A Study to Evaluate Safety, Tolerability, and Efficacy of AB-1009 Gene Therapy (GAA Gene) in Adult Participants With Late Onset Pompe Disease (PROGRESS-GT LOPD)

Status: RECRUITING

Eligibility Criteria

Age: 18 years and over

This study is NOT accepting healthy

Healthy Volunteers: volunteers

Inclusion Criteria:

1. Participant must be ≥18 years of age at the time of signing the informed consent form. 2. Confirmed GAA enzyme deficiency from any tissue source and/or confirmed biallelic GAA gene mutations. 3. Undergone enzyme replacement treatment (ERT) (either alglucosidase alfa (Lumizyme®) or avalglucosidase alfa-ngpt (Nexviazyme®)), for at least 6 months (at least 10 infusions) before signing the initial informed consent form. During the screening process, participants need to remain on their current ERT until close to dosing; 4. FVC in the upright position ≥30% and ≤80% of predicted; 5. Capable of walking at least 100 meters in the 6MWT (use of a cane, quad cane, or standard walker is permitted); 6. Contraceptive/barrier use by men and women requirements as per protocol. 7. Capable of giving informed consent and able to understand and comply with all study procedures.

Exclusion Criteria:

1. Severe cardiomyopathy, defined as left ventricular ejection fraction (LVEF) \<40% or New York Heart Association (NYHA) functional class 3 or above; 2. Require invasive mechanical ventilation, or rely on noninvasive ventilation during the day; 3. Intolerance to ERT or investigator-assessed intolerance to ERT, prior experience of serious ERT-related infusion-associated reactions (IARs); 4. Have known intrinsic liver diseases, including hepatitis, HIV-related liver disease, prior diagnosis of portal hypertension, splenomegaly, hepatic encephalopathy, severe fatty liver, cirrhosis or liver fibrosis ≥stage 2, ultrasound-identified liver neoplasms, or laboratory tests suggesting elevated alpha-fetoprotein. Patients with liver function tests including ALT or AST \>3× upper limit of normal (ULN) or any total bilirubin above ULN during screening will also be excluded; 5. Prior or ongoing medical condition(s), physical finding(s), assessment findings, or laboratory abnormality that, in the investigator's opinion, would impact participant's safety and compliance with the study procedures. 6. Have received gene therapy prior to screening; 7. Have received any systemic immunosuppressants (except inhalation or topical use) other than glucocorticoids or investigator-recommended immunosuppressants 30 days prior to screening through completion of screening, and/or known intolerance to immunosuppressants such as glucocorticoids; 8. Use of investigational drugs or drugs that could affect this study as evaluated by the investigator within 30 days prior to screening through completion of Week 52 or within 5 half-lives of the investigational drug (whichever is longer); 9. Have received any vaccine within 30 days prior to dosing; 10. Other conditions that make the participant not eligible for the study according to the investigator.

Conditions & Interventions

Interventions:
GENETIC: AB-1009 (GAA Gene)
Conditions:
Pompe Disease (Late-onset), Pompe Disease Late-Onset, LOPD
Keywords:
Pompe Disease, Glycogen Storage Disease, Lysosomal Storage Diseases, Acid Maltase Deficiency, Acid Maltase Deficiency Disease, Gene Therapy, AB-1009, Neuromuscular Disease, LOPD, Acid-Alpha Glucoside (GAA), GAA gene, Adeno-Associated Virus (AAV), Late-Onset Pompe Disease

More Information

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