European Medicines Agency

Doc. Ref. EMEA/158143/2009
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EUROPEAN MEDICINES AGENCY DECISION

of 24 March 2009

on the agreement of a Paediatric Investigation Plan and on the granting of a deferral and on the granting of a waiver for cysteamine hydrochloride (EMEA-000322-PIP01-08) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council as amended

(ONLY THE ENGLISH TEXT IS AUTHENTIC)

DISCLAIMER: This Decision does not entitle to the rewards and incentives referred to in Title V of Regulation (EC) No 1901/2006, as amended.

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THE EUROPEAN MEDICINES AGENCY,

Having regard to the Treaty establishing the European Community,

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 as amended 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 Orphan Europe SARL on 29 May 2008 under Article 16(1) of Regulation (EC) No 1901/2006 as amended also requesting a waiver under Article 13 of said Regulation, and a deferral under Article 20 of said Regulation,

Having regard to the Opinion of the Paediatric Committee of the European Medicines Agency, issued on 6 February 2009, in accordance with Article 18 of Regulation (EC) No 1901/2006 as amended, and Article 13 of said Regulation and Article 21 of said Regulation**,**

Having regard to Article 25 of Regulation (EC) No 1901/2006 as amended,

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 granting 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 cysteamine hydrochloride, eye gel, ocular 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 cysteamine hydrochloride, eye gel, ocular 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 cysteamine hydrochloride, eye gel, ocular 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 Orphan Europe SARL, Immeuble "Le Wilson", 70 av du Général de Gaulle, 92800 Puteaux, France.

Done at London, 24 March 2009

For the European Medicines Agency
Thomas Lönngren
Executive Director

(Signature on file)

European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

Doc. Ref. EMEA/PDCO/23424/2009
EMEA-000322-PIP01-08

OPINION OF THE PAEDIATRIC COMMITTEE ON THE AGREEMENT OF A PAEDIATRIC INVESTIGATION PLAN AND A DEFERRAL AND A WAIVER

Scope of the application

Active substance:
Cysteamine hydrochloride

Condition(s):
Cystinosis

Pharmaceutical form(s):
Eye gel

Route(s) of administration:
Ocular use

Name/corporate name of the PIP applicant:
Orphan Europe SARL

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Orphan Europe SARL submitted for agreement to the EMEA on 29 May 2008 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 1 July 2008.

Supplementary information was provided by the applicant on 27 November 2008.

A meeting with the Paediatric Committee took place on 4 February 2009.

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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 in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(b) of said Regulation, on the ground that the disease or condition for which the specific medicinal product is intended does not occur in the specified subset(s) of the paediatric population.

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 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 Agency, together with its annex(es) and appendix.

London, 6 February 2009

On behalf of the Paediatric Committee
Dr Daniel Brasseur, Chairman

(Signature on file)

ANNEX I

THE MEASURES AND TIMELINES OF THE AGREED PAEDIATRIC INVESTIGATION PLAN AND THE SUBSET(S) OF THE PAEDIATRIC POPULATION AND CONDITION(S) COVERED BY THE WAIVER

A. CONDITION(S)
Cystinosis

B. WAIVER
Cystinosis

- **Condition**

The waiver applies to: Preterm newborn infants, Term newborn infants (from birth to less than 28 days) and Infants to less than 6 months for eye gel for 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 cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need for this paediatric subset

C. PAEDIATRIC INVESTIGATION PLAN

- **Condition to be investigated**

Cystinosis

- **Proposed PIP indication**

Treatment of corneal cystine crystal deposits in cystinosis

- **Subset(s) of the paediatric population concerned by the paediatric development**

From 6 months to less than 18 years.

- **Formulation(s)**

Eye gel for ocular use

- **Studies**

Area	Number of studies	Description
Quality	0	Not applicable.
Non-clinical	0	Not applicable.
Clinical	1	Single blind, multi-center, randomised cross over, where randomisation is based on strata, active control non-inferiority trial in children from 3 years, and including adults.

Measures to address long term follow-up of potential safety issues, efficacy in relation to paediatric use: **No**

Date of completion of the paediatric investigation plan: **By December 2010**

Deferral for some or all studies contained in the paediatric investigation plan: **Yes**