# A Prospective, Observational Registry of Patients with Fabry Disease (AT1001-030)

**First published:** 12/09/2017

**Last updated:** 17/09/2025

Study Ongoing

## Administrative details

**EU PAS number**

EUPAS20599

**Study ID**

35885

**DARWIN EU® study**

No

**Study countries**

- [ ] Australia
- [ ] Austria
- [ ] Canada
- [ ] Croatia
- [ ] Denmark
- [ ] Finland

- [ ] Germany
- [ ] Greece
- [ ] Hungary
- [ ] Ireland
- [ ] Israel
- [ ] Italy
- [ ] Norway
- [ ] Portugal
- [ ] Spain
- [ ] Switzerland
- [ ] United Kingdom
- [ ] United States

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**Study description**

This is a prospective, multi centre, multinational, observational, safety, effectiveness, and outcomes registry enrolling approximately 450 Fabry disease patients (approximately 250 patients in the migalastat treated group, 100 patients in the ERT-treated group, and 100 patients in the untreated group). All patients will be followed for up to 5 years after enrolment.This is a registry to evaluate the effects of treatment on long-term safety, effectiveness, and quality of life (QOL) in patients with Fabry disease, with a main focus on migalastat, a medicine with a novel mechanism of action. Occurrence of key indicators of safety and effectiveness will be evaluated, such as cardiac, cerebrovascular and renal events, serious adverse events, and overall survival. A comparison of these events will be evaluated over a period of 5 years in migalastat-treated, ERT-treated (with amenable and non-amenable mutations), and untreated patients with Fabry disease who have amenable mutations.

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**Study status**

Ongoing

## Research institutions and networks

## Institutions

### Amicus Therapeutics

**First published:** 01/02/2024

**Last updated:** 01/02/2024

Institution

## Contact details

**Study institution contact**

Amicus Therapeutics Patient Advocacy

patientadvocacy@amicusrx.com

Study contact

patientadvocacy@amicusrx.com

**Primary lead investigator**

Jasmine Rutecki

Primary lead investigator

## Study timelines

**Date when funding contract was signed**

Actual: 01/12/2016

**Study start date**

Planned: 03/05/2018

Actual: 08/08/2018

**Date of final study report**
Planned: 03/05/2028

## Sources of funding

- Pharmaceutical company and other private sector

## More details on funding

Amicus Therapeutics UK Limited

## Regulatory

**Was the study required by a regulatory body?**

Yes

**Is the study required by a Risk Management Plan (RMP)?**

EU RMP category 3 (required)

## Other study registration identification numbers and links

EU/1/15/1082/001

# Methodological aspects

## Study type

## Study type list

**Study topic:**

Human medicinal product

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**Study type:**

Non-interventional study

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**Scope of the study:**

Disease epidemiology

Effectiveness study (incl. comparative)

Safety study (incl. comparative)

**Main study objective:**

This is a registry to evaluate the effects of treatment on long-term safety, effectiveness, andquality of life (QOL) in patients with Fabry disease, with a main focus on migalastat.Occurrence of key indicators of safety and effectiveness will be evaluated, such as serious adverse events, cardiac,cerebrovascular and renal events, and overall survival.

## Study Design

**Non-interventional study design**

Cohort

Other

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**Non-interventional study design, other**

This is a prospective, multi centre, multinational, observational, safety and effectiveness study

## Study drug and medical condition

**Name of medicine**

GALAFOLD

**Study drug International non-proprietary name (INN) or common name**

MIGALASTAT

**Anatomical Therapeutic Chemical (ATC) code**

(A16AX14) migalastat

migalastat

**Medical condition to be studied**

Fabry's disease

## Population studied

**Age groups**

Adolescents (12 to < 18 years)

Adults (18 to < 46 years)

Adults (46 to < 65 years)

Adults (65 to < 75 years)

Adults (75 to < 85 years)

Adults (85 years and over)

**Special population of interest**

Pregnant women

Renal impaired

**Estimated number of subjects**

450

## Study design details

**Data analysis plan**

Descriptive statistics will be presented. No formal hypothesis testing will be performed. Alldata will be summarized by treatment group. Continuous variables will be summarized usingthe number of observations (n), mean, standard deviation (SD), median, minimum, andmaximum. Categorical data will be summarized using counts and percents. For event data,ie, SAEs and the Fabry Associate Clinical Events (FACEs) of cardiac events, cerebrovascular events and renal events, exposure-adjusted incidence rates will be presented as counts of patients with a new event per 100 person-years exposure, with 95% confidence intervals using Ulm's method. An analysis of recurrent events will be conducted for SAEs and each specific FACE event and will be presented as total counts of each event per 100 person-years. A Cox proportional hazards ratio model will be used for the summary of survival data and will be adjusted forage at baseline and any previous cardiovascular events.

# Data management

## ENCePP Seal

The use of the ENCePP Seal has been discontinued since February 2025. The ENCePP Seal fields are retained in the display mode for transparency but are no longer maintained.

## Data sources

**Data sources (types)**

Electronic healthcare records (EHR)

Other

**Data sources (types), other**

Spontaneous reporting system, Prospective patient-based data collection

## Use of a Common Data Model (CDM)

**CDM mapping**

No

## Data quality specifications

**Check conformance**

Unknown

**Check completeness**

Unknown

**Check stability**

Unknown

**Check logical consistency**

Unknown

## Data characterisation

**Data characterisation conducted**

No