EMA/160450/2023

European Medicines Agency decision

P/0167/2023

of 15 May 2023

on the acceptance of a modification of an agreed paediatric investigation plan for autologous CD34+ hematopoietic stem and progenitor cells (HSPCs) genetically modified with the lentiviral vector IDUA LVV, encoding for the human α-L-iduronidase (IDUA) gene (OTL-203) (EMEA-003001-PIP01-21-M01) 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/0105/2022 issued on 13 April 2022,

Having regard to the application submitted by Orchard Therapeutics (Netherlands) B.V. on 16 December 2022 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a waiver and proposing a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 31 March 2023, in accordance with Article 22 of Regulation (EC) No 1901/2006 and Article 21 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 acceptance of changes to the agreed paediatric investigation plan and on the granting of a deferral.

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

(3) It is therefore appropriate to adopt a decision granting a 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 autologous CD34+ hematopoietic stem and progenitor cells (HSPCs) genetically modified with the lentiviral vector IDUA LVV, encoding for the human α-L-iduronidase (IDUA) gene (OTL-203), dispersion for infusion, intravenous 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

A deferral for autologous CD34+ hematopoietic stem and progenitor cells (HSPCs) genetically modified with the lentiviral vector IDUA LVV, encoding for the human α-L-iduronidase (IDUA) gene (OTL-203), dispersion for infusion, intravenous use, the details of which are set out in the opinion of the Paediatric Committee the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 3

This decision is addressed to Orchard Therapeutics (Netherlands) B.V., Basisweg 10, 1043 AP Amsterdam, The Netherlands.

EMA/PDCO/954462/2022
Amsterdam, 31 March 2023

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

EMEA-003001-PIP01-21-M01

Scope of the application

Active substance(s):

Autologous CD34+ hematopoietic stem and progenitor cells (HSPCs) genetically modified with the lentiviral vector IDUA LVV, encoding for the human α-L-iduronidase (IDUA) gene (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 16 December 2022 an application for modification of the agreed paediatric investigation plan with a waiver as set out in the European Medicines Agency's decision P/0105/2022 issued on 13 April 2022.

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

The procedure started on 30 January 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.
   - to grant a deferral, the details of which are 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 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	**Study 1 (TigetT10_MPSIH)** 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) **Study 2 (OTL-203-02)** 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)

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.