EMA/782279/2021

# European Medicines Agency decision

P/0019/2022

of 3 February 2022

on the acceptance of a modification of an agreed paediatric investigation plan for cysteamine (hydrochloride) (Cystadrops), (EMEA-000322-PIP01-08-M06) 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 Union procedures for the authorisation and supervision of medicinal products for human 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, the decision P/0153/2013 issued on 5 July 2013, the decision P/0287/2013 issued on 29 November 2013, the decision P/0322/2013 issued on 19 December 2013 and the decision P/0172/2017 issued on 3 July 2017,

Having regard to the application submitted by Recordati Rare Diseases SARL on 10 September 2021 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 December 2021, 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 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, as amended.
[2] OJ L 136, 30.4.2004, p. 1, as amended.

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 Recordati Rare Diseases SARL, Immeuble Le Wilson, 70 Avenue du Général de Gaulle, 92800 - Puteaux France.

EMA/PDCO/524170/2021
Amsterdam, 17 December 2021

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

EMEA-000322-PIP01-08-M06

## 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:**

Recordati Rare Diseases 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, Recordati Rare Diseases SARL submitted to the European Medicines Agency on 10 September 2021 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, the decision P/0322/2013 issued on 19 December 2013 and the decision P/0172/2017 issued on 3 July 2017.

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

The procedure started on 19 October 2021.

## Scope of the modification

Some timelines of the Paediatric Investigation Plan have been modified.

## 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 and to the deferral in the scope set out in the Annex I of this opinion.

   The Norwegian Paediatric Committee member agrees 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 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 of age;
- 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 (Cystadrops/09/choc-study):**<br>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.<br>**Measure 2:** (deleted during procedure EMEA-000322-PIP01-08-M05) |

| | | |
|---|---|---|
| | | **Measure 3:**<br><br>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 March 2023 |
| 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