1 ABSTRACT

Title

Fabrazyme (agalsidase beta) home infusion educational materials effectiveness evaluation: a survey of healthcare providers and patients / caregivers.

Keywords

Fabrazyme; home infusion; educational materials; effectiveness

Rationale and background

Fabrazyme (agalsidase beta) is a drug used to treat Fabry Disease. As part of the Fabrazyme risk management plan (RMP), educational materials for home infusion therapy have been developed to serve as resources for both treating healthcare providers (HCPs) and patients.

The Pharmacovigilance Risk Assessment Committee (PRAC) requested a study to measure the effectiveness of the Fabrazyme educational materials on home infusion. To comply with this request, two surveys were conducted among 1) HCPs and 2) patients and/or patient caregivers.

Research question and objectives

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

Cross-sectional survey to be completed independently by HCPs and patients / caregivers.

Subjects and study size

The study was conducted among two populations:

1. HCPs who prescribe, monitor, oversee the management, and / or provide in person medical supervision of patients on Fabrazyme home infusion (Treating physicians

and/or infusion nurses) across 5 European Union (EU) countries (France, United Kingdom (UK), Italy, Finland, Norway)
2. Patients (and or caregivers looking after patients) who receive Fabrazyme by home infusion across 3 EU countries (France, UK and Italy). Attempts were made to contact patients / caregivers in Finland and Norway but none were successfully recruited

The survey aimed to recruit a total sample of 34 HCPs (physicians and nurses) and 18 Fabry patients (or their caregivers). The final number of respondents recruited was 34 HCPs (25 physicians and 9 nurses) and 14 Fabry patients / caregivers (12 patients and 2 caregivers). [redacted]

Variables and data sources

Data were collected via an initial screener survey followed by a self-administered online survey questionnaire targeting either eligible HCPs or patients / caregivers. The screeners collected demographic and eligibility criteria data. The self-administered online survey questionnaires collected substantive data using the questions [redacted].

HCP survey:
- Key performance indicators; awareness and usefulness of materials
- Use of educational materials for evaluating and selecting patients for home infusion and training
- Handling Adverse Reactions (ARs)
- Implementation of the logbook.

Patient / caregiver survey:
- Material use and distribution and level of nurse involvement in the infusion
- Usefulness and understanding of educational materials
- Knowledge and behavior when preparing and administering Fabrazyme
- Implementation of the logbook
- Handling ARs

A **positive outcome** reported across key pre-defined questions for each of these objectives would support the effectiveness of the educational material. For this assessment, a positive outcome was defined as at >70% of the survey participants providing a 'correct' response [redacted] The 70% cut point is based on previous experience from other surveys evaluating the effectiveness of educational materials.

Results

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

HCP
Positive outcomes (i.e. >70% of HCPs providing a "correct response") were reported in 1 / 5 key areas that were being assessed for the HCP educational materials. This was:

- If the materials are being received and read

The 4 key areas where positive outcomes were not reported were:

- The level of training given to patients / caregivers
- Use of materials in evaluation of home infusion
- Knowledge on actions taken in case of infusion-associated reaction (IAR) and adverse event (AE)
- Implementation of the logbook

Patient / caregiver
Positive outcomes (i.e. >70% of patients / caregivers providing a "correct response") were not reported in any of the 4 key areas under assessment for the patient / caregiver educational materials. They were:

- If the materials are being received, read and referred to
- Knowledge on preparation of infusion
- Knowledge on actions taken in case of AE
- Implementation of the logbook

[redacted]

Discussion:

The materials are being distributed to a sufficient number of HCPs based on statistical considerations and read by those that do receive them. However, the materials are not always being used to assess eligibility for home infusion and an insufficient number of HCPs report that patients need to have received Fabrazyme several months prior to moving to home infusion. The results highlighted that HCPs in UK and France were least likely to use the educational materials for assessing a patient's eligibility for home infusion. These results have lowered the overall combined scores across the markets, however, due to the small base sizes these are only directional differences.

HCPs are using the materials when training patients / caregivers, however, are not covering all the main aspects of home infusions, the main area that is not being covered is on patient or caregiver completion of the logbook.

An insufficient proportion of HCPs know all of the correct steps to take in the event of an IAR, almost a quarter of HCPs said the infusion should be completed instead of being discontinued. HCPs frequently check the logbook and document changes but do not frequently review the logbook with the patient / caregiver. The results highlighted that HCPs in France were least aware of the correct steps to take in the event of an IAR. These results have lowered the overall combined scores across the markets, however, due to the small base sizes these are only directional differences.

The materials are not being distributed by HCPs to a sufficient number of patients / caregivers, based on statistical considerations, and are not being referred to frequently enough during home infusions.

However, those patients / caregivers that receive the materials are reading them. There is a need to improve the training that patients / caregivers are given regarding the correct procedure for preparing an infusion as well as the proper action in the event of an AE. The logbook is being implemented to record details around the infusion process but it is not always being taken by the patient to appointments with their physician.

The results highlighted that patients / caregivers in the UK were least likely to have received or be referring to the educational materials during home infusions. These results have lowered the overall combined scores across the markets, however, due to the small base sizes these are only directional differences.

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