EMADOC-1700519818-2039164

European Medicines Agency decision

EMA/PE/0000232761

of 15 April 2025

on the agreement of a paediatric investigation plan and on the granting of a waiver for etavopivat in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

Disclaimer

This decision does not constitute entitlement to the rewards and incentives referred to in Title V of Regulation (EC) No 1901/2006.

Only the English text is authentic.

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on the agreement of a paediatric investigation plan and on the granting of a waiver for etavopivat in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

The European Medicines Agency,

Having regard to the Treaty on the Functioning of the European Union,

Having regard to Regulation (EC) No 1901/2006 of the European Parliament and of the Council of 12 December 2006 on medicinal products for paediatric use and amending Regulation (EEC) No. 1768/92, Directive 2001/20/EC, Directive 2001/83/EC and Regulation (EC) No 726/2004[1],

Having regard to Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency[2],

Having regard to the application submitted by Novo Nordisk A/S on 24 April 2024 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 28 February 2025, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 13 of said Regulation,

Having regard to Article 25 of Regulation (EC) No 1901/2006,

Whereas:

(1) The Paediatric Committee of the European Medicines Agency has given an opinion on the agreement of a paediatric investigation plan and on the granting of a waiver.

(2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.

(3) It is therefore appropriate to adopt a decision granting a waiver.

[1] OJ L 378, 27.12.2006, p.1, as amended.
[2] OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

A paediatric investigation plan for etavopivat, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby agreed.

Article 2

A waiver for etavopivat, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 3

This decision is addressed to Novo Nordisk A/S, Novo Alle 1, 2880 – Bagsvaerd, Denmark.

EMADOC-1700519818-1810399 Corr[1]
Amsterdam, 28 February 2025

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan and a waiver

EMA/PE/0000232761

Scope of the application

Active substance(s):

Etavopivat

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of beta-thalassaemia intermedia and major

Treatment of alpha-thalassaemia intermedia and major

Pharmaceutical form(s):

Tablet

Granules

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Novo Nordisk A/S

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Novo Nordisk A/S submitted for agreement to the European Medicines Agency on 24 April 2024 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 27 May 2024.

[1] 8 April 2025

Supplementary information was provided by the applicant on 22 November 2024. The applicant proposed modifications to the paediatric investigation plan and withdrew its request for a deferral.

Opinion

1. The Paediatric Committee, having assessed the proposed paediatric investigation plan in accordance with Article 17 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree the paediatric investigation plan in accordance with Article 17(1) of said Regulation;
   - to grant a waiver for one or more subsets of the paediatric population in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(c) of said Regulation, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

   The Paediatric Committee member of Norway agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the agreed paediatric investigation plan and the subset(s) of the paediatric population and condition(s) covered by the waiver are set out in the Annex I.

This opinion is forwarded to the applicant and the Executive Director of the European Medicines Agency, together with its annexes and appendix.

Annex I

The subset(s) of the paediatric population and condition(s) covered by the waiver and the measures and timelines of the agreed paediatric investigation plan (PIP)

1. Waiver

1.1. Condition:

Treatment of alpha-thalassaemia intermedia and major

The waiver applies to:

- the paediatric population from birth to less than 6 months of age;
- tablet, granules, oral use;
- 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).

1.2. Condition:

Treatment of beta-thalassaemia intermedia and major

The waiver applies to:

- the paediatric population from birth to less than 6 months of age;
- tablet, granules, oral use;
- 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:

Treatment of alpha-thalassaemia intermedia and major

2.1.1. Indication(s) targeted by the PIP

Treatment of alpha-thalassaemia intermedia and major

2.1.2. Subset(s) of the paediatric population concerned by the paediatric development

From 6 months to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Tablet

Granules

2.1.4. Measures

Area	Description
Quality-related studies	Study 1 Development of an age-appropriate formulation

Non-clinical studies	Not applicable
Clinical studies	Study 2 (4202-HEM-201) Open-label, multiple dose trial to evaluate safety and efficacy of etavopivat in adolescents from 12 years to less than 18 years of age (and adults) with thalassemia. Study 3 (NN7536-8128) Double-blind, randomised, multiple dose, placebo concurrent controlled trial to evaluate safety and efficacy of etavopivat in adolescents from 16 years to less than 18 years of age (and adults) with transfusion-dependent thalassemia (TDT). Study 4 (NN7536-7949) Double-blind, randomised, multiple dose, placebo concurrent controlled trial to evaluate safety and efficacy, of etavopivat in adolescents from 12 years to less than 18 years of age (and adults) with non-transfusion dependent thalassemia (NTDT). Study 5 (NN7536-8129) Open-label, multiple dose trial to evaluate pharmacokinetics and safety of etavopivat in children from 6 months to less than 16 years of age with non-transfusion-dependent thalassaemia (NTDT), and with transfusion-dependent thalassaemia (TDT).
Modelling and simulation analyses	Modelling and simulation analyses, to evaluate the use of the product in children from 6 months to less than 16 years of age with thalassemia.
Other studies	Not applicable
Extrapolation plan	Studies 2, 3, 4 and 5 are part of an extrapolation plan covering the paediatric population from 6 months to less than 16 years of age, as agreed by the PDCO.

2.2. Condition:

Treatment of beta-thalassaemia intermedia and major

2.2.1. Indication(s) targeted by the PIP

Treatment of beta-thalassaemia intermedia and major

2.2.2. Subset(s) of the paediatric population concerned by the paediatric development

From 6 months to less than 18 years of age

2.2.3. Pharmaceutical form(s)

Tablet

Granules

2.2.4. Measures

Same as for condition 'treatment of alpha-thalassaemia intermedia and major'

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	By December 2029
Deferral for one or more measures contained in the paediatric investigation plan:	No

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

The product is not authorised anywhere in the European Community.