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 (MHRA-100214-PIP01-21-M04) and to the deferral

MHRA-100214-PIP01-21-M05

Scope of the Application

Active Substance(s)

RAVULIZUMAB

Condition(s)

Treatment of atypical haemolytic uraemic syndrome (aHUS)

Pharmaceutical Form(s)

Concentrate for solution for Injection

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 20/01/2025 11:43 GMT an application for a Modification

The procedure started on 17/02/2025 17:50 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-100214-PIP01-21-M05

Of 08/05/2025 09:35 BST

On the adopted decision for RAVULIZUMAB (MHRA-100214-PIP01-21-M05) 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 Injection , 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:

Not applicable.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of atypical haemolytic uremic syndrome (aHUS).

2.2 Indication(s) targeted by the PIP:

Treatment of atypical haemolytic uremic syndrome.

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

The paediatric population from birth 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	Study 4 Deleted during procedure MHRA-100214-PIP01-21-M05.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	1	Study 1 Deleted during procedure EMEA-001943-PIP01-16-M01. Study 2 (ALXN1210-aHUS-312) Open-label, single arm study to evaluate pharmacokinetics, pharmacodynamics, efficacy and safety of ravulizumab in children from birth to less than 18 years of age with aHUS. Study 5 (ALXN1210-PED-316) Deleted during procedure MHRA-100214-PIP01-21-M05.
Extrapolation, Modeling & Simulation Studies	1	Study 3 Modelling and simulation study to evaluate the use of intravenous ravulizumab in children from birth to less than 18 years of age. Same as Study 2 in MHRA-100213-PIP01-21-M01 and subsequent modifications. Study 6 Deleted during procedure MHRA-100214-PIP01-21-M05. Study 7 Deleted during procedure MHRA-100214-PIP01-21-M05.
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:	31/12/2020

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