EMADOC-1700519818-1810277

European Medicines Agency decision

EMA/PE/0000225245

of 3 January 2025

on the acceptance of a modification of an agreed paediatric investigation plan for nedosiran 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.

European Medicines Agency decision

EMA/PE/0000225245

of 3 January 2025

on the acceptance of a modification of an agreed paediatric investigation plan for nedosiran 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/0426/2019 issued on 6 December 2019, the decision P/0342/2020 issued on 9 September 2020, the decision P/0071/2021 issued on 17 March 2021, the decision P/0541/2021 issued on 31 December 2021, the decision P/0007/2023 issued on 10 February 2023 and the decision P/0064/2024 issued on 8 March 2024,

Having regard to the application submitted by Novo Nordisk A/S on 10 August 2024 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 15 November 2024, 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 nedosiran, 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, Vandtaarnsvej 108-110, 2860 - Soeborg, Denmark.

EMADOC-1700519818-1666337
Amsterdam, 15 November 2024

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

EMA/PE/000022524

Scope of the application

Active substance(s):

Nedosiran

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of primary hyperoxaluria

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 10 August 2024 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0426/2019 issued on 6 December 2019, the decision P/0342/2020 issued on 9 September 2020, the decision P/0071/2021 issued on 17 March 2021, the decision P/0541/2021 issued on 31 December 2021, the decision P/0007/2023 issued on 10 February 2023 and the decision P/0064/2024 issued on 8 March 2024.

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

The procedure started on 16 September 2024.

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 primary hyperoxaluria

2.1.1. Indication(s) targeted by the PIP

Treatment of primary hyperoxaluria

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 an age-appropriate solution for subcutaneous injection
Non-clinical studies	Not applicable
Clinical studies	**Study 2 (DCR-PHXC-101)** Open-label, single-arm trial to evaluate safety, tolerability, pharmacokinetics and pharmacodynamics of single ascending doses of nedosiran in paediatric subjects from 12 to less than 18 years of age with primary hyperoxaluria type 1 (PH1) or 2 (PH2) and healthy adult volunteers **Study 3 (DCR-PHXC-301)** Open-label, long-term rollover study to evaluate efficacy and safety of nedosiran in paediatric subjects from birth to less than 18 years of age (and adults) with primary hyperoxaluria type 1 (PH1), 2 (PH2) or 3 (PH3) **Study 4 (DCR-PHXC-201)** Double-blind, randomised, placebo-controlled trial to evaluate efficacy, safety and pharmacokinetics of nedosiran in paediatric subjects from 6 to less than 18

	years of age (and adults) with primary hyperoxaluria type 1 (PH1) or 2 (PH2) and relatively intact renal function **Study 5 (DCR-PHXC-104)** Double-blind, randomised, placebo-controlled study to evaluate safety, tolerability, pharmacokinetics, and pharmacodynamics of nedosiran in paediatric patients from 6 to less than 18 years of age (and adults) with primary hyperoxaluria type 3 (PH3) **Study 6 (DCR-PHXC-203)** Open-label, single-arm trial to evaluate pharmacokinetics, safety and efficacy of nedosiran in paediatric patients from birth to less than 12 years of age with primary hyperoxaluria type 1 (PH1), 2 (PH2) or 3 (PH3) and relatively intact renal function **Study 7 (DCR-PHXC-204)** Open-label, single-arm trial to evaluate pharmacokinetics, safety and efficacy of nedosiran in paediatric patients from birth to less than 18 years of age with primary hyperoxaluria type 1 (PH1) or 2 (PH2) and impaired renal function with or without dialysis **Study 11 (DCR-PHXC-502)** *This study was deleted as a result of procedure EMA/PE/0000225245 (i.e. the 7th modification procedure of this PIP).*
Extrapolation, modelling and simulation studies	**Study 8 (DIC201804)** Modelling and simulation study to support dose finding of nedosiran in children from 6 to less than 18 years of age with primary hyperoxaluria and relatively intact renal function **Study 9** Modelling and simulation study to evaluate the use of nedosiran in children from birth to less than 18 years of age with primary hyperoxaluria and impaired renal function **Study 10** Modelling and simulation study to evaluate the use of nedosiran in children from birth to less than 6 years of age with primary hyperoxaluria and relatively intact renal function (follow-up to Study 8)
Other studies	Not applicable
Other measures	Not applicable

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 November 2027
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.