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 a paediatric investigation plan (MHRA-100382-PIP01-21)

MHRA-100382-PIP01-21-M01

Scope of the Application

Active Substance(s)

RAVULIZUMAB

Condition(s)

Treatment of myasthenia gravis

Pharmaceutical Form(s)

Concentrate for solution for infusion

Route(s) of Administration

INTRAVENOUS USE

Name / Corporate name of the PIP applicant

Alexion Europe SAS

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Alexion Europe SAS submitted to the licensing authority on 25/10/2023 12:57 BST an application for a Modification

The procedure started on 01/02/2024 14:17 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

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-100382-PIP01-21-M01

Of 04/03/2024 17:20 GMT

On the adopted decision for RAVULIZUMAB (MHRA-100382-PIP01-21-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 RAVULIZUMAB, Concentrate for solution for infusion , INTRAVENOUS USE .

This decision is addressed to Alexion Europe SAS, 103-105 rue Anatole France, Levallois-Perret, FRANCE, 92300

ANNEX I

1. Waiver

1.1 Condition:

Treatment of myasthenia gravis The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 6 years of age Pharmaceutical form(s): Concentrate for solution for infusion Route(s) of administration: INTRAVENOUS 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 myasthenia gravis

2.2 Indication(s) targeted by the PIP:

Treatment of anti-acetylcholine receptor (AChR) antibody-positive generalized myasthenia gravis (gMG)

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):

Concentrate for solution for infusion

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	1	Study 1 (ALXN1210-MG-30X) Open-label, multi-centre study to evaluate pharmacokinetics, pharmacodynamics, safety and efficacy of ravulizumab in paediatric patients from 6 years to less than 18 years of age with anti-acetylcholine receptor (AChR) antibody-positive generalised myasthenia gravis (gMG).
Extrapolation, Modeling & Simulation Studies	1	Study 2 Extrapolation study to evaluate, pharmacokinetics/ pharmacodynamics, safety and efficacy of ravulizumab in children from 6 years to less than 18 years of age with AChR-Ab positive generalised myasthenia gravis (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:	Yes
Date of completion of the paediatric investigation plan:	31/07/2027

Deferral of one or more studies contained in the paediatric investigation plan:	Yes