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

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 clinicaltransparency@shire.com

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 type list

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

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

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