MHRA
10 South Colonnade
Canary Wharf
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United Kingdom

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Decision Cover Letter

Decision of the licensing authority to:

agree a paediatric investigation plan and grant a deferral and grant a waiver

MHRA-100267-PIP01-21

Scope of the Application

Active Substance(s)

Autologous CD34+ hematopoietic stem cells with a CRISPR-edited erythroid enhancer region of the BCL11A gene

Condition(s)

Treatment of beta-thalassemia intermedia and major

Pharmaceutical Form(s)

Dispersion for infusion

Route(s) of Administration

Intravenous use

Name / Corporate name of the PIP applicant

Vertex Pharmaceuticals (Europe) Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Vertex Pharmaceuticals (Europe) Limited submitted to the licensing authority on 18/10/2021 13:40 BST an application for a Paediatric Investigation Plan

The procedure started on 04/05/2022 11:07 BST

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 agree a paediatric investigation plan and grant a deferral and grant a waiver

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-100267-PIP01-21

Of 17/06/2022 08:35 BST

On the adopted decision for Autologous CD34+ hematopoietic stem cells with a CRISPR-edited erythroid enhancer region of the BCL11A gene (MHRA-100267-PIP01-21) 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 a paediatric investigation plan

This decision applies to a Paediatric Investigation Plan for Autologous CD34+ hematopoietic stem cells with a CRISPR-edited erythroid enhancer region of the BCL11A gene, Dispersion for infusion , Intravenous use .

This decision is addressed to Vertex Pharmaceuticals (Europe) Limited, 2 Kingdom Street, London, United Kingdom, W2 6BD

ANNEX I

1. Waiver

1.1 Condition:

Treatment of beta-thalassemia intermedia and major The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 6 months 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 over existing treatments

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of beta-thalassemia intermedia and major

2.2 Indication(s) targeted by the PIP:

Treatment of transfusion-dependent #-thalassemia in patients who are eligible for hematopoietic stem cell transplant (HSCT)

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

The paediatric population from 6 months 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 (CTX001-111) Open-label, non-randomised, single dose study to evaluate the safety and efficacy of CTX001 in adolescents from 12 years to less than 18 years of age (and adults) with transfusion-dependent #-thalassemia (TDT). Study 2 (VX21-CTX001-141) Open-label, non-randomised, single dose study to evaluate the safety and efficacy of CTX001 in children from 6 months to less than 12 years of age with transfusion-dependent #-thalassemia (TDT).
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:	Yes
Date of completion of the paediatric investigation plan:	31/03/2026
Deferral of one or more studies contained in the paediatric investigation plan:	Yes