EUropean REgistry in Children below six years of age treated with BeneFIX (EUREKIX)

First published: 08/04/2013

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Study Finalised

Administrative details

EU PAS number

EUPAS3788

Study ID

25611

DARWIN EU® study

No

Study countries

- Belgium
- Denmark
- Finland
- France

Italy
Netherlands
Spain
Sweden
United Kingdom

Study description

This is a two phase, non-interventional, multicenter trial including a retrospective (Phase I) and/or prospective (Phase II) data collection period. Retrospective data will be collected only if patients have been treated with BeneFIX for at least 12 months ahead of the inclusion in the study. In order to ensure consistent data quality, retrospective documentations must not cover a time period longer than 8 years ago i.e. if a patient is 8 years of age, his treatment with BeneFIX between 0-6 years of age may still be retrospectively documented.Prospective data will be collected if patients will be able to follow 12 to 24 months of treatment with BeneFIX before they reach 6 years of age.

Study status

Finalised

Research institutions and networks

Institutions

Great Ormond Street Hospital for Children NHS Foundation Trust

Multiple centres: 40 centres are involved in the study

Contact details

Study institution contact

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Study contact

Ri.Liesner@gosh.nhs.uk

Primary lead investigator

Ri Liesner

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 30/11/2011

Actual: 30/11/2011

Study start date

Planned: 15/12/2012

Actual: 12/07/2013

Data analysis start date

Planned: 31/08/2016

Actual: 16/03/2017

Date of final study report
Planned: 31/03/2017

Actual: 28/03/2018

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Pfizer

Study protocol

EUREKIX - approved ammended protocol+EU PAS registry number_15Apr2013.pdf (460.08 KB)

B1821046 - EUREKIX - NIS Amendement 2 21 Mar 2016.pdf (353.03 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:

Disease /health condition

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Other

Safety study (incl. comparative)

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

Efficacy of BeneFIX in children below 6 years of age

Data collection methods:

Secondary use of data

Main study objective:

The objective of the study is to collect data in Europe regarding safety (primary endpoint) and efficacy (secondary endpoint) of treatment with rFIX (BeneFIX®) in children below 6 years of age treated in the routine clinical setting

Study Design

Non-interventional study design

Other

Non-interventional study design, other

This is a two phase, non-interventional, multicenter trial including a retrospective (Phase I) and/or prospective (Phase II) data collection period.

Study drug and medical condition

Medical condition to be studied

Haemophilia B with anti factor IX

Population studied

Short description of the study population

Patients were eligible to take part in the retrospective data collection if they have been treated with BeneFIX for at least 12 months at an age below 6 years and are at time of consent not older than 8 years. Patients were eligible to participate in the prospective part of the study if they are able to accrue at least 12 months of data in the study before reaching the age of 6 years. The maximum time of prospective observation is 24 months.

Age groups

- Children (2 to < 12 years)

Special population of interest

Other

Special population of interest, other

Haemophilia B patients

Estimated number of subjects

50

Study design details

Data analysis plan

The efficacy of BeneFIX® will be descriptively assessed by different measurement parameters: –Annualized bleeding rates (ABRs) –Responses to the on-demand and prophylactic treatment with BeneFIX, respectively, for all bleeds and according to bleeding location (4-point scale of assessment: excellent, good, moderate, no response) –The incidence of less-than-expected therapeutic effect (LETE) will be assessed by the investigator using the criteria listed in Section 8.7.2.3. –Lack of effect, defined as the failure of expected pharmacologic action or therapeutic benefitIn the event of a bleed in the on-demand setting (including those occurring during the prophylaxis period), the 4-point response scale for an on-demand treatment of a bleeding episode is defined as follows: •Excellent•Good•Moderate•No Response

Documents

Study results

EUREKIX (DZR020)_CSR_Final Version_18 Sept18.pdf (1.58 MB)

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

Retrospective and Prospective patient-based data collection.Data will be collected from the patient’s treatment records and from their treatment diaries. All data collected have been assessed in routine clinical practice. Due to the non-interventional nature of this study, no additional visits or procedures are requested for the study.

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