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

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Decision Cover Letter

Decision of the licensing authority to:

accept of change(s) to the agreed paediatric investigation plan (MHRA-100069-PIP01-21) and to the deferral

MHRA-100069-PIP01-21-M01

Scope of the Application

Active Substance(s)

RIBOCICLIB SUCCINATE

Condition(s)

Treatment of neuroblastoma

Pharmaceutical Form(s)

Film coated tablet; Age-appropriate oral liquid dosage form

Route(s) of Administration

ORAL USE

Name / Corporate name of the PIP applicant

Novartis Pharmaceuticals UK Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Novartis Pharmaceuticals UK Limited submitted to the licensing authority on 19/08/2025 05:50 BST an application for a Modification

The procedure started on 02/09/2025 22:07 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-100069-PIP01-21-M01

Of 12/01/2026 11:43 GMT

On the adopted decision for RIBOCICLIB SUCCINATE (MHRA-100069-PIP01-21-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 RIBOCICLIB SUCCINATE, Film coated tablet; Age-appropriate oral liquid dosage form , ORAL USE .

This decision is addressed to Novartis Pharmaceuticals UK Limited, 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 neuroblastoma The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 1 year of age Pharmaceutical form(s): Film coated tablet Age-appropriate oral liquid dosage form Route(s) of administration: ORAL USE Reason for granting waiver: on the grounds that 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):

Treatment of neuroblastoma

2.2 Indication(s) targeted by the PIP:

Treatment of relapsed or refractory neuroblastoma in patients aged 18 months and above in combination with temozolomide and topotecan (TOTEM)

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

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

2.4 Pharmaceutical Form(s):

Film coated tablet Age-appropriate oral liquid dosage form

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Study 1 Deleted during procedure MHRA-100069-PIP01-21-M01.
Non-Clinical Studies	1	Study 2 Definitive juvenile toxicity study.
Clinical Studies	1	Study 3 Open-label, single arm trial (Part A) to determine the recommended Phase 2 dose (RP2D) and evaluate the pharmacokinetics, safety and activity, and of ribociclib as add-on to topotecan and temozolomide (TOTEM) in children from 1 year to less than 18 years of age (and adults) with relapsed or refractory neuroblastoma (and other solid tumours). Study 4 Deleted during procedure MHRA-100069-PIP01-21-M01. Study 5 Deleted during procedure MHRA-100069-PIP01-21-M01.
Extrapolation, Modeling & Simulation Studies	0	Study 6 Deleted during procedure MHRA-100069-PIP01-21-M01.
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:	31/12/2025

Deferral of one or more studies contained in the paediatric investigation plan:	No