

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

he 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

he 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

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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 proinflammatory, 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-Bel10-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, CBMdependent stimulation of NF- κ B 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,



Linda M. McAllister-Lucas, MD, PhD Division Chief, Hematology/Oncology

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*.

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

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

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

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

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- 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 Lxmyelocel-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 industrysponsored 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.

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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

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- "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



DEPARTMENT OF PEDIATRICS 2017 ANNUAL REPORT

DIVISION OF HEMATOLOGY/ONCOLOGY

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

embers 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.

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