INTRODUCTION

Fabry disease is a rare, X-linked lysosomal disorder resulting from α-galactosidase A (α-Gal A) deficiency caused by GLA gene variants.1 The clinical manifestations of Fabry disease include neuropathic pain, skin disorders, ophthalmologic disturbances, gastrointestinal signs and symptoms, pulmonary problems, renal failure, cardiomyopathy, cerebrovascular events, and early death.1–3 Until recently, enzyme replacement therapy (ERT) with agalsidasealfa or agalsidase beta administered via intravenous infusions every 2 weeks was the only treatment option for Fabry disease.4–6 Migalastat, a first-in-class, oral pharmacologic chaperone that binds to amenable variants of a Gal A, is now approved for the treatment of patients with Fabry disease and amenable GLA variants in 38 countries, including the United States.7 To provide a new pathway to understand Fabry disease and the effects of treatment on patients in real-world settings, an observational patient registry study called “followME” was initiated.

STUDY DESIGN

OBJECTIVES

• To assess the long-term safety of migalastat in patients with Fabry disease
• To obtain data on the background incidence of SAEs

SAFETY

• To evaluate the occurrence of Fabry-associated clinical events, including cardiac, cerebrovascular, and renal events
• To assess overall survival

EFFECTIVENESS

• To assess the QoL of patients with Fabry disease using PROs and health preference measures

PROS

Eligibility Criteria

Inclusion Criteria

- Age ≥16 years
- Fabry disease diagnosis
- eGFR_{CKD-EPI} ≥30 mL/min/1.73 m²
- Provided written, informed consent

Migalastat-Treated Patients

- Starting or taking migalastat treatment (for ≥24 months) at enrollment

Untreated Patients

- Never received treatment for Fabry disease
- Meet local treatment guidelines for Fabry disease

Interim Data Sources and Collection

- The followMe registry will share data through a secure platform.
- The registry will include data from 80 centers worldwide.
- Patient data will be fully anonymized; protected health information will not be shared.

Data Sharing

- Data requests are reviewed by the Registry Steering Committee, which is composed of clinical specialists and patient representatives.
- Once the proposal is approved, de-identified data sets will be made available to researchers.

Patient Involvement

- Participant data are collected via an electronic PRO system.
- An annual registry report will be made available to patients and the community.

DISCUSSION

- Often, patients with Fabry disease want to share their experience with physicians, researchers, and other patients in the Fabry disease community. followME allows this and aims to forge new paths to treat and understand Fabry disease
- followME will open a new chapter in research by advancing the understanding of treatments for Fabry disease and providing greater insight into the disease and its mechanisms
- followME, which began in August 2018, will add to the rapidly growing real-world experience with migalastat and other treatments for Fabry disease
- The followME’s unique data-sharing platform will empower patients and their physicians to gain a deeper understanding of Fabry disease, on both the individual patient level and the larger population level
- Additional information about followME can be found at https://www.followmeobservationalregistry.com/