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 changes to the agreed paediatric investigation plan and to the deferral

MHRA-100214-PIP01-21-M03

Scope of the Application

Active Substance(s)

RAVULIZUMAB

Condition(s)

atypical haemolytic uremic syndrome (aHUS)

Pharmaceutical Form(s)

Solution for injection; Concentrate for solution for infusion

Route(s) of Administration

SUBCUTANEOUS USE; 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 14/09/2022 09:50 BST an application for a Modification

The procedure started on 30/11/2022 12:01 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 changes 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-M03

Of 13/12/2022 10:22 GMT

On the adopted decision for RAVULIZUMAB (MHRA-100214-PIP01-21-M03) 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, Solution for injection; Concentrate for solution for infusion , SUBCUTANEOUS USE, PARENTERAL .

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 (aHUS)

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

All subsets of the paediatric population from birth to less than 18 years of age

2.4 Pharmaceutical Form(s):

Solution for injection Concentrate for solution for infusion

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 4 Development of an age-appropriate subcutaneous formulation
Non-Clinical Studies	0	Not applicable
Clinical Studies	2	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) Open-label multicentre, study to evaluate pharmacokinetics, pharmacodynamics, efficacy and safety of ravulizumab following subcutaneous administration in children from 2 years to less than 18 years of age with atypical haemolytic uraemic syndrome (aHUS) or PNH.
Extrapolation, Modeling & Simulation Studies	3	Study 3 Modelling and simulation study to evaluate the use of intravenous ravulizumab in children from birth to less than 18 years of age. Study 6 Modelling and simulation study to evaluate the use of subcutaneous ravulizumab in children from 2 years to less than 18 years of age in aHUS and PNH. Study 7 Extrapolation study to evaluate the use of subcutaneous ravulizumab in children from 2 years to less than 18 years of age with aHUS or PNH.
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:	31/12/2023
Deferral of one or more studies contained in the paediatric investigation plan:	Yes