EMADOC-1700519818-1810950

European Medicines Agency decision

EMA/PE/0000224283

of 3 January 2025

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

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/0149/2020 issued on 17 April 2020, the decision P/0210/2021 issued on 10 May 2021 and the decision P/0099/2024 issued on 5 April 2024,

Having regard to the application submitted by Swedish Orphan Biovitrum AB (publ) on 7 August 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 15 November 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 and to the deferral.

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

[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), including changes to the deferral, 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 is addressed to Swedish Orphan Biovitrum AB (publ), 112 76 – Stockholm, Sweden.

EMADOC-1700519818-1658230
Amsterdam, 15 November 2024

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

EMA/PE/0000224283

Scope of the application

Active substance(s):

Pegcetacoplan

Invented name and authorisation status:

See Annex II

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 (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 7 August 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/0149/2020 issued on 17 April 2020, the decision P/0210/2021 issued on 10 May 2021 and the decision P/0099/2024 issued on 5 April 2024.

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

The procedure started on 16 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 and to the deferral 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 paroxysmal nocturnal haemoglobinuria

The waiver applies to:

- the paediatric population from birth to less than 12 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 paroxysmal nocturnal haemoglobinuria

2.1.1. Indication(s) targeted by the PIP

Treatment of paroxysmal nocturnal haemoglobinuria

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

From 12 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	Not applicable
Clinical studies	**Study 1 (APL2-PNH-209)** Open-label, multiple dose trial to evaluate pharmacokinetics, safety and activity of pegcetacoplan in children from 12 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, modelling and simulation studies	**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	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 July 2029
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(s): Aspaveli
  - Authorised pharmaceutical form(s): solution for infusion
  - Authorised route(s) of administration: subcutaneous use
  - Authorised via centralised procedure