Fabrazyme (agalsidase beta) home infusion educational materials effectiveness evaluation: a survey for healthcare providers and patients/caregivers (Fabrazyme Home Infusion Ed Mat Survey)

First published: 14/03/2018

Last updated: 25/06/2024

Study Finalised

Administrative details

PURI

https://redirect.ema.europa.eu/resource/35382

EU PAS number

EUPAS23142

Study ID

35382

DARWIN EU® study

No

Study countries

- [ ] Finland
- [ ] France
- [ ] Italy
- [ ] Norway
- [ ] United Kingdom

Study description

The objective of this study is to assess the effectiveness of the agalsidase beta (Fabrazyme®) home infusion educational materials from both the healthcare provider (HCP) and the patient perspectives with regard to: distribution, understanding, usage, implementation of the logbook and opinions of the material.

Study status

Finalised

Research institutions and networks

Institutions

Sanofi

First published: 01/02/2024

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Institution

Contact details

Study institution contact

Trial Transparency Team

Study contact

Contact-US@sanofi.com

Primary lead investigator

Irene Shui

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 13/12/2017

Actual: 13/12/2017

Study start date

Planned: 01/12/2018

Actual: 01/11/2018

Date of final study report

Planned: 01/08/2019

Actual: 06/02/2020

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Sanofi Genzyme

Study protocol

rdctEUPASS-AGALSC08994 Protocol-Final PDFA version-29APR2020.pdf(375.72 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

AGALSC08994

Methodological aspects

Study type

Study type list

Study topic:

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 overall objective of this study was to assess the effectiveness of the home infusion educational materials from both the healthcare provider and the patient / caregiver perspectives. Effectiveness was measured through questions on knowledge and understanding of key content areas. Implementation of the logbook around the key content areas was also used to assess effectiveness of the materials.

The primary objectives were to determine:

- The level of knowledge and understanding regarding key content areas of the educational materials including: evaluation of home infusion (HCP) / elements to address when training the patient / caregiver / actions to be taken in case of an adverse event.
- Implementation of the logbook (owned by the patient but reviewed by the HCP).

Study Design

Non-interventional study design

Cross-sectional

Study drug and medical condition

Name of medicine

FABRAZYME

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

AGALSIDASE BETA

Anatomical Therapeutic Chemical (ATC) code

(A16AB04) agalsidase beta

agalsidase beta

Population studied

Short description of the study population

Online survey was conducted independently in two populations: 1) HCPs and 2) Patients or their caregivers

HCPs include both physicians and nurses:

A) Physicians: A target list of physicians known to be prescribing Fabrazyme home infusion will be provided by the MAH and used for recruitment.

B) Nurses: To recruit nurses we will use a mix of both targeted recruitment and via physician referral; for targeted recruitment the MAH will provide a list of external home care provider companies with contact details per market. Potentially eligible nurses will be contacted via telephone or email with information about this study.

Inclusion/exclusion criteria:

• Involved in the treatment of at least 1 patient with Fabry disease in the last 12 months

• Have prescribed Fabrazyme for home infusion and/or monitor, oversee the management, and/or provide in person medical supervision of patients on Fabrazyme home infusion in the last 12 months

• Not a current or ex-employee of Sanofi

Patient/caregivers recruitment

All HCPs from the target lists provided by the MAH and who are contacted regarding the study will also be asked to refer all patients they are treating for Fabry disease for inclusion within the study.

Patient/Caregiver inclusion/exclusion criteria include

• Diagnosed with Fabry disease / caring for a patient diagnosed with Fabry disease

• Patient received Fabrazyme in the home infusion setting in the past 12 months

• Not a current or ex-employee of Sanofi

Age groups

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)

Estimated number of subjects

52

Study design details

Outcomes

• The distribution and usage of the materials.

• The level of understanding regarding:

- Requirement and organisation for Fabrazyme home infusion,

- Preparation and administration of Fabrazyme home infusion,

- Safety reporting and actions to be taken in case of an adverse event.

• Opinions on how to improve the materials.

• Implementation of the logbook (owned by the patient but reviewed by the HCP).

Data analysis plan

The analysis will be cross-sectional and descriptive. Categorical data will be summarized by counts and percentages. Continuous data will be summarized using number, median, minimum and maximum values. Missing data will be noted for each variable.

Documents

Study results

rdctEUPASS-AGALSC08994 Study Report Abstract-Final PDFA version-29APR2020.pdf(426.05 KB)

Data management

Data sources

Data sources (types)

Other

Data sources (types), other

Online surveys of health care providers and patients

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