



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMADOC-1700519818-1855254 Corr¹

European Medicines Agency decision

EMA/PE/0000224175

of 28 January 2025

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

¹ 11 February 2025



European Medicines Agency decision

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on the acceptance of a modification of an agreed paediatric investigation plan for azamidugene autotemcel 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 726/2004²,

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 Agency³,

Having regard to the European Medicines Agency's decision P/0105/2022 issued on 13 April 2022 and the decision P/0167/2023 issued on 15 May 2023,

Having regard to the application submitted by Orchard Therapeutics (Netherlands) B.V. on 6 September 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 13 December 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.

² OJ L 378, 27.12.2006, p.1, as amended.

³ OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for azamidugene autotemcel, 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 Orchard Therapeutics (Netherlands) B.V., 10 Basisweg, 1043 - AP Amsterdam, Netherlands.



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMADOC-1700519818-1695435 ¹ Corr
Amsterdam, 13 December 2024

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA/PE/0000224175

Scope of the application

Active substance(s):

Azamidugene autotemcel (OTL-203)

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of mucopolysaccharidosis type I, Hurler syndrome

Pharmaceutical form(s):

Dispersion for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Orchard Therapeutics (Netherlands) B.V.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Orchard Therapeutics (Netherlands) B.V. submitted to the European Medicines Agency on 6 September 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/0105/2022 issued on 13 April 2022 and the decision P/0167/2023 issued on 15 May 2023.

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

The procedure started on 14 October 2024.

¹ 11 February 2025



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 members of Liechtenstein and Norway agree 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 of mucopolysaccharidosis type I, Hurler syndrome

The waiver applies to:

- the paediatric population from birth to less than 28 days;
- dispersion for infusion, intravenous use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

2. Paediatric investigation plan

2.1. Condition:

Treatment of Mucopolysaccharidosis type I, Hurler syndrome

2.1.1. Indication(s) targeted by the PIP

Treatment of Mucopolysaccharidosis type I, Hurler syndrome (MPS-IH)

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

From 28 days to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Dispersion for infusion

2.1.4. Measures

Area	Description
Quality-related studies	Not applicable
Non-clinical studies	Not applicable
Clinical studies	<p>Study 1 (TigetT10_MPSIH)</p> <p>Non-randomized, open-label study to evaluate safety and efficacy of OTL-203 for the treatment of patients from 28 days of age affected by mucopolysaccharidosis Type I, Hurler syndrome (MPS-IH)</p> <p>Study 2 (OTL-203-02)</p> <p>Multi-centre, randomized, active controlled clinical trial to evaluate the efficacy and safety of OTL-203 in subjects with mucopolysaccharidosis type I, Hurler syndrome (MPS-IH) compared to standard of care with allogeneic haematopoietic stem cell transplantation (allo-HSCT)</p>

Extrapolation, modelling and simulation studies	Not applicable
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 2030
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.