The Alpha-1 Study
Antitrypsin Deficiency-Related Liver Disease Treatment Using Carbamazepine
Quick Reference for Physicians

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Inclusion Criteria

• Age $\geq 14$ years $\leq 80$ years
• Alpha-1 antitrypsin deficiency (either ZZ or SZ type)
• Clinical evidence of portal hypertension
  — Varicies (esophageal or gastric)
  — Hyperslenism
  — Splenomegally with:
    – Platelet count $< 150$K or
    – Ascites

Exclusion Criteria

• Child Pugh score $\geq 12$
• 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.