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

**First published:** 04/03/2014

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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.

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**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

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**Study start date**

Planned: 16/08/2014

Actual: 23/01/2015

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**Data analysis start date**

Planned: 29/08/2016

Actual: 13/10/2015

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**Date of interim report, if expected**

Planned: 27/01/2015

Actual: 10/03/2016

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**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

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**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

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**Study type:**

Non-interventional study

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**Scope of the study:**

Assessment of risk minimisation measure implementation or effectiveness

Effectiveness study (incl. comparative)

**Data collection methods:**

Primary data collection

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**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

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**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

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**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.

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**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