EMA/724437/2017

European Medicines Agency decision

P/0364/2017

of 1 December 2017

on the acceptance of a modification of an agreed paediatric investigation plan for palovarotene (EMEA-001662-PIP01-14-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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on the acceptance of a modification of an agreed paediatric investigation plan for palovarotene (EMEA-001662-PIP01-14-M01) 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 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/0127/2015 issued on 5 June 2015,

Having regard to the application submitted by Clementia Pharmaceuticals Inc. on 24 July 2017 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan and proposing a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 13 October 2017, in accordance with Article 22 of Regulation (EC) No 1901/2006, and Article 21 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 acceptance of changes to the agreed paediatric investigation plan and on the granting of a deferral.

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

(3) It is therefore appropriate to adopt a decision on the granting of a deferral.

[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 palovarotene, capsule, hard, oral 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

A deferral for palovarotene, capsule, hard, oral use, the details of which are set out in the opinion of the Paediatric Committee the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 3

This decision is addressed to Clementia Pharmaceuticals Inc., 4150 Ste-Catherine Street West, Suite 550, H3Z 2Y5 – Montreal, Canada.

EMA/PDCO/501582/2017
London, 13 October 2017

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

EMEA-001662-PIP01-14-M01

Scope of the application

Active substance(s):

Palovarotene

Condition(s):

Treatment of fibrodysplasia ossificans progressiva

Pharmaceutical form(s):

Capsule, hard

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Clementia Pharmaceuticals Inc.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Clementia Pharmaceuticals Inc. submitted to the European Medicines Agency on 24 July 2017 an application for modification of the agreed paediatric investigation plan as set out in the European Medicines Agency's decision P/0127/2015 issued on 5 June 2015.

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

The procedure started on 15 August 2017.

Scope of the modification

Some measures and timelines of the Paediatric Investigation Plan have been modified.

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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;
   - to grant a deferral, the details of which are 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 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

Not applicable

2. Paediatric investigation plan

2.1. Condition:

Treatment of fibrodysplasia ossificans progressiva

2.1.1. Indication(s) targeted by the PIP

Treatment of fibrodysplasia ossificans progressiva

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

From birth to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Capsule, hard

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable
Non-clinical studies	2	**Study 1** Dose range-finding juvenile toxicity study (9000317) **Study 2** Juvenile toxicity study (6700132)
Clinical studies	4	**Study 3** Multicentre, randomized, double-blind, placebo-controlled dose-finding, proof-of-concept study to evaluate the ability of different doses of palovarotene to prevent heterotopic ossification (HO) at the flare-up site in subjects with fibrodysplasia ossificans progressiva (FOP) (PVO-1A-201) **Study 4** Multicentre, open-label study to evaluate the safety and activity of different palovarotene dosing regimens in subjects with FOP in three parts (Part A, B and C) (PVO-1A-202)

		Study 5 Multicentre, open label study to evaluate the efficacy of palovarotene in decreasing heterotopic ossification (HO) as compared to untreated subjects from study 6 and to evaluate the safety of palovarotene in subjects with FOP (PVO-1A-301) **Study 6** Longitudinal, non-interventional two-part (A and B) study to determine the natural course of the disease for 36 months in subjects with FOP (PVO-1A-001)
Extrapolation, modelling and simulation studies	0	Not applicable
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 June 2023
Deferral for one or more measures contained in the paediatric investigation plan:	Yes