MHRA
10 South Colonnade
Canary Wharf
London
E14 4PU
United Kingdom

gov.uk/mhra

Decision Cover Letter

Decision of the licensing authority to:

accept change(s) to the agreed paediatric investigation plan (MHRA-101319-PIP01-23-M01) and to the deferral

MHRA-101319-PIP01-23-M02

Scope of the Application

Active Substance(s)

denecimig

Condition(s)

Treatment of Haemophilia A

Pharmaceutical Form(s)

Solution for injection

Route(s) of Administration

SUBCUTANEOUS USE

Name / Corporate name of the PIP applicant

Novo Nordisk Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Novo Nordisk Limited submitted to the licensing authority on 18/08/2025 16:36 BST an application for a Modification

The procedure started on 03/09/2025 22:32 BST

1. The licensing authority, having assessed the application in accordance with the Human Medicines Regulations 2012, decides, as set out in the appended summary report:

to accept change(s) to the agreed paediatric investigation plan and to the deferral.

2. The measures and timelines of the paediatric investigation plan are set out in the Annex I.

This decision is forwarded to the applicant, together with its annex and appendix.

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Final Decision Letter

MHRA-101319-PIP01-23-M02

Of 12/09/2025 15:23 BST

On the adopted decision for denecimig (MHRA-101319-PIP01-23-M02) in accordance with the Human Medicines Regulations 2012.

The licensing authority, in accordance with the Human Medicines Regulations 2012, has adopted this decision:

Agreement on modification of a paediatric investigation plan (including modification of a waiver or deferral included in that paediatric investigation plan)

This decision applies to a Modification for denecimig , Solution for injection , SUBCUTANEOUS USE .

This decision is addressed to Novo Nordisk Limited, CMR, 3 City Place, Beehive Ring Road, West Sussex, Gatwick, UNITED KINGDOM, RH6 0PA

ANNEX I

1. Waiver

1.1 Condition:

Not applicable.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of Haemophilia A.

2.2 Indication(s) targeted by the PIP:

Routine prophylaxis to prevent or reduce frequency of bleeding episodes in patients with haemophilia A (congenital factor VIII deficiency) with or without factor VIII inhibitors.

2.3 Subset(s) of the paediatric population concerned by the paediatric development:

All subsets of the paediatric population from birth to less than 18 years of age.

2.4 Pharmaceutical Form(s):

Solution for injection.

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Development of 2 mg/ml strength suitable for the paediatric population.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	3	Study 2 (4513 MAD) Open-label multiple ascending dose trial to evaluate safety, pharmacokinetics and pharmacodynamics of denecimig in adolescents from 12 to less than 18 years of age (and adults) with haemophilia A. Study 3 (4514) Multicentre open-label trial in adolescents from 12 to less than 18 years of age (and adults) with haemophilia A to investigate efficacy and safety of denecimig prophylaxis compared to on-demand treatment and compared to standard-of-care prophylaxis. Study 4 (4516) Multicentre, open-label trial to investigate safety, efficacy, and exposure of denecimig prophylaxis in children from 1 to less than 12 years of age with haemophilia A.
Extrapolation, Modeling & Simulation Studies	2	Study 5 Modelling and simulation paediatric dose finding study. Study 6 Analysis of existing in-house data from children and adolescents from 2 to less than 18 years of age (and adults) and use of a literature maturation PK model of the Factor-VIII mimetic, emicizumab, to describe exposure in boys with haemophilia A from birth to less than 2 years of age.

Other Studies	0	Not applicable.
Other Measures	0	Not applicable.

3. Follow-up, completion and deferral of a PIP:

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	31/05/2025
Deferral of one or more studies contained in the paediatric investigation plan:	No