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

gov.uk/mhra

Decision Cover Letter

Decision of the licensing authority to:

accept change(s) to the agreed paediatric investigation plan (EMEA-001862-PIP03-20) and to the deferral

MHRA-101302-PIP01-23-M01

Scope of the Application

Active Substance(s)

BREXUCABTAGENE AUTOLEUCEL

Condition(s)

Treatment of mature B-cell neoplasms

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 21/12/2023 13:35 GMT an application for a Modification

The procedure started on 12/02/2024 12:15 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-101302-PIP01-23-M01

Of 03/06/2024 09:41 BST

On the adopted decision for BREXUCABTAGENE AUTOLEUCEL (MHRA-101302-PIP01-23-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 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 mature B-cell neoplasms 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 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 mature B-cell neoplasms

2.2 Indication(s) targeted by the PIP:

Treatment of relapsed or refractory B-cell non-Hodgkin lymphoma

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

The paediatric population less than 18 years of age and 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 (NHL portion of KTE-C19-104 [ZUMA-4]) Open-label, single arm trial to evaluate safety and activity of KTE-X19 in paediatric patients weighing at least 6 kg with relapsed or refractory B-cell non-Hodgkin lymphoma (r/r B-cell NHL).
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:	31/10/2026
Deferral of one or more studies contained in the paediatric investigation plan:	Yes