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-100610-PIP01-22-M01

Scope of the Application

Active Substance(s)

ECULIZUMAB

Condition(s)

Treatment of myasthenia gravis

Pharmaceutical Form(s)

Concentration 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 13/07/2022 13:03 BST an application for a Modification

The procedure started on 16/01/2023 08:17 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 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-100610-PIP01-22-M01

Of 20/02/2023 13:42 GMT

On the adopted decision for ECULIZUMAB (MHRA-100610-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 ECULIZUMAB, Concentration 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): Concentration 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 refractory acetylcholine receptor antibody (AChR-Ab) - positive generalised myasthenia gravis

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):

Concentration 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 (ECU-MG-303) Open-label, multi-centre study to evaluate pharmacokinetics, safety and effect of eculizumab in paediatric patients from 6 years to less than 18 years of age with refractory AChR-Ab positive generalised myasthenia gravis and to confirm the selected paediatric dosing in the modelling and simulation study.
Extrapolation, Modeling & Simulation Studies	2	Study 2 Modelling and simulation study to evaluate the use and support dosing regimen of eculizumab in paediatric patients from 6 years to less than 18 years of age with refractory AChR-Ab positive generalised myasthenia gravis. Study 3 Extrapolation study to evaluate efficacy, pharmacokinetics/ pharmacodynamic and safety of eculizumab in paediatric patients from 6 years to less than 18 years of age with AChR-Ab positive generalised myasthenia gravis.
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:	31/07/2022
Deferral of one or more studies contained in the paediatric investigation plan:	Yes