EMA/393021/2013

European Medicines Agency decision

P/0174/2013

of 30 July 2013

on the agreement of a paediatric investigation plan and on the granting of a deferral for alpha tocotrienol quinone (EMEA-001238-PIP01-12) 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 Edison Orphan Pharma BV on 15 August 2012 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 14 June 2013, in accordance with Article 18 of Regulation (EC) No 1901/2006, and of its own motion in accordance with 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 agreement of a paediatric investigation plan and on the granting of a deferral.

(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.

[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 alpha tocotrienol quinone, oral solution, oral use, enteral 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 alpha tocotrienol quinone, oral solution, oral use, enteral 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

This decision is addressed to Edison Orphan Pharma BV, Jenalaan 18c, 3584 CK - Utrecht, The Netherlands.

Done at London, 30 July 2013

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

EMA/PDCO/276509/2013

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

EMEA-001238-PIP01-12

Scope of the application

Active substance(s):

Alpha tocotrienol quinone

Condition(s):

Treatment of Leigh syndrome

Pharmaceutical form(s):

Oral solution

Route(s) of administration:

Oral use

Enteral use

Name/corporate name of the PIP applicant:

Edison Orphan Pharma BV

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Edison Orphan Pharma BV submitted for agreement to the European Medicines Agency on 15 August 2012 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 10 October 2012.

Supplementary information was provided by the applicant on 21 March 2013. 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 on its own motion in accordance with Article 21 of said Regulation.

   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 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, 14 June 2013

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

Not applicable

2. Paediatric Investigation Plan

2.1. Condition: treatment of Leigh syndrome

2.1.1. Indication(s) targeted by the PIP

Treatment of Leigh syndrome

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)

Oral solution

2.1.4. Measures

Area	Number of measures	Description
Quality	1	Measure 1 Development of an oral solution.
Non-clinical	0	Not applicable.
Clinical	2	Measure 2 Double-blind, randomised, multi-centre, placebo controlled trial to evaluate, safety, efficacy, pharmacokinetics and acceptability/palatability of alpha tocotrienol quinone in children from birth to less than 18 years with extension study to evaluate safety and two dose-levels. (EPI743-12-002-EU) Measure 3 Open label, long-term trial in children with genetically confirmed Leigh Syndrome to evaluate the long-term safety and neurodevelopmental effects of alpha tocotrienol quinone on children who complete measure 2 (EPI743-2012-002-EU-LTE).

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	By October 2016
Deferral for one or more measures contained in the paediatric investigation plan:	Yes