An Open Label Dose Escalation Study to Assess the Safety, Tolerability, and Pharmacologic Properties of High Dose Ambroxol Hydrochloride in Adult (≥18 Years of Age) Subjects With MPS III

NCT06614894

Status	RECRUITING
Phase	Phase 2, Phase 3
Sponsor	Ozlem Goker-Alpan
Enrollment	10 participants

Key Eligibility Criteria

Inclusion (6)

- IRB - approved informed consent/assent signed by subject and/or parent(s) or legal guardian(s).
- Genetically confirmed diagnosis of MPS III disease.
- Genomic DNA analysis demonstrating a homozygous or compound heterozygous pathogenic variants in SGSH (type A), NAGLU (type B), HGSNAT (type C), or GNS (type D) genes. Type E will not be studied.
- Male or female; eighteen years of age and older, who is able to take Ambroxol Hydrochloride orally.
- Negative urine pregnancy test at screening for female subjects with child-bearing potential.

... and 1 more (see full listing online)

Exclusion (11)

- Unwilling or unable to follow protocol requirements as per principal investigator.
- Any serious or chronic medical illness, including significant cardiac or severe debilitating pulmonary disease.
- Poorly controlled seizures, defined as more than one seizure per day for the past 6 months.
- Medications identified as a strong inducers or inhibitors of CYP3A, and changing to another alternative drug to treat the condition would place the subject at undue risk.
- Any medical condition that, in the opinion of the PI, would make the subject unsuitable to participate in the study.

... and 6 more (see full listing online)

Locations (1 total)

Lysosomal & Rare Disorders Research & Treatment Center, Inc., Fairfax, Virginia, United States