MHRA
10 South Colonnade
Canary Wharf
London
E14 4PU
United Kingdom

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Decision Cover Letter

Decision of the licensing authority to:

agree a paediatric investigation plan and grant a deferral

MHRA-101994-PIP01-25

Scope of the Application

Active Substance(s)

dorocubicel ; ECT-001-CB (Haematopoietic stem cells and blood progenitors umbilical cord-derived expanded with (1R,4R)-N1-(2-benzy|-7-(2-methyl-2H-tetrazol-5-yl) -9H-pyrimido[4,5-b] indol-4-yl)cyclohexane-1,4-diamine dihydrobromide dihydrate); Unexpanded CD34-cells

Condition(s)

Treatment of haematological malignancies requiring allogeneic haematopoietic stem cell transplantation

Pharmaceutical Form(s)

Dispersion for infusion

Route(s) of Administration

INTRAVENOUS USE

Name / Corporate name of the PIP applicant

Cordex Biologics International Ltd

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Cordex Biologics International Ltd submitted to the licensing authority on 13/06/2025 20:20 BST an application for a Paediatric Investigation Plan

The procedure started on 14/07/2025 13:57 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

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-101994-PIP01-25

Of 08/10/2025 10:58 BST

On the adopted decision for dorocubicel / allogeneic umbilical cord-derived CD34- cells, non-expanded (MHRA-101994-PIP01-25) 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 dorocubicel / allogeneic umbilical cord-derived CD34- cells, non-expanded, Dispersion for infusion , INTRAVENOUS USE .

This decision is addressed to Cordex Biologics International Ltd, 100 Longwater avenue, Reading, Berkshire, Reading, UNITED KINGDOM, RG2 6GP

ANNEX I

1. Waiver

1.1 Condition:

Not applicable

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of haematological malignancies requiring allogeneic haematopoietic stem cell transplantation

2.2 Indication(s) targeted by the PIP:

Treatment of patients with haematological malignancies requiring an allogeneic haematopoietic stem cell transplantation

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

All subsets of the paediatric population from birth 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 (ECT-001-CB.010) Open-label, randomised, active controlled trial to evaluate the efficacy of haematopoietic stem cells and blood progenitors umbilical cord-derived expanded with ECT-001-CB compared to best other stem cell source (either peripheral blood or bone marrow) in children from birth to less than 18 years of age (and adults) with relapsed or refractory acute myeloid leukaemia (AML) needing haematopoietic stem cell transplantation (HSCT). Study 2 (ECT-001-CB.007) Open-label, non-randomised, uncontrolled trial to evaluate safety and feasibility of ECT-001-CB infusion in children from birth to less than 18 years of age (and adults) with high-risk acute myeloid leukaemia needing 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:	31/12/2029
Deferral of one or more studies contained in the paediatric investigation plan:	Yes