A LONG-TERM, OPEN-LABEL STUDY TO EVALUATE THE SAFETY, PHARMACODYNAMICS, AND EFFICACY OF MIGALASTAT IN SUBJECTS > 12 YEARS OF AGE WITH FABRY DISEASE AND AMENABLE GLA VARIANTS

Status: Recruiting

Eligibility Criteria

Sex: All
Age: 12 Years to 17 Years old
This study is NOT accepting healthy volunteers

Inclusion Criteria:

• Male or female subjects diagnosed with Fabry disease > 12 years of age who completed Study AT1001-020

• Subject's parent or legally-authorized representative is willing and able to provide written informed consent and authorization for use and disclosure of personal health information or research-related health information, and subject provides assent, if applicable

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Exclusion Criteria:

• Has moderate or severe renal impairment (eGFR <60 ml/min/1.73 m2 at screening)

• Has advanced kidney disease requiring dialysis or kidney transplantation

• History of allergy or sensitivity to study medication (including excipients) or other iminosugars (eg, miglustat, miglitol)

• Has received any gene therapy at any time or anticipates starting gene therapy during the study period

• Requires treatment with Glyset (miglitol), Zavesca (miglustat) within 6 months before screening or throughout the study

• Requires treatment with Replagal (agalsidase alfa), or Fabrazyme (agalsidase beta) within 14 days before screening or throughout the study

• Subject is treated or has been treated with any investigational/experimental drug, biologic or device within 30 days before screening

• Any intercurrent illness or condition or concomitant medication use considered to be a contraindication at screening or baseline or that may preclude the subject from fulfilling the protocol requirements or suggests to the investigator that the potential subject may have an unacceptable risk by participating in this study

• Pregnant or breast-feeding

• Otherwise unsuitable for the study in the opinion of the investigator

Conditions & Interventions

Interventions:
Drug: migalastat HCl 150 mg
Conditions:
Fabry Disease
Keywords:
Lysosomal storage disease, migalastat

More Information

Description: This an extension study assessing the use of migalastat (AT1001) in pediatric populations. AT1001, under the trade name Galafold, is approved for use in the US in adults, but not children. The parent study is approved by the IRB under STUDY00006216.
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System ID: NCT04049760