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

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Decision Cover Letter

Decision of the licensing authority to:

agree a paediatric investigation plan and grant a deferral and grant a waiver

MHRA-101488-PIP01-24

Scope of the Application

Active Substance(s)

etavopivat

Condition(s)

Treatment of sickle cell disease

Pharmaceutical Form(s)

Tablet; Age-appropriate oral solid dosage form

Route(s) of Administration

ORAL 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 02/10/2024 09:35 BST an application for a Paediatric Investigation Plan

The procedure started on 05/11/2024 08:48 GMT

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 agree a paediatric investigation plan and grant a deferral and grant a waiver

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-101488-PIP01-24

Of 19/02/2025 15:39 GMT

On the adopted decision for etavopivat (MHRA-101488-PIP01-24) 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 a paediatric investigation plan

This decision applies to a Paediatric Investigation Plan for etavopivat , Tablet; Age-appropriate oral solid dosage form , ORAL USE .

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

ANNEX I

1. Waiver

1.1 Condition:

Treatment of sickle cell disease The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 6 months of age Pharmaceutical form(s): Tablet Age-appropriate oral solid dosage form Route(s) of administration: ORAL USE Reason for granting waiver: on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of sickle cell disease

2.2 Indication(s) targeted by the PIP:

Treatment of sickle cell disease (SCD)

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

The paediatric population from 6 months to less than 18 years of age

2.4 Pharmaceutical Form(s):

Tablet Age-appropriate oral solid dosage form

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Development of an age-appropriate oral solid dosage form (mini-tablets) for use in paediatric population from 6 months to less than 12 years of age.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	2	Study 2 (4202-HEM-301) Adaptive, randomised, double-blind, placebo-controlled study to assess efficacy and safety in patients from 12 years to less 18 years of age (and adults) with sickle cell disease. Study 3 (4202-HEM-202) Single-arm, open-label study to evaluate safety and pharmacokinetics in children from 6 months to less than 18 years of age with sickle cell disease.
Extrapolation, Modeling & Simulation Studies	3	Study 4 Modelling and simulation study to support dose selection of etavopivat in children and adolescents from 6 months to less than 18 years of age with sickle cell disease. Study 5 Analysis of existing in house and literature data. Extrapolation plan Studies 2, 3, 4 and 5 are part of an extrapolation plan covering the paediatric population from 6 months to less than 12 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:	31/07/2029
Deferral of one or more studies contained in the paediatric investigation plan:	Yes