A MULTICENTER, MULTICOUNTRY, POSTMARKETING ACTIVE SURVEILLANCE TALIGLUCERASE ALFA REGISTRY IN PATIENTS WITH GAUCHER DISEASE

First published: 18/09/2013

Last updated: 13/05/2024

Study Ongoing

Administrative details

EU PAS number

EUPAS4721

Study ID

47627

DARWIN EU® study

No

Study countries

Albania

Israel

Türkiye

United States

Study description

To gather data on the long term safety and effectiveness of taliglucerase alfa in the real world post-marketing setting, Pfizer will conduct a prospective non-interventional active surveillance drug registry of patients with Gaucher disease undergoing taliglucerase alfa treatment (referred to as the “Drug Registry”). The registry will be open for at least ten years. The pregnancy and lactation exposure related sub-study will be nested within the Drug Registry (and is referred to as the “Pregnancy/Lactation Sub Study”). The Pregnancy/Lactation Sub-Study will be open for the maximum of 11 years (ie, will extend a maximum of 1 year beyond the end of data collection in the Drug Registry if a woman becomes pregnant during the last 9 months of the Drug Registry).

Study status

Ongoing

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

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Muhammad Younus

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 01/10/2013

Study start date

Planned: 31/10/2013

Actual: 18/09/2013

Date of interim report, if expected

Planned: 31/07/2019

Actual: 31/07/2019

Date of final study report

Planned: 24/07/2024

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Pfizer

Regulatory

Was the study required by a regulatory body?

Yes

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

Non-EU RMP only

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Scope of the study:

Effectiveness study (incl. comparative)

Other

If ‘other’, further details on the scope of the study

Evaluation of long-term safety and effectiveness of taliglucerase alfa, including effects on pregnancy and fetal outcomes, and newborns and infants who breastfeed from mothers treated with taliglucerase alfa

Main study objective:
The main objectives of the registry are to characterize the safety profile of taliglucerase alfa through the solicited collection and summary of non serious and serious adverse event data and to characterize the effectiveness of taliglucerase alfa through the collection and analysis of Gaucher disease measures

Study Design

Non-interventional study design
Cohort

Study drug and medical condition

Medicinal product name, other
Elelyso

Medical condition to be studied
Gaucher's disease

Population studied

Age groups

- Children (2 to < 12 years)
- 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

Estimated number of subjects

1

Study design details

Outcomes

Reports of all serious adverse events and non serious adverse events that occur during follow up and Gaucher disease measures including hematologic (hemoglobin and platelet count) and organ volume (spleen and liver) assessments, Sub-study outcomes that include reports of all relevant pregnancy, fetal, neonatal and infant outcomes

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Data analysis plan

Data will be analyzed using descriptive statistics. For outcomes of interest, summary statistics, including counts and frequencies will be calculated. Crude cumulative incidence, and crude incidence rates per person-time will be calculated as appropriate. Depending on the outcome of interest, stratified analyses may be performed. Further exploratory analyses will be developed as necessary

Documents

Study report

B3031002 Interim Study Report Abstract_EU PAS Register.pdf (136.49 KB)

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)

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