

**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 and grant a waiver

MHRA-101413-PIP01-24

### **Scope of the Application**

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

BLINATUMOMAB

#### **Condition(s)**

Treatment of acute lymphoblastic leukaemia

#### **Pharmaceutical Form(s)**

Powder for solution for injection

#### **Route(s) of Administration**

SUBCUTANEOUS USE

#### **Name / Corporate name of the PIP applicant**

Amgen Limited

#### **Basis for the Decision**

Pursuant to the Human Medicines Regulations 2012, Amgen Limited submitted to the licensing authority on 29/04/2024 18:27 BST an application for a Paediatric Investigation Plan

The procedure started on 13/01/2025 11:49 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 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-101413-PIP01-24

Of 20/03/2025 16:46 GMT

On the adopted decision for BLINATUMOMAB (MHRA-101413-PIP01-24) 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 BLINATUMOMAB, Powder for solution for injection , SUBCUTANEOUS USE .

This decision is addressed to Amgen Limited, 216 Cambridge Science Park, Milton Road , Cambridge, UNITED KINGDOM, CB4 0WA

## ANNEX I

### 1. Waiver

#### 1.1 Condition:

Treatment of acute lymphoblastic leukaemia The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 28 days of age Pharmaceutical form(s): Powder for solution for injection Route(s) of administration: SUBCUTANEOUS USE Reason for granting waiver: on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

### 2. Paediatric Investigation Plan:

#### 2.1 Condition(s):

Treatment of acute lymphoblastic leukaemia

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

Treatment of children with relapsed/ refractory B-cell precursor acute lymphoblastic leukaemia

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

The paediatric population from 28 days to less than 18 years of age

**2.4 Pharmaceutical Form(s):**

Powder for solution for injection

**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 (20180257) Open-label, uncontrolled, single arm study to evaluate safety, activity and pharmacokinetic (PK) parameters following subcutaneous (SC) administration of blinatumomab in adolescent patients from 12 years to less than 18 years of age (and adults) with relapsed or refractory B-cell precursor acute lymphoblastic leukaemia (R/R B-ALL). Study 2 (20220107) Open label, uncontrolled, single arm trial to evaluate safety and tolerability and determine the recommended Phase 2 dose (Phase 1) and evaluate the activity (Phase 2) of SC blinatumomab in patients from 28 days to less than 18 years of age with R/R B-ALL.
Extrapolation, Modeling & Simulation Studies	3	Study 3 Modelling and simulation population pharmacokinetic (Pop-PK) study to predict the initial paediatric doses to be used in further clinical studies in children from 28 days to less than 18 years of age with R/R B-ALL. Study 4 Modelling and simulation Pop-PK/PD study to confirm or modify the paediatric posology compared to the regimen used in clinical trials in children from 28 days to less than 18 years of age

		with R/R B-ALL. Extrapolation plan Study 1 (Study 20180257), Study 2 (Study 20220107) and Study 4 are part of an extrapolation plan covering the paediatric population from 28 days to less than 18 years of age.
<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>	Yes
<b>Date of completion of the paediatric investigation plan:</b>	31/03/2029
<b>Deferral of one or more studies contained in the paediatric investigation plan:</b>	Yes