

**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 and to grant a product specific waiver  
MHRA-100418-PIP01-22-M01

### **Scope of the Application**

#### **Active Substance(s)**

axicabtagene ciloleucel

#### **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 18/01/2022 15:49 GMT an application for a Modification

The procedure started on 12/08/2022 15:16 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 grant a product specific 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-100418-PIP01-22-M01

Of 19/09/2022 08:56 BST

On the adopted decision for axicabtagene ciloleucel (MHRA-100418-PIP01-22-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 a modification of a waiver to include all age groups for the listed condition)

This decision applies to a Modification for axicabtagene ciloleucel, 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): All subsets of the paediatric population from birth to less than 18 years 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):

Not Applicable

**2.2 Indication(s) targeted by the PIP:**

Not Applicable
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**2.3 Subset(s) of the paediatric population concerned by the paediatric development:**

Not Applicable
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**2.4 Pharmaceutical Form(s):**

Not Applicable
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**2.5 Studies:**

<b>Study Type</b>	<b>Number of Studies</b>	<b>Study Description</b>
<b>Quality Measures</b>	0	Not Applicable
<b>Non-Clinical Studies</b>	0	Not Applicable
<b>Clinical Studies</b>	0	Not Applicable
<b>Extrapolation, Modeling &amp; Simulation Studies</b>	0	Not Applicable
<b>Other Studies</b>	0	Not Applicable
<b>Other Measures</b>	0	Not Applicable

**3. Follow-up, completion and deferral of a PIP:**

<b>Concerns on potential long term safety and efficacy issues in relation to paediatric use:</b>	
<b>Date of completion of the paediatric investigation plan:</b>	
<b>Deferral of one or more studies contained in the paediatric investigation plan:</b>	

