GENEr8-GTR: A Retrospective Cohort Study of Patients Treated with ROCTAVIAN™ (valoctocogene roxaparvovec): An Analysis of Patient Registries (BMN 270-801)

First published: 15/11/2022

Last updated: 23/04/2024

Study | Ongoing

Administrative details

EU PAS number

EUPAS49243

Study ID

49244

DARWIN EU® study

No

Study countries

- ☐ Austria
- ☐ Belgium
- ☐ Brazil

- [ ] Bulgaria
- [ ] Croatia
- [ ] Cyprus
- [ ] Czechia
- [ ] Denmark
- [ ] Finland
- [ ] France
- [ ] Germany
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- [ ] Ireland
- [ ] Italy
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- [ ] Netherlands
- [ ] Norway
- [ ] Poland
- [ ] Portugal
- [ ] Romania
- [ ] Russian Federation
- [ ] Slovakia
- [ ] Slovenia
- [ ] Spain
- [ ] Sweden
- [ ] Switzerland
- [ ] Türkiye
- [ ] United Kingdom
- [ ] United States

Study description

ROCTAVIAN™ is a commercially available gene therapy for the treatment of patients with hemophilia A (HA). As gene therapies of this type remain novel, long-term follow-up data are limited. This study is being undertaken to better characterize the long-term effectiveness and safety outcomes of patients treated with ROCTAVIAN in a real-world setting based on the safety profile outlined in the Pharmacovigilance and Risk Management Plan, based on the data collected in the World Federation of Hemophilia Gene Therapy Registry (WFH GTR) for people with HA (PwHA) administered ROCTAVIAN in a real-world setting.

Study status

Ongoing

Research institutions and networks

Institutions

BioMarin Pharmaceuticals

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Last updated: 01/02/2024

Institution

Contact details

Study institution contact

270-801 Program Director medinfo@bmrn.com

Study contact

medinfo@bmrn.com

Primary lead investigator

270-801 Program Director

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 15/12/2022

Study start date

Planned: 30/09/2023

Actual: 30/10/2023

Data analysis start date

Planned: 30/09/2023

Actual: 30/10/2023

Date of interim report, if expected

Planned: 30/06/2029

Date of final study report

Planned: 30/06/2044

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

BioMarin International Ltd.

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)

Safety study (incl. comparative)

Main study objective:

To characterize the long-term effectiveness and safety outcomes of patients treated with ROCTAVIAN in a real-world setting.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medicinal product name

ROCTAVIAN

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

VALOCTOCOGENE ROXAPARVOVEC

Medical condition to be studied

Factor VIII deficiency

Additional medical condition(s)

Hemophilia A

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

720

Study design details

Outcomes

Targeted adverse events (TAEs) of hepatoxicity, thromboembolic events, infusion reactions, new malignancies, and development of FVIII inhibitors. Suspected adverse drug reactions, bleeding events, FVIII expression, use of haemostatic medications, and patient reported quality of life.

Data analysis plan

Full statistical methods and data specifications will be detailed further in an SAP. Analysis population will include: Safety Analysis Population: patients administered ROCTAVIAN and in the WFH GTR. Outcomes Analysis Population: patients administered ROCTAVIAN in the WFH GTR, and with data collected on the specific endpoint of interest captured in the WFH GTR database. Summary statistics (annual and overall) of the proportion, event rate, and incidence rate of the TAEs, and the event rate and/or incidence of suspected ADRs among all patients administered ROCTAVIAN will be provided. Summary statistics of annualised bleeding rate for treated bleeds and percentage of patients with zero (0) bleeds by treatment duration, FVIII level change over time, use of exogenous factor and non-factor replacement treatment(s) and change in EQ-

5D-5L will be described (as allowed by the data).

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 source(s)

The World Federation of Hemophilia Gene Therapy Registry

Data source(s), other

- American Thrombosis and Hemostasis Network (ATHN), United States
- EUHASS - Blood disorders

Data sources (types)

Disease registry

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