EMA/364312/2024

European Medicines Agency decision

P/0297/2024

of 16 August 2024

on the acceptance of a modification of an agreed paediatric investigation plan for narsoplimab, (EMEA-002479-PIP01-18-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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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/0400/2019 issued on 4 December 2019 and the decision P/0370/2021 issued on 8 September 2021,

Having regard to the application submitted by Omeros Ireland Limited on 18 March 2024 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 28 June 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 and to the deferral.

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

[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 narsoplimab, solution for injection/infusion, intravenous use, including changes to the deferral, 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 Omeros Ireland Limited, Ormond Building 31-36 Ormond Quay Upper, D07 EE37 - Dublin 7, Ireland.

EMA/PDCO/144984/2024
Amsterdam, 28 June 2024

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

EMEA-002479-PIP01-18-M02

Scope of the application

Active substance(s):

Narsoplimab

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment in haematopoietic stem cell transplantation

Pharmaceutical form(s):

Solution for injection/infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Omeros Ireland Limited

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Omeros Ireland Limited submitted to the European Medicines Agency on 18 March 2024 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/0400/2019 issued on 4 December 2019 and the decision P/0370/2021 issued on 8 September 2021.

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

The procedure started on 29 April 2024.

Scope of the modification

Some 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 and to the deferral 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 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 in haematopoietic stem cell transplantation

The waiver applies to:

- the paediatric population from birth to less than 28 days;
- solution for injection/infusion, intravenous use;
- on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset.

2. Paediatric investigation plan

2.1. Condition

Treatment in haematopoietic stem cell transplantation

2.1.1. Indication(s) targeted by the PIP

Treatment of paediatric patients aged from 28 days to less than 18 years of age with haematopoietic stem cell transplant associated thrombotic microangiopathy (HSCT-TMA)

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

From 28 days to less than 18 years old

2.1.3. Pharmaceutical form(s)

Solution for injection/infusion

2.1.4. Measures

Area	**Description**
Quality-related studies	Not applicable.
Non-clinical studies	**Study 1** Definitive juvenile mice toxicity study.
Clinical studies	**Study 2** Open-label pharmacokinetic and pharmacodynamics study of narsoplimab in paediatric subjects who received an allogeneic hematopoietic stem cell transplant for the treatment of benign or malignant disease and are at high-risk of HSCT-TMA (OMS721-HCT-002).

Extrapolation, modelling and simulation studies	Not applicable.
Other studies	**Study 3** Retrospective medical record review to collect data from paediatric patients with high-risk HSCT-TMA to build a control group for Study 2.
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 December 2026
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.