

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

gov.uk/mhra

Decision Cover Letter

Decision of the licensing authority to:

agree a paediatric investigation plan and grant a deferral and grant a waiver

MHRA-101902-PIP01-25

Scope of the Application

Active Substance(s)

Bleximenib

Condition(s)

Treatment of acute myeloid leukaemia

Pharmaceutical Form(s)

Age-appropriate oral dosage form; Film-coated tablet

Route(s) of Administration

ORAL USE

Name / Corporate name of the PIP applicant

Janssen Cilag Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Janssen Cilag Limited submitted to the licensing authority on 23/04/2025 23:45 BST an application for a Paediatric Investigation Plan

The procedure started on 10/06/2025 13:10 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 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-101902-PIP01-25

Of 21/08/2026 07:32 BST

On the adopted decision for Bleximenib (MHRA-101902-PIP01-25) 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 Bleximenib, Age-appropriate oral liquid dosage form Film-coated tablet , Oral use Gastric use .

This decision is addressed to Janssen Cilag Limited, 50-100 Holmers Farm Way, High Wycombe, Buckinghamshire, UNITED KINGDOM, HP12 4EG

ANNEX I

1. Waiver

1.1 Condition:

Treatment of acute myeloid leukaemia The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 28 days of age Pharmaceutical form(s): Age-appropriate oral liquid dosage form Film-coated tablet Route(s) of administration: Oral use Gastric 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 myeloid leukaemia

2.2 Indication(s) targeted by the PIP:

Treatment of patients from 28 days to less than 18 years of age with relapsed or refractory acute myeloid leukaemia with a histone-lysine N-methyltransferase 2A (KMT2A) rearrangement or a nucleophosmin 1 (NPM1) mutation or nucleoporin 98 (NUP98) or nucleoporin 214 (NUP214) alterations. Treatment of patients from 28 days to less than 18 years of age with newly diagnosed acute myeloid leukaemia with a KMT2A rearrangement or a NPM1 mutation or NUP98 or NUP214 alterations.

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):

Age-appropriate dosage oral liquid dosage form Film-coated tablet

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Development of an age-appropriate, oral liquid formulation to be used in patients from 28 days to less than 18 years of age.
Non-Clinical Studies	1	Study 2 Definitive juvenile mouse toxicity study.
Clinical Studies	1	Study 3 Open-label, two-part study in paediatric patients from 28 days to less than 18 years of age with acute myeloid leukaemia (AML) harbouring KMT2A (histone-lysine N-methyltransferase 2A) rearrangements (KMT2Ar), or NPM1 (nucleophosmin 1) mutation (NPM1m), or nucleoporin 98 (NUP98), or nucleoporin 124 (NUP214) alterations including: • Part 1 single arm trial in paediatric patients with relapsed/refractory acute myeloid leukaemia to evaluate the pharmacokinetics, safety, and preliminary clinical activity of bleximenib used in combination with standard chemotherapy. • Part 2, randomised active controlled trial in paediatric patients with newly diagnosed AML to assess the pharmacokinetics, safety and efficacy

		of bleximenib in combination with standard chemotherapy compared to chemotherapy. The design, combination treatment, sample size, control and endpoints of the part 2 of the study to be agreed by the Regulatory Agency prior to part 2 initiation and once results of study part 1 and nonclinical study 1 are available.
Extrapolation, Modeling & Simulation Studies	1	Study 4 Population PK (popPK) analysis to confirm the bleximenib dose to be used in patients from 28 days to less than 18 years of age which is intended to match the recommended phase 2 dose exposure observed in adults.
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:	No
Date of completion of the paediatric investigation plan:	30/09/2031
Deferral of one or more studies contained in the paediatric investigation plan:	Yes