



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

EMA/173232/2020

## European Medicines Agency decision P/0149/2020

of 17 April 2020

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for pegcetacoplan (EMEA-002600-PIP01-19) 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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# European Medicines Agency decision

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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<sup>1</sup>,

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<sup>2</sup>,

Having regard to the application submitted by Apellis Ireland Limited on 23 April 2019 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said regulation and a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 28 February 2020, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 21 of said Regulation and Article 13 of said Regulation,

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 agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver.
- (2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.
- (3) It is therefore appropriate to adopt a decision granting a deferral.
- (4) It is therefore appropriate to adopt a decision granting a waiver.

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<sup>1</sup> OJ L 378, 27.12.2006, p.1.

<sup>2</sup> OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

**Article 1**

A paediatric investigation plan for pegcetacoplan, solution for infusion, subcutaneous use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby agreed.

**Article 2**

A deferral for pegcetacoplan, solution for infusion, subcutaneous use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

**Article 3**

A waiver for pegcetacoplan, solution for infusion, subcutaneous use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

**Article 4**

This decision is addressed to Apellis Ireland Limited, 10 Earlsfort Terrace, D02 T380 - Dublin 2, Ireland.



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

EMA/PDCO/686195/2019  
Amsterdam, 28 February 2020

## Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan and a deferral and a waiver

EMA-002600-PIP01-19

### 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 16(1) of Regulation (EC) No 1901/2006 as amended, Apellis Ireland Limited submitted for agreement to the European Medicines Agency on 23 April 2019 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 28 May 2019.

Supplementary information was provided by the applicant on 27 November 2019. The applicant proposed modifications to the paediatric investigation plan and to the waiver.



## Opinion

1. The Paediatric Committee, having assessed the proposed paediatric investigation plan in accordance with Article 17 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

- to agree the paediatric investigation plan in accordance with Article 17(1) of said Regulation;
- to grant a deferral in accordance with Article 21 of said Regulation;
- to grant a waiver for one or more subsets of the paediatric population in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(a) of said Regulation, on the grounds that the specific medicinal product is likely to be ineffective or unsafe in part or all of the paediatric population.

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

2. The measures and timelines of the agreed 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	Number of measures	Description
Quality-related studies	0	Not applicable.
Non-clinical studies	0	Not applicable.
Clinical studies	1	<b>Study 1 (APL2-PNH-209)</b> 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.

<b>Area</b>	<b>Number of measures</b>	<b>Description</b>
Extrapolation, modelling and simulation studies	2	<p><b>Study 2 (APL2-PNH-003)</b></p> <p>Use of population pharmacokinetic (PK) model to analyse PK data collected in paediatric studies to inform dosing recommendation in paediatric subjects.</p> <p><b>Study 3 (APL2-PNH-004)</b></p> <p>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.</p>
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