GENEr8-COAS: A Non-Interventional, Multi-National, Longitudinal Study of Patients Treated with ROCTAVIAN™ (valoctocogene roxaparvovec) (GENEr8-COAS Observational Cohort Study)

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Study Planned

Administrative details

EU PAS number

EUPAS49071

Study ID

49072

DARWIN EU® study

No

Study countries

- ☐ Germany
- ☐ Italy

United States

Study description

This study is a non-interventional, multi national, longitudinal cohort study. The study will enroll patients diagnosed with HA and treated with ROCTAVIAN™. Patients will be offered the opportunity to enroll in this study only after a clinical decision has been made that they will receive ROCTAVIAN™. Only subjects who are eventually administered ROCTAVIAN™ will be included in the analysis. The study will enroll subjects until the total number of 200 planned subjects administered ROCTAVIAN™ has been reached. Data will be collected directly from subjects and health care providers as part of routine clinical practice. Where possible to avoid duplication of entry, necessary data may be extracted from relevant electronic health record systems where variables match, however, the source data will remain the initial written case history. Bleed events, FVIII levels, exogenous factor and non-factor replacement treatments, and COAs (e.g. PROs and HJHS) will be utilized to describe effectiveness. Measurements of effectiveness endpoints at or prior to, the index date (i.e. baseline data) will be utilized to quantify changes over time, as warranted. SAEs (regardless of causality or assessed relatedness), suspected ADRs of any type, and TAEs of hepatotoxicity, thromboembolic events, infusion reactions (including hypersensitivity), new malignancies, and development of FVIII inhibitors, during or after administration of ROCTAVIAN™ (index date) will be collected to describe safety endpoints. The end of the study is defined as the date on which the last subject is assessed for the purposes of the final collection of data for the primary endpoints, appropriate follow-up of safety events as required by applicable regulations and described herein notwithstanding.

Study status

Planned

Research institutions and networks

Institutions

BioMarin Pharmaceuticals

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Institution

Contact details

Study institution contact

270-601 Program Director medinfo@bmrn.com

Study contact

medinfo@bmrn.com

Primary lead investigator

270-601 Program Director

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 15/12/2022

Study start date

Planned: 13/09/2023

Data analysis start date
Planned: 01/01/2027

Date of interim report, if expected
Planned: 30/06/2027

Date of final study report
Planned: 30/12/2042

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

BioMarin Pharmaceutical Inc.

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)

Regulatory procedure number

EMA/H/C/005830

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Scope of the study:

Effectiveness study (incl. comparative)

Main study objective:

To describe the bleeding profile and long-term durability of coagulation factor VIII (FVIII) expression in subjects administered with ROCTAVIAN™

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medicinal product name

ROCTAVIAN

Medical condition to be studied

Factor VIII deficiency

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

200

Study design details

Outcomes

Annualized Bleeding Rate (treated and all bleeds) Percentage of subjects with zero (0) bleeds (treated and all bleeds), by treatment duration Change over time in FVIII activity levels Use of exogenous factor and non-factor replacement treatment Change in clinical outcome assessments over time. Proportion, event rate, and incidence rate of SAEs. Proportion, event rate, and incidence rate of suspected ADRs Proportion, event rate, and incidence rate of TAEs of hepatotoxicity, thromboembolic events, infusion reactions (including hypersensitivity), new malignancies, and development of FVIII inhibitors

Data analysis plan

Due to the observational nature of this study and inclusion criteria, results will represent a broad range of real-world practice, which may reflect or be influenced by site heterogeneity (e.g. clinic structure, local clinical practice)), differences in clinical practice and underlying differences in the patient population. Consistent and thorough data collection should allow results to be described in such a way that these differences can be characterized, analyzed, and reported. Full analysis will be described in the SAP.

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

Prospective patient-based data collection, Retrospective 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