World Federation of Hemophilia Gene Therapy Registry (WFH GTR)

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Study Planned

Administrative details

PURI

https://redirect.ema.europa.eu/resource/48684

EU PAS number

EUPAS48683

Study ID

48684

DARWIN EU® study

No

Study countries

- ☐ Australia
- ☐ Austria
- ☐ Belgium

- [ ] Canada
- [ ] Denmark
- [ ] France
- [ ] Germany
- [ ] Greece
- [ ] Ireland
- [ ] Israel
- [ ] Italy
- [ ] Japan
- [ ] Netherlands
- [ ] Saudi Arabia
- [ ] Spain
- [ ] Sweden
- [ ] Switzerland
- [ ] Türkiye
- [ ] United Kingdom
- [ ] United States

Study description

The primary objective of the GTR is to determine the long-term safety of factor VIII and factor IX gene therapies in PWH. Secondary objectives are to determine the long-term efficacy and the durability of factor VIII and factor IX gene therapies in PWH and to assess long-term quality of life and burden of disease post gene-therapy infusion.

Study status

Planned

Research institutions and networks

Institutions

World Federation of Hemophilia (WFH)

Canada

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Institution | Not-for-profit

Contact details

Study institution contact

Donna Coffin

Study contact

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Primary lead investigator

Donna Coffin

Primary lead investigator

Study timelines

Date when funding contract was signed

Actual: 28/02/2022

Study start date

Planned: 01/12/2022

Date of final study report
Planned: 30/11/2037

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Biomarin, Pfizer, CSL Behring, Spark, Takeda

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 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 of the WFH GTR is to determine the long-term safety of factor VIII and factor IX gene therapies in patients with hemophilia. The secondary objectives of the WFH GTR are to determine the long-term efficacy and the durability of factor VIII and factor IX gene therapies in patients with hemophilia and to assess long-term quality of life.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medical condition to be studied

Haemophilia

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

The primary outcome/endpoint is safety events over the long-term. The secondary outcomes/endpoints are efficacy and durability of efficacy of factor VIII and factor IX gene therapies in patients with hemophilia

Data analysis plan

The WFH GTR study is a prospective, observational, and longitudinal registry of patients diagnosed with hemophilia, who have received gene therapy for hemophilia. The primary objective is to determine the long-term safety of factor VIII and factor IX gene therapies. Safety will be analyzed from adverse events (AE) and mortality using Kaplan-Meier plots. The secondary objectives are to determine the long-term efficacy and the durability of factor VIII and factor IX gene therapies. Bleeding rate and plasma factor activity level will be assessed together with long-term quality of life. Bleeding event rate will be calculated as events by patient-year(s) as number of incident cases divided by the amount of person-time at risk. Median time to occurrence of each bleed will be calculated using Kaplan-Meier plots. For long-term quality of life, changes in the composite health related quality of life scores will be reported as mean, median, standard deviation and interquartile range.

Data management

Data sources

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