mellon Foundation Institute	Hand, Timothy	National Institutes of Health	Regulation of Commensal-Specific T Cells during Gastrointestinal Inflammation	$100,000.00	$8,000.00	$108,000.00	20-Aug-15	31-Jul-17

NATIONAL INSTITUTES OF HEALTH (continued)

DIVISION	NAME	AGENCY TITLE	GRANT TITLE	ANNUAL DIRECT COST	ANNUAL INDIRECT COST	ANNUAL TOTAL COST	GRANT BEGIN DATE	GRANT END DATE
Richard King Mellon Foundation Institute	Kolls, Jay	National Institutes of Health	Th17 Cytokines and Lung Immunity	$288,539.00	$152,312.00	$440,851.00	01-Jan-15	31-Dec-19
Richard King Mellon Foundation Institute	Kolls, Jay	National Institutes of Health	T Cells and *P. carinii* Pneumonia	$279,433.00	$150,894.00	$430,327.00	01-Feb-16	31-Jan-20
Richard King Mellon Foundation Institute	Kolls, Jay	National Institutes of Health	Improved Therapeutics and Diagnostics for *Pneumocystis* Pneumonia	$310,531.00	$166,015.00	$476,546.00	01-Feb-16	31-Jan-21
Richard King Mellon Foundation Institute	Kolls, Jay	National Institutes of Health	Generation of Novel Human Monoclonals for Lung Disease	$131,674.00	$72,584.00	$204,258.00	01-Apr-16	31-Mar-18
Weight Management and Wellness	Lee, SoJung	National Institutes of Health	Resistance and Cardiorespiratory Time-Matched Exercise Training in Youth: A Randomized, Controlled Trial	$745,378.00	$331,768.00	$1,077,146.00	15-Aug-13	31-May-18

OTHER FEDERAL FUNDING

DIVISION	NAME	AGENCY TITLE	GRANT TITLE	ANNUAL DIRECT COST	ANNUAL INDIRECT COST	ANNUAL TOTAL COST	GRANT BEGIN DATE	GRANT END DATE
Adolescent Medicine	Miller, Elizabeth	Health Resources and Services Administration	Behavioral Health and Workforce Education and Training for Professionals	$14,022.00	$1,123.00	$15,145.00	30-Sep-14	29-Sep-17
Adolescent Medicine	Miller, Elizabeth	Centers for Disease Control and Prevention	A Cluster-Randomized Trial of a Middle School Gender Violence Prevention Program	$232,732.00	$117,146.00	$349,878.00	01-Sep-14	31-Aug-17
Adolescent Medicine	Miller, Elizabeth	Centers for Disease Control and Prevention	Engendering Healthy Masculinities to Prevent Sexual Violence	$294,474.00	$155,206.00	$449,680.00	30-Sep-14	29-Sep-18
Child Development Unit	Noll, Robert	Health Resources and Services Administration	LEND Center of Pittsburgh	$81,220.00	$4,252.00	$85,472.00	01-Jul-11	31-Dec-16
Endocrinology	Dong, Hengjiang	Pennsylvania Department of Health	Development of Anti-Fox01 Small-Molecule Drugs for Diabetic Hyperlipidemia	$70,833.00	$14,167.00	$85,000.00	01-Jan-17	31-Dec-20
Infectious Diseases	Lin, Philana Ling	Pennsylvania Department of Health	Blood Signature of Reactivation Tuberculosis Risk	$54,847.00	$10,969.00	$65,816.00	01-Jan-17	31-Dec-20
Infectious Diseases	Williams, John	Centers for Disease Control and Prevention	New Vaccine Surveillance Network (Supplement)	$509,377.00	$276,338.00	$785,715.00	01-Sep-16	31-Aug-17
Infectious Diseases	Williams, John	Centers for Disease Control and Prevention	New Vaccine Surveillance Network	$150,682.00	$63,603.00	$214,285.00	01-Sep-16	31-Aug-21
Neurology	Asato, Miya	Department of Health and Human Services	LEND Center at the University of Pittsburgh	$570,732.00	$45,659.00	$616,391.00	01-Jul-16	30-Jun-21
Neurology	Pandey, Udai	Pennsylvania Department of Health	Determining Pathogenic Mechanisms of Amyotrophic Lateral Sclerosis	$70,834.00	$14,166.00	$85,000.00	01-Jan-17	31-Dec-20
Pulmonology	Alcorn, John	Pennsylvania Department of Health	The Role of IL-22 in Influenza, Bacterial Super-Infection	$70,833.00	$14,167.00	$85,000.00	01-Jan-17	31-Dec-20
Rheumatology	Vallejo, Abbe	Pennsylvania Department of Health	Unraveling Innate Immunopathways of Healthy Longevity in a Mouse Model	$70,831.00	$14,166.00	$84,997.00	01-Jan-17	31-Dec-20

FOUNDATIONS AND OTHER FUNDING

DIVISION	NAME	AGENCY TITLE	TITLE	ANNUAL DIRECT COST	ANNUAL INDIRECT COST	ANNUAL TOTAL COST	GRANT BEGIN DATE	GRANT END DATE
Adolescent Medicine	Miller, Elizabeth	National Institutes of Health	Predicting Suicide Risk in Sexual Minority and Nonminority Youth: Associations among Neural Sensitivity to Social Rejection, Peer Rejection, and Suicidality	$4,103.00	$328.00	$4,431.00	01-Jun-17	31-May-18
Adolescent Medicine	Miller, Elizabeth	National Institutes of Health	Substance Use Disparities among Transgender Youth	$11,458.00	$6,216.00	$17,674.00	15-Sep-15	31-Aug-17
Adolescent Medicine	Miller, Elizabeth	National Institutes of Health	Pittsburgh Girls Study: Substance Use and HIV Risk Behaviors/STI in Young Adulthood	$10,313.00	$5,569.00	$15,882.00	01-Jul-16	30-Apr-21
Adolescent Medicine	Miller, Elizabeth	National Institutes of Health	Pittsburgh Girls Study: Substance Use and HIV Risk Behaviors/STI in Young Adulthood	$11,661.00	$6,443.00	$18,104.00	01-Jul-16	30-Apr-21
Adolescent Medicine	Miller, Elizabeth	National Institutes of Health	University of Pittsburgh Clinical and Translational Science Institute, administrative budget	$10,503.00	$5,672.00	$16,175.00	12-Jul-16	31-May-18
Adolescent Medicine	Miller, Elizabeth	National Institutes of Health	University of Pittsburgh Clinical and Translational Science Institute, administrative budget	$11,670.00	$6,462.00	$18,132.00	12-Jul-16	31-May-18
Adolescent Medicine	Miller, Elizabeth	National Institutes of Health	University of Pittsburgh Clinical and Translational Science Institute, core budget	$30,233.00	$16,326.00	$46,559.00	12-Jul-16	31-May-21
Adolescent Medicine	Miller, Elizabeth	National Institutes of Health	University of Pittsburgh Clinical and Translational Science Institute, core budget	$23,339.00	$12,924.00	$36,263.00	12-Jul-16	31-May-21
Adolescent Medicine	Miller, Elizabeth	Johns Hopkins University	The IPV Provider Network: Engaging the Health Care Provider Response to Interpersonal Violence Against Women	$56,157.00	$16,847.00	$73,004.00	15-Aug-15	31-Jul-18
Adolescent Medicine	Miller, Elizabeth	Grable Foundation	Sisterhood 2.0	$20,000.00	$0.00	$20,000.00	01-Jan-17	31-Dec-17
Adolescent Medicine	Miller, Elizabeth	American Academy of Pediatrics	Community-Pediatric Partnerships for Child Well-Being	$17,500.00	$0.00	$17,500.00	01-Jul-15	14-Jan-17
Adolescent Medicine	Miller, Elizabeth	Blue Shield of California	Improving Systems of Care: Exploring Local-Level Impacts	$20,970.00	$2,950.00	$23,920.00	01-Jan-16	30-Nov-17
Adolescent Medicine	Pulcini, Christian	American Academy of Pediatrics	Trauma-Informed Home Visitation for At-Risk Families	$2,000.00	$0.00	$2,000.00	01-Sep-16	28-Feb-17
Allergy and Immunology	Chen, Wei	National Institutes of Health	Parameters That Underlie Treg Insufficiency in Autoimmune Diabetes	$12,915.00	$7,071.00	$19,986.00	01-Jan-17	31-Dec-17
Allergy and Immunology	Chen, Wei	National Institutes of Health	Endogenous Alpha-to-Beta Cells Transdifferentiation in Diabetes	$8,599.00	$4,741.00	$13,340.00	01-Apr-17	31-Mar-22
Allergy and Immunology	Green, Todd	Food Allergy Research and Education	FARE Clinical Center of Excellence FCN	$109,091.00	$10,909.00	$120,000.00	01-Jul-15	30-Jun-17
Allergy and Immunology	Manni, Michelle L.	National Institutes of Health	Anti-Inflammatory Lipid Mediators in Asthma	$9,285.00	$5,014.00	$14,299.00	05-Aug-16	30-Jun-17
Allergy and Immunology	Manni, Michelle L.	Parker B. Francis Foundation	The Role of IL-22Ra2 in Allergic Airway Disease	$50,000.00	$0.00	$50,000.00	01-Jul-16	30-Jun-19
Bone Marrow Transplant/Cell Therapy	Byersdorfer, Craig	Hyundai Hope on Wheels	Metabolic Inhibition to Preserve Anti-Leukemia Responses while Minimizing GVHD	$125,000.00	$0.00	$125,000.00	01-Jan-17	31-Dec-18
Bone Marrow Transplant/Cell Therapy	Szabolcs, Paul	National Institutes of Health	Dendritic Cell (Dcreg) Therapy in Live Donor Renal Transplant Recipients	$4,583.00	$2,481.00	$7,064.00	15-Aug-16	31-Jul-17
Bone Marrow Transplant/Cell Therapy	Szabolcs, Paul	Emory University	Hematopoietic Stem Cell Transplantation for Young Adults with Sickle Cell Disease-Capitation Portion	$0.00	$0.00	$0.00	01-Sep-15	31-Jul-17
Bone Marrow Transplant/Cell Therapy	Szabolcs, Paul	Emory University	Hematopoietic Stem Cell Transplantation for Young Adults with Sickle Cell Disease	$22,488.00	$12,171.00	$34,659.00	01-Sep-15	31-Jul-17
Cardiology	Feingold, Brian	Wayne State University	Cardiac Biomarkers in Pediatric Cardiomyopathy	$949.00	$512.00	$1,461.00	01-Apr-15	30-Jun-17

FOUNDATIONS AND OTHER FUNDING (continued)

DIVISION	NAME	AGENCY TITLE	TITLE	ANNUAL DIRECT COST	ANNUAL INDIRECT COST	ANNUAL TOTAL COST	GRANT BEGIN DATE	GRANT END DATE
Cardiology	Feingold, Brian	Vanderbilt University Medical Center	Chronic Graft Destruction: Interplay of Allo- and Autoantibodies and Nonadherence	$26,644.00	$14,388.00	$41,032.00	01-Feb-13	31-Jan-17
Cardiology	Feingold, Brian	New England Research Institute	Pediatric Health Network FUEL Trial	$69,052.00	$37,806.00	$106,858.00	01-Jan-17	30-Jun-17
Child Advocacy Center	Berger, Rachel	National Institutes of Health	Validation of Serum Biomarkers to Classify Outcome after Pediatric Cardiac Arrest	$3,563.00	$1,924.00	$5,487.00	01-Jun-16	31-May-21
Child Advocacy Center	Berger, Rachel	Pennsylvania Department of Public Welfare	Pennsylvania Child Welfare Training Program - IV-B Project	$5,571.00	$836.00	$6,407.00	01-Jul-11	30-Jun-17
Child Advocacy Center	Berger, Rachel	Pennsylvania Department of Public Welfare	Pennsylvania Child Welfare Training Program - IV-B Project	$12,999.00	$1,950.00	$14,949.00	01-Jul-11	30-Jun-17
Child Advocacy Center	Berger, Rachel	Patient-Centered Outcomes Research Institute	Using the EMR to Decrease Disparities in Screening for Child Physical Abuse	$27,768.00	$11,107.00	$38,875.00	01-Sep-13	31-Aug-17
Child Advocacy Center	Berger, Rachel	Hillman Center	Decreasing Unsafe Sleep in Allegheny County: A Multidisciplinary, Multipronged Approach	$181,819.00	$18,181.00	$200,000.00	01-Apr-16	31-Mar-18
Child Development Unit	Johnson, Cynthia	Autism Speaks	Autism Treatment Network 2014 Cycle	$35,905.00	$3,590.00	$39,495.00	01-Sep-14	31-Aug-17
Child Development Unit	Noll, Robert	University of Rochester School of Medicine	Problem-Solving Skills Training for Clinicians Providing Psychosocial Care in Pediatric Oncology	$70,646.00	$5,652.00	$76,298.00	01-Apr-15	31-Mar-20
Child Development Unit	Noll, Robert	Children's Research Institute	Neurocognitive Assessments within COG: An Intensive Model for Successfully Evaluating Children with High-Risk ALL	$6,415.00	$0.00	$6,415.00	15-Dec-14	14-Dec-17
Cystic Fibrosis Research Center	Bertrand, Carol	National Institutes of Health	Building Multilevel Models of Therapeutic Response in the Lungs	$17,035.00	$9,199.00	$26,234.00	01-Sep-16	30-Jun-17
Cystic Fibrosis Research Center	Bertrand, Carol	Cystic Fibrosis Foundation	Regulation of a Cystic Fibrosis Disease Modifier, SLC26A9	$125,000.00	$0.00	$125,000.00	01-Mar-17	28-Feb-18
Cystic Fibrosis Research Center	Frizzell, Raymond	National Institutes of Health	Basic and Translational Studies of Cystic Fibrosis, operating account, Core A	$291,177.00	$157,236.00	$448,413.00	01-Jul-16	31-May-17
Cystic Fibrosis Research Center	Frizzell, Raymond	National Institutes of Health	Basic and Translational Studies of Cystic Fibrosis, operating account, Core A	$299,912.00	$161,952.00	$461,864.00	01-Jul-16	31-May-17
Cystic Fibrosis Research Center	Frizzell, Raymond	Cystic Fibrosis Foundation	Selective Steps in Wild-Type and F508del CFTR Processing	$230,238.00	$18,419.00	$248,657.00	01-Sep-15	31-Aug-16
Cystic Fibrosis Research Center	Frizzell, Raymond	Cystic Fibrosis Foundation	PITT RDP Research and Development Program Component II	$500,000.00	$0.00	$500,000.00	01-Jul-15	30-Jun-19
Emergency Medicine	Hickey, Robert	University of Virginia	Established Status Epilepticus Treatment Trial	$649.00	$351.00	$1,000.00	01-Sep-14	28-Feb-18
Emergency Medicine	Hickey, Robert	University of Michigan	Emergency Department Screen for Teens at Risk for Suicide	$107,840.00	$58,234.00	$166,074.00	01-Sep-14	30-Jun-17
Emergency Medicine	Hickey, Robert	University of Michigan	Supplement-Emergency Department Screen for Teens at Risk for Suicide	$14,873.00	$8,031.00	$22,904.00	01-Jul-16	30-Jun-17
Emergency Medicine	Hickey, Robert	Centers for Disease Control and Prevention	Outpatient VE for Seasonal Flu, Pandemic Flu, and RSV in a Large, Diverse Network	$42,976.00	$23,261.00	$66,237.00	01-Aug-16	31-Jul-21
Emergency Medicine	Hickey, Robert	Nationwide Children's Hospital	Great Lakes Emergency Medical Services for Children Research Network	$86,409.00	$25,923.00	$112,332.00	01-Sep-16	31-Aug-19
Emergency Medicine	Manole, Mioara	National Institutes of Health	Mitochondria-Targeted Redox Therapy for Cerebral Ischemia in the Developing Brain	$4,706.00	$2,541.00	$7,247.00	15-Mar-14	31-Dec-18
Endocrinology	Arslanian, Silva	National Institutes of Health	University of Pittsburgh Clinical and Translational Science Institute	$78,303.00	$42,284.00	$120,587.00	01-Jul-11	30-Jun-18
Endocrinology	Arslanian, Silva	George Washington University	Treatment Options for Type 2 Diabetes in Adolescents and Youth TODAY2 PHASE 2 (T2P2) Long-Term Post-Intervention Follow-Up Protocol	$155,898.00	$79,951.00	$235,849.00	01-Aug-15	30-Apr-20

FOUNDATIONS AND OTHER FUNDING (continued)

DIVISION	NAME	AGENCY TITLE	TITLE	ANNUAL DIRECT COST	ANNUAL INDIRECT COST	ANNUAL TOTAL COST	GRANT BEGIN DATE	GRANT END DATE
Endocrinology	Arslanian, Silva	George Washington University	TODAY2 Phase 2 (T2P2): Long-Term Post-Intervention Follow-Up	$194,009.00	$107,190.00	$301,199.00	01-May-16	30-Apr-21
Endocrinology	Arslanian, Silva	University of Colorado Denver	Brief Intensive Glycemic Control and Beta Cell Function in Pediatric Type 2 Diabetes	$280,735.00	$133,915.00	$414,650.00	24-Sep-11	30-Jun-17
Endocrinology	Becker, Dorothy	University of South Florida	Diabetes Prevention Trial Supplement	$86,863.00	$0.00	$86,863.00	17-Apr-09	06-Feb-18
Endocrinology	Becker, Dorothy	Carnegie Mellon University	From Adolescence to Adulthood: Persons with and without Diabetes	$55,412.00	$28,537.00	$83,949.00	01-Jul-12	30-Jun-17
Endocrinology	Becker, Dorothy	University of South Florida	The Environmental Determinants of Diabetes in the Young	$3,585.00	$1,936.00	$5,521.00	01-Sep-13	30-Jun-17
Endocrinology	Becker, Dorothy	University of South Florida	Clinic Affiliate Prediction and Prevention 1 Diabetes (TrialNet)	$72,727.00	$39,273.00	$112,000.00	23-Mar-15	30-Jun-17
Endocrinology	Becker, Dorothy	University of Bristol	What Protects Islet Autoantibody-Positive T1D Relatives Who Do Not Progress?	$18,940.00	$1,894.00	$20,834.00	01-Apr-15	31-Aug-17
Endocrinology	Dong, Hengjiang	American Diabetes Association	Hepatic Insulin for Diabetes	$104,545.00	$10,455.00	$115,000.00	01-Jan-17	31-Dec-19
Endocrinology	Witchel, Selma	National Institutes of Health	Substance Use Disparities Among Transgender Youth	$8,973.00	$4,868.00	$13,841.00	15-Sep-15	31-Aug-17
Endocrinology	Witchel, Selma	American Academy of Pediatrics	Persistent Symptoms of Hyperandrogenism in Girls with a History of Premature Adrenarche or Adolescent Hyperandrogenism	$0.00	$0.00	$0.00	01-Aug-15	31-May-17
Gastroenterology	Keljo, David	Cornell University, Weill Cornell Medicine	Sex Differences in Statural Growth Impairment in Pediatric Crohn's Disease	$14,719.00	$3,798.00	$18,517.00	01-Sep-16	30-Apr-19
Gastroenterology	Keljo, David	Cornell University, Weill Cornell Medicine	Sex Differences in Statural Growth Impairment in Pediatric Crohn's Disease	$9,875.00	$5,333.00	$15,208.00	01-Sep-16	30-Apr-19
Gastroenterology	Keljo, David	Cornell University, Weill Cornell Medicine	Sex Differences in Statural Growth Impairment in Pediatric Crohn's Disease, Per Patient	$7,957.00	$0.00	$7,957.00	01-Sep-16	30-Apr-17
Gastroenterology	Keljo, David	Emory University	Risk Stratification and Identification of Immunogenetic and Microbial Markers of Complicated Disease Course in Pediatric Crohn's Disease	$7,636.00	$764.00	$8,400.00	01-Jul-16	30-Jun-17
Gastroenterology	Keljo, David	Mount Sinai School of Medicine	A Multidisciplinary Human Study on the Genetic, Environmental, and Microbial Interactions That Cause IBC	$0.00	$0.00	$0.00	01-Sep-14	30-Sep-17
Gastroenterology	Keljo, David	Mount Sinai School of Medicine	A Multidisciplinary Human Study on the Genetic, Environmental, and Microbial Interactions That Cause IBD (GEM Project) Per Patient	$0.00	$0.00	$0.00	01-Sep-14	30-Sep-17
Gastroenterology	Khan, Zahida	Alpha-1 Foundation	Advanced Single-Cell Analysis of Proliferation in PiZ Mouse Hepatocytes	$40,000.00	$0.00	$40,000.00	01-Jul-16	30-Jun-17
Gastroenterology	Lowe, Mark	National Institutes of Health	Consortium for the Study of Pancreatitis: Pittsburgh Clinical Center	$4,583.00	$2,486.00	$7,069.00	01-Sep-15	31-Mar-17
Gastroenterology	Siebold, Leah	Hospital for Sick Children	NEOPICS-Intestinal Disease Biobank: Genetic, Functional, and Microbial Studies in Intestinal Disease	$9,091.00	$909.00	$10,000.00	01-Oct-14	30-Sep-17
Gastroenterology	Siebold, Leah	Hospital for Sick Children	Defining Very Early Onset IBD	$1,500.00	$150.00	$1,650.00	01-Sep-16	28-Feb-17
Gastroenterology	Squires, James	National Institutes of Health	Creating Models of Rare Childhood Liver Diseases Using the Human Liver-on-a-Chip	$2,101.00	$1,134.00	$3,235.00	01-Jul-16	30-Jun-17
Gastroenterology	Squires, James	Pittsburgh Liver Research Center	Pilot and Feasibility Grant	$25,000.00	$0.00	$25,000.00	01-Sep-16	31-Aug-17
Gastroenterology	Squires, Robert	National Institutes of Health	Hepatitis B Clinical Research Network, Data Coordinating Center	$11,670.00	$6,302.00	$17,972.00	01-Sep-15	31-May-20
Gastroenterology	Venkat, Veena	University of California San Francisco	Immunosuppression Withdrawal for Stable Pediatric Liver Transplant Recipients	$1,084.00	$558.00	$1,642.00	01-Jul-14	30-Jun-17

FOUNDATIONS AND OTHER FUNDING (continued)

DIVISION	NAME	AGENCY TITLE	TITLE	ANNUAL DIRECT COST	ANNUAL INDIRECT COST	ANNUAL TOTAL COST	GRANT BEGIN DATE	GRANT END DATE
Gastroenterology	Xiao, Xunjun	National Pancreas Foundation	CEL-HYB Confer Susceptibility to Chronic Pancreatitis through Proteotoxicity	$537.00	$0.00	$537.00	01-Jul-16	31-Mar-17
General Academic Pediatrics	Bogen, Debra	National Institutes of Health	Factors Contributing to Oral Health Disparities in Appalachia	$4,802.00	$2,593.00	$7,395.00	01-Jul-16	30-Jun-21
General Academic Pediatrics	Bogen, Debra	National Institutes of Health	University of Pittsburgh Clinical and Translational Science Institute	$63,431.00	$34,253.00	$97,684.00	12-Jul-16	31-May-21
General Academic Pediatrics	Bogen, Debra	National Institutes of Health	University of Pittsburgh Clinical and Translational Science Institute	$73,901.00	$40,923.00	$114,824.00	12-Jul-16	31-May-21
General Academic Pediatrics	Bogen, Debra	Tufts Medical Center, Inc.	Improving Outcomes in Neonatal Abstinence Syndrome	$12,870.00	$6,950.00	$19,820.00	01-Oct-14	30-Jun-17
General Academic Pediatrics	Bogen, Debra	University of Massachusetts Worcester	Stochastic Vibro-Tactile Stimulation: A Non-Pharmacological Intervention for Abstinence and Drug Withdrawal in Newborn Infants	$137,948.00	$74,493.00	$212,441.00	01-Aug-16	30-Jun-21
General Academic Pediatrics	Bogen, Debra	Magee-Womens Research Institute and Foundation	A Phase 1 Pharmacokinetic Trial of Ledipasvir/Sofosbuvir (LDV/SOF) Fixed-Dose Combination in Pregnant Women with Chronic Hepatitis C Infections	$9,667.00	$5,244.00	$14,911.00	21-Sep-16	31-Aug-17
General Academic Pediatrics	Bogen, Debra	National Science Foundation	Optimal Management of Donor Milk Banks	$3,953.00	$2,135.00	$6,088.00	01-Sep-15	31-Aug-18
General Academic Pediatrics	Bogen, Debra	RAND Corporation	Expanding Rural Access to Breastfeeding Support: A Pilot RCT	$45,176.00	$24,904.00	$70,080.00	01-Apr-16	31-Mar-19
General Academic Pediatrics	Bogen, Debra	Magee-Womens Research Institute and Foundation	A Phase 1 Pharmacokinetic Trial of Ledipasvir/Sofosbuvir Fixed-Dose Combination in Pregnant Women with Chronic Hepatitis C Virus Infection	$14,750.00	$2,452.00	$17,202.00	01-Aug-16	31-Jul-17
General Academic Pediatrics	Hoberman, Alejandro	Ricoh Innovations Corporation	Acquisition and Classification of Light-field Data of the Tympanic Membrane ™ and Middle Ear Processes of Children Suspected of Having Otitis Media	$202,230.00	$124,371.00	$326,601.00	28-Mar-14	31-Mar-17
General Academic Pediatrics	Malinow, Ana	American Academy of Pediatrics	Making it to the Doctor	$10,000.00	$0.00	$10,000.00	01-Sep-16	31-Aug-17
General Academic Pediatrics	Martin, Judith	Centers for Disease Control and Prevention	Outpatient VE for Seasonal Flu, Pandemic Flu, and RSV in a Large, Diverse Network (CORE-VE)	$50,043.00	$27,086.00	$77,129.00	01-Aug-16	31-Jul-17
General Academic Pediatrics	Martin, Judith	Vanderbilt University	Vaccine and Treatment Evaluation Units, Task Area B-C Task Order 14-0079. B1C1.0028	$118,334.00	$63,899.00	$182,233.00	01-Sep-15	31-Jul-18
General Academic Pediatrics	Ray, Kristin	Harvard Medical School	Evaluation the Los Angeles Department of Health Services eConsult Program Project	$8,143.00	$1,222.00	$9,365.00	01-Oct-16	30-Sep-18
General Academic Pediatrics	Reis, Evelyn	National Institutes of Health	University of Pittsburgh Clinical and Translational Science Institute	$10,521.00	$5,681.00	$16,202.00	12-Jul-16	31-May-21
General Academic Pediatrics	Reis, Evelyn	National Institutes of Health	University of Pittsburgh Clinical and Translational Science Institute	$11,233.00	$6,220.00	$17,453.00	12-Jul-16	31-May-21
General Academic Pediatrics	Reis, Evelyn	Centers for Disease Control and Prevention	Outpatient VE for Seasonal Flu, Pandemic Flu, and RSV in a Large, Diverse Network	$7,671.00	$4,152.00	$11,823.00	01-Aug-16	31-Jul-21
General Academic Pediatrics	Shope, Timothy	American Academy of Pediatrics	Improving the Pediatric Community's Readiness and Response to Seasonal and Pandemic Influenza	$14,286.00	$7,714.00	$22,000.00	01-Nov-14	30-Jun-17
Genetics	Madan-Khetarpal, Suneeta	National Institutes of Health	Genetics of Extracellular Matrix in Health and Disease	$25,068.00	$13,787.00	$38,855.00	01-Apr-15	31-Mar-20
Genetics	Nicholls, Robert	Spastic Paraplegia Foundation, Inc.	Gene Co-Regulation in the Hereditary Spastic Paraplegias	$0.00	$0.00	$0.00	01-Oct-10	31-Dec-17
Genetics	Nicholls, Robert	Foundation for Prader-Willi Research	Activation of Silenced Genes in Prader-Willi Syndrome	$0.00	$0.00	$0.00	01-Sep-15	30-Jun-17
Genetics	Nicholls, Robert	Foundation for Prader-Willi Research	Activation of Silenced Genes in Prader-Willi Syndrome	$0.00	$0.00	$0.00	01-Sep-15	30-Jun-17

FOUNDATIONS AND OTHER FUNDING (continued)

DIVISION	NAME	AGENCY TITLE	TITLE	ANNUAL DIRECT COST	ANNUAL INDIRECT COST	ANNUAL TOTAL COST	GRANT BEGIN DATE	GRANT END DATE
Genetics	Nicholls, Robert	Foundation for Prader-Willi Research	Understanding Multiple Hormone Secretion Deficits in Prader-Willi Syndrome	$100,000.00	$8,000.00	$108,000.00	01-Oct-16	30-Sep-17
Genetics	Vockley, Gerard	National Institutes of Health	Pre-Clinical Studies of Novel Mitochondrial Gene Therapies	$5,156.00	$2,784.00	$7,940.00	01-Apr-15	31-Jan-19
Genetics	Vockley, Gerard	Baebies, Incorporated	Point-of-Birth Newborn Screening for Inborn Errors of Metabolism Using Digital Microfluidic Technology	$22,525.00	$12,219.00	$34,744.00	16-Sep-16	31-Aug-18
Genetics	Vockley, Gerard	Nebraska Medical Center	Sterol and Isoprenoid Diseases Consortium	$2,152.00	$1,162.00	$3,314.00	04-Sep-14	31-Aug-19
Hematology/ Oncology	Bailey, Kelly	Alex's Lemonade Stand	Micro-Environmental Regulators of Ewing Sarcoma Metastasis	$51,000.00	$0.00	$51,000.00	01-Oct-16	30-Sep-19
Hematology/ Oncology	Cohen, Debra	Emory University	Patient-Centered Comprehensive Medication Adherence Management System to Improve Effectiveness of Disease-Modifying Therapy with Hydroxyurea in Patients with Sickle Cell Disease	$48,862.00	$19,544.00	$68,406.00	01-Jul-14	31-Jan-17
Hematology/ Oncology	Cooper, James	All Children's Research Institute, Inc.	Prospective Evaluation of the Duration of Therapy for Thrombosis in Children Study	$6,310.00	$1,893.00	$8,203.00	11-Jan-17	30-Jun-17
Hematology/ Oncology	Cooper, James	All Children's Research Institute, Inc.	Multicenter Evaluation of the Duration of Therapy for Thrombosis in Children	$8,166.00	$0.00	$8,166.00	01-May-14	31-Dec-18
Hematology/ Oncology	Goyal, Rakesh	Children's Hospital of Philadelphia	Reduced-Intensity Conditioning for Children and Adults with Hemophagocytic Syndromes or Selected Primary Immune Deficiencies	$9,399.00	$5,044.00	$14,443.00	01-Jul-14	30-Jun-17
Hematology/ Oncology	Hillery, Cheryl	Medical College of Wisconsin	Mechanisms of Inflammation in Sickle Cell Disease	$50,000.00	$27,000.00	$77,000.00	01-Apr-16	31-Mar-21
Hematology/ Oncology	Lucas, Peter	U.S. Army	Transcriptomic Profiling and Functional Characterization of Fusion Genes in Recurrent Ovarian Cancer (OC150095)	$12,380.00	$6,709.00	$19,089.00	15-Aug-16	14-Aug-18
Hematology/ Oncology	Lucas, Peter	Breast Cancer Research Foundation	Intra-Tumor Heterogeneity (ITH) in Primary and Metastatic Breast Cancer	$12,650.00	$2,530.00	$15,180.00	01-Oct-15	30-Sep-17
Hematology/ Oncology	Lucas, Peter	Breast Cancer Research Foundation	Endocrine-Resistant Invasive Lobular Breast Cancer	$12,650.00	$2,530.00	$15,180.00	01-Oct-16	30-Sep-17
Hematology/ Oncology	Maurer, Scott	National Institutes of Health	Peptide Vaccine Immunotherapy for Children with Recurrent Low-Grade Astrocytomas	$44,672.00	$24,121.00	$68,793.00	08-Jan-16	31-Dec-17
Hematology/ Oncology	Prochownik, Edward	Washington University	Project 1: Preclinical and Clinical Imaging and Treatment of Multiple Myeloma with cMyc-Max Nanoparticles	$49,263.00	$26,602.00	$75,865.00	01-Sep-15	31-Jul-20
Hematology/ Oncology	Ritchey, Kim	Children's Hospital of Philadelphia	AALL1122 BMS CA180-372: A Phase 2, Multicenter, Historically Controlled Study of Dasatinib Added to Standard Chemotherapy in Pediatric Patients with Newly Diagnosed Philadelphia Chromosome-Positive Acute Lymphoblastic Leukemia	$1,667.00	$333.00	$2,000.00	21-Jun-12	30-Jun-17
Hematology/ Oncology	Tersak, Jean	Children's Hospital of Philadelphia	Children's Oncology Group Phase 1/ Pilot Consortium Grant	$22,728.00	$12,272.00	$35,000.00	01-Aug-16	31-Jul-17
Hematology/ Oncology	Tersak, Jean	Children's Hospital of Philadelphia	Per Case Reimbursement NIH National Clinical Trials Network BIQSFP Grant U10CA180886-02S7-COG AALL0932	$5,826.00	$3,175.00	$9,001.00	01-Mar-16	28-Feb-18
Hematology/ Oncology	Tersak, Jean	Children's Hospital of Philadelphia	CHOP/COG-NIH-NCTN-Per Case Reimbursement AALL1131	$19,252.00	$10,589.00	$29,841.00	01-Mar-16	28-Feb-18
Hematology/ Oncology	Tersak, Jean	Children's Hospital of Philadelphia	Per Case Children's Oncology Group Phase I	$25,000.00	$5,000.00	$30,000.00	01-Aug-10	31-Jul-17
Hematology/ Oncology	Tersak, Jean	Children's Hospital of Philadelphia	COG NCORP Research Base, Per Case Reimbursement	$20,779.00	$11,221.00	$32,000.00	01-Aug-14	31-Jul-19

FOUNDATIONS AND OTHER FUNDING (continued)

DIVISION	NAME	AGENCY TITLE	TITLE	ANNUAL DIRECT COST	ANNUAL INDIRECT COST	ANNUAL TOTAL COST	GRANT BEGIN DATE	GRANT END DATE
Hematology/ Oncology	Tersak, Jean	Children's Hospital of Philadelphia	ALTE1621 Reducing Risk of Anthracycline-Related Heart Failure after Childhood Cancer	$14,529.00	$7,937.00	$22,466.00	01-Dec-16	30-Nov-21
Hematology/ Oncology	Tersak, Jean	Children's Hospital of Philadelphia	CHOP/COG Chairs Grant Workload Intensity CA 180886	$36,472.00	$19,695.00	$56,167.00	11-Apr-14	28-Feb-19
Hematology/ Oncology	Tersak, Jean	Children's Hospital of Philadelphia	CHOP/COG NIH National Clinical Trials Network Per Case Reimbursement Grant CA 180886 Successor to COG Chairs Grant CA 98543	$51,949.00	$28,051.00	$80,000.00	01-Mar-14	28-Feb-18
Hematology/ Oncology	Tersak, Jean	Children's Hospital of Philadelphia	EVERYCHILD (APEC14B1) COG Foundation	$30,000.00	$0.00	$30,000.00	26-Oct-15	25-Oct-20
Hematology/ Oncology	Tersak, Jean	Children's Hospital of Philadelphia	Cancer Trials Support Unit (CTSU) Phase II Supplemental Payments (N01-CM-62212)	$11,688.00	$6,312.00	$18,000.00	01-Jul-14	30-Jun-17
Hematology/ Oncology	Tersak, Jean	Children's Hospital of Philadelphia	St. Baldrick's Foundation Supplemental Reimbursement	$0.00	$0.00	$0.00	26-Apr-12	25-Apr-18
Hematology/ Oncology	Tersak, Jean	Children's Hospital of Philadelphia	ADVL1315-Pfizer, Inc. No. WI181667: A Phase I Study of the VEGF Receptor Tyrosine Kinase Inhibitor Axitinib (INLYTA) in Children with Relapsed/ Refractory Solid Tumors	$13,600.00	$3,400.00	$17,000.00	29-Apr-15	28-Apr-18
Hematology/ Oncology	Tersak, Jean	Children's Hospital of Philadelphia	A Phase 3 Randomized Trial for Newly Diagnosed High-Risk B-Precursor Acute Lymphoblastic Leukemia Testing Clofarabine (IND#73789, NSC#606869) in the Very High Risk Stratum	$22,889.00	$5,722.00	$28,611.00	04-May-15	03-May-20
Hematology/ Oncology	Tersak, Jean	Children's Hospital of Philadelphia	ADVL0912 Pfizer Phase 1/2 Study of PF-02341066, an Oral Small Molecule Inhibitor of Anaplastic Lymphoma Kinase and C-Met, in Children with Relapsed/Refractory Solid Tumors and Anaplastic Large Cell Lymphoma	$5,480.00	$1,370.00	$6,850.00	05-May-15	04-May-18
Hematology/ Oncology	Tersak, Jean	Children's Hospital of Philadelphia	ADVL1212: A Phase 1 Study of Crizotinib (IND #105573) in Combination with Conventional Chemotherapy for Relapsed or Refractory Solid Tumors or Anaplastic Large Cell Lymphoma	$665.00	$166.00	$831.00	22-Jul-15	04-May-18
Hematology/ Oncology	Tersak, Jean	Children's Hospital of Philadelphia	ADVL114: A Phase 1 Study of Eribulin Mesylate (E7389, IND#116,292), a Novel Microtubule Targeting Chemotherapeutic Agent in Children with Refractory or Recurrent Solid Tumors (Excluding CNS), Including Lymphomas	$5,500.00	$1,375.00	$6,875.00	22-Jul-15	04-May-18
Hematology/ Oncology	Tersak, Jean	Children's Hospital of Philadelphia	A Phase III, Randomized, Open-Label, Multicenter Study of the Safety and Efficacy of Apixaban for Thromboembolism Prevention Versus No Systemic Anticoagulant Prophylaxis during Induction Chemotherapy in Children with Newly Diagnosed Acute Lymphoblastic	$18,217.00	$4,554.00	$22,771.00	17-Sep-15	16-Sep-20
Hematology/ Oncology	Tersak, Jean	Children's Hospital of Philadelphia	AAML 1522:CC-5013-AML-002: A Phase 2, Multicenter, Single-Arm, Open-Label Study to Evaluate the Activity, Safety, and Pharmacokinetics of Lenalidomide (Revlimid) in Pediatric Subjects with Relapsed or Refractory Acute Myeloid Leukemia	$6,815.00	$1,704.00	$8,519.00	25-Feb-16	24-Feb-21
Hematology/ Oncology	Tersak, Jean	Children's Hospital of Philadelphia	ADVL1412: A Phase 1/2 Study of Nivolumab in Children, Adolescents, and Young Adults with Recurrent or Refractory Solid Tumors as a Single Agent and in Combination with Ipilimumab	$20,965.00	$5,241.00	$26,206.00	03-Mar-16	02-Mar-19
Hematology/ Oncology	Tersak, Jean	Children's Hospital of Philadelphia	ADVL1416: A Phase 1 Study of Ramucirumab, a Human Monoclonal Antibody against the Vascular Endothelial Growth Factor-2 (VEGFR-2) Receptor in Children with Refractory Solid Tumors, Including CNS Tumors	$15,680.00	$4,000.00	$19,680.00	19-Apr-16	18-Apr-19

FOUNDATIONS AND OTHER FUNDING (continued)

DIVISION	NAME	AGENCY TITLE	TITLE	ANNUAL DIRECT COST	ANNUAL INDIRECT COST	ANNUAL TOTAL COST	GRANT BEGIN DATE	GRANT END DATE
Hematology/ Oncology	Tersak, Jean	Children's Hospital of Philadelphia	AHOD1331: A Randomized Phase III Study of Brentuximab Vedotin (SGN-35, IND #117117) for Newly Diagnosed High-Risk Classical Hodgkin Lymphoma (cHL) in Children and Adolescents: A Groupwide Phase III Study	$2,560.00	$640.00	$3,200.00	25-Aug-16	24-Aug-18
Hematology/ Oncology	Tersak, Jean	Children's Hospital of Philadelphia	ADVL1621: A Phase I/II Study of Pembrolizumab (MK-3475) in Children with Advanced Melanoma or PD-L1-Positive Advanced, Relapsed, or Refractory Solid Tumor or Lymphoma (Keynote-051) MK-3475-051	$35,702.00	$8,925.00	$44,627.00	01-Nov-16	31-Oct-21
Hematology/ Oncology	Tersak, Jean	Children's Hospital of Philadelphia	AALL1331: Risk-Stratified Randomized Phase III Testing of Blinatumomab (IND# 117467, NSC#765986) in First Relapse of Childhood B-Lymphoblastic Leukemia (B-ALL)	$12,966.00	$3,241.00	$16,207.00	06-Dec-16	31-Oct-21
Hematology/ Oncology	Tersak, Jean	Children's Hospital of Philadelphia	COG AALL1521: A Phase 2 Study of the JAK1/JAK2 Inhibitor Ruxolitinib with Chemotherapy in Children with *De Novo* High-Risk CRLF2-Rearranged and/or JAK Pathway-Mutant Acute Lymphoblastic Leukemia	$16,765.00	$4,191.00	$20,956.00	01-Mar-17	31-Jan-22
Hematology/ Oncology	Tersak, Jean	Children's Hospital of Philadelphia	AAML1031: A Phase III Randomized Trial for Patients with *De Novo* AML Using Bortezomib and Sorafenib (IN D#11480:NSC#681239,NSC#74772) for Patients with High Alleclic Ration FLT3/ITD	$5,807.00	$1,452.00	$7,259.00	01-Mar-17	31-Jan-22
Hematology/ Oncology	Tersak, Jean	Alex's Lemonade Stand	Children's Hospital of Pittsburgh of UPMC Phase 1-2 Infrastructure Support Grant Reapplication	$125,000.00	$0.00	$125,000.00	01-Jan-14	31-Dec-18
Infectious Diseases	Coyne, Carolyn	National Institutes of Health	Exosome-Based Placental Maternal Communication	$12,977.00	$7,121.00	$20,098.00	01-Feb-17	31-Jan-18
Infectious Diseases	Coyne, Carolyn	Magee-Womens Research Institute and Foundation	Primary Human Trophoblasts and the Transfer of Viral Resistance	$26,077.00	$13,430.00	$39,507.00	01-Mar-17	31-May-17
Infectious Diseases	Dermody, Terence	Burroughs Wellcome Fund	2017 International Congress of Virology (ICV)	$15,000.00	$0.00	$15,000.00	01-Mar-17	31-Oct-17
Infectious Diseases	Green, Michael	Duke University	Multicenter Studies to Improve Diagnosis and Treatment of Pediatric Candidiasis	$1,085.00	$585.00	$1,670.00	01-Jun-15	31-May-18
Infectious Diseases	Green, Michael	Duke University	Multicenter Study to Improve Diagnosis and Treatment of Pediatric Candidiasis, Biopic Study	$542.00	$293.00	$835.00	01-Jun-15	31-May-18
Infectious Diseases	Green, Michael	University of South Florida	NIDDK Type 1 Diabetes TrialNet Data Coordinating Center	$0.00	$0.00	$0.00	01-Jan-15	28-Feb-18
Infectious Diseases	Green, Michael	University of South Florida	NIDDK Type 1 Diabetes TrialNet Data Coordinating Center	$5,865.00	$3,167.00	$9,032.00	01-Jan-15	28-Feb-18
Infectious Diseases	Lin, Philana Ling	National Institutes of Health	Predicting Immune Responses That Correlate with Protection against Tuberculosis	$24,567.00	$13,266.00	$37,833.00	01-Aug-12	30-Jun-17
Infectious Diseases	Lin, Philana Ling	National Institutes of Health	PET Probes Targeting Immune Cells for Imaging Tuberculosis	$8,189.00	$4,422.00	$12,611.00	01-Jul-15	30-Jun-20
Infectious Diseases	Lin, Philana Ling	National Institutes of Health	The Consequences of Reinfection with *M. tuberculosis*	$16,378.00	$8,844.00	$25,222.00	01-Jul-15	31-Dec-19
Infectious Diseases	Lin, Philana Ling	National Institutes of Health	An Adjuvant That Promotes TH1/TH17 and CD8 T Cells in a Tuberculosis Vaccine	$16,179.00	$8,737.00	$24,916.00	01-Feb-13	31-Jan-18
Infectious Diseases	Lin, Philana Ling	National Institutes of Health	The Effects of *M. tuberculosis* Infection on Lung Microbiome in Macaques	$3,263.00	$1,795.00	$5,058.00	01-Mar-16	28-Feb-18
Infectious Diseases	Lin, Philana Ling	National Institutes of Health	Rational Design of Combination Regimens Targeting Both Host and Pathogen	$16,298.00	$8,842.00	$25,140.00	25-Sep-15	31-Aug-17

FOUNDATIONS AND OTHER FUNDING (continued)

DIVISION	NAME	AGENCY TITLE	TITLE	ANNUAL DIRECT COST	ANNUAL INDIRECT COST	ANNUAL TOTAL COST	GRANT BEGIN DATE	GRANT END DATE
Infectious Diseases	Lin, Philana Ling	University of Michigan	Predicting Protective T-Cell Responses in Tuberculosis Using a Biology Approach	$2,750.00	$1,485.00	$4,235.00	01-Oct-16	31-Jan-18
Infectious Diseases	Lin, Philana Ling	University of Michigan	Predicting Protective T-Cell Responses in Tuberculosis Using a Biology Approach	$8,232.00	$4,445.00	$12,677.00	01-Oct-16	31-Jan-18
Infectious Diseases	Lin, Philana Ling	Baylor College of Medicine	Surveillance of Invasive Pneumococcal Isolates after Pneumococcal Vaccination	$9,600.00	$2,400.00	$12,000.00	18-Jun-15	17-Jun-18
Infectious Diseases	Lin, Philana Ling	Harvard School of Public Health	Immune Signatures of Differential NHP Granuloma States	$17,343.00	$1,734.00	$19,077.00	01-Jan-16	30-Dec-17
Infectious Diseases	Lin, Philana Ling	Aeras Global TB Vaccine Foundation	Efficacy of BCG Administrated by Different Routes in the Rhesus Macaque Model of TB	$16,450.00	$1,645.00	$18,095.00	06-Dec-16	30-Nov-17
Infectious Diseases	Michaels, Marian	Children's Hospital of Philadelphia	BAA: A Multicenter Prospective Study of Human Adenovirus Infection and Disease in Pediatric Human Stem Cell Transplant Recipients	$76,297.00	$42,510.00	$118,807.00	01-Jul-16	30-Jun-21
Infectious Diseases	Michaels, Marian	University of Alabama	A Pharmacokinetic, Pharmacodynamic, and Resistance Evaluation of Intravenous Ganciclovir in Premature Infants	$15,102.00	$2,946.00	$18,048.00	01-Feb-12	27-Sep-17
Infectious Diseases	Michaels, Marian	Duke University	Prospective Observational Study of the Risk Factors for Hospital-Acquired and Ventilator-Associated Bacterial Pneumonia	$27,695.00	$14,955.00	$42,650.00	08-Dec-16	28-Mar-17
Infectious Diseases	Nowalk, Andrew John	National Institutes of Health	Colistin-Resistant *Acinetobacter baumannii*	$3,175.00	$1,715.00	$4,890.00	15-Mar-14	28-Feb-18
Infectious Diseases	Williams, John	Centers for Disease Control and Prevention	Optional Component: Flu Vaccine Effectiveness in Those Hospitalized in a Large Diverse Health System	$4,412.00	$2,382.00	$6,794.00	01-Aug-16	31-Jul-18
Infectious Diseases	Williams, John	Centers for Disease Control and Prevention	Flu Vaccine Effectiveness in Those Hospitalized in a Large Diverse Health System	$18,529.00	$10,006.00	$28,535.00	01-Aug-16	31-Jul-18
Infectious Diseases	Williams, John	Centers for Disease Control and Prevention	Outpatient VE for Seasonal Flu, Pandemic Flu, and RSV in a Large, Diverse Network	$63,993.00	$34,636.00	$98,629.00	01-Aug-16	31-Jul-21
Neonatology	Ghazi, Arjumand	Ellison Medical Foundation	miRNAs and Lipophilic Pathways That Transmit Reproductive Signals to Control Aging	$0.00	$0.00	$0.00	01-Jul-12	31-Dec-16
Neonatology	Ghazi, Arjumand	Aging Institute of UPMC Senior Services and the University of Pittsburgh	Suppression of Age-Related Germline Tumorigenesis by Mitochondria-Targeted ROS Modulators	$20,000.00	$0.00	$20,000.00	01-Feb-16	31-Jan-18
Neonatology	Lamitina, Samuel T.	United States-Israel Binational Science Foundation	Biomechanical Profiling of *C. elegans* motility	$7,794.00	$0.00	$7,794.00	01-Oct-14	30-Sep-17
Neonatology	Silverman, Gary	National Institutes of Health	New Therapies for Liver Fibrosis, Project 2	$0.00	$0.00	$0.00	24-Sep-12	31-Aug-17
Neonatology	Yanowitz, Toby Debra	University of California San Francisco	High-Dose Erythropoietin for Asphyxia and Encephalopathy	$38,021.00	$20,674.00	$58,695.00	30-Sep-16	30-Jun-21
Neonatology	Yanowitz, Toby Debra	UPMC Enterprises	A Non-Invasive Diagnostic Test for Necrotizing Enterocolitis	$6,222.00	$3,827.00	$10,049.00	01-Sep-16	31-May-17
Nephrology	Airik, Rannar	National Institutes of Health	Pittsburgh Center for Kidney Research	$30,000.00	$16,200.00	$46,200.00	01-Aug-16	31-Jul-18
Nephrology	Bates, Carl	National Institutes of Health	Pittsburgh Center for Kidney Research, Core C	$15,000.00	$8,100.00	$23,100.00	01-Aug-13	31-Jul-18
Nephrology	Bates, Carl	March of Dimes	17th Congress of the International Pediatric Nephrology Association	$5,000.00	$0.00	$5,000.00	01-Jul-16	30-Jun-17
Nephrology	Ho, Jacqueline	National Institutes of Health	Role of MicroRNAs in Kidney Sodium Regulation	$8,350.00	$4,509.00	$12,859.00	01-May-15	30-Apr-20
Nephrology	Ho, Jacqueline	National Institutes of Health	University of Pittsburgh as the GUDMAP Tissue Hub and Collection Site	$25,912.00	$13,992.00	$39,904.00	15-Sep-16	31-May-21

FOUNDATIONS AND OTHER FUNDING (continued)

DIVISION	NAME	AGENCY TITLE	TITLE	ANNUAL DIRECT COST	ANNUAL INDIRECT COST	ANNUAL TOTAL COST	GRANT BEGIN DATE	GRANT END DATE
Nephrology	Ho, Jacqueline	National Institutes of Health	University of Pittsburgh as the GUDMAP Tissue Hub and Collection Site	$36,506.00	$20,220.00	$56,726.00	15-Sep-16	31-May-21
Nephrology	Miyashita, Yosuke	Emory University	Cystinosis: Clinical Outcomes in a Contemporary Group of American Patients	$3,714.00	$2,286.00	$6,000.00	25-Oct-16	24-Oct-17
Nephrology	Nguyen, Christina	McGill University	Teen Adherence in Kidney Transplant: Improving Tracking to Optimize Outcomes	$3,611.00	$1,950.00	$5,561.00	01-Sep-16	30-Jun-21
Nephrology	Phua, Yu Leng	American Society of Nephrology Foundation	The Role of miR-17-92 in Nephron Progenitor Self-Renewal	$50,000.00	$0.00	$50,000.00	01-Jul-15	30-Jun-17
Nephrology	Santos, Deborah	University of Michigan	The Role of the miR-17-92 MicroRNA Cluster in Glomerulopathies	$28,935.00	$2,315.00	$31,250.00	01-Jan-17	31-Dec-17
Nephrology	Sims-Lucas, Sunder	National Institutes of Health	Effect of Aging on Urothelial Function	$19,480.00	$10,520.00	$30,000.00	30-Sep-15	31-Aug-20
Nephrology	Sims-Lucas, Sunder	National Institutes of Health	University of Pittsburgh as the GUDMAP Tissue Hub and Collection Site	$114,089.00	$61,607.00	$175,696.00	15-Sep-16	31-May-21
Nephrology	Sims-Lucas, Sunder	National Institutes of Health	University of Pittsburgh as the GUDMAP Tissue Hub and Collection Site	$103,494.00	$57,305.00	$160,799.00	15-Sep-16	31-May-21
Nephrology	Swiatecka-Urban, Agnieszka	National Institutes of Health	Basis and Clinical Studies of Cystic Fibrosis	$0.00	$0.00	$0.00	01-Jul-15	31-May-16
Nephrology	Swiatecka-Urban, Agnieszka	University of Pennsylvania	Primary Outcomes in Glomerulonephritis Study	$4,137.00	$1,720.00	$5,857.00	01-Jun-14	31-May-18
Nephrology	Swiatecka-Urban, Agnieszka	American Heart Association	Lemur Tyrosine Kinase and TGF Beta Signaling	$70,000.00	$7,000.00	$77,000.00	01-Jul-15	30-Jun-17
Neurology	Abdel-Hamid, Hoda	National Institutes of Health	Network of Excellence In Neuroscience Clinical Trials (University of Pittsburgh CRS)	$3,188.00	$1,642.00	$4,830.00	30-Sep-11	30-Jun-18
Neurology	Asato, Miya	Autism Speaks Network	Autism Speaks Network	$5,662.00	$566.00	$6,228.00	01-Sep-14	31-Aug-17
Neurology	Cummings, Dana	National Institutes of Health	Stroke Trials Network, Regional Coordinating Stroke Center	$3,283.00	$1,773.00	$5,056.00	30-Sep-13	31-Jul-17
Neurology	Escolar, Maria	PPD Investigator Services, LLC	Natural History of Metachromatic Leukodystrophy and the Assessment of Disease Progression in Untreated and Hematopoietic Stem Cell Therapy-Treated Patients	$2,000.00	$1,230.00	$3,230.00	01-Jul-16	30-Jun-17
Neurology	Escolar, Maria	Legacy of Angels Foundation	NDRD Krabbe Disease Patient Care and Research	$347,773.00	$17,389.00	$365,162.00	01-Oct-15	30-Sep-18
Neurology	Escolar, Maria	Legacy of Angels Foundation	Development Program for the Treatment of Krabbe Disease Using Intravenous Adeno-Associated Virus Gene Therapy	$544,491.00	$11,314.00	$555,805.00	01-Oct-15	30-Sep-18
Neurology	Escolar, Maria	Legacy of Angels Foundation	Treatment of Krabbe Disease Using Gene Therapy: Rat Toxicology Studies and Pre-Clinical Drug Production	$1,965,567.00	$98,278.00	$2,063,845.00	01-Oct-16	30-Sep-18
Neurology	Escolar, Maria	Legacy of Angels Foundation	A Pilot Engraftment Study in Immunosuppressed Rats	$62,354.00	$3,118.00	$65,472.00	01-Oct-16	30-Sep-17
Neurology	Goldstein, Amy	National Institutes of Health	Creating Models of Rare Childhood Liver Diseases Using the Human Liver Chip	$1,238.00	$669.00	$1,907.00	01-Jul-16	30-Jun-17
Neurology	Goldstein, Amy	Columbia University	North American Mitochondrial Disease Consortium	$1,299.00	$701.00	$2,000.00	01-Sep-13	31-Aug-17
Neurology	Pandey, Udai	Johns Hopkins University	Investigating the Molecular Mechanisms of FUS-Related ALS	$0.00	$0.00	$0.00	01-Apr-14	31-Dec-17
Pulmonology	Alcorn, John	National Institutes of Health	Immature Infant Alveolar Macrophage Clearance of Cell Debris during RSV Infection	$378.00	$204.00	$582.00	01-Apr-15	30-Jun-17
Pulmonology	Alcorn, John	National Institutes of Health	Basic and Clinical Studies of Cystic Fibrosis	$0.00	$0.00	$0.00	01-Jul-15	31-May-18
Pulmonology	Alcorn, John	American Heart Association	The Role of the Receptor for Advanced Glycation Endproducts in Asthma	$7,492.00	$749.00	$8,241.00	01-Jul-15	30-Jun-17

FOUNDATIONS AND OTHER FUNDING (continued)

DIVISION	NAME	AGENCY TITLE	TITLE	ANNUAL DIRECT COST	ANNUAL INDIRECT COST	ANNUAL TOTAL COST	GRANT BEGIN DATE	GRANT END DATE
Pulmonology	Celedon, Juan C.	Massachusetts General Hospital	Airway Microbiome and Age 6y Asthma Phenotypes in Two Diverse Multicenter Cohorts	$4,583.00	$2,486.00	$7,069.00	01-Sep-16	31-Aug-23
Pulmonology	Celedon, Juan C.	Icahn School of Medicine at Mt. Sinai	Pulmonary Diseases in WTC Workers: Symptoms, Function, and Chest CT Correlates	$22,915.00	$12,432.00	$35,347.00	01-Sep-16	31-Aug-17
Pulmonology	Celedon, Juan C.	Massachusetts General Hospital	Infant Specific-IgE, Rhinovirus-C Bronchiolitis, and Incident Asthma in MARC-35	$2,214.00	$1,196.00	$3,410.00	01-Dec-14	30-Nov-19
Pulmonology	Celedon, Juan C.	Massachusetts General Hospital	Nasal MicroRNA during Bronchiolitis and Age 6y Asthma Phenotypes: MARC-35 Cohort	$6,870.00	$3,752.00	$10,622.00	01-Dec-16	30-Nov-21
Pulmonology	Larkin, Allyson	National Institutes of Health	Implications and Stability of Clinical and Molecular Phenotypes of Severe Asthma	$33,011.00	$17,001.00	$50,012.00	09-Aug-11	31-May-17
Pulmonology	Orenstein, David	National Institutes of Health	Basic and Translational Studies of Cystic Fibrosis, Core B	$9,908.00	$5,350.00	$15,258.00	01-Jul-15	31-May-18
Pulmonology	Orenstein, David	Seattle Children's Hospital	OPTIMIZing Treatment for Early Pseudomonas aeruginosa Infection in Cystic Fibrosis: The OPTIMIZE Multicenter Randomized Trial, Clinical Coordinating Center	$22,283.00	$8,175.00	$30,458.00	15-Sep-13	30-Jun-18
Pulmonology	Orenstein, David	Seattle Children's Hospital	The EPIC Observational Study: Longitudinal Assessment of Risk Factors for and Impact of Pseudomonas aeruginosa Acquisition and Early Anti-Pseudomonal Treatment in Children with CF, Year 6-10	$12,818.00	$1,026.00	$13,844.00	01-Sep-13	31-Aug-18
Pulmonology	Orenstein, David	Cystic Fibrosis Foundation	ACTIVATE-CF: Effects of a Six-Month Partially Supervised Conditioning Program in CF: An International Multicenter, Randomized, Controlled Trial	$0.00	$0.00	$0.00	01-Nov-14	31-Oct-17
Pulmonology	Orenstein, David	Cystic Fibrosis Foundation	University of Pittsburgh Additional TDN Coordinator Support	$0.00	$0.00	$0.00	01-Dec-14	30-Nov-17
Pulmonology	Pilewski, Joseph	Seattle Children's Hospital	Proof of Principal Evaluation of IV Gallium Nitrate in Patients with Cystic Fibrosis	$40,386.00	$3,231.00	$43,617.00	01-Jul-16	30-Jun-17
Pulmonology	Pilewski, Joseph	Seattle Children's Hospital	Testing the Effect of Adding Chronic Azithromycin to Inhaled Tobramycin: A Randomized, Placebo-Controlled, Double-Blinded Trial of Azithromycin 500 MG Thrice Weekly in Combination with Inhaled Tobramycin Study	$9,700.00	$776.00	$10,476.00	01-Jan-16	31-Dec-18
Pulmonology	Pilewski, Joseph	Seattle Children's Hospital	Standardized Treatment of Pulmonary Exacerbations II	$5,945.00	$475.00	$6,420.00	01-Jan-16	31-Dec-18
Pulmonology	Pilewski, Joseph	Cystic Fibrosis Foundation	Additional Research Coordinator Award	$39,122.00	$3,130.00	$42,252.00	01-Jan-16	31-Dec-17
Pulmonology	Pilewski, Joseph	Cystic Fibrosis Foundation	Translational Therapeutics Development Center	$192,529.00	$15,402.00	$207,931.00	01-Jan-17	31-Dec-17
Pulmonology	Spahr, Jonathan	Cystic Fibrosis Foundation	CFF First- and Second-Year Clinical Fellowship	$62,144.00	$0.00	$62,144.00	01-Jul-15	30-Jun-17
Pulmonology	Wang, Jieru	National Institutes of Health	Combined Viral and Bacterial Infection and Zinc Homeostasis in Distal Lung	$13,202.00	$7,129.00	$20,331.00	01-Apr-15	31-Mar-20
Pulmonology	Weiner, Daniel	Department of Defense	Respiratory Ciliary Dysfunction and Pulmonary Risks in Congenital Heart Disease Patients	$2,443.00	$1,319.00	$3,762.00	30-Sep-15	29-Sep-18
Pulmonology	Weiner, Daniel	Seattle Children's Hospital	A Two-Part, Multicenter, Prospective, Longitudinal Study of CFTR-Dependent Disease Profiling in Cystic Fibrosis	$0.00	$0.00	$0.00	01-Jul-14	01-Jun-18
Pulmonology	Weiner, Daniel	Seattle Children's Hospital	Saline Hypertonic in Preschoolers	$7,806.00	$624.00	$8,430.00	01-Nov-14	31-Oct-18
Pulmonology	Weiner, Daniel	Cystic Fibrosis Foundation	Implementation of the Depression and Anxiety Guidelines: Award for a Mental Health Coordinator	$50,000.00	$4,000.00	$54,000.00	01-Jan-16	31-Dec-18
Pulmonology	Weiner, Daniel	Cystic Fibrosis Foundation	Outpatient Clinical Pharmacy Services	$52,000.00	$4,160.00	$56,160.00	01-Nov-16	31-Oct-19

FOUNDATIONS AND OTHER FUNDING (continued)

DIVISION	NAME	AGENCY TITLE	TITLE	ANNUAL DIRECT COST	ANNUAL INDIRECT COST	ANNUAL TOTAL COST	GRANT BEGIN DATE	GRANT END DATE
Rheumatology	Brunner, Emily	Cure JM Foundation	Stepping It Up: Physical Activity Monitors as Outcome Measures in Juvenile Myositis	$26,667.00	$0.00	$26,667.00	01-Dec-16	30-Jun-18
Rheumatology	Rosenkranz, Margalit	Children's Hospital Medical Center	Gene Expression in Pediatric Arthritis	$0.00	$0.00	$0.00	01-Sep-12	31-Aug-17
Rheumatology	Torok, Kathryn	Nancy Taylor Foundation for Chronic Diseases, Inc.	Serum Cytokine Profiles in Localized Scleroderma	$152,963.00	$9,037.00	$162,000.00	01-Sep-16	31-Aug-18
Rheumatology	Torok, Kathryn	Scleroderma Foundation	Identifying Juvenile Scleroderma Immunophenotyped Subsets	$243,219.00	$19,458.00	$262,677.00	01-Apr-16	31-Mar-18
Rheumatology	Vallejo, Abbe	National Institutes of Health	Translational Evaluation of Aging, Inflammation, and HIV in Lung Dysfunction	$9,188.00	$4,962.00	$14,150.00	04-Sep-13	30-Jun-18
Rheumatology	Vallejo, Abbe	Nancy Taylor Foundation for Chronic Diseases, Inc.	Maladaptive DN T Cells in Juvenile Idiopathic Arthritis	$150,000.00	$12,000.00	$162,000.00	01-Aug-10	31-Jul-17
Richard King Mellon Foundation Institute	Kolls, Jay	National Institutes of Health	Immune Airway-Epithelial Interactions in Steroid-Refractory Severe Asthma	$173,212.00	$93,534.00	$266,746.00	01-Jun-15	31-May-20
Richard King Mellon Foundation Institute	Kolls, Jay	National Institutes of Health	Basic and Clinical Studies of Cystic Fibrosis, Core B	$26,213.00	$14,155.00	$40,368.00	01-Jul-15	31-May-18
Richard King Mellon Foundation Institute	Kolls, Jay	National Institutes of Health	Lung Epithelial-Immune Interactions in Respiratory Virus Infection	$15,146.00	$8,179.00	$23,325.00	15-Aug-15	31-May-19
Richard King Mellon Foundation Institute	Kolls, Jay	Tulane University Health Sciences Center	Il-22: A Therapeutic Cytokine for the Amelioration of Pulmonology Influenza Injury	$11,255.00	$6,148.00	$17,403.00	01-Aug-15	30-Nov-18
Richard King Mellon Foundation Institute	Kolls, Jay	Cystic Fibrosis Foundation	Transcriptomic Responses to Kalydeco: Role in Predicting Outcomes	$100,000.00	$8,000.00	$108,000.00	01-Jan-16	31-Dec-17
Richard King Mellon Foundation Institute	Kolls, Jay	Cystic Fibrosis Foundation	Genome-Wide Analyses of Epigenetic Landscape of CF Airways	$50,000.00	$4,000.00	$54,000.00	01-Apr-16	31-Mar-18
Weight Management and Wellness	Arslanian, Silva	Wake Forest University	B-Cell Function and Cognition in the Restoring Insulin Secretion Study	$24,125.00	$13,028.00	$37,153.00	01-Sep-13	31-Aug-17
Weight Management and Wellness	Hughan, Kara	National Institutes of Health	Nitrite Benefits to Mediate Fatigability in Older HfpEF Patients	$2,628.00	$1,419.00	$4,047.00	15-Aug-16	31-Jul-17
Weight Management and Wellness	Hughan, Kara	Cystic Fibrosis Foundation	Nitrite Modulation of Mitochondrial Function and Insulin Sensitivity in Patients with Cystic Fibrosis	$29,059.00	$2,325.00	$31,384.00	01-Sep-16	31-Aug-19
Rheumatology	Rosenkranz, Margalit	Children's Hospital Medical Center	Gene Expression in Pediatric Arthritis	$0.00	$0.00	$0.00	01-Sep-12	31-Aug-17
Rheumatology	Rosenkranz, Margalit	Duke University	Observational Study of Pediatric Rheumatic Diseases: The CARRA Registry	$82,600.00	$20,650.00	$103,250.00	01-Jul-15	30-Jun-16
Rheumatology	Torok, Kathryn	Rheumatology Research Foundation	Immunophenotyping Active Disease in Localized Scleroderma	$50,000.00	$0.00	$50,000.00	15-Sep-14	31-Oct-16
Rheumatology	Torok, Kathryn	Scleroderma Foundation	Identifying Juvenile Scleroderma Immunophenotype Subsets	$228,697.00	$8,626.00	$237,323.00	01-Apr-16	31-Mar-18
Rheumatology	Vallejo, Abbe	National Institutes of Health	Translational Evaluation of Aging, Inflammation, and HIV in Lung Dysfunction	$9,114.00	$4,922.00	$14,036.00	04-Sep-13	30-Jun-18
Rheumatology	Vallejo, Abbe	Nancy Taylor Foundation for Chronic Diseases, Inc.	Maladaptive DN T cells in Juvenile Idiopathic Arthritis	$150,000.00	$12,000.00	$162,000.00	01-Aug-10	31-Jul-17
Rheumatology	Vallejo, Abbe	American Federation for Aging Research	Thymic Preservation and Immune Function in a Long-Lived Mouse Model	$125,000.00	$12,500.00	$137,500.00	01-Jul-12	30-Jun-16
Rheumatology	Vallejo, Abbe	CR Way	CR Way Immune Pilot Study of Successful Aging	$36,000.00	$0.00	$36,000.00	01-May-16	30-Nov-16
Richard King Mellon Foundation Institute	Kolls, Jay	National Institutes of Health	Mesenchymal Stem Cell Secretome in Lung Fibrosis: Mitochondria and RNA Shuttle	$11,089.00	$5,989.00	$17,078.00	01-Jul-13	30-Jun-16

FOUNDATIONS AND OTHER FUNDING (continued)

DIVISION	NAME	AGENCY TITLE	TITLE	ANNUAL DIRECT COST	ANNUAL INDIRECT COST	ANNUAL TOTAL COST	GRANT BEGIN DATE	GRANT END DATE
Richard King Mellon Foundation Institute	Kolls, Jay	National Institutes of Health	Immune Airway–Epithelial Interactions in Steroid-refractory Severe Asthma	$174,000.00	$91,530.00	$265,530.00	01-Jun-15	31-May-20
Richard King Mellon Foundation Institute	Kolls, Jay	National Institutes of Health	Basic and Clinical Studies of Cystic Fibrosis—Core B	$31,180.00	$16,837.00	$48,017.00	01-Jul-15	30-Jun-20
Richard King Mellon Foundation Institute	Kolls, Jay	National Institutes of Health	Lung Epithelial–Immune Interactions in Respiratory Virus Infection	$14,235.00	$7,687.00	$21,922.00	15-Aug-15	31-May-19
Richard King Mellon Foundation Institute	Kolls, Jay	National Institutes of Health	Lung Epithelial–Immune Interactions in Respiratory Virus Infection	$14,874.00	$8,032.00	$22,906.00	15-Aug-15	31-May-19
Richard King Mellon Foundation Institute	Kolls, Jay	National Institutes of Health	Mechanisms of IL-17–Mediated Host Defense in the Kidney	$4,221.00	$2,279.00	$6,500.00	20-Sep-15	30-Jun-16
Richard King Mellon Foundation Institute	Kolls, Jay	National Institutes of Health	Understanding Severe Asthma Using an Experimental Mouse Model	$14,611.00	$7,890.00	$22,501.00	07-Jan-13	31-Jan-17
Richard King Mellon Foundation Institute	Kolls, Jay	National Institutes of Health	UPITT Rheumatoid Arthritis Combined Center (UPITT RACC)	$22,915.00	$12,375.00	$35,290.00	24-Sep-14	31-May-17
Richard King Mellon Foundation Institute	Kolls, Jay	Tulane University Health Sciences Center	Il-22: A Therapeutic Cytokine for the Amelioration of Pulmonology Influenza Injury	$6,445.00	$3,480.00	$9,925.00	01-Aug-15	30-Nov-18
Richard King Mellon Foundation Institute	Kolls, Jay	Fondazione Centro San Raffaele	Targeting IL17-Producing T Cells during *Pseudomonas aeruginosa* Lung Disease	$4,597.00	$368.00	$4,965.00	01-Apr-16	31-Mar-18
Richard King Mellon Foundation Institute	Kolls, Jay	Cystic Fibrosis Foundation	RDP Renewal–Core B–Clinical Studies Core	$22,220.00	$0.00	$22,220.00	01-Jul-15	30-Jun-16
Richard King Mellon Foundation Institute	Kolls, Jay	Cystic Fibrosis Foundation	Transcriptomic Responses to Kalydeco—Role in Predicting Outcomes	$100,000.00	$8,000.00	$108,000.00	01-Jan-16	31-Dec-17
Richard King Mellon Foundation Institute	Kolls, Jay	Cystic Fibrosis Foundation	Genome-wide Analyses of Epigenetic Landscape of Cystic Fibrosis Airways	$50,000.00	$4,000.00	$54,000.00	01-Apr-16	31-Mar-18
Richard King Mellon Foundation Institute	Kuhn, Bernhard	University of Pennsylvania	Role of Single-Cell mRNA Variation in Systems Associated Electrically Excitable C	$215,000.00	$116,100.00	$331,100.00	01-Sep-14	31-May-17
Richard King Mellon Foundation Institute	Kuhn, Bernhard	Fondation Leducq	Elicitin Heart Regeneration through Cardiomyocyte	$105,050.00	$10,505.00	$115,555.00	24-Feb-16	23-Feb-17
Weight Management and Wellness	Arslanian, Silva	Wake Forest University	B-Cell Function and Cognition in the Restoring Insulin Secretion (RISE) Study	$24,125.00	$13,028.00	$37,153.00	01-Sep-13	31-Aug-17

INDUSTRY-SPONSORED STUDIES

DIVISION	NAME	AGENCY TITLE	TITLE	ANNUAL DIRECT COST	ANNUAL INDIRECT COST	ANNUAL TOTAL COST	GRANT BEGIN DATE	GRANT END DATE
Allergy and Immunology	Green, Todd	DBV Technologies	A Double-Blind, Placebo-Controlled, Randomized Trial to Study the Viaskin Milk Efficacy and Safety for Treating IgF-Mediated Cow's Milk Allergy in Children	$140,738.00	$35,185.00	$175,923.00	17-Jul-15	16-Jul-17
Bone Marrow Transplant/Cell Therapy	Vander Lugt, Mark	Chimerix	A Phase 3, Open-Label, Multicenter Study of the Safety/Tolerability and Efficacy of Brincidofovir (CMX001) for the Prevention of Adenovirus (AdV) Disease in Subjects with Asymptomatic AdV Infection at Risk of Progression and for the Treatment of Subjects	$97,224.00	$26,943.00	$124,167.00	01-Dec-14	31-Dec-17
Endocrinology	Escobar, Oscar	Endo Pharmaceuticals	A Multicenter, Observational Registry of Pediatric Female Subjects with Central Precocious Puberty Receiving Supprelin La (Histrelin Acetate), a Hydrogen Subcutaneous Implant	$0.00	$0.00	$0.00	25-Sep-13	24-Sep-18
Endocrinology	Escobar, Oscar	Versartis, Inc.	An Open-Label, Long-Term Safety Study of Long-Acting Human Growth Hormone Somavaratan (VRS-317) in Children with Growth Hormone, PED-16-028	$49,736.00	$13,934.00	$63,670.00	01-Dec-16	30-Nov-18
Gastroenterology	King, Dale	Chiltern International, Inc.	A Safety and Efficacy Study of a Bowel-Cleansing Preparation (BL1800) in Pediatric Subjects Undergoing Colonoscopy (PED-16-020)	$43,649.00	$10,912.00	$54,561.00	24-Oct-16	30-Jun-17
Gastroenterology	Rudolph, Jeffrey	Fresenius Kabi Deutschland GmbH	A Prospective, Randomized, Controlled, Double-Blind, Parallel-Group, Phase 3 Study to Compare Safety and Efficacy of Smoflipid 20% to Intralipid 20% in Hospitalized Neonates and Infants Requiring 28 days of Parenteral Nutrition	$329,912.00	$82,103.00	$412,015.00	17-Mar-16	31-Jan-18
Gastroenterology	Shah, Sapana	Janssen Research and Development, LLC	A Phase 4, Multicenter, Open-Label Study of Serum Infliximab Concentrations and Efficacy and Safety of Dose Escalation in Pediatric Patients with Inflammatory Bowel Disease	$102,111.00	$25,153.00	$127,264.00	19-Apr-16	31-Aug-18
Gastroenterology	Squires, Robert	Lumena Pharmaceuticals, Inc.	Therapeutic Effect of LUM001-Letter Agreement IMAGE	$0.00	$0.00	$0.00	01-Sep-15	31-Aug-17
Gastroenterology	Srinath, Arvid	Forest Research Institute, Inc.	A Multicenter, Randomized, Double-Blind, Placebo-Controlled, Parallel-Group, Safety and Efficacy Study of a Range of Linaclotide Doses Administered Orally to Children, Ages 7 to 17 Years, with Irritable Bowel Syndrome with Constipation	$26,877.00	$6,720.00	$33,597.00	08-Mar-16	31-Dec-17
Gastroenterology	Srinath, Arvid	Forest Research Institution, Inc.	A Multicenter, Randomized, Double-Blind, Placebo-Controlled, Parallel-Group, Safety and Efficacy Study of a Range of Linaclotide Doses Administered Orally to Children, Ages 6 to 17 Years, Who Fulfill Modified Rome III Criteria for Child/Adolescent Functional Constipation	$21,916.00	$5,479.00	$27,395.00	08-Mar-16	31-Dec-17
General Academic Pediatrics	Hoberman, Alejandro	MedImmune, Inc.	A Phase 2b Randomized, Double-Blind, Placebo-Controlled Study to Evaluate the Safety and Efficacy of MEDI8897, a Monoclonal Antibody with an Extended Half-Life against Respiratory Syncytial Virus, in Healthy Preterm Infants	$319,073.00	$79,768.00	$398,841.00	01-Nov-16	01-Nov-17
Genetics	Gonzalez, Lina	Horizon Therapeutics, Inc.	Characterization of the Burden of Genetic Disease in Mercer County Amish	$90,483.00	$22,621.00	$113,104.00	01-Mar-16	28-Feb-18
Genetics	Vockley, Gerard	Pfizer	A Multicenter, Multicounty, Post-Marketing Active Surveillance Taliglucerase Alpha Registry in Patients with Gaucher Disease	$0.00	$0.00	$0.00	16-Jul-14	15-Jul-17

INDUSTRY-SPONSORED STUDIES (continued)

DIVISION	NAME	AGENCY TITLE	TITLE	ANNUAL DIRECT COST	ANNUAL INDIRECT COST	ANNUAL TOTAL COST	GRANT BEGIN DATE	GRANT END DATE
Genetics	Vockley, Gerard	Stealth BioTherapeutics Inc.	A Phase 2, Randomized, Double-Blind, Placebo-Controlled Crossover Study to Evaluate the Safety, Tolerability, and Efficacy of Subcutaneous Injections of Elamipretide (MTP-131) in Subjects with Genetically Confirmed Mitochondrial Disease Previously Treated	$117,195.00	$29,295.00	$146,490.00	16-Aug-16	15-Aug-18
Genetics	Vockley, Gerard	Reata Pharmaceuticals, Inc.	A Phase 2 Study of the Safety, Efficacy, and Pharmacodynamics of RTA 408 in the Treatment of Mitochondrial Myopathy	$189,217.00	$47,304.00	$236,521.00	28-Jul-15	27-Jul-17
Genetics	Vockley, Gerard	PROTALIX	A Randomized, Double-Blind, Active Control Study of the Safety and Efficacy of PRX-102 Compared to Agalsidase Beta on Renal Function in Patients with Fabry Disease Previously Treated with Agalsidase Beta	$269,591.00	$67,398.00	$336,989.00	11-Jan-17	11-Jan-19
Genetics	Vockley, Gerard	ArmaGen, Inc.	An Extension Study of AGT-181-102 Evaluating Safety and Glycosaminoglycans (GAGS) in Adult Patients with Hurler-Scheie or Scheie Syndrome Who Have Completed Eight Weeks of Dosing with AGT-181 in Study AGT-181-102	$66,153.00	$16,538.00	$82,691.00	31-Mar-16	30-Mar-18
Genetics	Vockley, Gerard	Alexion Pharmaceuticals	An Observational, Longitudinal, Prospective, Long-Term Registry of Patients with Hypophosphatasia	$24,900.00	$6,225.00	$31,125.00	19-Oct-15	18-Oct-17
Genetics	Vockley, Gerard	Shire Human Genetic Therapies, Inc.	An Open-Label Extension of Study HGT-HIT-045 Evaluating Long-Term Safety and Clinical Outcomes of Intrathecal Idursulfase-IT, Administered in Conjunction with Intravenous Elaprase, in Pediatric Patients with Hunter Syndrome and Early Cognitive Impairment	$0.00	$0.00	$0.00	01-Feb-13	28-Feb-18
Genetics	Vockley, Gerard	Alexion Pharmaceuticals	An Open-Label, Multicenter, Expanded Access Program for Asfotase Alfa (Human Recombinant Tissue-Nonspecific Alkaline Phosphatase Fusion Protein) Treatment for Patients with Infantile or Juvenile-Onset Hypophosphatasia (HPP)	$34,099.00	$8,525.00	$42,624.00	07-Oct-15	06-Oct-17
Genetics	Vockley, Gerard	BioMarin Pharmaceuticals, Inc.	Burden of PAH Deficiency/PKU among Adults: A Retrospective Chart Review and Cross-Sectional Patient Survey	$9,790.00	$2,447.00	$12,237.00	07-Nov-16	06-Nov-18
Genetics	Vockley, Gerard	Hyperion Therapeutics, Inc.	Long-Term Registry of Patients with Urea Cycle Disorders	$30,396.00	$7,524.00	$37,920.00	18-Sep-13	17-Sep-23
Genetics	Vockley, Gerard	ICON Clinical Research, LLC	The Clinical and Economic Burden of Illness of MPS II (Hunter Syndrome) in the United States	$7,170.00	$1,793.00	$8,963.00	17-Oct-16	16-Oct-18
Genetics	Vockley, Gerard	Ultragenyx Pharmaceuticals, Inc.	Dietary Therapy for Inherited Disorders of Mitochondrial Fatty Acid Oxidation and Glycogenoses	$18,902.00	$4,725.00	$23,627.00	02-Aug-13	30-Jun-18
Genetics	Vockley, Gerard	Moderna TX, Inc.	MCAD Study	$195,492.00	$120,227.00	$315,719.00	11-Oct-16	10-Oct-18
Genetics	Vockley, Gerard	Moderna TX, Inc.	VLCAD Study	$200,492.00	$123,302.00	$323,794.00	11-Oct-16	10-Oct-18
Hematology/ Oncology	Cohen, Debra	Mast Therapeutics, Inc.	An Open-Label Safety Extension Trial Assessing Repeat Administration of MST-188 (Purified Poloxamer 188) Injection in Subjects with Sickle Cell Disease Experiencing Vaso-Occlusive Crisis	$35,834.00	$8,708.00	$44,542.00	23-Mar-16	22-Mar-19
Hematology/ Oncology	Cohen, Debra	Pfizer	PED-15-015: A Phase 3, Multicenter, Randomized, Double-Blind, Placebo-Controlled, Parallel-Group Study to Evaluate the Efficacy and Safety of Rivipansel (GMI-1070) in the Treatment of Vaso-Occlusive Crisis in Hospitalized Subjects with Sickle Cell Disease	$33,142.00	$8,036.00	$41,178.00	01-Sep-16	31-Aug-19

INDUSTRY-SPONSORED STUDIES (continued)

DIVISION	NAME	AGENCY TITLE	TITLE	ANNUAL DIRECT COST	ANNUAL INDIRECT COST	ANNUAL TOTAL COST	GRANT BEGIN DATE	GRANT END DATE
Hematology/ Oncology	Cohen, Debra	Pfizer	PED-15-016: A Phase 3, Multicenter, Randomized, Double-Blind, Placebo-Controlled, Parallel-Group Study to Evaluate the Efficacy and Safety of Rivipansel (GMI 1070) in the Treatment of Vaso-Occlusive Crisis in Hospitalized Subjects with Sickle Cell Disease	$36,740.00	$8,575.00	$45,315.00	01-Nov-16	31-Oct-18
Hematology/ Oncology	Malec, Lynn	Biogen MA, Inc.	Recombinant Factor VIII Fc Fusion Protein, rFVIIIFc, in Immune Tolerance Induction in Hemophilia A Patients with Inhibitors	$19,427.00	$4,857.00	$24,284.00	12-May-16	30-Apr-19
Hematology/ Oncology	Ritchey, Kim	Amgen Corporation	A Single-Arm, Open-Label, Long-Term Efficacy and Safety Study of Romiplostim in Thrombocytopenic Pediatric Subjects with Immune Thrombocytopenia	$111,438.00	$27,453.00	$138,891.00	01-Oct-15	30-Sep-18
Nephrology	Ellis, Demetrius	AVI BioPharma	Open-Label, Multiple-Dose, Efficacy, Safety, and Tolerability Study of Eteplirsen in Subjects with Duchenne Muscular Dystrophy Who Participated in Study 4658-US-201	$14,429.00	$3,297.00	$17,726.00	01-Jun-12	31-May-18
Neurology	Abdel-Hamid, Hoda	AVI BioPharma, Inc.	Open-Label, Multiple-Dose Efficacy, Safety, and Tolerability Study of Eteplirsen in Patients with Duchenne Muscular Dystrophy Who Participated in Study 4658-US-201	$14,430.00	$3,297.00	$17,727.00	01-Jun-12	31-May-18
Neurology	Escolar, Maria	Synageva BioPharma Corporation	A Phase I/II Open-Label Study in MPS IIIB Subjects to Investigate the Safety, Pharmacokinetics, and Pharmacodynamics of SBC-103 Administered Intravenously	$415,342.00	$103,585.00	$518,927.00	17-Dec-14	16-Dec-17
Neurology	Goldstein, Amy	Stealth BioTherapeutics, Inc.	A Prospective, Observational Study of Patients with Primary Mitochondrial Disease	$33,919.00	$8,480.00	$42,399.00	30-Jan-17	30-Jan-19
Pulmonology	Alcorn, John	MedImmune, Inc.	Efficacy of Novel Antibodies against Influenza, *Staphylococcus aureus* Super-Infection	$89,369.00	$54,961.00	$144,330.00	03-Jun-16	02-Jun-18
Pulmonology	Orenstein, David	Vertex	A Phase 3, Double-Blind, Placebo-Controlled, Parallel-Group Study to Evaluate the Efficacy and Safety of Lumacaftor in Combination with Ivacaftor in Subjects Aged 6 through 11 Years with Cystic Fibrosis, Homozygous for the F508del-CFTR Mutation	$49,260.00	$12,315.00	$61,575.00	27-Oct-15	26-Oct-17
Pulmonology	Orenstein, David	Anthera Pharmaceuticals, Inc.	A Phase 3, Randomized, Open-Label, Assessor-Blind, Non-Inferiority, Active-Comparator Study Evaluating the Efficacy and Safety of Liprotamase in Subjects with Cystic Fibrosis-Related Exocrine Pancreatic Insufficiency	$30,581.00	$7,646.00	$38,227.00	09-Dec-15	08-Dec-17
Pulmonology	Orenstein, David	Alcresta Pharmaceuticals, Inc.	Study to Evaluate Safety, Tolerability, and Fat Absorption Using a Novel Enteral Feeding In-Line Digestive Enzyme Cartridge (RELIZORB) in Patients with Cystic Fibrosis Receiving Enteral Feeding	$22,491.00	$5,623.00	$28,114.00	19-May-16	18-May-18
Pulmonology	Orenstein, David	Alcresta Pharmaceuticals, Inc.	Study to Evaluate Safety, Tolerability, and Fat Absorption Using a Novel Enteral Feeding In-Line Digestive Enzyme Cartridge in Patients with Cystic Fibrosis Receiving Enteral Feeding	$22,035.00	$5,509.00	$27,544.00	21-Dec-15	20-Dec-17
Pulmonology	Pilewski, Joseph	Celtaxsys, Inc.	A Phase 2, Multicenter, Randomized, Double-Blind, Placebo-Controlled, Parallel-Group Study to Evaluate the Efficacy, Safety, and Tolerability of CTX-4430 Administered Orally Once Daily for 48 Weeks in Adult Patients with Cystic Fibrosis	$36,419.00	$9,105.00	$45,524.00	17-Feb-16	16-Feb-18

INDUSTRY-SPONSORED STUDIES (continued)

DIVISION	NAME	AGENCY TITLE	TITLE	ANNUAL DIRECT COST	ANNUAL INDIRECT COST	ANNUAL TOTAL COST	GRANT BEGIN DATE	GRANT END DATE
Pulmonology	Pilewski, Joseph	Nivalis Therapeutics	A Phase 2, Randomized, Double-Blind, Placebo-Controlled, Parallel-Group Study of N91115 to Evaluate Efficacy and Safety in Patients with Cystic Fibrosis Who Are Homozygous for the F508del-CFTR Mutation and Being Treated with Lumacaftor/Ivacaftor	$22,777.00	$5,695.00	$28,472.00	14-Jun-16	13-Jun-18
Pulmonology	Pilewski, Joseph	Nivalis Therapeutics	A Phase 2, Randomized, Double-Blind, Placebo-Controlled, Parallel-Group Study of N91115 to Evaluate Efficacy and Safety in Patients with Cystic Fibrosis Who Are Homozygous for the F508del-CFTR Mutation and Being Treated with Lumacaftor/Ivacaftor	$38,426.00	$9,607.00	$48,033.00	15-Jan-16	14-Jan-18
Pulmonology	Pilewski, Joseph	Vertex	A Phase 2a, Randomized, Double-Blind, Placebo-Controlled, Crossover Study to Evaluate the Safety and Efficacy of VX-371 in Subjects Aged 12 Years or Older with Cystic Fibrosis, Homozygous for the F508del-CFTR Mutation, and Being Treated with Orkambi	$57,242.00	$14,311.00	$71,553.00	26-May-16	25-May-18
Pulmonology	Pilewski, Joseph	Vertex	A Phase 3, Open-Label, Rollover Study to Evaluate the Safety and Efficacy of Long-Term Treatment with VX-661 in Combination with Ivacaftor in Subjects Aged 12 Years and Older with Cystic Fibrosis, Homozygous or Heterozygous for the F508del-CFTR Mutation	$57,011.00	$14,253.00	$71,264.00	28-Oct-15	27-Oct-17
Pulmonology	Pilewski, Joseph	Seattle Children's Hospital	G551D Observational (GOAL) Study	$15,908.00	$1,272.00	$17,180.00	01-Sep-11	31-Dec-20
Pulmonology	Weiner, Daniel	RespirTech Technologies	Peak Cough Flow and Airway Clearance in Pediatric Neuromuscular Disease	$0.00	$0.00	$0.00	01-Aug-13	31-Jul-18
Rheumatology	Kietz, Daniel	Bristol-Myers Squibb	An Observational Registry of Abatacept in Patients with Juvenile Idiopathic Arthritis	$34,236.00	$10,171.00	$44,407.00	01-May-13	30-Apr-23
Rheumatology	Rosenkranz, Margalit	Pfizer	A Long-Term, Open-Label Follow-Up Study of CP-690, 550 for Treatment of Juvenile Idiopathic Arthritis	$7,695.00	$1,799.00	$9,494.00	24-Sep-15	20-Mar-22
Rheumatology	Rosenkranz, Margalit	Pfizer	Efficacy, Safety, and Tolerability of Tofacitinib for Treatment of Polyarticular Course Juvenile Idiopathic Arthritis in Children and Adolescent Subjects	$55,024.00	$13,756.00	$68,780.00	11-Jan-17	10-May-19
Rheumatology	Rosenkranz, Margalit	Pfizer	Efficacy, Safety, and Tolerability of Tofacitinib for Treatment of Polyarticular Course Juvenile Idiopathic Arthritis in Children and Adolescent Subjects	$55,025.00	$13,756.00	$68,781.00	11-Jan-17	10-May-19
Richard King Mellon Foundation Institute	Kolls, Jay	Novartis Pharmaceuticals	Exploring Antibody-Mediated B-Cell Depletion and Impact of PCP in Mice	$52,439.00	$32,250.00	$84,689.00	15-Sep-16	14-Sep-17
Weight Management and Wellness	Arslanian, Silva	Novo Nordisk Pharmaceuticals, Inc.	Effect of liraglutide for Weight Management in Pubertal Adolescent Subjects with Obesity: A 56-Week, Double-Blind, Randomized, Parallel-Group, Placebo-Controlled Multinational Trial Followed by a 26-Week Period off Study Drug, Phase 3a	$96,592.00	$24,148.00	$120,740.00	20-Jan-17	19-Jan-19
Weight Management and Wellness	Hughan, Kara	Boehringer Ingelheim Pharmaceuticals, Inc.	An Open-Label, Randomized, Multicenter, Single-Dose, Parallel-Group Trial to Evaluate Pharmacokinetics and Pharmacodynamics of Empagliflozin in Children and Adolescents from 10 to Less Than 18 Years of Age with Type 2 Diabetes Mellitus	$0.00	$0.00	$0.00	15-Aug-14	14-Aug-17
Weight Management and Wellness	Ismail, Heba	Novo Nordisk Pharmaceuticals, Inc.	A Randomized, Double-Blind, Placebo-Controlled Trial to Assess Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of Liraglutide in Obese Children Aged 7 to 11 Years	$27,628.00	$6,907.00	$34,535.00	03-Jun-16	02-Jun-18

INDUSTRY-SPONSORED STUDIES (continued)

DIVISION	NAME	AGENCY TITLE	TITLE	ANNUAL DIRECT COST	ANNUAL INDIRECT COST	ANNUAL TOTAL COST	GRANT BEGIN DATE	GRANT END DATE
Weight Management and Wellness	Ismail, Heba	Novo Nordisk Pharmaceuticals, Inc.	Efficacy and Safety of Faster-Acting Insulin Aspart Compared to NovoRapid Both in Combination with Insulin Degludec in Children and Adolescents with Type 1 Diabetes	$128,860.00	$32,215.00	$161,075.00	29-Jun-16	28-Jun-18
Pulmonology	Orenstein, David	Alcresta Pharmaceuticals, Inc.	Study to Evaluate Safety, Tolerability, and Fat Absorption Using a Novel Enteral Feeding In-Line Digestive Enzyme Cartridge (RELIZORB) in Patients with Cystic Fibrosis Receiving Enternal Feeding	$22,491.00	$5,622.00	$28,113.00	19-May-16	18-May-18
Pulmonology	Orenstein, David	Alcresta Pharmaceuticals, Inc.	Study to Evaluate Safety, Tolerability, and Fat Absorption Using a Novel Enteral Feeding In-Line Digestive Enzyme Cartridge in Patients with Cystic Fibrosis Receiving Enteral Feeding	$36,335.00	$9,084.00	$45,419.00	21-Dec-15	20-Dec-17
Pulmonology	Pilewski, Joseph	Vertex	A Phase 2, Randomized, Double-blind, Placebo-controlled, Parallel-group, Exploratory Study to Evaluate Effects of VX-661 in Combination with Ivacaftor on Lung and Extrapulmonary Systems in Subjects Aged 18 Years and Older with Cystic Fibrosis	$127,997.00	$31,999.00	$159,996.00	23-Nov-15	22-Nov-16
Pulmonology	Pilewski, Joseph	Celtaxsys, Inc.	A Phase 2, Multicenter, Randomized, Double-Blind, Placebo-Controlled, Parallel-Group Study to Evaluate the Efficacy, Safety, and Tolerability of CTX-4430 Administered Orally Once Daily for 48 Weeks in Adult Patients with Cystic Fibrosis	$36,419.00	$9,105.00	$45,524.00	17-Feb-16	16-Feb-18
Pulmonology	Pilewski, Joseph	Nivalis Therapeutics	A Phase 2, Randomized, Double-Blind, Placebo-Controlled, Parallel-Group Study of N91115 to Evaluate Efficacy and Safety in Patients with Cystic Fibrosis Who Are Homozygous for the F508del-CFTR Mutation and Being Treated with Lumacaftor/Ivacaftor	$22,776.00	$5,694.00	$28,470.00	14-Jun-16	13-Jun-18
Pulmonology	Pilewski, Joseph	Nivalis Therapeutics	A Phase 2, Randomized, Double-Blind, Placebo-Controlled, Parallel-Group Study of N91115 to Evaluate Efficacy and Safety in Patients with Cystic Fibrosis Who Are Homozygous for the F508del-CFTR Mutation and Being Treated with Lumacaftor/Ivaftor	$38,426.00	$9,606.00	$48,032.00	15-Jan-16	14-Jan-18
Pulmonology	Pilewski, Joseph	Vertex	A Phase 2a, Randomized, Double-Blind, Placebo-controlled, Crossover Study to Evaluate the Safety and Efficacy of VX-371 in Subjects Aged 12 Years or Older with Cystic Fibrosis, Homozygous for the F508del-CFTR Mutation, and Being Treated with Orkambi	$57,242.00	$14,310.00	$71,552.00	26-May-16	25-May-18
Pulmonology	Pilewski, Joseph	Vertex	A Phase 3, Open-Label, Rollover Study to Evaluate the Safety and Efficacy of Long-term Treatment with VX-661 in Combination with Ivacaftor in Subjects Aged 12 Years and Older with Cystic Fibrosis, Homozygous or Heterozygous for the F508del-CFTR Mutation	$57,010.00	$14,252.00	$71,262.00	28-Oct-15	27-Oct-17
Pulmonology	Pilewski, Joseph	Vertex	A Phase 3, Randomized, Double-Blind, Placebo-Controlled, Crossover Study to Evaluate the Efficacy and Safety of Ivacaftor and VX-661 in Combination with Ivacaftor in Subjects Aged 12 Years and Older with Cystic Fibrosis	$56,337.00	$14,085.00	$70,422.00	26-Jun-15	25-Jun-17
Pulmonology	Pilewski, Joseph	Vertex	A Phase 3b, Open-Label Study to Evaluate Lumacaftor and Ivacaftor Combination Therapy in Subjects 12 Years and Older with Cystic Fibrosis and Advanced Lung Disease, Homozygous for the F508del-CFTR Mutation	$26,341.00	$7,741.00	$34,082.00	05-Mar-15	04-Mar-17

INDUSTRY-SPONSORED STUDIES (continued)

DIVISION	NAME	AGENCY TITLE	TITLE	ANNUAL DIRECT COST	ANNUAL INDIRECT COST	ANNUAL TOTAL COST	GRANT BEGIN DATE	GRANT END DATE
Pulmonology	Pilewski, Joseph	Seattle Children's Hospital	G551D Observational (GOAL) Study	$32,345.00	$1,787.00	$34,132.00	01-Sep-11	31-Dec-20
Pulmonology	Weiner, Daniel	RespirTech Technologies	Peak Cough Flow and Airway Clearance in Pediatric Neuromuscular Disease	$0.00	$0.00	$0.00	01-Aug-13	31-Jul-18
Rheumatology	Kietz, Daniel	Bristol-Myers Squibb	An Observational Registry of Abatacept in Patients with Juvenile Idiopathic Arthritis	$34,236.00	$10,171.00	$44,407.00	01-May-13	30-Apr-23
Rheumatology	Rosenkranz, Margalit	Pfizer	A Long-term, Open-Label Follow-up Study of CP-690, 550 for Treatment of Juvenile Idiopathic Arthritis (JIA)	$7,693.00	$1,799.00	$9,492.00	24-Sep-15	20-Mar-22
Richard King Mellon Foundation Institute	Kolls, Jay	Implicit Bioscience	Evaluation of ODS in Infection	$11,886.00	$7,310.00	$19,196.00	11-Nov-15	10-Nov-16
Weight Management and Wellness	Hughan, Kara	Boehringer Ingelheim Pharmaceuticals, Inc.	An Open-Label, Randomized, Multicenter, Single-Dose, Parallel-Group Trial to Evaluate Pharmacokinetics and Pharmacodynamics of Empagliflozin in Children and Adolescents from 10 to Less Than 18 Years of Age with Type 2 Diabetes Mellitus	$18,215.00	$4,179.00	$22,394.00	15-Aug-14	14-Aug-17
Weight Management and Wellness	Ismail, Heba	Novo Nordisk Pharmaceuticals, Inc.	A Randomized, Double-Blind, Placebo-Controlled Trial to Assess Safety, Tolerability, Pharmacokinetics, and pPharmacodynamics of Liraglutide in Obese Children Aged Seven to 11 Years	$41,768.00	$10,442.00	$52,210.00	03-Jun-16	02-Jun-18
Weight Management and Wellness	Ismail, Heba	Novo Nordisk Pharmaceuticals, Inc.	Efficacy and Safety of Faster-Acting Insulin Aspart Compared to NovoRapid Both in Combination with Insulin Degludec in Children and Adolescents with Type 1 Diabetes	$128,859.00	$32,215.00	$161,074.00	29-Jun-16	28-Jun-18

Notes

Annual Report 17 Department of Pediatrics

University of Pittsburgh
School of Medicine

Children's Hospital of Pittsburgh of UPMC