Drug utilisation study of Eliglustat for the treatment of Gaucher Disease Type 1 in Europe using electronic healthcare records (ELIGLC06913)

First published: 14/04/2020

Last updated: 26/04/2024

Study Planned

Administrative details

PURI

https://redirect.ema.europa.eu/resource/50547

EU PAS number

EUPAS34611

Study ID

50547

DARWIN EU® study

No

Study countries

- [ ] Belgium
- [ ] France
- [ ] Germany

Study description

Eliglustat (Cerdelga®) is an oral treatment indicated for the long-term treatment of Gaucher Disease Type 1 (GD1). This treatment was approved by the EMA on 19/01/2015. Today, the EMA requested a drug utilisation study (DUS) on Eliglustat and its concomitant treatments in Europe. Eliglustat is metabolised primarily by CYP2D6, and to a lesser extent by CYP3A. The concomitant use of drugs affecting CYP2D6 or CYP3A4 activity may alter Eliglustat plasma concentrations. Inversely, Eliglustat may alter the activity of these substances. Therefore, it is important that the use of inhibitors of CYP2D6 and/or CYP3A, strong CYP3A inducers, P-gp and CYP2D6 substrates as concomitant medications among patients treated with Eliglustat be carefully monitored. Moreover, a pilot study conducted in Lombardia (Italy) in 2015 showed that a lot of patients with GD were treated with several concomitant medications such as inhibitors of CYP2D6 and of CYP3A. Consequently, this DUS would inform on the usage patterns prevailing in Europe and evaluate the effectiveness of risk minimisation measures that have been put in place. The aim of the DUS is to estimate the dose and duration of Eliglustat therapy as well as the proportion, type, and duration of past and concomitant medication use in GD1 patients treated with Eliglustat. The concomitant medications of interest are strong and moderate inhibitors of CYP2D6 and/or CYP3A inhibitors, strong CYP3A inducers, P-gp substrates and CYP2D6 substrates. Also, the DUS will describe the healthcare service pattern for the prescriptions of concomitant medications in patients treated with Eliglustat. The DUS is planned in five European countries (Belgium, France, Denmark, Germany and UK) using existing health databases. In each country, the period of study will be from one

year before the launching of Eliglustat to Q3 2022. The final report to EMA is planned in Q4 2024.

Study status

Planned

Research institutions and networks

Institutions

Parexel International

United States

First published: 19/10/2010

Last updated: 10/12/2024

Institution **Non-Pharmaceutical company** **ENCePP partner**

International Prevention Research Institute (IPRI)

France

First published: 19/03/2010

Last updated: 05/04/2012

Institution **EU Institution/Body/Agency** **ENCePP partner**

Contact details

Study institution contact

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Primary lead investigator

Katja M Hakkarainen

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 01/08/2013

Actual: 01/08/2013

Study start date

Planned: 03/10/2022

Data analysis start date

Planned: 01/04/2024

Date of final study report

Planned: 27/11/2024

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Sanofi-Genzyme

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

EMEA/H/C/003724

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Scope of the study:

Drug utilisation

Main study objective:

The main objective of the DUS is to estimate the dose and duration of Eliglustat therapy as well as the proportion, type, and duration of past and concomitant medication use in GD1 patients treated with Eliglustat. The concomitant medications of interest are strong and moderate inhibitors of CYP2D6 and/or CYP3A inhibitors, strong CYP3A inducers, P-gp substrates and CYP2D6 substrates.

Study drug and medical condition

Name of medicine

CERDELGA

Medical condition to be studied

Gaucher's disease type I

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

Data analysis plan

Only descriptive analyses will be performed: - Descriptive analysis of Eliglustat therapy in terms of duration and dose. - Descriptive analysis of past (one year prior to Eliglustat initiation) and concomitant medication use in terms of proportion, type, and duration. The treatments of interest will be strong and moderate inhibitors of CYP2D6 and/or CYP3A, strong CYP3A inducers, P-gp substrates and CYP2D6 substrates. The proportions will be computed taking into account all treatments of interest and also by type of treatments and by duration. Proportions for the group CYP inhibitors will also be computed. - Descriptive analysis of the health care service pattern (i.e. prescriber's specialty and types of patient visit) for the use of concomitant medications in patients treated with Eliglustat. All these analyses will be conducted by country. Stratification analyses based on sex and age will be performed if the number of GD1 patients taking Eliglustat allows it.

Data management

Data sources

Data sources (types)

Administrative healthcare records (e.g., claims)

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