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 grant a product specific waiver

MHRA-101520-PIP01-24-M01

Scope of the Application

Active Substance(s)

ECULIZUMAB

Condition(s)

Treatment of neuromyelitis optica spectrum disorders.

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 19/06/2024 15:05 BST an application for a Modification

The procedure started on 22/08/2024 19:37 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 grant a product specific 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-101520-PIP01-24-M01

Of 27/08/2024 10:12 BST

On the adopted decision for ECULIZUMAB (MHRA-101520-PIP01-24-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 and to grant a product specific waiver.

This decision applies to a Modification for ECULIZUMAB, Concentrate for solution for infusion , INTRAVENOUS .

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

ANNEX I

1. Waiver

1.1 Condition:

Treatment of neuromyelitis optica spectrum disorders. The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 18 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 the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Studies 1, 2, 3 and 4 were deleted during procedure MHRA-101520-PIP01-24-M01 and replaced by a full product specific waiver.

2.2 Indication(s) targeted by the PIP:

Not applicable.

2.3 Subset(s) of the paediatric population concerned by the paediatric development:

Not applicable.

2.4 Pharmaceutical Form(s):

Not applicable.

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	0	Not applicable.
Extrapolation, Modeling & Simulation Studies	0	Not applicable.
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:	
Date of completion of the paediatric investigation plan:	
Deferral of one or more studies contained in the paediatric investigation plan:	