A Global Prospective Observational Registry of Patients With Pompe Disease

Status: RECRUITING

Eligibility Criteria

Sex: ALL
Age:
Healthy Volunteers: This study is NOT accepting healthy volunteers
Inclusion Criteria:

- Diagnosis of LOPD or IOPD based on documented deficiency of GAA enzyme activity and/or GAA genotyping

Exclusion Criteria:

- Patients who are currently receiving investigational therapy for Pompe disease in a clinical trial, a compassionate use program, or an expanded access program (EAP)

Conditions & Interventions

Interventions:
BIOLOGICAL: Cipaglucosidase alfa, DRUG: Miglustat, BIOLOGICAL: Alglucosidase alfa or Avalglucosidase alfa, OTHER: Untreated
Conditions:
Pompe Disease

More Information

Description:
This is a global, multicenter, prospective, observational registry of patients with Pompe disease, including those with late-onset pompe disease (LOPD) and infantile-onset pompe disease (IOPD). Both untreated patients and those being treated with an approved therapy for Pompe disease are eligible to participate. The objectives of the registry are: * To evaluate the long-term safety of Pompe disease treatments through collection of data that describe the frequency of adverse events (AEs)/serious adverse events (SAEs) occurring in Pompe disease patients * To evaluate the long-term real-world effectiveness of Pompe disease treatments * To evaluate the long-term real-world impact of Pompe disease treatments on quality of life (QOL) and patient-reported outcomes (PROs) * To describe the natural history of untreated Pompe disease

Contact(s): Jenel Facey - jenel.facey@cchmc.org
Principal Investigator:
Principal Investigator ID:
Phase:
IRB Number:
System ID: NCT06121011