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-102497-PIP01-26

Scope of the Application

Active Substance(s)

PONATINIB; PONATINIB HYDROCHLORIDE

Condition(s)

Treatment of chronic myeloid leukaemia (CML), Treatment of acute lymphoblastic leukaemia

Pharmaceutical Form(s)

Capsule, hard; Film-coated tablet; Age-appropriate formulation

Route(s) of Administration

ORAL USE

Name / Corporate name of the PIP applicant

INCYTE BIOSCIENCES UK LIMITED

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, INCYTE BIOSCIENCES UK LIMITED submitted to the licensing authority on 28/04/2026 16:41 BST an application for a

The procedure started on 01/06/2026 10:54 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 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-102497-PIP01-26

Of 19/06/2026 10:00 BST

On the adopted decision for PONATINIB; PONATINIB HYDROCHLORIDE (MHRA-102497-PIP01-26) 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 for PONATINIB; PONATINIB HYDROCHLORIDE, Capsule, hard; Film-coated tablet; Age-appropriate formulation , ORAL USE .

This decision is addressed to INCYTE BIOSCIENCES UK LIMITED, First Floor Q1, The Square, Randalls Way, Leatherhead, UNITED KINGDOM, KT22 7TW

ANNEX I

1. Waiver

1.1 Condition:

1.1. Treatment of chronic myeloid leukaemia The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 1 year of age. Pharmaceutical form(s): Capsule, hard; Film-coated-tablet; Age-appropriate formulation Route(s) of administration: ORAL USE Reason for granting waiver: On the grounds the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s). 1.2Treatment of acute lymphoblastic leukaemia The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 1 year of age. Pharmaceutical form(s): Capsule, hard; Film-coated-tablet; Age-appropriate formulation Route(s) of administration: ORAL USE Reason for granting waiver: On the grounds the specific medicinal product does not represent a significant therapeutic benefit as the needs are already covered.

2. Paediatric Investigation Plan:

2.1 Condition(s):

2.1. Treatment of chronic myeloid leukaemia (CML) 2.2. Treatment of acute lymphoblastic leukaemia (ALL)

2.2 Indication(s) targeted by the PIP:

2.1. For the treatment of the paediatric population with chronic (CP), accelerated (AP), or blast phase (BP) CML who are resistant or intolerant to prior tyrosine kinase inhibitor (TKI) therapy, or who have the T315I mutation. 2.2. For the treatment of the paediatric population with Philadelphia positive (Ph+) ALL who are resistant or intolerant to prior TKI therapy, or who have the T315I mutation.

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

2.1. The paediatric population from 1 year to less than 18 years of age. 2.2. The paediatric population from 1 year to less than 18 years of age.

2.4 Pharmaceutical Form(s):

2.1. Capsule, hard Film-coated-tablet Age-appropriate formulation 2.2. Capsule, hard Film-coated-tablet Age-appropriate formulation

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	2	Studies for Conditions 1 and 2 Study 1 Development of an age-appropriate formulation for oral use. Study 5 (This study was added during procedure EMEA-001186-PIP01-11-M02) Development of capsules of 5 mg strength.
Non-Clinical Studies	1	Study for Conditions 1 and 2 Study 2 Toxicity study in juvenile rats.
Clinical Studies	2	Study for Conditions 1 and 2 Study 3 Open-label, single-agent, dose-escalation, multi-centre trial to investigate tolerability, safety and activity of ponatinib in the paediatric population from 1 year to less than 18 years of age with malignant disease for which no effective treatment is known, and with an expansion cohort of the paediatric population with chronic phase chronic myeloid leukaemia and

		other leukaemia and solid tumours. Study for Condition 2 Study 4 Open-label, multi-centre trial including a safety lead-in phase to investigate the safety, the activity and the efficacy of ponatinib as add-on to standard therapy in the paediatric population from 1 year to less than 18 years of age with relapsed or refractory BCR-ABL translocation-positive ALL.
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:	30/06/2025
Deferral of one or more studies contained in the paediatric investigation plan:	Yes