# Pompe Disease Registry Protocol

Status: RECRUITING

## Eligibility Criteria

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

All patients with a confirmed diagnosis of Pompe disease who have signed the informed consent and authorization form(s) are eligible for inclusion. Confirmed diagnosis is defined as documented GAA enzyme deficiency from blood, skin, or muscle tissue and/or documentation of 2 GAA gene mutations.

Exclusion Criteria:

There are no exclusion criteria in this Registry

## Conditions & Interventions

Conditions:
Glycogen Storage Disease Type II, Pompe Disease
Keywords:
Glycogen Storage Disease Type II (GSD-II), GSD-II, Pompe Disease, Pompe Disease (late-onset), Acid Maltase Deficiency Disease, Glycogenosis II

## More Information

Description:
The Pompe Registry is a global, multicenter, international, longitudinal, observational, and voluntary program for patients with Pompe disease, designed to track the disease's natural history and outcomes in patients, both treated and not. Data from the Registry are also used to fulfill various global regulatory commitments, to support product development/reimbursement, and for other research and non-research related purposes. The objectives of the Registry are: * To enhance understanding of the variability, progression, identification, and natural history of Pompe disease, with the ultimate goal of better guiding and assessing therapeutic intervention. * To assist the Pompe medical community with the development of recommendations for monitoring patients, and to provide reports on patient outcomes, to optimize patient care. * To characterize the Pompe disease population. * To evaluate the long-term effectiveness of alglucosidase alfa.

Contact(s): Trial Transparency email recommended (Toll free number for US & Canada) - contact-us@sanofi.com
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