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

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Last updated: 18/06/2025

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

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.

Study status

Ongoing

Research institutions and networks

Institutions

Amicus Therapeutics

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Institution

Contact details

Study institution contact

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Study contact

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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 type:

Non-interventional study

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

Other

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

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