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

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Decision Cover Letter

Decision of the licensing authority to:

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

MHRA-102111-PIP01-25

Scope of the Application

Active Substance(s)

remibrutinib

Condition(s)

Treatment of myasthenia gravis

Pharmaceutical Form(s)

Film-coated tablet Age-appropriate oral solid 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 03/09/2025 20:41 BST an application for a Paediatric Investigation Plan

The procedure started on 04/11/2025 10:29 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 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-102111-PIP01-25

Of 29/01/2026 06:37 GMT

On the adopted decision for remibrutinib (MHRA-102111-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 remibrutinib, Film-coated tablet Age-appropriate oral solid 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 myasthenia gravis The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 6 years of age Pharmaceutical form(s): Film-coated tablet Age-appropriate oral solid dosage form Route(s) of administration: ORAL USE Reason for granting waiver: on the grounds that the specific medicinal product is likely to be unsafe.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of myasthenia gravis

2.2 Indication(s) targeted by the PIP:

Treatment of generalised myasthenia gravis in children from 6 years and older who are anti-acetylcholine receptor antibody-positive (AChR+), or anti-muscle-specific tyrosine kinase antibody-positive (MuSK+), or double-seronegative.

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

The paediatric population from 6 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

Film-coated tablet Age-appropriate oral solid dosage form

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Development of an age-appropriate oral solid dosage form suitable for children from 6 years to less than 12 years of age.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	1	Study 2 Open label, single-arm, uncontrolled trial to evaluate pharmacokinetics, pharmacodynamics, exploratory efficacy and safety of remibrutinib in children from 6 years to less than 18 years of age with generalised myasthenia gravis (gMG) who are antiacetylcholine receptor antibody-positive (AChR+), anti-muscle specific tyrosine kinase antibody-positive (MuSK+), or double seronegative.
Extrapolation, Modeling & Simulation Studies	3	Study 3 Development of a popPK model based on the PK data from adult patients with gMG and other indications for: • Prediction of initial paediatric dose(s) to be used in Study 2 • use of PopPK(/PD) for PK simulation in children from 6 to less than 18 years of age with gMG as a basis for extrapolation. Study 4 Characterisation of PK and confirmation of the dose to be used in paediatric gMG patients, including update of the model developed in Study 3. Extrapolation Plan The planned phase III study in adult

		gMG patients (CLOU064O12301), the paediatric clinical Study 2, Study 3 and Study 4 are part of the extrapolation plan of efficacy data from adults covering the paediatric population from 6 years to less than 18 years of age with gMG.
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:	31/12/2033
Deferral of one or more studies contained in the paediatric investigation plan:	Yes