Fabry Disease Registry & Pregnancy Sub-registry

Status: RECRUITING

Eligibility Criteria

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

- Fabry Registry: All patients with a confirmed diagnosis of Fabry disease who have signed the informed consent and patient authorization form(s) are eligible for inclusion. Confirmed diagnosis is defined as a documented deficiency in plasma or leukocyte αGAL (alpha-galactosidase) enzyme activity and/or mutation(s) in the gene coding for αGAL.
- Fabry Pregnancy Sub-registry:
    - Eligible women must:
    - be enrolled in the Fabry Registry.
    - be pregnant, or have been pregnant with appropriate medical documentation available.
    - provide a signed informed consent and authorization form(s) to participate in the Sub-Registry prior to any Sub-Registry-related data collection being performed.

Exclusion Criteria Fabry Registry: There are no exclusion criteria. Fabry Pregnancy Sub-registry: There are no exclusion criteria.

Conditions & Interventions

Conditions:
Fabry Disease
Keywords:
alpha Galactosidase A, aGAL (alpha-galactosidase), Fabry, GL3 (globotriaosylceramide), Anderson-Fabry Disease, angiokeratomas, GLA deficiency (gene deficiency), errors in metabolism

More Information

Description:
The Fabry Registry is an ongoing, international multi-center, strictly observational program that tracks the routine clinical outcomes for patients with Fabry disease, irrespective of treatment status. No experimental intervention is involved; patients in the Registry undergo clinical assessments and receive care as determined by the patient's treating physician. The primary objectives of the Registry are: * To enhance the understanding of the variability, progression, and natural history of Fabry disease, including heterozygous females with the disease; * To assist the Fabry medical community with the development of recommendations for monitoring patients and reports on patient outcomes to help optimize patient care; * To characterize and describe the Fabry population as a whole; * To evaluate the long-term safety and effectiveness of Fabrazyme® Fabry Pregnancy Sub-registry: This Sub-registry is a multicenter, international, longitudinal, observational, and voluntary program designed to track pregnancy outcomes for any pregnant woman enrolled in the Fabry Registry, regardless of whether she is receiving disease-specific therapy (such as enzyme replacement therapy with agalsidase beta) and irrespective of the commercial product with which she may be treated. Data from the Sub-registry are also used to fulfill various global regulatory requirements, to support product development/reimbursement, and for other research and non-research-related purposes. No experimental intervention is given; thus a patient will undergo clinical assessments and receive standard of care treatment as determined by the patient's physician. If a patient consents to this Sub-registry, information about the patient's medical and obstetric history, pregnancy, and birth will be collected, and, if a patient consents to data collection for her infant, data on infant growth through month 36 postpartum will be collected.

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