

MHRA
10 South Colonnade
Canary Wharf
London
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United Kingdom

gov.uk/mhra

Decision Cover Letter

Decision of the licensing authority to:

accept of change(s) to the agreed paediatric investigation plan (MHRA-100266-PIP01-21) and to the deferral

MHRA-100266-PIP01-21-M01

Scope of the Application

Active Substance(s)

EXAGAMGLOGENE AUTOTEMCEL

Condition(s)

Treatment of Sickle Cell Disease

Pharmaceutical Form(s)

Dispersion for infusion

Route(s) of Administration

INTRAVENOUS USE

Name / Corporate name of the PIP applicant

Vertex Pharmaceuticals Incorporated

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Vertex Pharmaceuticals Incorporated submitted to the licensing authority on 15/01/2026 15:34 GMT an application for a Modification

The procedure started on 29/01/2026 09:31 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 and to the deferral.

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-100266-PIP01-21-M01

Of 29/01/2026 15:36 GMT

On the adopted decision for EXAGAMGLOGENE AUTOTEMCEL (MHRA-100266-PIP01-21-M01) 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 EXAGAMGLOGENE AUTOTEMCEL, Dispersion for infusion , INTRAVENOUS USE .

This decision is addressed to Vertex Pharmaceuticals Incorporated, 2 Kingdom Street, London, UNITED KINGDOM, W2 6BD

ANNEX I

1. Waiver

1.1 Condition:

Treatment of Sickle Cell Disease 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 Sickle Cell Disease

2.2 Indication(s) targeted by the PIP:

Treatment of severe sickle cell disease in patients 6 months to less than 18 years of age 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 (CTX001-121) Open-label, non-randomised, single dose study to evaluate safety and efficacy of exagamglogene autotemcel in adolescents from 12 years to less than 18 years (and adults) with severe sickle cell disease (SCD). Study 2 (VX21-CTX001-151) Open-label, non-randomised, single dose study to evaluate safety and efficacy of exagamglogene autotemcel in children from 6 months to less than 12 years of age with severe sickle cell disease (SCD).
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:	31/12/2029
Deferral of one or more studies contained in the paediatric investigation plan:	Yes

