EMA/218859/2021

European Medicines Agency decision

P/0210/2021

of 10 May 2021

on the acceptance of a modification of an agreed paediatric investigation plan for pegcetacoplan, (EMEA-002600-PIP01-19-M01) 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 Community procedures for the authorisation and supervision of medicinal products for human and veterinary 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,

Having regard to the application submitted by Apellis Ireland Limited on 21 December 2020 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 26 March 2021, 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.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for pegcetacoplan, 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 is addressed to Apellis Ireland Limited, 10 Earlsfort Terrace, D02 T380 - Dublin 2, Ireland.

EMA/PDCO/5019/2021
Amsterdam, 26 March 2021

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

EMEA-002600-PIP01-19-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:

Apellis Ireland Limited

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Apellis Ireland Limited submitted to the European Medicines Agency on 21 December 2020 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 application for modification proposed changes to the agreed paediatric investigation plan

The procedure started on 26 January 2021.

Scope of the modification

Some measures 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 Norwegian Paediatric Committee member 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 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 annex 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	Number of measures	Description
Quality-related studies	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 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	2	**Study 2 (APL2-PNH-003)** Use of population pharmacokinetic (PK) model to analyse PK data

simulation studies		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 PIP

Concerns on potential long-term safety/efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By October 2024
Deferral for one or more measures contained in the paediatric investigation plan:	Yes