A Study of Migalastat in Pediatric Subjects (2 to <12 Yrs) With Fabry Disease and Amenable GLA Variants

Status: RECRUITING

Eligibility Criteria

Sex: ALL
Age: 2 years to 11 years old
Healthy Volunteers: This study is NOT accepting healthy volunteers
Inclusion Criteria

- Male or female subjects, diagnosed with Fabry disease who are between ages 2 and < 12 years at randomization (subjects aged 11 years must have birthdays > 30 days after randomization)
- 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.
- Subject has a GLA variant documented in his/her medical record that is amenable to migalastat prior to Visit 2.
- Subject has not received ERT (eg, Replagal® [agalsidase alfa] or Fabrazyme® [agalsidase beta]) for at least 14 days prior to Baseline visit.
- Subject has at least 1 documented complication (ie, historical or current laboratory abnormality or sign/symptom) of Fabry disease
- If of reproductive potential, both male and female subjects agree to use a medically accepted method of contraception throughout the duration of the study and for up to 30 days after their last dose of migalastat.

Exclusion Criteria

- Has moderate or severe renal impairment (eGFR < 60 mL/min/1.73 m2 at Visit 1 [screening]).
- Has advanced kidney disease requiring dialysis or kidney transplantation.
- History of allergy or sensitivity to migalastat (including excipients) or other iminosugars (eg, miglustat, miglitol).
- Has received any investigational/experimental drug, biologic, or device within 30 days or 5 half-lives of the investigational product (whichever is longer) before Visit 1 (screening).
- Has received any gene therapy at any time or anticipates starting gene therapy during the study period.
- Requires treatment with Glyset (miglitol) or Zavesca (miglustat), within 6 months before Visit 1(screening) or throughout the study.
- Has any intercurrent illness or condition at Visit 1 (screening) or Visit 2 (baseline) 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 breastfeeding
- Otherwise unsuitable for the study in the opinion of the investigator

Conditions & Interventions

Interventions:
DRUG: Migalastat HCl 20 mg
Conditions:
Fabry Disease
Keywords:
migalastat, AT1001, Galafold, lysosomal disease

More Information

Description:
An open-label study to evaluate the safety, pharmacokinetics (PK), pharmacodynamics (PD), and efficacy of migalastat treatment in pediatric subjects 2 to \< 12 years of age with Fabry disease and with amenable GLA variants.

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