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 waiver

MHRA-101430-PIP01-24

Scope of the Application

Active Substance(s)

RAVULIZUMAB

Condition(s)

Treatment in haematopoietic stem cell transplant

Pharmaceutical Form(s)

Concentrate for solution for infusion; solution for injection

Route(s) of Administration

INTRAVENOUS

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 29/04/2024 20:28 BST an application for a Paediatric Investigation Plan

The procedure started on 02/05/2025 13:10 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 agree a paediatric investigation plan 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-101430-PIP01-24

Of 03/03/2025 11:45 GMT

On the adopted decision for RAVULIZUMAB (MHRA-101430-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; 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:

Treatment in haematopoietic stem cell transplant

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment in haematopoietic stem cell transplant

2.2 Indication(s) targeted by the PIP:

Treatment of paediatric patients with haematopoietic stem cell transplant associated thrombotic microangiopathy (HSCT-TMA)

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

The paediatric population from 28 days to less than 18 years

2.4 Pharmaceutical Form(s):

Concentrate for solution for infusion; solution for injection

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not Applicable
Non-Clinical Studies	0	Not Applicable
Clinical Studies	2	Study 1 Open-label, single-arm trial to evaluate efficacy, safety, pharmacokinetics and pharmacodynamics of ravulizumab as add-on to best supportive care (BSC) in paediatric patients from 28 days to less than 18 years of age with thrombotic microangiopathy (TMA) after haematopoietic stem cell transplantation (HSCT) Study 2 Double-blind, randomised, placebo-controlled trial to evaluate efficacy and safety of ravulizumab in adolescents from 12 years to less than 18 years of age (and adults) with thrombotic microangiopathy (TMA) after haematopoietic stem cell transplantation (HSCT)
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:	No
Date of completion of the paediatric investigation plan:	29/11/2024
Deferral of one or more studies contained in the paediatric investigation plan:	No