EMA/355150/2015

European Medicines Agency decision

P/0127/2015

of 5 June 2015

on the agreement of a paediatric investigation plan for palovarotene (EMEA-001662-PIP01-14) 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 Clementia Pharmaceuticals Inc. on 7 August 2014 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 17 April 2015, in accordance with Article 18 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 agreement of a paediatric investigation plan.

(2) It is therefore appropriate to adopt a decision agreeing a 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

A paediatric investigation plan for palovarotene, capsule, hard, oral 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

This decision is addressed to Clementia Pharmaceuticals Inc., 1375 Transcanada Highway, Suite 200, H9P 2W8 – Dorval, Canada.

Done at London, 5 June 2015

For the European Medicines Agency
Jordi Llinares Garcia
Head of Division (ad interim)
Human Medicines Research and Development Support
(Signature on file)

EMA/PDCO/94152/2015
London, 17 April 2015

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan

EMEA-001662-PIP01-14

Active substance(s):

Palovarotene

Condition(s):

Treatment of fibrodysplasia ossificans progressiva

Pharmaceutical form(s):

Capsule, hard

Pouch

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Clementia Pharmaceuticals Inc.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Clementia Pharmaceuticals Inc. submitted for agreement to the European Medicines Agency on 7 August 2014 an application for a paediatric investigation plan for the above mentioned medicinal product and a waiver under Article 13 of said Regulation.

The procedure started on 16 September 2014.

Supplementary information was provided by the applicant on 26 January 2015. 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.

   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.

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

Pouch

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 extension study to evaluate the long-term safety and efficacy of prior palovarotene treatment in FOP patients who completed Study PVO-1A-201 and who have experienced a new, distinct flare-

Area	Number of measures	Description
		up during the 12-month follow-up. (PVO-1A-202) Study 5 Multicentre, randomised, double-blind, placebo-controlled study to evaluate the ability of palovarotene to maintain or improve range of motion at the flare-up site in subjects with FOP. (PVO-1A-301) Study 6 Longitudinal, non-interventional, multicentre, natural history study of FOP to use the baseline assessments from two imaging modalities (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 September 2018
Deferral for one or more measures contained in the paediatric investigation plan:	No