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 and to the deferral.

MHRA-101131-PIP01-23-M01

Scope of the Application

Active Substance(s)

BLINATUMOMAB

Condition(s)

Treatment of acute lymphoblastic leukaemia

Pharmaceutical Form(s)

Powder for solution for infusion

Route(s) of Administration

INTRAVENOUS 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 29/08/2023 17:19 BST an application for a Modification

The procedure started on 17/11/2023 16:19 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.

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

gov.uk/mhra

Final Decision Letter

MHRA-101131-PIP01-23-M01

Of 24/11/2023 09:57 GMT

On the adopted decision for BLINATUMOMAB (MHRA-101131-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 BLINATUMOMAB, Powder for solution for infusion , INTRAVENOUS USE .

This decision is addressed to Amgen Limited, 216 Cambridge Science Park, 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): Preterm newborn infants and term newborn infants (from birth to less than 28 days). Pharmaceutical form(s): Powder for solution for infusion Route(s) of administration: INTRAVENOUS USE Reason for granting waiver: On the grounds clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfill 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 previously untreated high-risk first relapse of B precursor acute lymphoblastic leukaemia.

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

The paediatric population from 1 month to less than 18 years of age.

2.4 Pharmaceutical Form(s):

Powder for solution for infusion

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 (MT103-205, 2010-024264-18) Multi-centre, open-label, multiple-dose, dose-escalation trial to evaluate pharmacokinetics, pharmacodynamics, toxicity, safety and anti-tumour activity of blinatumomab in children from birth to less than 18 years of age with a relapse of B precursor acute lymphoblastic leukaemia involving the bone marrow or a refractory acute lymphoblastic leukaemia and for whom no effective treatment is known, with an extension phase. Study 2 (20120215) Randomised, controlled, open-label trial to evaluate the pharmacokinetics, safety and efficacy of blinatumomab compared to multi-agent consolidation chemotherapy in children from 1 month to less than 18 years of age with a first, high-risk relapse of B-precursor acute lymphoblastic leukaemia.
Extrapolation, Modeling & Simulation Studies	1	Study 3 (Pharmacokinetic-pharmacodynamic analysis of blinatumomab paediatric data from subjects with acute lymphoblastic

		leukaemia) Pharmacokinetic-pharmacodynamic analysis to inform the dose for study 2.
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:	31/07/2023
Deferral of one or more studies contained in the paediatric investigation plan:	Yes