EMA/21173/2023

European Medicines Agency decision

P/0046/2023

of 24 January 2023

on the acceptance of a modification of an agreed paediatric investigation plan for exagamglogene autotemcel (EMEA-002730-PIP04-21-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/0549/2021 issued on 31 December 2021,

Having regard to the application submitted by Vertex Pharmaceuticals (Ireland) Limited on 10 January 2023 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 20 January 2023, 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.

[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 exagamglogene autotemcel, 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

This decision covers all conditions, indications, pharmaceutical forms, routes of administration, measures, timelines, waivers and deferrals, as agreed in the decision P/0548/2021 issued on 31 December 2021, including subsequent modifications thereof.

Article 3

This decision is addressed to Vertex Pharmaceuticals (Ireland) Limited, Unit 49, Block F2, Northwood Court, Santry, D09 T665 - Dublin 9, Ireland.

EMA/PDCO/14793/2023
Amsterdam, 20 January 2023

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

EMEA-002730-PIP04-21-M01

Scope of the application

Active substance(s):

Exagamglogene autotemcel

Invented name and authorisation status:

See Annex II

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 (Ireland) Limited

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Vertex Pharmaceuticals (Ireland) Limited submitted to the European Medicines Agency on 10 January 2023 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/0549/2021 issued on 31 December 2021.

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

The procedure started on 11 January 2023.

Scope of the modification

An administrative modification to link two distinct paediatric investigation plans for regulatory purposes.

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 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 beta-thalassemia intermedia and major

The waiver applies to:

- the paediatric population from birth to less than 6 months of age;
- dispersion for infusion, intravenous use;
- 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:

Treatment of beta-thalassemia intermedia and major

2.1.1. Indication(s) targeted by the PIP

Treatment of transfusion-dependent β-thalassemia (TDT) in patients who are eligible for hematopoietic stem cell transplantation (HSCT)

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

From 6 months to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Dispersion for infusion

2.1.4. Measures

Area	**Number of measures**	**Description**
Quality-related studies	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 safety and efficacy of autologous CRISPR Cas9 modified CD34+ human hematopoietic stem and progenitor cells (hHSPCs) (hereafter CTX001) in adolescents from 12 years to less than 18 years of age (and adults) with transfusion-dependent β-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 β-thalassemia (TDT)
Extrapolation, modelling and simulation studies	0	Not applicable
Other studies	0	Not applicable
Other measures	0	Not applicable

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By March 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.