

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-102189-PIP01-25

Scope of the Application

Active Substance(s)

IPTACOPAN HYDROCHLORIDE MONOHYDRATE

Condition(s)

Treatment of generalised myasthenia gravis

Pharmaceutical Form(s)

Capsule, hard; Age appropriate oral 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 21/11/2025 04:46 GMT an application for a Paediatric Investigation Plan

The procedure started on 08/12/2025 13:06 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-102189-PIP01-25

Of 03/03/2026 14:37 GMT

On the adopted decision for IPTACOPAN HYDROCHLORIDE MONOHYDRATE (MHRA-102189-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 IPTACOPAN HYDROCHLORIDE MONOHYDRATE, Capsule, hard; Age appropriate oral dosage form , ORAL USE .

This decision is addressed to NOVARTIS PHARMACEUTICALS UK LIMITED, 2nd Floor, The WestWorks Building, London, UNITED KINGDOM, W12 7FQ

ANNEX I

1. Waiver

1.1 Condition:

Treatment of generalised myasthenia gravis The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 2 years of age Pharmaceutical form(s): Capsule, hard Age appropriate oral dosage form Route(s) of administration: ORAL 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 generalised myasthenia gravis

2.2 Indication(s) targeted by the PIP:

Treatment of generalised myasthenia gravis in children and adolescents aged 2 years to less than 18 years who are anti-acetylcholine receptor (AChR) antibody-positive

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

The paediatric population from 2 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

Capsule, hard Age appropriate oral 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.
Non-Clinical Studies	1	Study 2 Definitive 52-week toxicity study in juvenile dogs with a 27 week recovery period.
Clinical Studies	1	Study 3 Open-label, single-arm study to assess the pharmacokinetics, pharmacodynamics, safety, tolerability and exploratory efficacy of iptacopan in paediatric patients 2 years to less than 18 years of age with generalised myasthenia gravis (gMG).
Extrapolation, Modeling & Simulation Studies	3	Study 4 Modelling and simulation analyses (population PK) to establish the dose for Study 3 in paediatric patients from 2 years to less than 18 years of age with generalised myasthenia gravis (gMG). Study 5 Modelling and simulation analyses (population PK) to confirm the dose required for paediatric patients from 2 years to less than 18 years of age with generalised myasthenia gravis (gMG). Extrapolation Plan The phase III study planned in adult gMG patients (CLNP023Q12301), PIP Study 3, PIP Study 4 and PIP Study 5 are part of the extrapolation plan of efficacy data from adults covering the paediatric population from 2

		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:	No
Date of completion of the paediatric investigation plan:	30/09/2032
Deferral of one or more studies contained in the paediatric investigation plan:	Yes