MHRA
10 South Colonnade
Canary Wharf
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United Kingdom

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Decision Cover Letter

Decision of the licensing authority to:

accept change(s) to the agreed paediatric investigation plan (EMEA-002600-PIP01-19) and to the deferral

MHRA-101720-PIP01-24-M01

Scope of the Application

Active Substance(s)

PEGCETACOPLAN

Condition(s)

Treatment of paroxysmal nocturnal haemoglobinuria

Pharmaceutical Form(s)

Solution for infusion

Route(s) of Administration

SUBCUTANEOUS USE

Name / Corporate name of the PIP applicant

Swedish Orphan Biovitrum AB

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Swedish Orphan Biovitrum AB submitted to the licensing authority on 29/11/2024 12:33 GMT an application for a Modification

The procedure started on 13/01/2025 14:18 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-101720-PIP01-24-M01

Of 01/04/2025 09:56 BST

On the adopted decision for PEGCETACOPLAN (MHRA-101720-PIP01-24-M01) 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 PEGCETACOPLAN, Solution for infusion , SUBCUTANEOUS USE .

This decision is addressed to Swedish Orphan Biovitrum AB, Norra Stationsgatan 93A, Stockholm, SWEDEN, 11276

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 12 years of age Pharmaceutical form(s): Solution for infusion Route(s) of administration: SUBCUTANEOUS USE Reason for granting waiver: on the grounds that the specific medicinal product is likely to be unsafe.

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 12 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

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 (APL2-PNH-209) Open-label, multiple dose trial to evaluate pharmacokinetics, safety and activity of pegcetacoplan in children from 12 years to less than 18 years of age with anaemia due to paroxysmal nocturnal haemoglobinuria (PNH) who are treatment naïve or who remain anaemic despite treatment with a complement inhibitor.
Extrapolation, Modeling & Simulation Studies	2	Study 2 (APL2-PNH-003) Use of population pharmacokinetic (PK) model to analyse PK data collected in paediatric studies to inform dosing recommendation in paediatric subjects. Study 3 (APL2-PNH-004) Use of population PK/pharmacodynamic (PD) and exposure-response (E-R) model of existing in-house clinical data on pegcetacoplan to support efficacy assumptions in the paediatric population based on extrapolation.
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:	31/07/2029
Deferral of one or more studies contained in the paediatric investigation plan:	Yes