EMA/98104/2024

European Medicines Agency decision

P/0101/2024

of 12 April 2024

on the acceptance of a modification of an agreed paediatric investigation plan for afamelanotide (Scenesse), (EMEA-000737-PIP02-11-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/0292/2011 issued on 2 December 2011, and the decision P/0060/2023 issued on 3 February 2023,

Having regard to the application submitted by Clinuvel Europe Limited on 20 November 2023 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 23 February 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 afamelanotide (Scenesse), implant, age-appropriate prolonged release formulation, 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 Clinuvel Europe Limited, 10 Earlsfort Terrace, D02 T380 - Dublin 2, Ireland.

EMA/PDCO/539785/2023
Amsterdam, 23 February 2024

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

EMEA-000737-PIP02-11-M03

Scope of the application

Active substance(s):

Afamelanotide

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of erythropoietic protoporphyria

Pharmaceutical form(s):

Implant

Age-appropriate prolonged release formulation

Route(s) of administration:

Subcutaneous use

Name/corporate name of the PIP applicant:

Clinuvel Europe Limited

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Clinuvel Europe Limited submitted to the European Medicines Agency on 20 November 2023 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0292/2011 issued on 2 December 2011, and the decision P/0060/2023 issued on 3 February 2023.

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

The procedure started on 3 January 2024.

Scope of the modification

Some 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 erythropoietic protoporphyria

The waiver applies to:

- all subsets of the paediatric population from birth to less than 2 years of age;
- for implant, subcutaneous use, age appropriate prolonged release formulation, subcutaneous use;
- on the grounds that clinical studies cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the paediatric population.

2. Paediatric Investigation Plan

2.1. Condition: treatment of erythropoietic protoporphyria

2.1.1. Indication(s) targeted by the PIP

Treatment of erythropoietic protoporphyria

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)

Implant

Age appropriate prolonged release formulation

2.1.4. Studies

Area	Description
Quality	Study 1. Development of age appropriate prolonged release formulation for subcutaneous use.
Non-clinical	Study 2. Juvenile repeat-dose toxicity study in rats followed by 4-week recovery.
Clinical	Study 3. Comparative study to evaluate the pharmacokinetics of afamelanotide and the pharmacodynamic response to afamelanotide between subcutaneous administration of solid implant and the age appropriate prolonged release formulation in healthy adults. Study 4. Open-label, multicentre, multiple dose, dose-escalation pharmacokinetic and pharmacodynamic study of afamelanotide age appropriate prolonged release formulation in children from 6 to less than 18 years with erythropoietic protoporphyria. Study 5. Open-label, multicentre, multiple dose, dose-escalation pharmacokinetic and pharmacodynamic study of afamelanotide age appropriate prolonged release

	formulation in children from 2 to less than 6 years with erythropoietic protoporphyria. Study 6. Placebo controlled, randomised, double-blind safety, pharmacodynamics and efficacy trial of afamelanotide age appropriate prolonged release formulation in children from 6 to less than 18 years with erythropoietic protoporphyria, and with an open-label active-only arm in children from 2 to less than 6 years, with 12 month open-label extension to evaluate safety.

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By March 2028
Deferral for one or more studies contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

Condition and authorised indication

1. Prevention of phototoxicity in erythropoietic protoporphyria (EPP)

Authorised indication:

- Scenesse is indicated for prevention of phototoxicity in adult patients with erythropoietic protoporphyria (EPP).
  - Invented name: Scenesse
  - Authorised pharmaceutical form(s): implant
  - Authorised route of administration: subcutaneous use
  - Authorised via centralised procedure.