EMA/504444/2023

European Medicines Agency decision

P/0477/2023

of 1 December 2023

on the acceptance of a modification of an agreed paediatric investigation plan for denecimig (EMEA-002762-PIP02-20-M02) 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 acceptance of a modification of an agreed paediatric investigation plan for denecimig (EMEA-002762-PIP02-20-M02) 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 European Medicines Agency's decision P/0450/2020 issued on 1 December 2020 and the decision P/0215/2023 issued on 14 June 2023,

Having regard to the application submitted by Novo Nordisk A/S on 28 June 2023 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 13 October 2023, in accordance with Article 22 of Regulation (EC) No 1901/2006,

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 acceptance of changes to the agreed paediatric investigation plan.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

[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

Changes to the agreed paediatric investigation plan for denecimig, solution for injection, subcutaneous use, are hereby accepted in the scope set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices.

Article 2

This decision is addressed to Novo Nordisk A/S, 108-110 Vandtårnsvej, 2860 – Soborg, Denmark.

EMA/PDCO/316821/2023
Amsterdam, 13 October 2023

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan

EMEA-002762-PIP02-20-M02

Scope of the application

Active substance(s):

Denecimig

Invented name and authorisation status:

See Annex II

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 A/S

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Novo Nordisk A/S submitted to the European Medicines Agency on 28 June 2023 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0450/2020 issued on 1 December 2020 and the decision P/0215/2023 issued on 14 June 2023.

The application for modification proposed changes to the agreed paediatric investigation plan.

The procedure started on 14 August 2023.

Scope of the modification

Some measures and timelines of the Paediatric Investigation Plan have been modified.

Opinion

1. The Paediatric Committee, having assessed the application in accordance with Article 22 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree to changes to the paediatric investigation plan in the scope set out in the Annex I of this opinion.

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

2. The measures and timelines of the paediatric investigation plan 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

Not applicable

2. Paediatric investigation plan

2.1. Condition:

Treatment of haemophilia A

2.1.1. Indication(s) targeted by the PIP

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

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

From birth to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Solution for injection

2.1.4. Measures

Area	Description
Quality-related studies	***Study 1*** Development of 2 mg/ml strength suitable for the paediatric population.
Non-clinical studies	Not applicable
Clinical studies	***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, modelling and simulation studies	***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.

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 May 2025
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

The product is not authorised anywhere in the European Community.