A prospective multicenter observational post authorization safety sub-registry to characterize the long-term safety profile of commercial use of eliglustat (Cerdelga®) in adult patients with Gaucher disease (ELISAFE)

First published: 11/02/2016

Last updated: 25/06/2024

Study Ongoing

Administrative details

EU PAS number

EUPAS11998

Study ID

48611

DARWIN EU® study

No

Study countries

- [ ] Belgium
- [ ] Denmark
- [ ] Greece
- [ ] Italy
- [ ] Portugal
- [ ] Romania
- [ ] Spain
- [ ] United Kingdom

Study description

This is a non-interventional, multicenter, prospective post-authorization safety study of Cerdelga. The aim of the study is to collect long term safety data from the International Collaborative Gaucher Group (ICGG) Gaucher Registry patients treated with Cerdelga and Cerezyme, and to describe the utilization of Cerdelga. The study will enroll patients who will be receiving Cerdelga or Cerezyme for the first time or as part of ongoing treatment of their Gaucher disease (GD). Since this is a sub-registry of the ICGG Gaucher Registry, Health Care Providers (HCPs) for the sub-registry will be chosen among the enrolling sites within the ICGG Gaucher Registry and in countries where Cerdelga will be available on the market. Inclusion criteria for the study are very wide to enable HCP’s to enroll as many adult patients treated or to be treated with Cerdelga or Cerezyme as possible in order to collect data in as many patients as possible. The exclusion criteria are limited only to patients currently enrolled in another clinical trial and patients treated with Cerezyme and Cerdelga in combination at the time of enrolment. At least 100 patients enrolled will be on Cerdelga. Patients will receive treatment as determined by the patient’s HCP and guided by the provisions of the prevailing locally approved product labelling and the ICGG Gaucher Registry recommendations for patient monitoring. This observational study will operate under real-world clinical practice conditions. There are no imposed protocol visits or procedures. It will collect only available

data at the time of patient visits occurring as per local practice visit schedule or following the recommended schedule of assessments of the ICGG Gaucher Registry. There is no set duration for this study. Data collection will be concluded four years after the last Cerdelga patient has been enrolled in the study at which time analysis will be performed. Information will be collected in case report forms (CRFs).

Study status

Ongoing

Research institutions and networks

Institutions

Sanofi

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Institution

Contact details

Study institution contact

Trial Transparency Team Contact-US@Sanofi.com

Study contact

Contact-US@Sanofi.com

Primary lead investigator

Trial Transparency Team

Primary lead investigator

Study timelines

Date when funding contract was signed
Planned: 03/10/2016

Actual: 25/07/2017

Study start date
Planned: 09/01/2018

Actual: 18/04/2018

Date of interim report, if expected
Actual: 03/07/2023

Date of final study report
Planned: 03/07/2025

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Sanofi Genzyme

Study protocol

OBS14099 protocol_PASS_PRAC03_23Aug2016.pdf (626.47 KB)

rdct-obs14099-16-1-1-pass-amended-protocol04-pdfa.pdf (640.63 KB)

Regulatory

Was the study required by a regulatory body?

Yes

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

EU RMP category 1 (imposed as condition of marketing authorisation)

Other study registration identification numbers and links

OBS14099

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Drug utilisation

Main study objective:

1. To evaluate the long term safety of Cerdelga in real-world clinical practice

2. To describe the utilization of Cerdelga (compliance/adherence of Health Care Providers):

- to the labelling with regard to CYP2D6 genotyping prior to initiation of Cerdelga

- to the Cerdelga label with regard to patients' CYP2D6 predicted phenotype

- to the labelling with regard to use in Gaucher disease type 1

Study drug and medical condition

Medicinal product name

CERDELGA

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

ELIGLUSTAT

Anatomical Therapeutic Chemical (ATC) code

(A16AX10) eliglustat

eliglustat

Medical condition to be studied

Gaucher'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

150

Study design details

Outcomes

• Incidence rate of any AE.

• Frequencies and percentages of timing of CYP2D6 genotyping (prior to or following) initiation of Cerdelga.

• Frequencies and percentages of CYP2D6 predicted phenotype among Cerdelga-treated patients.

• Frequencies and percentages of types of GD among Cerdelga-treated patients.

• Incidence rate of any serious adverse event.

Data analysis plan

Analysis will be performed on the Safety Set defined as all patients enrolled in the sub-registry who have received at least one dose of Cerdelga or Cerezyme following enrollment. The main analysis of interest will be the incidence of any AE report for Cerdelga treated patients during their participation in the subregistry, considering treatment status at sub-registry enrollment if applicable. Similar calculations will be performed for the Cerezyme treated

patients. Summaries will also be presented for three categories of treatment status within each of the Cerdelga and Cerezyme groups. Data will be analyzed as available, missing data will not be imputed. All summaries will be descriptive, no formal hypothesis testing will be performed.

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

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