A Post authorization safety surveillance registry with BeneFIX in hemophilia B patients in usual care settings

First published: 04/03/2014

Last updated: 23/04/2024

Study Finalised

Administrative details

EU PAS number

EUPAS5890

Study ID

26423

DARWIN EU® study

No

Study countries

China

Study description

This is a non-interventional,voluntary prospective registry study conducted in major hemophilia treatment centers in China. The objective of this registry is to evaluate the safety and efficacy of BeneFIX in hemophilia B patients in the Chinese population. It will enroll Chinese patients with Hemophilia B of all severities. Enrolled subjects will be treated with intravenous infusions of BeneFIX at a dose and frequency prescribed by the subject’s treating physician in accordance with the BeneFIX label and will be adjusted solely according to medical and therapeutic necessity. The registry study will capture observations that will be used for evaluating recombinant FIX replacement product safety, including: subject demographics, medical history, hemophilia history and medications. Safety assessments, treatment data and any laboratory-based FIX inhibitor determinations will be collected at all visits. All subjects in this registry will be followed for 1 year.

Study status

Finalised

Research institutions and networks

Institutions

Institute of Hematology and Hospital of Blood Diseases, Chinese Academy of Medical Sciences

Multiple centres: 16 centres are involved in the study

Contact details

Study institution contact

Pablo Rendo pablo.rendo@pfizer.com

Study contact

pablo.rendo@pfizer.com

Primary lead investigator

Pablo Rendo

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 16/01/2013

Actual: 15/01/2015

Study start date

Planned: 16/08/2014

Actual: 23/01/2015

Data analysis start date

Planned: 29/08/2016

Actual: 13/10/2015

Date of interim report, if expected

Planned: 27/01/2015

Actual: 10/03/2016

Date of final study report
Planned: 12/12/2016

Actual: 19/12/2016

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Pfizer

Study protocol

A Post authorization safety surveillance registry with BeneFIX in hemophilia B patients in usual care settings.pdf (383.42 KB)

Administrative Amendment.pdf (436.98 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)

Methodological aspects

Study type

Study type list

Study topic:

Disease /health condition

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Effectiveness study (incl. comparative)

Data collection methods:

Primary data collection

Main study objective:

Primary study objective is to evaluate the product medically important events (FIX inhibitor development, FIX hypersensitivity allergic reaction, thrombogenicity, lack of effect and red blood cell RBCagglutination) in Chinese hemophilia B patients during treatment with BeneFIX.

Study Design

Non-interventional study design

Other

Non-interventional study design, other

This is a non-interventional, voluntary prospective registry study conducted in major hemophilia treatment centers in China.

Study drug and medical condition

Medicinal product name

BENEFIX

Medical condition to be studied

Haemophilia B without inhibitors

Population studied

Short description of the study population

Any patient with Hemophilia B in China who is eligible and willing to participate in the registry.

Patients must meet all of the following inclusion criteria to be eligible for inclusion in the study:

1. Male subjects of all ages and severity with Hemophilia B.
2. Subjects using or intending to use BeneFIX for Factor IX replacement therapy.
3. Subjects/parents/legal representatives must be able to comply with registry procedures (informed consent/assent process, clinical visits, reporting of infusion and bleed data, reporting of adverse events, etc).
4. Evidence of a personally signed and dated informed consent document indicating that the patient (or a legally acceptable representative) has been informed of all pertinent aspects of the study.

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

Hepatic impaired

Renal impaired

Estimated number of subjects

60

Study design details

Outcomes

The primary outcomes are development of clinically significant FIX inhibitors, FIX hypersensitivity allergic reactions, thrombogenicity, lack of effect and red cell agglutination. Secondary safety outcomes include the frequency of AEs and SAEs,etc.Secondary efficacy outcomes will be:Annualized bleeding rates in subjects receiving treatment with BeneFIX,The responses to all on-demand treatment with BeneFIX for all bleeds,Number of BeneFIX infusions to treat each new bleed,Number of spontaneous/non traumatic breakthrough bleeds within 48 hours of a prophylaxis dose of BeneFIX

Data analysis plan

All safety and efficacy analyses, except the analysis for Annualized Bleeding Rates(ABR),will be performed on all subjects who receive at least one dose of BeneFIX. ABR analysis will be performed on subjects who participated in prophylaxis period for at least 6 months. The efficacy results of this study will be presented using descriptive statistics. The following descriptive statistics will

be used:1)For numeric endpoints: sample size, mean, standard deviation, median, minimum and maximum,2)For categorical endpoints: count and percentage.Safety data will be tabulated and listed according to Pfizer’s standard reporting algorithms. The reasons for discontinuation of BeneFIX therapy will be described.Subgroup analysis will be done by several sub-populations.

Documents

Study, other information

ANNEX 1 Subject Diary.pdf (175.88 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.

Signed checklist for study protocols

ANNEX 2 ENCePP checklist.pdf (1.62 MB)

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

Unknown