Comparative Effectiveness of Roctavian to Standard of Care

First published: 03/11/2026

Last updated: 03/11/2026

Study Discontinued

Administrative details

EU PAS number

EUPAS1000001075

Study ID

1000001075

DARWIN EU® study

No

Study countries

Germany

Study description

Comparative Effectiveness of Roctavian to Standard of Care Hemostatic Therapies in Germany Among People with Severe Hemophilia A: A Prospective Non-Interventional Study Utilizing Data Collected in the German Hemophilia

Register.

Study status

Discontinued

Research institutions and networks

Institutions

IQVIA

United Kingdom

First published: 12/11/2021

Last updated: 22/04/2024

Institution | **Non-Pharmaceutical company** | **ENCePP partner**

Contact details

Study institution contact

Program 270-603 Director medinfo@bmrn.com

Study contact

medinfo@bmrn.com

Primary lead investigator

Program 270-603 Director

Primary lead investigator

Study timelines

Date when funding contract was signed
Planned: 01/10/2024

Actual: 01/01/2025

Study start date
Planned: 09/01/2025

Actual: 09/01/2025

Data analysis start date
Actual: 07/09/2025

Date of final study report
Planned: 08/06/2026

Actual: 08/06/2026

Sources of funding

- Pharmaceutical company and other private sector

Study protocol

270-603 Protocol with Appendices.pdf (1.33 MB)

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 topic:

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Effectiveness study (incl. comparative)

Evaluation of patient-reported outcomes

Safety study (incl. comparative)

Data collection methods:

Secondary use of data

Study design:

Prospective non-interventional study

Main study objective:

• Primary objective: To compare the annualized bleeding rate for treated bleeds.

• Secondary objectives:

o To compare the annualized bleeding rate for major, life threatening, and joint bleeds.

o To compare the proportion of people with zero bleeds for treated, major, life threatening, and joint bleeds.

o To compare the use of hemostatic medications.

o To compare joint health, quality of life, and pain.

o To compare safety events of interest.

o To describe time to resumption of prophylactic treatment among persons administered Roctavian.

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

Anatomical Therapeutic Chemical (ATC) code

(B02BD15) valoctocogene roxaparvovec

valoctocogene roxaparvovec

Medical condition to be studied

Haemophilia A without inhibitors

Population studied

Short description of the study population

Adult PwSHA without a history of (or current) inhibitors to FVIII in Germany

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Age groups

- **Adult and elderly population (≥18 years)**
  - Adults (18 to < 65 years)
    - Adults (18 to < 46 years)
    - Adults (46 to < 65 years)
  - Elderly (≥ 65 years)
    - Adults (65 to < 75 years)
    - Adults (75 to < 85 years)
    - Adults (85 years and over)

Special population of interest

Other

Special population of interest, other

Males with Haemophilia A

Estimated number of subjects

300

Study design details

Setting

Germany; Data collection beginning August 30, 2024

Comparators

Standard of Care (SoC) hemostatic prophylaxis treatments

Outcomes

• Bleeding Events

• SoC Hemostatic Treatment Usage

• Clinical Outcome Assessment Tools

o Hemophilia Quality of Life assessment (Haemo-QoL-A)

o Hemophilia Joint Health Score (HJHS)

o Brief Pain Inventory-Short Form (BPI-SF)

o Safety Events

Data analysis plan

Comparison of outcomes were planned between the Roctavian cohort and SoC cohort after propensity score adjustment for differences in baseline characteristics (e.g., demographic and clinical variables recorded in the DHR prior to the index date). Due to the small sample sizes, no comparative analyses were conducted.

Summary results

Uptake and enrollment in 270-603 was <10% of the sample size required per the sample size calculation conducted by the Institute for Quality and Efficiency in Health Care (IQWiG) and detailed in the study protocol, at the time of study closure. The limited uptake and small sample size constrains interpretability of the results and indicated that the study was futile in providing meaningful data on the comparative effectiveness of Roctavian to SoC hemostatic prophylaxis after the AbD specified recruitment period.

Documents

Study report

270-603 Final Report.pdf (1.01 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 source(s)

Deutsches Hämophilieregister

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