PASS Progress Report no. 04

Study ID: NN7999-4031

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

Non-interventional post authorisation safety study (PASS)

Title page

Study ID	NN7999-4031
ClinicalTrials.gov identifier	NCT03745924
EU PAS register number	EUPAS26592
EU PAS register link	http://www.encepp.eu/encepp/viewResource.htm?id=29248
Study initiated	Actual FPFV: 01-Apr-2019
Sponsor	Novo Nordisk A/S Novo Allé DK-2880 Bagsværd Denmark
Data cut-off date	1 December 2022

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1 Background

This progress report number 04 is related to the non-interventional Post Authorisation Safety Study (NIS PASS) (NN7999-4031), which is conducted in accordance with the marketing authorisation for Refixia® (EMEA/H/C/004178) granted by the Commission Decision in June 2017.

This progress report number 04 has been prepared in agreement with the commitment as requested by PRAC assessor in the 3rd PSUR. (29th May 2019).

Furthermore, table 2.1 format/wording has been updated to align with the study protocol. The changes are: Numbers of screened and treated have been corrected to enrolled, current in study, and withdrawn.

The cut-off for the data presented in this report is 1-December-2022.

2 Study progress

2.1 Study Schedule

The paradigm8 study (NN7999-4031) started on 1st April 2019 when the first patient was enrolled into the study.

- LPFV: 10th November 2022
- Planned LPLV: Q4 2027
- Planned final study report: Q2 2028

The planned timelines may be subject for adjustment during the course of the study.

2.2 Enrolling countries

The following countries are actively (have enrolled patients) participating in the study: Austria, Belgium, Canada, Croatia, Czech Republic, Denmark, Finland, Germany, Greece, Norway, Portugal & United Kingdom

2.3 Study Progress

Table 2–1 Number of patients in study

	Planned enrolled in Study	Actual enrolled in Study	Current in Study	Withdrawn	Planned Completed	Actual Completed
Total	60	65	60	5	50	0

Study recruitment accelerated significantly at end of recruitment. In total 65 patients were enrolled. Out of the 65 patients enrolled in the study, five patients have withdrawn from the study before completing 4 years in the study. The reason for withdrawal for these patients: One was "withdrawal of consent by patient" – no further reason was provided, four was "Physician decision". Of these; one patient was moving far away from site, one patient had GI bleedings with unknown causes, another patient went through a period of excess bleeds which led to a shift in treatment, and one patient was withdrawn due to switch in treatment. By recruiting five participants more than planned it is expected to mitigate the withdrawals.

2.4 Status

Closing the global recruitment with a total of 65 participants, hence we are positive towards conducting the study per planned timelines. Therefore, Novo Nordisk is of the opinion that the study is progressing according to plan.