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

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Decision Cover Letter

Decision of the licensing authority to:

agree a paediatric investigation plan and grant a deferral and grant a waiver

MHRA-101727-PIP01-24

Scope of the Application

Active Substance(s)

RAVULIZUMAB

Condition(s)

Treatment of primary immunoglobulin A nephropathy

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 18/12/2024 10:00 GMT an application for a Paediatric Investigation Plan

The procedure started on 13/01/2025 15:01 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 agree a paediatric investigation plan and grant a deferral and grant a 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-101727-PIP01-24

Of 04/06/2025 10:41 BST

On the adopted decision for RAVULIZUMAB (MHRA-101727-PIP01-24) 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 a paediatric investigation plan

This decision applies to a Paediatric Investigation Plan for RAVULIZUMAB, Concentrate 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 primary immunoglobulin A nephropathy 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 primary immunoglobulin A nephropathy

2.2 Indication(s) targeted by the PIP:

Treatment of primary immunoglobulin A nephropathy (IgAN)

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 Open-label, uncontrolled, single-arm, multicentre study to evaluate pharmacokinetics, pharmacodynamics, safety, and efficacy of ravulizumab in children and adolescents from 2 years to less than 18 years of age with primary immunoglobulin A nephropathy (IgAN).
Extrapolation, Modeling & Simulation Studies	2	Study 2 Modelling and simulation study to confirm the paediatric doses for use in children and adolescents from 2 years to less than 18 years of age with IgAN. Extrapolation Plan Study 1 and Study 2 are part of the extrapolation plan covering the paediatric population from 2 years to less than 18 years of age with primary IgAN.
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:	No
Date of completion of the paediatric investigation plan:	31/08/2029
Deferral of one or more studies contained in the paediatric investigation plan:	Yes