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-101289-PIP01-23-M01

Scope of the Application

Active Substance(s)

Remibrutinib

Condition(s)

Treatment of chronic spontaneous urticaria.

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 23/12/2023 04:42 GMT an application for a Modification

The procedure started on 11/04/2024 11:07 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-101289-PIP01-23-M01

Of 24/04/2024 08:08 BST

On the adopted decision for Remibrutinib (MHRA-101289-PIP01-23-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 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 chronic spontaneous urticaria. 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 the specific medicinal product is likely to be unsafe.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of chronic spontaneous urticaria.

2.2 Indication(s) targeted by the PIP:

Treatment of chronic spontaneous urticaria in adolescents and children 6 years of age and above with an inadequate response to H1-antihistamine treatment.

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	2	Study 1 Development of an appropriate strength of an oral solid dosage form for adolescents. Study 2 Development of an appropriate strength of an oral solid dosage form for children from 6 years to less than 12 years of age.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	2	Study 3 (CLOU064F12301) Double-blind, randomised, placebo-controlled trial to evaluate pharmacokinetics, safety and efficacy of LOU064 as add-on to best standard of care in adolescents from 12 years to less than 18 years of age with Chronic Spontaneous Urticaria (CSU) and inadequate response to H1-antihistamine therapy. Study 4 (CLOU064F12302) Open-label, uncontrolled trial to evaluate pharmacokinetics, activity and safety of LOU064 in children from 6 years to less than 12 years of age with Chronic Spontaneous Urticaria (CSU) and inadequate response to H1-antihistamine therapy.
Extrapolation, Modeling & Simulation Studies	4	Study 5 Modelling and Simulation Activity Part 1 (adolescents). Study 6 Modelling and Simulation Activity Part 2 (children). Study 7 Analysis of existing exposure, efficacy and safety data on LOU064 in adolescents

		from 12 years to less than 18 years of age with Chronic Spontaneous Urticaria (CSU). Study 8 Analysis of existing exposure, efficacy and safety data on LOU064 in children from 6 years to less than 12 years of age with Chronic Spontaneous Urticaria (CSU).
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:	31/08/2031
Deferral of one or more studies contained in the paediatric investigation plan:	Yes