NN7999-4031: A Non-Interventional Post-Authorisation Safety Study (PASS) in male haemophilia B patients receiving Nonacog Beta Pegol (N9-GP) prophylaxis treatment

First published: 27/11/2018

Last updated: 02/07/2024

Study Ongoing

Administrative details

EU PAS number

EUPAS26592

Study ID

42488

DARWIN EU® study

No

Study countries

- ☐ Austria
- ☐ Belgium
- ☐ Canada

- [ ] Denmark
- [ ] Germany
- [ ] Greece
- [ ] Netherlands
- [ ] Norway
- [ ] Portugal
- [ ] Spain
- [ ] Sweden
- [ ] Switzerland
- [ ] United Kingdom

Study description

This study will collect information on side effects and how well Refixia/REBINYN works during long-term treatment (prophylaxis) in males with haemophilia B. While taking part in this study, participants will receive the same treatment as given to them by their study doctor. All visits at the clinic are done in the same way as the participants are used to. During visits at the clinic, participants might be asked for some relevant tests if considered useful by their study doctor. During the visits, the participants study doctor might ask if the participants had any side effects since their last study visit. The participants will be asked to note down the number of bleeds and the treatment of their bleeds as well as their regular prophylaxis. During the visits to the clinic, the participants will be asked to answer some questionnaires about their quality of life and their ability to be physically active. The participant’s participation in the study will last for 4-9 years, depending on when they 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 Reporting Novo Nordisk

clinicaltrials@novonordisk.com

Study contact

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Primary lead investigator

Clinical Reporting Anchor and Disclosure (1452) Novo Nordisk A/S

Primary lead investigator

Study timelines

Date when funding contract was signed

Actual: 08/07/2015

Study start date
Planned: 01/04/2019

Actual: 01/04/2019

Date of final study report
Planned: 15/12/2028

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Novo Nordisk A/S

Study protocol

4031-protocol-eu-pas-reg-redacted.pdf (426.11 KB)

4031-protocol-eu-pas-reg-redacted-version-2.0.pdf (458.26 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)

Other study registration identification numbers and links

ClinicalTrials.gov identifier: NCT03745924WHO UTN number: U1111-1165-8657

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Drug utilisation

Effectiveness study (incl. comparative)

Main study objective:

The primary objective of the study is to investigate the safety of N9-GP in prophylaxis and during long-term routine use (Adverse Drug Reactions) in patients with haemophilia B in the manner it isprescribed by physicians. This will include assessment of specific pharmacological risks for FIX replacement products (FIX inhibitors, allergic reactions, and thromboembolic events).

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

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

NONACOG BETA PEGOL

Medical condition to be studied

Haemophilia B without inhibitors

Population studied

Age groups

- Adolescents (12 to < 18 years)
- Children (2 to < 12 years)
- Infants and toddlers (28 days – 23 months)
- Preterm newborn infants (0 – 27 days)
- Term newborn infants (0 – 27 days)
- 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

70

Study design details

Outcomes

Number of Adverse Drug Reactions (ADRs) (FIX inhibitors, allergic reactions, and thromboembolic events) reported during the study period, No.of SAEs.-No.of bleeding episodes,long-term routine N9-GP use assessed by ABR.-No.of treatment requiring bleeding episodes,long-term routine N9-GP use,ABR assessed.-Haemostatic effect of N9-GP for treatment assessed by success/failure on a 4-point scale.-Haemostatic response of N9-GP in perioperative management assessed as success/failure on a 4-point scale.-All reported for up to 9 years

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Data analysis plan

No formal testing of statistical hypotheses will be performed. Evaluation of data will be based upon descriptive statistics, i.e. summary tables, listings and figures. Categorical data will be summarised by frequency tables while continuous data will be summarized by mean, standard deviation, median, minimum and maximum value separated into age groups.

Documents

Study report

4031 regulatory progress report pass progress report no.04 eu-pas-reg redacted.pdf (429.16 KB)

4031-eu-pass-progress-report-20191206.pdf (102.31 KB)

4031EU pass progress report no.02.pdf (349.97 KB)

_ Public Registration of Results Regulatory pass progress report redacted.pdf (315.53 KB)

Study, other information

4031-eu-pass-progress-report-20191206.pdf (102.31 KB)

4031EU pass progress report no.02.pdf (349.97 KB)

_ Public Registration of Results Regulatory pass progress report redacted.pdf
(315.53 KB)

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

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