EMA/434697/2024

European Medicines Agency decision

P/0348/2024

of 24 September 2024

on the acceptance of a modification of an agreed paediatric investigation plan for pegcetacoplan (Aspaveli), (EMEA-002600-PIP03-21-M03) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

Disclaimer

This decision does not constitute entitlement to the rewards and incentives referred to in Title V of Regulation (EC) No 1901/2006.

Only the English text is authentic.

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The European Medicines Agency,

Having regard to the Treaty on the Functioning of the European Union,

Having regard to Regulation (EC) No 1901/2006 of the European Parliament and of the Council of 12 December 2006 on medicinal products for paediatric use and amending Regulation (EEC) No. 1768/92, Directive 2001/20/EC, Directive 2001/83/EC and Regulation (EC) No 726/2004[1],

Having regard to Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency[2],

Having regard to the European Medicines Agency's decision P/0284/2022 issued on 11 August 2022, the decision P/0030/2024 issued on 31 January 2024 and the decision P/0195/2024 issued on 14 June 2024,

Having regard to the application submitted by Swedish Orphan Biovitrum AB (publ) on 3 June 2024 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 6 September 2024, in accordance with Article 22 of Regulation (EC) No 1901/2006,

Having regard to Article 25 of Regulation (EC) No 1901/2006,

Whereas:

(1) The Paediatric Committee of the European Medicines Agency has given an opinion on the acceptance of changes to the agreed paediatric investigation plan.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

[1] OJ L 378, 27.12.2006, p.1, as amended.
[2] OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for pegcetacoplan (Aspaveli), solution for infusion, subcutaneous use, are hereby accepted in the scope set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices.

Article 2

This decision covers all conditions, indications, pharmaceutical forms, routes of administration, measures, timelines, waivers and deferrals, as agreed in the decision P/0149/2020 issued on 17 April 2020, including subsequent modifications thereof.

Article 3

This decision is addressed to Swedish Orphan Biovitrum AB (publ), Swedish Orphan Biovitrum AB (publ), SE-112 76 – Solna, Sweden.

EMA/PDCO/276116/2024 Corr[1]
Amsterdam, 6 September 2024

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan

EMEA-002600-PIP03-21-M03

Scope of the application

Active substance(s):

Pegcetacoplan

Invented name and authorisation status:

See Annex II

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 (publ)

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Swedish Orphan Biovitrum AB (publ) submitted to the European Medicines Agency on 3 June 2024 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/0284/2022 issued on 11 August 2022, the decision P/0030/2024 issued on 31 January 2024 and the decision P/0195/2024 issued on 14 June 2024.

The application for modification proposed changes to the agreed paediatric investigation plan.

The procedure started on 8 July 2024.

[1] 18 September 2024

Scope of the modification

Some measures and timelines of the Paediatric Investigation Plan have been modified.

Opinion

1. The Paediatric Committee, having assessed the application in accordance with Article 22 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree to changes to the paediatric investigation plan in the scope set out in the Annex I of this opinion.

   The Paediatric Committee member of Norway agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the paediatric investigation plan and the subset(s) of the paediatric population and condition(s) covered by the waiver are set out in the Annex I.

This opinion is forwarded to the applicant and the Executive Director of the European Medicines Agency, together with its annexes and appendix.

Annex I

The subset(s) of the paediatric population and condition(s) covered by the waiver and the measures and timelines of the agreed paediatric investigation plan (PIP)

1. Waiver

1.1. Condition:

Treatment of glomerulonephritis and nephrotic syndrome

The waiver applies to:

- the paediatric population from birth to less than 2 years of age;
- solution for infusion, subcutaneous use;
- on the grounds that the specific medicinal product is likely to be unsafe.

2. Paediatric investigation plan

2.1. Condition:

Treatment of glomerulonephritis and nephrotic syndrome

2.1.1. Indication(s) targeted by the PIP

Treatment of primary C3 glomerulopathy

Treatment of primary immune-complex membranoproliferative glomerulonephritis

2.1.2. Subset(s) of the paediatric population concerned by the paediatric development

From 2 years to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Solution for infusion

2.1.4. Measures

Area	Description
Quality-related studies	Not applicable.
Non-clinical studies	**Study 1:** Dose-range finding juvenile toxicity study in rats. **Study 2:** Definitive juvenile toxicity study in rats.
Clinical studies	**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, modelling and simulation studies	**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 pharmacokinetics (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	Not applicable.
Other measures	Not applicable.

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By September 2031
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

Condition(s) and authorised indication(s)

1. Treatment of paroxysmal nocturnal haemoglobinuria

Authorised indication(s):

- ASPAVELI is indicated as monotherapy in the treatment of adult patients with paroxysmal nocturnal haemoglobinuria (PNH) who have haemolytic anaemia.
  - Invented name: Aspaveli
  - Authorised pharmaceutical form(s): Solution for infusion.
  - Authorised route(s) of administration: Subcutaneous use.
  - Authorised via centralised procedure.