A Global Prospective Observational Study Of Women With Fabry Disease And Their Infants During Pregnancy And Breastfeeding (AT1001-037)

First published: 16/12/2020

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Study Ongoing

Administrative details

EU PAS number

EUPAS38668

Study ID

38669

DARWIN EU® study

No

Study countries

United Kingdom

United States

Study description

This is a global prospective observational study of women with Fabry disease and their infants during pregnancy and/or breastfeeding. The study will evaluate outcomes of pregnancy and/or breastfeeding in women and infants exposed to migalastat. All pregnant women with Fabry disease are eligible to enroll, an unexposed cohort potentially can be used for comparisons. Cases will be reported voluntarily to the Pregnancy Coordinating Center (PCC) from any country by Healthcare Providers (HCPs), by patients and secondary contacts, as well as, from any Fabry Amicus Registry, and from any ongoing Amicus clinical trials or expanded access programs. Patient enrollment and follow-up is facilitated remotely by the PCC and PCC Investigator. The PCC Investigator will oversee study operations and the PCC will be responsible for enrolling patients and collecting data as described in the protocol. The PCC will follow patients throughout their pregnancies and/or breastfeeding and infants through 1 year of age. All PCC activities will follow country-specific regulatory and ethics committee procedures. As the study is observational, medical treatment for each patient and infant will be consistent with routine clinical practice, and will not be mandated or required in any way by the study protocol. The study will enroll patients for a minimum of 10 years and may need to extend beyond 10 years if there are few participants compared to pharmacovigilance cases or an increased usage of migalastat in females of reproductive potential. Amicus will discuss with the Agency the status and interim results prior to closing the study.

Study status

Ongoing

Research institutions and networks

Institutions

United BioSource Corporation (UBC)

Switzerland

First published: 25/04/2013

Last updated: 06/03/2024

Institution | Non-Pharmaceutical company | ENCePP partner

Contact details

Study institution contact

Fabry Pregnancy Registry Pregnancy Coordinating Center

fabrypregnancy@ubc.com

Study contact

fabrypregnancy@ubc.com

Primary lead investigator

Janine Collins

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 01/05/2019

Actual: 01/05/2019

Study start date

Planned: 30/04/2020

Actual: 17/04/2020

Date of interim report, if expected
Planned: 31/07/2020

Actual: 31/07/2020

Date of final study report
Planned: 31/10/2020

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Amicus Therapeutics, Inc.

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)

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Scope of the study:

Drug utilisation

Main study objective:

Describe & estimate the frequency of pregnancy complications in women with Fabry disease who were exposed to at least 1 dose of migalastat during pregnancy & fetal/infant outcomes in infants through 1 year of age > pregnancy outcomes > fetal/neonatal and infant outcomes

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medical condition to be studied

Fabry's disease

Population studied

Age groups

- 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)

Estimated number of subjects

100

Study design details

Outcomes

Describe and estimate the frequency of pregnancy complications in women with Fabry disease who were exposed to at least 1 dose of migalastat during pregnancy and fetal and infant outcomes in infants through 1 year of age > pregnancy outcomes > fetal/neonatal and infant outcomes

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Data analysis plan

A formal Statistical Analysis Plan (SAP) will include details of all planned analyses and presentation of study data. Since this is an observational study, descriptive analyses will be provided. Descriptive statistics will comprise the number of observations (n), mean, standard deviation (SD), median, minimum, and maximum for continuous variables, and n and percent for categorical variables.

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)

Disease registry

Other

Data sources (types), other

Prospective patient-based data collection, Exposure registry

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