MHRA
10 South Colonnade
Canary Wharf
London
E14 4PU
United Kingdom

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Decision Cover Letter

Decision of the licensing authority to:

agree a paediatric investigation plan and grant a deferral and grant a waiver

MHRA-100382-PIP01-21

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 09/12/2021 15:35 GMT an application for a Paediatric Investigation Plan

The procedure started on 20/06/2022 10:47 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 agree a paediatric investigation plan and grant a deferral and grant a waiver

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

Of 07/07/2022 08:11 BST

On the adopted decision for RAVULIZUMAB (MHRA-100382-PIP01-21) 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 a paediatric investigation plan

This decision applies to a Paediatric Investigation Plan 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 generalized 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