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

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Decision Cover Letter

Decision of the licensing authority to:

accept change(s) to the agreed paediatric investigation plan.

MHRA-101123-PIP01-23-M02

Scope of the Application

Active Substance(s)

BREXUCABTAGENE AUTOLEUCEL

Condition(s)

Treatment of acute lymphoblastic leukaemia.

Pharmaceutical Form(s)

Dispersion for infusion

Route(s) of Administration

INTRAVENOUS USE

Name / Corporate name of the PIP applicant

Gilead Sciences Ltd

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Gilead Sciences Ltd submitted to the licensing authority on 04/04/2024 19:29 BST an application for a Modification

The procedure started on 19/06/2024 11:38 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 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-101123-PIP01-23-M02

Of 05/07/2024 15:58 BST

On the adopted decision for BREXUCABTAGENE AUTOLEUCEL (MHRA-101123-PIP01-23-M02) 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 BREXUCABTAGENE AUTOLEUCEL, Dispersion for infusion , INTRAVENOUS USE .

This decision is addressed to Gilead Sciences Ltd, 280 High Holborn, London , UNITED KINGDOM, WC1V 7EE

ANNEX I

1. Waiver

1.1 Condition:

Treatment of acute lymphoblastic leukaemia The waiver applies / applied to: Paediatric Subset(s): The paediatric population weighing less than 6 kg. Pharmaceutical form(s): Dispersion for infusion Route(s) of administration: INTRAVENOUS USE Reason for granting waiver: On the grounds the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of acute lymphoblastic leukaemia

2.2 Indication(s) targeted by the PIP:

Treatment of relapsed or refractory B-precursor acute lymphoblastic leukaemia (r/r ALL).

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

The paediatric population less than 18 years of age weighing at least 6 kg.

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 formulation of KTE-X19 suitable for administration to paediatric patients with a minimum weight of 6 kg.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	1	Study 2 Open-label, single arm, 2-phase trial to evaluate safety and activity, of KTE-X19 in children weighing at least 6 kg with B-cell acute lymphoblastic leukaemia (cohort 1) or with B-cell non-Hodgkin lymphoma (cohort 2) whose disease is refractory to a standard chemotherapy regimen, relapsed after stem cell transplantation (SCT).
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:	31/12/2023
Deferral of one or more studies contained in the paediatric investigation plan:	Yes