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 change(s) to the agreed paediatric investigation plan and to the deferral

MHRA-101711-PIP01-24-M02

Scope of the Application

Active Substance(s)

IPTACOPAN HYDROCHLORIDE MONOHYDRATE

Condition(s)

Treatment of C3 glomerulopathy

Pharmaceutical Form(s)

Capsule, hard

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 09/06/2026 17:06 BST an application for a Modification

The procedure started on 26/06/2026 14:20 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-101711-PIP01-24-M02

Of 06/10/2026 17:55 BST

On the adopted decision for IPTACOPAN HYDROCHLORIDE MONOHYDRATE (MHRA-101711-PIP01-24-M02) 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 IPTACOPAN HYDROCHLORIDE MONOHYDRATE, Capsule, hard , 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, W127FQ

ANNEX I

1. Waiver

1.1 Condition:

Treatment of C3 glomerulopathy 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 solid dosage form Route(s) of administration: Oral use

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of C3 glomerulopathy

2.2 Indication(s) targeted by the PIP:

Treatment of C3 glomerulopathy

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

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	2	Study 2 8-week dose range-finding juvenile toxicity study. Study 3 (1870009) 52-week definitive juvenile toxicity study with 27-week recovery period in juvenile dogs.
Clinical Studies	2	Study 4 (CLNP023B12301) Randomised, placebo-controlled, 12-month study (6 months double-blind plus 6 months open-label) to evaluate the efficacy and safety of iptacopan compared to placebo in adolescent (and adult) patients with C3 glomerulopathy. Study 5 Open-label, single-arm study to assess the safety, tolerability and exposure of iptacopan in paediatric patients from 2 years to less than 12 years of age with C3 glomerulopathy.
Extrapolation, Modeling & Simulation Studies	2	Study 6 Modelling and simulation study to select dose in adolescents. Study 7 Modelling and simulation study for dose selection of Study 5 in children from 2 years to less than 12 years of age.
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:	31/07/2034
Deferral of one or more studies contained in the paediatric investigation plan:	Yes