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

gov.uk/mhra

Decision Cover Letter

Decision of the licensing authority to:

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

MHRA-100866-PIP01-23

Scope of the Application

Active Substance(s)

obecabtagene autoleucel

Condition(s)

Treatment of acute lymphoblastic leukaemia (ALL)

Pharmaceutical Form(s)

Dispersion for infusion

Route(s) of Administration

INTRAVENOUS USE

Name / Corporate name of the PIP applicant

AUTOLUS LIMITED

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, AUTOLUS LIMITED submitted to the licensing authority on 04/04/2023 16:14 BST an application for a Paediatric Investigation Plan

The procedure started on 26/09/2023 07:50 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-100866-PIP01-23

Of 06/11/2023 07:57 GMT

On the adopted decision for obecabtagene autoleucel (MHRA-100866-PIP01-23) 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 obecabtagene autoleucel, Dispersion for infusion , INTRAVENOUS USE .

This decision is addressed to AUTOLUS LIMITED, The MediaWorks, 191 Wood Ln, London W12 7FP, London, UNITED KINGDOM, W12 7FP

ANNEX I

1. Waiver

1.1 Condition:

Treatment of acute lymphoblastic leukaemia (ALL) The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 6 kg of bodyweight. 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 acute lymphoblastic leukaemia (ALL)

2.2 Indication(s) targeted by the PIP:

Treatment of relapsed or refractory B-cell acute lymphoblastic leukaemia (B-ALL)

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

The paediatric population from a body weight of 6 kg and above 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	1	Study 1 Development of a presentation of the formulation suitable for paediatric use.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	1	Study 2 (AUTO1-PY1) Open-label, single arm trial to evaluate safety, tolerability and activity of obecabtagene autoleucel in children with a body weight of at least 6 kg to less than 18 years of age with CD19-positive relapsed/ refractory B-ALL and relapsed/ refractory aggressive, mature B Non Hodgkin Lymphoma (B-NHL).
Extrapolation, Modeling & Simulation Studies	0	Not applicable.
Other Studies	0	Not applicable.
Other Measures	1	On completion of Study 2 and based on the review of the study results, the applicant must discuss with the Regulatory Authority a proposal for inclusion of a randomised controlled study in the paediatric population.

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:	31/12/2031
Deferral of one or more studies contained in the paediatric investigation plan:	Yes