An Observational Post-authorization Long-term Follow-up Study to Characterize the Effectiveness and Safety of HEMGENIX® (Etranacogene Dezaparvovec) in Patients with Hemophilia B (CSL222_4001)

First published: 15/08/2023

Last updated: 10/06/2024

Study Planned

Administrative details

PURI

https://redirect.ema.europa.eu/resource/106067

EU PAS number

EUPAS106066

Study ID

106067

DARWIN EU® study

No

Study countries

- [ ] Austria
- [ ] Belgium
- [ ] Denmark
- [ ] Finland
- [ ] France
- [ ] Germany
- [ ] Ireland
- [ ] Italy
- [ ] Netherlands
- [ ] Norway
- [ ] Spain
- [ ] Sweden
- [ ] Switzerland
- [ ] United Kingdom
- [ ] United States

Study description

This observational, post-authorization, long-term follow-up study aims to investigate the short and long-term effectiveness and safety of HEMGENIX in patients with hemophilia B. The study will also include a cohort of patients with hemophilia B treated with FIX prophylaxis to enable interpretation of relevant efficacy and safety findings of HEMGENIX.

Study status

Planned

Research institutions and networks

Institutions

CSL Behring

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Institution

Contact details

Study institution contact

Study Registration Coordinator

Study contact

clinicaltrials@cslbehring.com

Primary lead investigator

Study Registration Coordinator

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 15/08/2023

Study start date

Planned: 15/09/2023

Data analysis start date

Planned: 24/08/2043

Date of final study report
Planned: 15/01/2044

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

CSL Behring

Regulatory

Was the study required by a regulatory body?

Yes

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

EU RMP category 1 (imposed as condition of marketing authorisation)

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Effectiveness study (incl. comparative)

Main study objective:

The primary objective is: To investigate the short and long-term effectiveness profile of HEMGENIX by following adults with hemophilia B who are treated with HEMGENIX or are on continuous FIX prophylaxis for a period of 15 years.

Study Design

Non-interventional study design

Cohort

Other

Non-interventional study design, other

Observational, post-authorization, long-term follow-up study

Study drug and medical condition

Name of medicine

HEMGENIX

Name of medicine, other

FIX prophylaxis

Study drug International non-proprietary name (INN) or common name

ETRANACOGENE DEZAPARVOVEC

Anatomical Therapeutic Chemical (ATC) code

(B02BD) Blood coagulation factors

Blood coagulation factors

Medical condition to be studied

Factor IX deficiency

Additional medical condition(s)

Hemophilia B

Population studied

Age groups

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)

Estimated number of subjects

500

Study design details

Outcomes

Bleeding Rate,

Efficacy: Bleeding Rate, FIX activity, Annualized consumption of FIX replacement therapy, Number of patients remaining free of previous continuous routine prophylaxis, Target joints

Safety: Incidence of related SAEs during 15 years of follow-up, Incidence of AESIs during 15 years of follow-up

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Data analysis plan

Detailed description of the statistical analyses is included in the Statistical analysis plan (SAP) document. Formal hypothesis testing is not planned for this study, and therefore no control for type I error will be done for either primary or secondary endpoint analysis. The efficacy of interest of the study is to estimate the ABR after HEMGENIX treatment (full dose or partial dose), regardless of additional FIX replacement therapy used for bleeding count. Five half-lives of FIX time will be excluded from the time at risk. Baseline demographics and characteristics will be summarized for both the HEMGENIX and FIX Prophylaxis cohorts of the study using descriptive statistics (number of observations in each group, mean, standard deviation, median, 1st quartile, 3rd quartile). No statistical comparisons are planned between the 2 cohorts. No case-matched controlling will be performed between the HEMGENIX and FIX Prophylaxis cohorts for this study.

Data management

Data sources

Data source(s), other

ATHN Transcends: A Natural History Study of Non-Neoplastic Hematologic Disorders (Hemophilia Cohort, Gene Therapy Outcomes Arm and Natural History Arm), United States

Data sources (types)

Disease registry

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