EMA/927100/2011

European Medicines Agency decision

P/292/2011

of 2 December 2011

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for afamelanotide (EMEA-000737-PIP02-11) 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 application submitted by Clinuvel (UK) Limited on 10 May 2011 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 11 November 2011, in accordance with Article 18 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.

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

Has adopted this decision:

Article 1

A paediatric investigation plan for afamelanotide, implant, age appropriate prolonged release formulation, 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 afamelanotide, implant, age appropriate prolonged release formulation, 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 afamelanotide, implant, age appropriate prolonged release formulation, 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 Clinuvel (UK) Limited, The Broadgate Tower, Third Floor, 20 Primrose St, EC2A 2RS – London, United Kingdom.

Done at London, 2 December 2011

For the European Medicines Agency
Guido Rasi
Executive Director
(Signature on file)

EMA/PDCO/760687/2011

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

EMEA-000737-PIP02-11

Scope of the application

Active substance(s):

Afamelanotide

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 (UK) Limited

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Clinuvel (UK) Limited submitted for agreement to the European Medicines Agency on 10 May 2011 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 15 June 2011.

Supplementary information was provided by the applicant on 26 August 2011. The applicant proposed modifications to the paediatric investigation plan and withdrew its request for a waiver.

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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 18 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 on its own motion in accordance with Article 12 of said Regulation and concluded in accordance with Article 11(1)(c) of said Regulation, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

   The Icelandic and the Norwegian Paediatric Committee members agree with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the agreed paediatric investigation plan 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.

London, 11 November 2011

On behalf of the Paediatric Committee
Dr Daniel Brasseur, Chairman
(Signature on file)

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

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	Number of studies	Description
Quality	1	Study 1. Development of age appropriate prolonged release formulation for subcutaneous use.
Non-clinical	1	Study 2. Juvenile repeat-dose toxicity study in rats followed by 4-week recovery.
Clinical	4	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.

Area	Number of studies	Description
		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 June 2022
Deferral for one or more studies contained in the paediatric investigation plan:	Yes