Myozyme (alglucosidase alfa) Safety Information Packet effectiveness evaluation: a health care professional survey

First published: 15/07/2015

Last updated: 02/07/2024

Study Finalised

Administrative details

EU PAS number

EUPAS10301

Study ID

49116

DARWIN EU® study

No

Study countries

- ☐ France
- ☐ Germany
- ☐ Italy

- [ ] Poland
- [ ] Spain
- [ ] United Kingdom

Study description

The Pharmacovigilance Risk Assessment Committee (PRAC) requested Genzyme, a Sanofi Company to further update the Myozyme Safety Information Packet (SIP) and to propose a study to evaluate the effectiveness of the updated version of the SIP. To comply with PRAC requirements, a health care professional survey that assesses the effectiveness of the updated SIP is proposed. The survey consists of two waves (wave 1 and wave 2) which are to be carried out pre- and post-implementation of the updated SIP, at least 18 months apart. The survey is intended to assess whether implementation of the updated SIP has led to increased awareness, usage, usefulness, readability, understanding, clinical knowledge and behavioural implementation of key safety messages compared with the previous version of the SIP. Distribution and opinion of HCPs about the SIP will also be evaluated.

Study status

Finalised

Research institutions and networks

Institutions

OXON Epidemiology

- [ ] Spain
- [ ] United Kingdom

First published: 06/12/2010

Last updated: 15/03/2024

Institution | Laboratory/Research/Testing facility | Non-Pharmaceutical company | ENCePP partner

Contact details

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Primary lead investigator

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Study timelines

Date when funding contract was signed

Planned: 09/12/2014

Actual: 09/12/2014

Study start date

Planned: 01/05/2015

Actual: 17/06/2015

Date of interim report, if expected
Planned: 20/09/2015

Actual: 14/09/2015

Date of final study report
Planned: 08/03/2017

Actual: 31/03/2017

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Genzyme Europe B.V., The Netherlands.

Study protocol

Myozyme HCP Survey_Protocol_V3-2_(30Jan2015).pdf (1.08 MB)

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 topic:

Disease /health condition

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Effectiveness study (incl. comparative)

Data collection methods:

Primary data collection

Main study objective:

The main objective of this survey is to assess awareness, readability, usage, usefulness, understanding, knowledge of the management of risks associated with Myozyme and behavioral implementation of key safety information contained in the updated safety information packet (SIP) as compared to the previous version of the SIP among HCPs.

Study Design

Non-interventional study design

Cross-sectional

Study drug and medical condition

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

ALGLUCOSIDASE ALFA

Medical condition to be studied

Enzyme level decreased

Population studied

Short description of the study population

A survey of health care professional prescribing and monitoring myozyme patients to evaluate awareness, readability, usage, usefulness, understanding, knowledge of the management of risks associated with myozyme and behavioral implementation of key safety information contained in the updated Myozyme Safety Information Packet (SIP) as compared to the previous version of the SIP. The survey was conducted in the France, Germany, Italy, Spain, UK and Poland.

Inclusion criteria:

• Managed at least one patient in the preceding year on Myozyme for Pompe disease through prescribing, monitoring or administering Myozyme therapy.

Exclusion criteria:

• Current or ex-employee of Genzyme, a Sanofi Company or Sanofi.

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

Study design details

Outcomes

The updated safety information packet (SIP) will be compared to the previous version of the SIP for differences in: awareness, usage of the SIP, levels of HCPs' knowledge and understanding related to the key messages, behavior of HCPs around key safety messages and levels of readability, degree of usefulness, The updated safety information packet (SIP) will be compared to the previous version of the SIP for differences in proportion of HCPs who have received the SIP, determinants of response, opinion and qualitative reasons for lack of immunological testing.

Data analysis plan

The primary analysis will assess, for each survey wave, awareness, readability, usage, usefulness, understanding, patient management and behavioural implementation by percentage and mean scores. Comparisons of these endpoints between the two waves will be made. For each survey, distribution and opinion will be assessed by percentage.

Documents

Study results

Summary of Study report_EUPAS10301_ALGMYC08432.pdf (98.66 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

Two-wave cross-sectional survey

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