MHRA
10 South Colonnade
Canary Wharf
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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-101671-PIP01-24

Scope of the Application

Active Substance(s)

PEGCETACOPLAN

Condition(s)

Treatment of glomerulonephritis and nephrotic syndrome

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 22/11/2024 11:21 GMT an application for a Paediatric Investigation Plan

The procedure started on 13/01/2025 12:15 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-101671-PIP01-24

Of 13/03/2025 17:02 GMT

On the adopted decision for PEGCETACOPLAN (MHRA-101671-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 PEGCETACOPLAN, Solution for infusion , SUBCUTANEOUS USE .

This decision is addressed to Swedish Orphan Biovitrum AB, Tomtebodavägen 23A, Solna, SWEDEN, 11276

ANNEX I

1. Waiver

1.1 Condition:

Treatment of glomerulonephritis and nephrotic syndrome 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 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 glomerulonephritis and nephrotic syndrome

2.2 Indication(s) targeted by the PIP:

Treatment of primary C3 glomerulopathy Treatment of primary immune-complex membranoproliferative glomerulonephritis

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 infusion

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable.
Non-Clinical Studies	2	Study 1 (23CATX001) Dose range-finding juvenile toxicity study in rats. Study 2 (24CATX001) Definitive juvenile toxicity study in rats.
Clinical Studies	2	Study 3 (APL2-C3G-310) Randomised, placebo-controlled, double-blind, multicentre study, to assess pharmacokinetics, efficacy and safety of pegcetacoplan in adolescents from 12 years to less than 18 years of age (and in adults) with glomerulonephritis and nephrotic syndrome, with a 26-week open-label period to evaluate durability of response,pharmacokinetics and long-term safety and efficacy. Study 4 Open-label, historical controlled, single-arm, multicentre study to assess pharmacokinetics, efficacy and safety of pegcetacoplan in children from 2 years to less than 12 years of age with glomerulonephritis and nephrotic syndrome.
Extrapolation, Modeling & Simulation Studies	2	Study 5 Population pharmacokinetic (PK) model for selection of dose regimens of pegcetacoplan for children from 2 years to less than 18 years of age with glomerulonephritis and nephrotic syndrome. Study 6 Extrapolation study to combine

		population PK and exposure-response models derived from clinical data on pegcetacoplan to support pharmacokinetic, pharmacodynamic, and efficacy assumptions in the paediatric population with glomerulonephritis and nephrotic syndrome.
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:	30/09/2031
Deferral of one or more studies contained in the paediatric investigation plan:	Yes