A Prospective Safety Sub-Registry to Assess Anaphylaxis and Severe Allergic Reactions, and Severe Cutaneous and Systemic Immune-Mediated Reactions with Alglucosidase Alfa Treatment (Pompe Safety Sub-Registry)

First published: 02/04/2015

Last updated: 14/03/2024

Study Finalised

Administrative details

PURI

https://redirect.ema.europa.eu/resource/44514

EU PAS number

EUPAS9194

Study ID

44514

DARWIN EU® study

No

Study countries

- [ ] Belgium
- [ ] Germany
- [ ] Italy
- [ ] Taiwan
- [ ] United States

Study description

The objectives of this Safety Sub-Registry are to collect uniform and meaningful data on patients with Pompe disease who experience anaphylaxis, severe allergic reactions, and/or signals of severe cutaneous and/or systemic immune-mediated reactions following treatment with alglucosidase alfa. This Safety Sub-Registry also will assess: • the symptoms, severity, outcome, and occurrence of those adverse events (AEs, anaphylaxis, severe allergic reactions, and signals of severe cutaneous and systemic immune mediated reactions), • the effect of antibody responses (both timing and pattern of responses) and cross reacting immunologic material (CRIM) status (in patients with age at symptom onset less than or equal to 12 months only) on the occurrence of such AEs.

Study status

Finalised

Research institutions and networks

Institutions

Sanofi

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Institution

Contact details

Study institution contact

Trial Transparency Team Trial Transparency Team

Study contact

Contact-US@sanofi.com

Primary lead investigator

Trial Transparency Team Trial Transparency Team

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 08/12/2010

Actual: 09/09/2015

Study start date

Planned: 01/06/2011

Actual: 20/03/2015

Date of final study report
Planned: 30/09/2022

Actual: 02/11/2021

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Genzyme, a Sanofi company

Study protocol

rdct- lts13930-aglu06909-amended-protocol2-PDFA.pdf(466.25 KB)

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

LTS13930

Methodological aspects

Study type

Study topic:

Disease /health condition

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Safety study (incl. comparative)

Data collection methods:

Combined primary data collection and secondary use of data

Main study objective:

The objectives of this Safety Sub-Registry are to collect uniform and meaningful data on patients with Pompe disease who experience anaphylaxis, severe allergic reactions, and/or signals of severe cutaneous and/or systemic immune-mediated reactions following treatment with alglucosidase alfa.

Study Design

Non-interventional study design

Other

Non-interventional study design, other

Sub-Registry, Non-interventional, Observational study

Study drug and medical condition

Name of medicine

MYOZYME

Name of medicine, other

Lumizyme

Medical condition to be studied

Glycogen storage disease type II

Population studied

Short description of the study population

At least 100 patients enrolled in the Pompe Registry at selected sites around the world who meet the inclusion criteria for this Safety Sub-Registry are eligible to participate in the Safety SubRegistry. This includes patients with onset of clinical signs/symptoms at ≤12 months of age (infantile-onset Pompe disease), as well as those with symptom onset at >12 months of age (lateonset Pompe disease). No single participating site is allowed to enroll more than 20% of the total Safety Sub-Registry patient population. Patients currently treated with alglucosidase alfa and treatment-naïve patients who initiate treatment at time of enrollment in the Safety Sub-Registry are targeted for enrollment at each site.

INCLUSION CRITERIA

Patients must meet all of the following criteria to be eligible for inclusion in this Safety SubRegistry:

• be enrolled in the Pompe Registry;

• provide a signed Patient Information and Authorization form;

• have a confirmed diagnosis of Pompe disease (confirmation of diagnosis is defined as documented GAA enzyme deficiency from any tissue source and/or

documentation of 2 GAA gene mutations);
• be naïve to and plan to be treated with alglucosidase alfa at or prior to enrollment, or are being treated with alglucosidase alfa.
EXCLUSION CRITERIA
Patients will be excluded if they have received an investigational drug (excluding alglucosidase alfa) within 30 days prior to signing a Safety Sub-Registry Patient Information and Authorization form, or if they are taking or plan to take any investigational product while enrolled in the Safety Sub-Registry.

Age groups

Term newborn infants (0 – 27 days)

Infants and toddlers (28 days – 23 months)

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

Other

Special population of interest, other

Patients with Glycogen storage disease type II/Pompe disease

Estimated number of subjects

100

Study design details

Outcomes

•the symptoms, severity, outcome, and occurrence of those adverse events (AEs, anaphylaxis, severe allergic reactions, and signals of severe cutaneous and systemic immune mediated reactions), •the effect of antibody responses and cross reacting immunologic material (CRIM) status (in patients with age at symptom onset less than or equal to 12 months only) on the occurrence of such AEs.

Data analysis plan

Genzyme Registry staff will perform the statistical analysis of the data derived from the Registry, using the SAS® statistical software.

Documents

Study results

rdct- lts13930-CSR Abstract-PDFA.pdf(629.01 KB)

Data management

Data sources

Data sources (types)

Disease registry

Electronic healthcare records (EHR)

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