NN7088-4029 A multinational, prospective, open labelled, non-controlled, non-interventional post-authorisation study of turoctocog alfa pegol (N8-GP) during long-term routine prophylaxis and treatment of bleeding episodes in patients with haemophilia A (pathfinder 9)

First published: 21/09/2020

Last updated: 08/01/2025

Study Ongoing

Administrative details

PURI

https://redirect.ema.europa.eu/resource/39327

EU PAS number

EUPAS36536

Study ID

39327

DARWIN EU® study

No

Study countries

- [ ] Austria
- [ ] Croatia
- [ ] Czechia
- [ ] Estonia
- [ ] Germany
- [ ] Greece
- [ ] Hungary
- [ ] Italy
- [ ] Lithuania
- [ ] Portugal
- [ ] Slovakia
- [ ] Slovenia
- [ ] Spain
- [ ] Switzerland

Study description

This study will collect information on side effects and how well Esperoct® (turoctocog alfa pegol (N8-GP)) works during long-term treatment (prophylaxis) in males with haemophilia A. Participants in this study will get the same treatment as they would normally get, if they were not participating in the study. All visits at the clinic are done in the same way as participants are used to, when visiting their doctor. During visits at the clinic participants might be asked for some relevant tests if considered useful by the study doctor. During the visits the study doctor might ask if participants had any side effects since the last study visit. Participants will be asked to note down in their own diary the number of bleeds and how these were treated, as well as their regular

prophylaxis. Participation in the study will last for about 5-7 years, depending on when participants join the study. Participants are free to leave the study at any time and for any reason. This will not affect their current and future medical care.

Study status

Ongoing

Research institutions and networks

Institutions

Novo Nordisk

First published: 01/02/2024

Last updated: 01/02/2024

Institution

Contact details

Study institution contact

Clinical Transparency and Medical Writing Office (1452)
Novo Nordisk A/S

Study contact

pactadmin@novonordisk.com

Primary lead investigator

Clinical Transparency and Medical Writing Office (1452)
Novo Nordisk A/S

Primary lead investigator

Study timelines

Date when funding contract was signed
Actual: 17/04/2020

Study start date
Planned: 01/10/2020

Actual: 23/10/2020

Date of final study report
Planned: 02/06/2028

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Novo Nordisk A/S

Study protocol

4029-protocol-eu-pas-reg-redacted.pdf(1.07 MB)

_ Protocol 4029 protocol eu-pas-reg redacted (1).pdf(715.55 KB)

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

EMEA/H/C/004883/0000

Other study registration identification numbers and links

UTN: U1111-1235-6007

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Safety study (incl. comparative)

Main study objective:

The primary objective of the study is to investigate the safety of N8-GP including the PEG moiety during prophylaxis and long-term use in patients with haemophilia A as prescribed by the physician.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

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

TUROCTOCOG ALFA PEGOL

Medical condition to be studied

Factor VIII deficiency

Population studied

Age groups

Term newborn infants (0 – 27 days)

Infants and toddlers (28 days – 23 months)

Children (2 to < 12 years)

Adolescents (12 to < 18 years)

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

60

Study design details

Outcomes

Number of Adverse Events (AEs) reported during the study period from inclusion of the patient until end of study, Number of Serious Adverse Events (SAEs) reported during the study period from inclusion of the patient until end of study

Data analysis plan

No formal testing of statistical hypothesis will be performed. All data will be presented using descriptive statistics. Categorial data will be summarized by frequency tables while continuous data will be summarized by mean, standard deviation, median, minimum and maximum value. Subgroup analysis will be presented: - By age groups (< 6 years, 6 to < 12 years, 12 to < 18 years, 18 to < 65 years, ≥ 65 years) - By severity of disease (moderate and severe) Patients who previously developed inhibitors before entering this study might be presented separately if deemed necessary.

Documents

Study report

4029 progress report eu-pas-reg 01 redacted.pdf(263.77 KB)

4029 progress report eu-pas-reg 02 redacted.pdf(476.39 KB)

Study, other information

4029 progress report eu-pas-reg 02 redacted.pdf(476.39 KB)

Data management

Data sources

Data sources (types)

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