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

gov.uk/mhra

Decision Cover Letter

Decision of the licensing authority to:

accept change(s) to the agreed paediatric investigation plan (MHRA-100445-PIP01-22) and to the deferral.

MHRA-100445-PIP01-22-M01

Scope of the Application

Active Substance(s)

RAVULIZUMAB

Condition(s)

Treatment of Neuromyelitis Optica Spectrum Disorder (NMOSD).

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 29/01/2024 11:03 GMT an application for a

The procedure started on 11/03/2024 19:26 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.

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

gov.uk/mhra

Final Decision Letter

MHRA-100445-PIP01-22-M01

Of 27/03/2024 14:43 GMT

On the adopted decision for RAVULIZUMAB (MHRA-100445-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 for RAVULIZUMAB, Concentrate for solution for infusion. , INTRAVENOUS .

This decision is addressed to Alexion Europe SAS, Alexion Europe SAS, Levallois-Perret, FRANCE, 92300

ANNEX I

1. Waiver

1.1 Condition:

Treatment of Neuromyelitis Optica Spectrum Disorder (NMOSD). The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 2 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):

Treatment of Neuromyelitis Optica Spectrum Disorder (NMOSD).

2.2 Indication(s) targeted by the PIP:

Treatment of aquaporin-4 antibody-positive neuromyelitis optica spectrum disorder.

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):

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-NMO-317) Open-label, historical-controlled trial to evaluate efficacy, pharmacokinetics, pharmacodynamics, and safety of ravulizumab in children and adolescents from 2 years to less than 18 years of age with anti-aquaporin-4 antibody-positive [AQP4-Ab (+)] neuromyelitis optica spectrum disorder (NMOSD).
Extrapolation, Modeling & Simulation Studies	1	Study 2 Extrapolation study to evaluate the pharmacokinetics (PK) / pharmacodynamics (PD), safety and efficacy of ravulizumab in paediatric patients with AQP4-Ab (+) NMOSD from 2 years to less than 18 years of age.
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:	30/06/2026
Deferral of one or more studies contained in the paediatric investigation plan:	Yes