091301: FEIBA NF GLOBAL OUTCOME STUDY (FEIBA-GO) (091301: FEIBA-GO)

First published: 24/06/2014

Last updated: 22/02/2024

Study Finalised

Administrative details

PURI

https://redirect.ema.europa.eu/resource/43046

EU PAS number

EUPAS6691

Study ID

43046

DARWIN EU® study

No

Study countries

- ☐ France
- ☐ Germany
- ☐ Hungary

- [ ] Italy
- [ ] Norway
- [ ] Poland
- [ ] Portugal
- [ ] Russian Federation
- [ ] Spain
- [ ] United Kingdom

Study description

The study addresses the need to measure long-term effectiveness, safety and quality of life outcome measures for haemophilia A or B patients with high-responding inhibitors treated on-demand and in prophylaxis with FEIBA NF. The purpose of the study is to document the natural history of hemophilia A or B disease in subjects with high responding inhibitors either to Factor VIII or Factor IX and to describe long-term outcomes in terms of effectiveness, safety and quality of life in subjects receiving FEIBA NF in routine clinical practice.

Study status

Finalised

Research institutions and networks

Institutions

Shire

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Institution

Multiple centres: 40 centres are involved in the study

Contact details

Study institution contact

Study Contact Shire

Study contact

clinicaltransparency@shire.com

Primary lead investigator

Study Contact Shire

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 30/09/2013

Actual: 30/09/2013

Study start date

Planned: 30/06/2014

Actual: 03/09/2014

Data analysis start date
Planned: 28/02/2020

Actual: 28/02/2020

Date of final study report
Planned: 30/10/2020

Actual: 26/11/2020

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Baxalta Innovations GmbH, now part of Shire

Study protocol

091301-protocol-original-redact.pdf(964.17 KB)

091301-protocol-amendment 2-redact.pdf(1017.02 KB)

Regulatory

Was the study required by a regulatory body?

No

Is the study required by a Risk Management Plan (RMP)?

Not applicable

Methodological aspects

Study type

Study topic:

Human medicinal product

Disease /health condition

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Effectiveness study (incl. comparative)

Safety study (incl. comparative)

Other

If ‘other’, further details on the scope of the study

Pharmacodynamic study, Health-Related Quality of Life Analysis

Data collection methods:

Primary data collection

Main study objective:

The primary objective of the study is to describe the hemostatic effectiveness of FEIBA NF in a variety of clinical settings including on-demand therapy, prophylaxis and immune tolerance induction (ITI) in haemophilia A or B patients with high-responding inhibitors.

Study Design

Non-interventional study design

Cohort

Other

Non-interventional study design, other

Post-authorization, prospective, uncontrolled, observational, non-interventional, open-label, multicenter cohort study

Study drug and medical condition

Name of medicine, other

FEIBA NF

Additional medical condition(s)

Haemophilia A or B with high responding inhibitors

Population studied

Short description of the study population

Patients were male hemophilia A or B patients with high-responding inhibitors who had been prescribed FEIBA for the treatment or prevention of bleeding events by a treating physician prior to the decision to enroll in the study. No additional diagnostic or monitoring procedures were applied to patients, except those that were part of normal/routine clinical practice.

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

Hemophilia A or B patients

Estimated number of subjects

55

Study design details

Outcomes

(A) 1) Prophylaxis: Treatment of breakthrough bleeds 2) On-demand: Treatment of BEs: -Bleed rates -Types of bleeding events -Number of BEs with corresponding hemostatic efficacy ratings(B) 1) Prophylaxis: Totals & breakthrough bleeds 2) On-demand: -Number of infusions- Weight adjusted dose- Total units of FEIBA NF infused, 1) Joint clinical outcomes in routine clinical practice setting, using any therapeutic regimen, assessed as in common practice2) Health-Related Quality of Life using standardized assessment questionnaires/ tools

Data analysis plan

Descriptive statistics will include specifically but not exclusively, arithmetic mean, standard deviations, medians, minimum, maximum, 25th and 75th percentiles, proportions, frequency counts and 95% confidence intervals of

select point estimates. Figures will be prepared to illustrate the patterns of data over time where appropriate.

Documents

Study results

091301-clinical-study-report-redact.pdf(812.48 KB)

Data management

Data sources

Data sources (types)

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