

# 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

### EU PAS number

EUPAS23142

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### Study ID

35382

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### DARWIN EU® study

No

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### Study countries

Finland

France

- Italy
  - Norway
  - United Kingdom
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### **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.

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### **Study status**

Finalised

## Research institutions and networks

### Institutions

Sanofi

**First published:** 01/02/2024

**Last updated:** 01/02/2024

Institution

## Contact details

### **Study institution contact**

Trial Transparency Team Contact-US@sanofi.com

**Study contact**

[Contact-US@sanofi.com](mailto: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

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### **Study start date**

Planned: 01/12/2018

Actual: 01/11/2018

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### **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

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### **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

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**Study type:**

Non-interventional study

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**Scope of the study:**

Effectiveness study (incl. comparative)

**Data collection methods:**

Primary data collection

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**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

**Medicinal product name**

FABRAZYME

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

AGALSIDASE BETA

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**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
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### **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)
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### **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).
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### **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)

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## 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

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### **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

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### **Check completeness**

Unknown

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### **Check stability**

Unknown

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### **Check logical consistency**

Unknown

## Data characterisation

### **Data characterisation conducted**

No