Study of S-606001 as an Add-on to Enzyme Replacement Therapy (ERT) in Participants With Late-onset Pompe Disease (LOPD)

Status: RECRUITING

Eligibility Criteria

Sex: ALL
Age: 18 years and over
Healthy Volunteers: This study is NOT accepting healthy volunteers
Key Inclusion Criteria:

- Participant must be ≥18 years of age and ≥40 kilograms (kg) of body weight at the time of signing the informed consent.
- Participant must have a diagnosis of LOPD based on documentation of 1 of the following:

1. Deficiency of acid alpha-glucosidase (GAA) enzyme
2. GAA genotype
    - Participant has a %FVC ≥30% and ≤80% in an upright position without mechanical ventilation at screening; or Participant has ≥10% %FVC drop from upright position to supine position and %FVC ≥20% in a supine position.
    - Participant performs the 6MWT at screening, as determined by the clinical evaluator, and meets all of the following criteria:
3. Screening values of 6-minute walk distance (6MWD) are ≥75 meters
4. Screening values of 6MWD are ≤90% of the predicted value for healthy adults
    - Participants must be ERT-experienced, defined as currently receiving ERT and having been receiving ERT for ≥24 months, with no regimen change in the last 6 months.

Key Exclusion Criteria:

- Has a medical condition or any other extenuating circumstance that may pose an undue safety risk to the participant or may compromise his/her ability to comply with or adversely impact protocol requirements.
- Has active infections at screening.
- Malignancy within the past 5 years except for basal cell or squamous epithelial carcinomas of the skin that have been resected with no evidence of metastatic disease for 3 years.
- Current or chronic history of liver disease.
- Known biallelic loss of function mutations whether in glycogenin gene (GYG) or in glycogen phosphorylase muscle associated gene(PYGM) .
- Has received any investigational therapy or pharmacological treatment for Pompe disease, within 30 days or 5 half-lives of the therapy or treatment, whichever is longer, before day 1 or is anticipated to do so during the study.
- Has received gene therapy or small interfering ribonucleic acid (RNA) therapy for Pompe disease.
- Participant, if female, is pregnant or breastfeeding at screening.
- Participant, whether male or female, is planning to conceive a child during the study.

Note: Other protocol-specified inclusion and exclusion criteria may apply.

Conditions & Interventions

Interventions:
DRUG: S-606001, DRUG: Placebo
Conditions:
Pompe Disease
Keywords:
Late-onset Pompe disease, Enzyme replacement therapy, LOPD, ERT, S-606001, Muscle glycogen synthase, Liver glycogen synthase, Rare disease, Autosomal disease, Acid alpha-glucosidase, GAA, Glycogen storage disorder, GSD

More Information

Description:
The purpose of this study is to evaluate the safety, pharmacodynamics (PD), and exploratory clinical efficacy of S-606001 in adult participants with LOPD as an add-on to ERT.

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