MHRA
10 South Colonnade
Canary Wharf
London
E14 4PU
United Kingdom

gov.uk/mhra

Decision Cover Letter

Decision of the licensing authority to:

accept change(s) to the agreed paediatric investigation plan and to the deferral.

MHRA-102064-PIP01-25-M01

Scope of the Application

Active Substance(s)

MERCAPTAMINE 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

Recordati Rare Diseases

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Recordati Rare Diseases submitted to the licensing authority on 30/07/2025 14:44 BST an application for a Modification

The procedure started on 30/07/2025 16:52 BST

1. The licensing authority, having assessed the application in accordance with the Human Medicines Regulations 2012, decides, as set out in the appended summary report:

to accept change(s) to the agreed paediatric investigation plan and to the deferral

2. The measures and timelines of the paediatric investigation plan are set out in the Annex I.

This decision is forwarded to the applicant, together with its annex and appendix.

MHRA
10 South Colonnade
Canary Wharf
London
E14 4PU
United Kingdom

gov.uk/mhra

Final Decision Letter

MHRA-102064-PIP01-25-M01

Of 01/08/2025 11:49 BST

On the adopted decision for MERCAPTAMINE HYDROCHLORIDE (MHRA-102064-PIP01-25-M01) in accordance with the Human Medicines Regulations 2012.

The licensing authority, in accordance with the Human Medicines Regulations 2012, has adopted this decision:

Agreement on modification of a paediatric investigation plan (including modification of a waiver or deferral included in that paediatric investigation plan).

This decision applies to a Modification for MERCAPTAMINE HYDROCHLORIDE, Eye drops, solution , OCULAR USE .

This decision is addressed to Recordati Rare Diseases, Tour Hekla, 52, Avenue du Général de Gaulle 92800 Puteaux, France, Puteaux, FRANCE, 92800

ANNEX I

1. Waiver

1.1 Condition:

Treatment of cystinosis. The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 6 months of age. Pharmaceutical form(s): Eye drops, solution Route(s) of administration: OCULAR USE Reason for granting waiver: 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(s):

Treatment of cystinosis.

2.2 Indication(s) targeted by the PIP:

Treatment of corneal cystine crystal deposits in cystinosis.

2.3 Subset(s) of the paediatric population concerned by the paediatric development:

The paediatric population from 6 months to less than 18 years of age.

2.4 Pharmaceutical Form(s):

Eye drops, solution

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	2	Study 1 (Cystadrops/09/choc-study) 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. Study 2, deleted during procedure EMEA-000322-PIP01-08-M05. Study 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.
Extrapolation, Modeling & Simulation Studies	0	Not applicable.
Other Studies	0	Not applicable.
Other Measures	0	Not applicable.

3. Follow-up, completion and deferral of a PIP:

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	31/03/2023
Deferral of one or more studies contained in the paediatric investigation plan:	Yes