A Study to Evaluate the Safety, Efficacy, PK, PD and Immunogenicity of Cipaglucosidase Alfa/Miglustat in IOPD Subjects Aged 0 to <18

Status: RECRUITING

Eligibility Criteria

Sex: ALL
Age: Up to 17 years old
Healthy Volunteers: This study is NOT accepting healthy volunteers
Inclusion Criteria:

Cohort 1:

1. Male or female subjects who are aged 6 months to < 18 years on Day 1
2. Subject must have documentation of IOPD genotype
3. Subject must have had hypertrophic cardiomyopathy at the time of diagnosis
4. Subject must have received ERT for at least 6 months immediately before enrollment. For subjects whose ERT dosage has been modified, the subject must have been on the modified dosage and regimen for at least 3 months before enrollment
5. Subjects aged ≥ 12 to < 18 years must perform one valid 6-minute walk test (6MWT) (≥ 75 meters) at screening; Subjects aged ≥ 5 to < 12 years must perform one valid 6MWT (≥ 40 meters) at screening; Subjects aged 18 months to < 5 years must be ambulatory and assessed to be likely to be able to perform 6MWT (≥ 40 meters) when they turn 5 years old
6. Subjects must have experienced a clinical decline on their current rhGAA dose and frequency

Cohort 2:

1. Male or female subjects who are aged 0 to <6 months at Day 1
2. Subject must have documentation of IOPD genotype
3. Subject must have had hypertrophic cardiomyopathy at the time of diagnosis
4. Subject is ERT-naïve

Long-term Extension (Cohort 1 or Cohort 2):

1. Subject must have, in the opinion of the investigator, benefited from therapy with cipaglucosidase alfa/miglustat during the 104-week primary treatment period with no significant safety concerns.

Exclusion Criteria:

Cohort 1 and Cohort 2, unless specified

1. Subject requires invasive ventilation (eg, tracheostomy)
2. Subject is CRIM negative and has not received prophylactic immunomodulation (Cohort 1); Subject is CRIM negative and will not be receiving prophylactic immunomodulation (Cohort 2)
3. Subject has a history of life-threatening IARs/hypersensitivity (eg, anaphylaxis and severe cutaneous reactions) to ERT (eg, alglucosidase alfa, cipaglucosidase alfa, miglustat) or other iminosugars, or to any of the excipients, where rechallenge was unsuccessful
4. Subject has prior history of illness or condition known to affect motor function
5. Female subject is pregnant (or intends to get pregnant) or breastfeeding at screening (Cohort 1)

Conditions & Interventions

Interventions:
BIOLOGICAL: Cipaglucosidase alfa, DRUG: Miglustat
Conditions:
Glycogen Storage Disease Type II Infantile Onset

More Information

Description:
This is a Phase 3, open-label, multicenter study to evaluate the safety, efficacy, PK, PD, and immunogenicity of cipaglucosidase alfa/miglustat treatment in ERT-experienced and ERT-naïve pediatric subjects with IOPD.

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