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-M01

Scope of the Application

Active Substance(s)

IPTACOPAN HYDROCHLORIDE MONOHYDRATE

Condition(s)

Treatment of C3 glomerulopathy

Pharmaceutical Form(s)

Capsule, hard; 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 19/11/2024 03:54 GMT an application for a Modification

The procedure started on 03/12/2024 16:05 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.

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Final Decision Letter

MHRA-101711-PIP01-24-M01

Of 17/12/2024 09:43 GMT

On the adopted decision for IPTACOPAN HYDROCHLORIDE MONOHYDRATE (MHRA-101711-PIP01-24-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 IPTACOPAN HYDROCHLORIDE MONOHYDRATE, Capsule, hard; 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, London, UNITED KINGDOM, W12 7FQ

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 6 months of age. Pharmaceutical form(s): Capsule, hard; Age-appropriate oral solid dosage form Route(s) of administration: ORAL USE Reason for granting waiver: On the grounds the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible.

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 6 months to less than 18 years of age.

2.4 Pharmaceutical Form(s):

Capsule, hard 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.
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) Randomized, placebo-controlled, 12-month study (6 months doubleblind + 6 months open-label) to evaluate the efficacy and safety of iptacopan (LNP023) 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 6 months 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 6 months 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:	31/12/2029
Deferral of one or more studies contained in the paediatric investigation plan:	Yes