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 adopted paediatric investigation plan and to the deferral.

MHRA-102170-PIP01-25-M01

Scope of the Application

Active Substance(s)

asciminib hydrochloride

Condition(s)

Treatment of chronic myeloid leukaemia.

Pharmaceutical Form(s)

Film-coated granules; Film-coated tablet

Route(s) of Administration

ORAL USE

Name / Corporate name of the PIP applicant

Novartis Pharmaceuticals UK Ltd

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Novartis Pharmaceuticals UK Ltd submitted to the licensing authority on 09/10/2025 17:41 BST an application for a Modification

The procedure started on 04/11/2025 17:56 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-102170-PIP01-25-M01

Of 28/11/2025 08:25 GMT

On the adopted decision for asciminib hydrochloride (MHRA-102170-PIP01-25-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 asciminib hydrochloride, Film-coated granules; Film-coated tablet , ORAL USE .

This decision is addressed to Novartis Pharmaceuticals UK Ltd , 2nd Floor, The WestWorks Building White City Place, 195 Wood Lane , London, UNITED KINGDOM, W12 7FQ

ANNEX I

1. Waiver

1.1 Condition:

Treatment of chronic myeloid leukaemia. The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 3 years of ages Pharmaceutical form(s): Film-coated granules Film-coated tablet Route(s) of administration: ORAL USE Reason for granting waiver: on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of chronic myeloid leukaemia.

2.2 Indication(s) targeted by the PIP:

Treatment of Philadelphia chromosome-positive chronic myelogenous leukaemia in chronic phase (Ph+ CML-CP) previously treated with one or more tyrosine kinase inhibitors (TKIs).

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

The paediatric population from 3 years to less than 18 years of age.

2.4 Pharmaceutical Form(s):

Film-coated granules Film-coated tablet

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Development of an age-appropriate oral formulation.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	1	Study 2 Open-label, multiple dose trial to evaluate pharmacokinetics, safety, activity, acceptability/ palatability of asciminib in children from 3 years to less than 18 years of age with Philadelphia chromosome-positive chronic myelogenous leukaemia in chronic phase (Ph + CML-CP) previously treated with one or more tyrosine kinase inhibitors (TKIs).
Extrapolation, Modeling & Simulation Studies	3	Study 3 Physiologically based PK (PBPK) study to predict the initial dose of asciminib for study 2 in children from 3 years to less than 18 years of age with Philadelphia chromosome-positive chronic myelogenous leukaemia in chronic phase (Ph+ CML-CP) previously treated with one or more tyrosine kinase inhibitors (TKIs). Study 4 Population-PK/PD model of nilotinib adult data to predict paediatric nilotinib response in order to further support the applicability of efficacy extrapolation for TKIs, such as asciminib in children from 3 year

		to less than 18 years of age with Philadelphia chromosome-positive chronic myelogenous leukaemia in chronic phase (Ph+ CML-CP) previously treated with one or more tyrosine kinase inhibitors (TKIs). Study 5 Extrapolation study to support the use of asciminib in paediatric patients from 3 years to less than 18 years of age with Philadelphia chromosome-positive chronic myelogenous leukaemia in chronic phase (Ph+ CML-CP) previously treated with one or more tyrosine kinase inhibitors (TKIs).
Other Studies	1	Study 6 Systematic review of the literature to assess the similarity of response to TKIs (dasatinib, imatinib and nilotinib) between adult and paediatric patients with CML when treated with the same BCR-ABL1 TKI at a comparable exposure.
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:	31/03/2027
Deferral of one or more studies contained in the paediatric investigation plan:	Yes