An Open-Label Study Of The Safety, Pharmacokinetics, Pharmacodynamics, And Efficacy Of 12-Month Treatment With Migalastat In Pediatric Subjects (Aged 2 To < 12 Years) With Fabry Disease And Amenable GLA Variants

Status: Recruiting

Eligibility Criteria

Sex: Male or Female
Age Group: Up to 18 years old
This study is NOT accepting healthy volunteers

Inclusion Criteria:

- 2 to under 12 years of age - diagnosis of Fabry disease - see the ClinicalTrials.gov listing for complete inclusion criteria

Exclusion Criteria:

- moderate to severe kidney disease or requiring dialysis or a kidney transplant - previous gene therapy or certain medications that may interfere with the study - pregnant or breastfeeding - see the ClinicalTrials.gov listing for complete exclusion criteria

Conditions & Interventions

Interventions:
Drug: Migalastat HCl 20 mg
Conditions:
Children's Health, Rare Diseases, Rare Diseases
Keywords:
Fabry disease

More Information

Description: The purpose of this study is to evaluate the safety and effectiveness of migalastat, an investigational treatment for children with Fabry disease. Researchers will also study how the medication is processed by the body and how it affects the disease. Participants will receive migalastat for up to 12 months and complete regular study visits and health assessments throughout the study.
Study Contact: Brenda Diethelm-Okita - dieth001@umn.edu
Principal Investigator: Chester Whitley, MD, PhD
Phase: PHASE3
IRB Number: STUDY00025577