Minimum Recommended Schedule of Assessments for Monitoring Patients with Fabry Disease

FABRY REGISTRY

	All Patients	Patients not on Enzyme Therapy		Patients on Enzyme Therapy		
	Upon Enrollment	Every 12 months	At time of an event	Baseline and every 6 months	Baseline and every 12 - 24 months	At time of an event or therapy change
General						
Demographics	●					
Enzyme Activity	●					
Genotype	●					
Diagnosis	●					
Medical History	●	●		●		
Physical Examination	●					
Fabry Disease Clinical Assessment [A]						
Cerebrovascular - TIA, Stroke	●	●	●	●		●
Neurology - Sweating, Heat/Cold Intolerence, Pain	●	●		●		
Gastroenterology	●	●		●		
Cardiology - ECHO[B], ECG[B]	●	●[B]	●	●	●[B]	●
Renal - Dialysis, Transplant	●	●	●	●		●
Skin	●	●		●		
Respiratory - Spirometry	●	●	●	●		●
Ophthalmology	●	●	●		●	●
Vital Signs and Laboratory Tests						
Height/Weight	●	●	●	●		●
Blood Pressure	●	●	●	●		●
Serum Creatinine and BUN	●	●	●	●		●
Urinary Protein Excretion [C]	●	●	●	●		●
GFR [D]	●	●	●	●		●
Specialized Tests						
Plasma GL-3	Plasma samples for GL-3 testing should be drawn prior to the first infusion, then every 3 months for the first 18 months of treatment, then every 6 months thereafter.					
Antibody Testing	Serum samples for IgG testing should be drawn prior to the first infusion, then every 3 months for the first 18 months of treatment, then every 6 months until a negative result is confirmed, and annually thereafter.					
Immune Complex Testing	If signs and symptoms of immune complex are evident, appropriate laboratory assessments for circulating immune complexes, such as Raji and C1q binding methods, will be undertaken in consultation with the Genzyme Safety Officer.					
Pain/Quality of Life (QOL) [E]						
SF-36® Health Survey	●	●		●		●
Brief Pain Inventory (Short Form)	●	●		●		●
PedsQL™ Measurement Model	●	●		●		●
Enzyme Replacement Therapy Status	●			●		●
Adverse Event Reporting	Ongoing/continuous monitoring with reporting through Genzyme Pharmacovigilance Department. Refer to Safety section of Protocol and Manual for specific reporting guidelines and instructions.					

[A] Relates to a series of questions of Fabry specific symptoms that are delineated in the CRFs attached. The Clinical Assessments represent the core Fabry-related disease manifestations that are assessed to stage disease progression over the life-long course of the disease. Physicians will determine the actual frequency of necessary assessments according to a patient's individualized need for medical care and routine follow-up.

[B] ECHO and ECG are recommended for patients ≥ 35 years of age every other year.

[C] 24 hour or first morning void urine for urine protein, creatinine and microalbumin

[D] GFR can be estimated using equations such as the MDRD equation for adults and Schwartz formula for children

[E] Ideally, pain, Quality of Life and Health-Related assessments should be measured at Baseline and every 6 months.

FABRY REGISTRY

A Collective Resource to Help Optimize Outcomes

The Fabry Registry is an ongoing, observational database sponsored by Genzyme that tracks natural history and outcomes of patients with Fabry disease. All Fabry patients are eligible for enrollment irrespective of their ERT status, and all physicians managing patients with Fabry disease are encouraged to participate in the Registry. The Fabry Registry is a global outcomes assessment and disease management program that compiles patient outcomes data from routine clinical practice to provide the medical community with resources to help optimize patient care. Patients should be encouraged to participate and advised that their participation is voluntary and may involve long-term follow up. Physicians are encouraged to collaborate, share observations, and generate hypotheses for evaluation, as well as assist in the collection of clinical data in an effort to guide and assess future therapeutic intervention.

Overview

The primary objectives of the Fabry 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 ERT (agalsidase beta).

The registry will also monitor the effect of ERT on pregnant women and their offspring, and determine if ERT is excreted in breast milk.

Please refer to www.fabryregistry.com for more detailed information on the Fabry Registry.

Benefits of Participation

Your contribution of participating patients' data to the Fabry Registry database benefits all other Registry participants, since it is pooled with other data to study trends or address specific questions. As a participating physician, you are encouraged to query the database for specific information to facilitate the management of your Fabry patients. Other benefits of participation include:

- Providing physicians with their own patient-specific reports to monitor disease status
- Encouraging dialog among participating physicians to facilitate clinical discussion
- Providing access to information on evolving patient care and practice patterns

Role of Participating Physicians

Participating physicians are requested to submit participating patients' data on a regular basis. It is recommended that data be submitted to the Registry according to the Recommended Schedule of Assessments found in the Fabry Registry Protocol (see reverse side). In order to provide routine reports regarding participating patient status and current information to fulfill data requests, it is important that the data are submitted on a regular basis. Members of the Fabry Registry staff are available upon request to assist with data collection questions. Physicians may be contacted by Registry staff to answer questions or to clarify information that was submitted to the Registry.

The Fabry Registry Board of Advisors

Scientific direction and monitoring recommendations are provided to the Fabry Registry by a group of physicians in conjunction with Genzyme who have extensive experience in managing patients with Fabry disease. These physicians serve as primary liaisons between the Fabry community and the Registry in their geographic regions. Some of the activities in which the Advisory Board is involved include:

- Developing recommendations for monitoring Fabry disease patients
- Reviewing Fabry Registry Clinical Summary Reports and publications on Fabry disease
- Develop new Case Report Forms (CRFs) to address specific unanswered clinical questions related to Fabry disease
- Consulting with other physicians and health care providers on the management of Fabry patients

A list of the members of the Board can be found on the reverse side.

Accessing Registry Data

Data provided by participating physicians to the Fabry Registry is designed to help optimize the quality of patient care. Routinely, participating physicians will receive summary reports of their own patient data, as well as de-identified and aggregate Fabry Registry reports. Physician and patient information is maintained as confidential and is only used and disclosed in accordance with the signed patient authorization. Data that is provided in response to data requests will be furnished by identification numbers only in a de-identified format. No physician to physician data comparisons will be made.

Any health care professional interested in querying, publishing or presenting aggregate Fabry Registry data should contact the Fabry Registry. Requests for publishing or presenting Registry data will be considered in consultation with the Fabry Registry Board of Advisors according to the Fabry Registry Publication Policy found at www.fabryregistry.com.

To enroll in the Registry or for future information, please contact:

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