EMA/339827/2013

European Medicines Agency decision

P/0153/2013

of 5 July 2013

on the acceptance of a modification of an agreed paediatric investigation plan for cysteamine hydrochloride (EMEA-000322-PIP01-08-M02) 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 cysteamine hydrochloride (EMEA-000322-PIP01-08-M02) 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/47/2009 issued on 24 March 2009,

Having regard to the application submitted by Orphan Europe SARL on 21 February 2013 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 17 May 2013, 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.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for cysteamine hydrochloride, eye drops, solution, ocular 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 agreed PIP covers all conditions, indications, pharmaceutical forms, routes of administration, measures, timelines, waivers and deferrals, as agreed in the decision P/47/2009 issued on 24 March 2009 including subsequent modifications thereof.

Article 3

This decision is addressed to Orphan Europe SARL, Immeuble Le Wilson, 70 avenue du Général de Gaulle, 92800 – Puteaux, France.

Done at London, 5 July 2013

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

EMA/PDCO/136031/2013 Corr

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

EMEA-000322-PIP01-08-M02

Scope of the application

Active substance(s):

Cysteamine hydrochloride

Condition(s):

Treatment of cystinosis

Pharmaceutical form(s):

Eye drops, solution

Route(s) of administration:

Ocular use

Name/corporate name of the PIP applicant:

Orphan Europe SARL

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Orphan Europe SARL submitted to the European Medicines Agency on 21 February 2013 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/47/2009 issued on 24 March 2009.

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

The procedure started on 20 March 2013.

Scope of the modification

The clinical trial design in the Paediatric Investigation Plan has 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.

   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 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 annex and appendix.

London, 17 May 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

1.1. Condition: treatment of cystinosis

The waiver applies to:

- the paediatric population from birth to 6 months;
- for eye drops, solution, ocular use;
- on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s);
- on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

2. Paediatric Investigation Plan

2.1. Condition: treatment of cystinosis

2.1.1. Indication(s) targeted by the PIP

Treatment of corneal cystine crystal deposits in cystinosis

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

From 6 months to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Eye drops, solution

2.1.4. Measures

Area	Number of measures	Description
Quality	0	Not applicable.
Non-clinical	0	Not applicable.
Clinical	2	Measure 1: Randomised, multi-centre, assessor-blind, active controlled trial to evaluate, safety, efficacy, and tolerability of Cystadrops to determine superiority over Cysteamine hydrochloride 0.10% in children from 2 years to 18 years of age, and adults with cystinosis. Measure 2: Open-label, safety and tolerability of Cystadrops in children from 6 months to less than 6 years with cystinosis.

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 March 2017
Deferral for one or more measures contained in the paediatric investigation plan:	Yes