

The Alpha-1 Study

Antitrypsin Deficiency-Related Liver
Disease Treatment Using Carbamazepine

Quick Reference for Physicians



Robert H. Squires, MD

Primary Investigator

1-855-428-2281

liverstudy@chp.edu

www.chp.edu/liverstudy



Children's | *of*
Hospital of Pittsburgh | **UPMC**

Inclusion Criteria

- Age ≥ 14 years ≤ 80 years
- Alpha-1 antitrypsin deficiency (either ZZ or SZ type)
- Clinical evidence of portal hypertension
 - Varicies (esophageal or gastric)
 - Hypersplenism
 - Splenomegally with:
 - Platelet count $< 150K$ or
 - Ascites

Exclusion Criteria

- Child Pugh score ≥ 12
- Serum total bilirubin > 5 mg/dl
- INR > 2.2
- Serum creatinine > 1.5 mg/dl

Key Study Points

What we know

- ATD can lead to liver fibrosis/cirrhosis as well as respiratory problems
- Accumulation of the misfolded protein in liver cells causes the damage
- Currently, liver transplantation is the only therapeutic option
- Mouse models demonstrated that carbamazepine (CBZ) could degrade the misfolded protein and reverse liver fibrosis

Purpose of study

- Evaluate effects of CBZ vs. placebo for 12 months
- Determine if CBZ is safe in patients with pre-existing liver disease and whether it reduces hepatic ATZ load, fibrosis, and portal pressure

What will happen

- 30 participants will enroll at UPMC facilities
- Screening visit including liver biopsy
- Randomization visit – either CBZ or placebo
- 4 follow-up visits, which include scans and bloodwork
- Week 52 final study visit including liver biopsy
- Close monitoring by Hepatology team

Questions and Referrals

To refer a patient or to discuss eligibility, please call **1-855-428-2281** or send an email to **liverstudy@chp.edu**.

