



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/462362/2017

European Medicines Agency decision

P/0172/2017

of 31 July 2017

on the acceptance of a modification of an agreed paediatric investigation plan for cysteamine (hydrochloride) (Cystadrops), (EMA-000322-PIP01-08-M05) 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 726/2004¹,

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 Agency²,

Having regard to the European Medicines Agency's decision P/47/2009 issued on 24 March 2009, the decision P/0153/2013 issued on 5 July 2013, the decision P/0287/2013 issued on 29 November 2013, and the decision P/0322/2013 issued on 19 December 2013,

Having regard to the application submitted by Orphan Europe SARL on 27 February 2017 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 21 July 2017, 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, following a re-examination procedure of the Paediatric Committee's opinion according to Article 25(3) Regulation (EC) No 1901/2006, an opinion on the acceptance of changes to the agreed paediatric investigation plan and to the deferral.
- (2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan, including changes to the 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 cysteamine (hydrochloride) (Cystadrops), eye drops, solution, ocular use, including changes to the deferral, 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 decision is addressed to Orphan Europe SARL, Immeuble Le Wilson, 70 avenue du Général de Gaulle, 92800 - Puteaux, France.



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/PDCO/410154/2017

London, 21 July 2017

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

EMA-000322-PIP01-08-M05

Scope of the application

Active substance(s):

Cysteamine (hydrochloride)

Invented name:

Cystadrops

Condition(s):

Treatment of corneal cystine crystal deposits in cystinosis

Authorised indication(s):

See Annex II

Pharmaceutical form(s):

Eye drops, solution

Route(s) of administration:

Ocular use

Name / corporate name of the PIP applicant:

Orphan Europe SARL

Information about the authorised medicinal product:

See Annex II



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 27 February 2017 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 decision P/0153/2013 issued on 5 July 2013, the decision P/0287/2013 issued on 29 November 2013 and the decision P/0322/2013 issued on 19 December 2013.

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

An Opinion was adopted by the Paediatric Committee on 19 May 2017 for the above mentioned product. Orphan Europe SARL received the Paediatric Committee Opinion on 24 May 2017.

On 22 June 2017 Orphan Europe SARL submitted to the European Medicines Agency a written request including detailed grounds for a re-examination of the Opinion.

The re-examination procedure started on 23 June 2017.

Scope of the modification

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

Final Opinion

1. The Paediatric Committee, having assessed the detailed grounds for re-examination, in accordance with Article 25(3) of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

1.1. to revise its opinion and

- to agree to the changes regarding the measures of the paediatric investigation plan and the timelines of the deferral in the scope set out in the Annex I of this opinion;
- to refuse the changes to the waiver.

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 European Medicines Agency, together with its annexes 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

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).

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 the 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: (deleted during procedure EMEA-000322-PIP01-08-M05) Measure 3: Open-label, single-arm study to assess safety and efficacy of Cystadrops in children from 6 months to less than 2 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 December 2021
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Condition(s) and authorised indication(s):

1. Treatment of cystinosis

Authorised indication(s):

- Cystadrops is indicated for the treatment of corneal cystine crystal deposits in adults and children from 2 years of age with cystinosis.

Authorised pharmaceutical form(s):

Eye drops, solution

Authorised route(s) of administration:

Ocular use