MHRA
10 South Colonnade
Canary Wharf
London
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United Kingdom

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Decision Cover Letter

Decision of the licensing authority to:

accept change(s) to the agreed paediatric investigation plan (MHRA-100330-PIP01-21-M01)

MHRA-100330-PIP01-21 -M02

Scope of the Application

Active Substance(s)

Azamidugene autotemcel (OTL-203)

Condition(s)

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

Pharmaceutical Form(s)

Dispersion for infusion

Route(s) of Administration

INTRAVENOUS USE

Name / Corporate name of the PIP applicant

Orchard Therapeutics (Europe) Ltd

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Orchard Therapeutics (Europe) Ltd submitted to the licensing authority on 10/09/2024 18:19 BST an application for a Modification

The procedure started on 29/10/2024 10:41 GMT

1. The licensing authority, having assessed the application in accordance with the Human Medicines Regulations 2012, decides, as set out in the appended summary report:

to accept change(s) to the agreed paediatric investigation plan

2. The measures and timelines of the paediatric investigation plan are set out in the Annex I.

This decision is forwarded to the applicant, together with its annex and appendix.

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Final Decision Letter

MHRA-100330-PIP01-21 -M02

Of 06/02/2025 15:58 GMT

On the adopted decision for Azamidugene autotemcel (OTL-203) (MHRA-100330-PIP01-21 -M02) in accordance with the Human Medicines Regulations 2012.

The licensing authority, in accordance with the Human Medicines Regulations 2012, has adopted this decision:

Agreement on modification of a paediatric investigation plan (including modification of a waiver or deferral included in that paediatric investigation plan)

This decision applies to a Modification for Azamidugene autotemcel (OTL-203), Dispersion for infusion , INTRAVENOUS USE .

This decision is addressed to Orchard Therapeutics (Europe) Ltd, 245 Hammersmith Road, London, UNITED KINGDOM, W6 8PW

ANNEX I

1. Waiver

1.1 Condition:

Treatment of Mucopolysaccharidosis type I, Hurler syndrome (MPS-IH) The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 28 days of age Pharmaceutical form(s): Dispersion for infusion Route(s) of administration: INTRAVENOUS USE Reason for granting waiver: 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(s):

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

2.2 Indication(s) targeted by the PIP:

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

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

The paediatric population from 28 days to less than 18 years of age

2.4 Pharmaceutical Form(s):

Dispersion for infusion

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	2	Study 1 (TigetT10_MPSIH) Non-randomised, 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) Multicentre, randomised, 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, Modeling & Simulation Studies	0	Not applicable.
Other Studies	0	Not applicable.
Other Measures	0	Not applicable.

3. Follow-up, completion and deferral of a PIP:

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	30/11/2030
Deferral of one or more studies contained in the paediatric investigation plan:	Yes