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-101781-PIP01-25

Scope of the Application

Active Substance(s)

pozelimab

Condition(s)

Treatment of paroxysmal nocturnal haemoglobinuria

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

Regeneron UK Ltd.

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Regeneron UK Ltd. submitted to the licensing authority on 07/03/2025 10:48 GMT an application for a Paediatric Investigation Plan

The procedure started on 11/04/2025 08:14 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 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-101781-PIP01-25

Of 08/07/2025 07:56 BST

On the adopted decision for pozelimab (MHRA-101781-PIP01-25) 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 pozelimab, Solution for injection, Concentrate for solution for infusion , SUBCUTANEOUS USE; INTRAVENOUS USE .

This decision is addressed to Regeneron UK Ltd., The Charter Building, Vine Street, Uxbridge , London, UNITED KINGDOM, UB8 1JG

ANNEX I

1. Waiver

1.1 Condition:

Treatment of paroxysmal nocturnal haemoglobinuria The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 2 years of age Pharmaceutical form(s): Solution for injection Concentrate for solution for infusion Route(s) of administration: SUBCUTANEOUS USE 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 paroxysmal nocturnal haemoglobinuria

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 2 years 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	0	Not applicable.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	1	Study 1 Open-label, single arm trial to evaluate pharmacokinetics/ pharmacodynamics, safety and descriptive efficacy of cemdisiran given in combination with pozelimab in children from 2 years to less than 18 years of age with paroxysmal nocturnal haemoglobinuria (PNH).
Extrapolation, Modeling & Simulation Studies	1	Study 2 Modelling and simulation dose finding study.
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:	30/06/2030
Deferral of one or more studies contained in the paediatric investigation plan:	Yes