

**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-100213-PIP01-21-M04) and to the deferral.

MHRA-100213-PIP01-21-M05

### **Scope of the Application**

#### **Active Substance(s)**

RAVULIZUMAB

#### **Condition(s)**

Treatment of paroxysmal nocturnal haemoglobinuria

#### **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 21/01/2025 10:56 GMT an application for a Modification

The procedure started on 05/03/2025 17:03 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-100213-PIP01-21-M05

Of 08/05/2025 09:34 BST

On the adopted decision for RAVULIZUMAB (MHRA-100213-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 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:

Not applicable.

### 2. Paediatric Investigation Plan:

#### 2.1 Condition(s):

Treatment of paroxysmal nocturnal haemoglobinuria (PNH).

#### 2.2 Indication(s) targeted by the PIP:

Treatment of paroxysmal nocturnal haemoglobinuria.

### 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 5 Deleted during procedure MHRA-100213-PIP01-21-M05.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	2	Study 1 (ALXN1210-PNH-304) Open-label multicentre, single arm trial to evaluate pharmacokinetic (PK) and pharmacodynamic (PD) parameters, efficacy and safety of ravulizumab in children from birth to less than 18 years of age with PNH. Study 6 (ALXN1210-PED-316) Deleted during procedure MHRA-100213-PIP01-21-M05.
Extrapolation, Modeling & Simulation Studies	5	Study 2 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 3 in MHRA-100214-PIP01-21-M01 and any subsequent modifications. Study 3 Extrapolation study to evaluate the efficacy, PK/PD and safety of ravulizumab in paediatric PNH patients from 12 years to less than 18 years of age. Study 4 Extrapolation study to evaluate the efficacy, PK/PD and safety of ravulizumab in paediatric PNH patients from birth to less than 12 years of age. Study 7 Deleted during procedure MHRA-100213-PIP01-21-M05. Study 8 Deleted during procedure MHRA-100213-PIP01-21-M05.
Other Studies	0	Not applicable.
Other Measures	0	Not applicable.

### 3. Follow-up, completion and deferral of a PIP:

<b>Concerns on potential long term safety and efficacy issues in relation to paediatric use:</b>	Yes
<b>Date of completion of the paediatric investigation plan:</b>	31/03/2022
<b>Deferral of one or more studies contained in the paediatric investigation plan:</b>	Yes