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-100426-PIP01-22-M01

Scope of the Application

Active Substance(s)

ligelizumab

Condition(s)

Treatment of chronic spontaneous urticaria

Pharmaceutical Form(s)

Solution for injection; Age appropriate dosage form

Route(s) of Administration

Subcutaneous 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 28/01/2022 12:17 GMT an application for a Modification

The procedure started on 15/03/2022 15:30 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-100426-PIP01-22-M01

Of 29/03/2022 07:52 BST

On the adopted decision for ligelizumab (MHRA-100426-PIP01-22-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 ligelizumab, Solution for injection; Age appropriate dosage form , Subcutaneous 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 2 years of age Pharmaceutical form(s): Solution for injection; Age appropriate dosage form Route(s) of administration: Subcutaneous use, parenteral 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 chronic spontaneous urticaria

2.2 Indication(s) targeted by the PIP:

Treatment of chronic spontaneous urticaria in patients 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 2 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

Solution for injection; Age appropriate dosage form for parenteral use.

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Development of an appropriate strength of solution for injection for the paediatric population from 2 to less than 12 years of age.
Non-Clinical Studies	0	Not applicable
Clinical Studies	4	Study 2 (CQGE031C2202) Double-blind, randomised, placebo-controlled, parallel-group trial to evaluate pharmacokinetics, safety and activity of ligelizumab in children from 12 to less than 18 years of age with chronic spontaneous urticaria Study 3 Deleted during procedure EMEA-001811-PIP02-15-M02 Study 4 (CQGE031C2305) Open-label, uncontrolled trial to evaluate pharmacokinetics, safety and activity of ligelizumab in children from 2 to less than 12 years of age with chronic spontaneous urticaria Study 9 (CQGE031C2302) Added during procedure EMEA-001811-PIP02-15-M02 Double-blind, randomised, active and placebo-controlled, parallel-group trial to evaluate efficacy and safety of ligelizumab in children from 12 to less than 18 years of age (and adults) with chronic spontaneous urticaria Study 10 (CQGE031C2303) Added during procedure EMEA-001811-PIP02-15-M02 Double-blind, randomised, active and placebo-controlled,

		parallel-group trial to evaluate efficacy and safety of ligelizumab in children from 12 to less than 18 years of age (and adults) with chronic spontaneous urticaria.
Extrapolation, Modeling & Simulation Studies	5	Study 5 Modelling and simulation study to establish the dose of ligelizumab in Study 2. Study 6 Modelling and simulation study to establish the dose of ligelizumab in Study 4. Study 7 Modelling and simulation study to establish the dose of ligelizumab in children from 2 to less than 6 years of age in Study 4. Study 8 Extrapolation analysis of existing PK/PD data on ligelizumab on chronic spontaneous urticaria (children from 2 to less than 12 years of age). Study 11 Extrapolation analysis of existing PK/PD adolescent data on ligelizumab on chronic spontaneous urticaria.
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:	31/08/2027
Deferral of one or more studies contained in the paediatric investigation plan:	Yes