Clinical Policy: Cysteamine Ophthalmic (Cystaran, Cystadrops)

Reference Number: ERX.NPA.111
Effective Date: 06.01.19
Last Review Date: 05.20
Line of Business: Commercial, Medicaid

Revision Log

See Important Reminder at the end of this policy for important regulatory and legal information.

Description
Cysteamine (Cystaran®, Cystadrops®) ophthalmic solution is a cystine-depleting agent.

FDA Approved Indication(s)
Cystaran and Cystadrops are indicated for the treatment of corneal cystine crystal accumulation in patients with cystinosis.

Policy/Criteria
Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

Health plan approved formularies should be reviewed for all coverage determinations. Requirements to use preferred alternative agents apply only when such requirements align with the health plan approved formulary.

It is the policy of health plans affiliated with Envolve Pharmacy Solutions™ that Cystaran and Cystadrops are **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Corneal Cystine Crystal Accumulation (must meet all)**:**

1. Diagnosis of cystinosis;
2. Prescribed by or in consultation with an ophthalmologist;
3. Presence of corneal cysteine accumulation;
4. Dose does not exceed one of the following (a or b):
   a. Cystaran: 1 drop in each eye every hour while awake (1 bottle per week);
   b. Cystadrops: 1 drop in each eye 4 times a day while awake (3 bottles/month).

Approval duration: 6 months

B. Other diagnoses/indications

1. Refer to ERX.PA.01 if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized).

II. Continued Therapy

A. Corneal Cystine Crystal Accumulation (must meet all)**:**

1. Currently receiving medication via a health plan affiliated with Envolve Pharmacy Solutions or member has previously met initial approval criteria;
2. Member is responding positively to therapy;
3. If request is for a dose increase, new dose does not exceed one of the following (a or b):
   a. Cystaran: 1 drop in each eye every hour while awake (1 bottle per week);
   b. Cystadrops: 1 drop in each eye 4 times a day while awake (3 bottles/month).

Approval duration: 12 months

B. Other diagnoses/indications (must meet 1 or 2)**:**

1. Currently receiving medication via a health plan affiliated with Envolve Pharmacy Solutions and documentation supports positive response to therapy.
   Approval duration: Duration of request or 12 months (whichever is less); or

2. Refer to ERX.PA.01 if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized).

III. Diagnoses/Indications for which coverage is NOT authorized:

A. Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off-label use policy – ERX.PA.01 or evidence of coverage documents.

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key
FDA: Food and Drug Administration

Appendix B: Therapeutic Alternatives
Not applicable

Appendix C: Contraindications/Boxed Warnings
None reported

V. Dosage and Administration

Drug Name	Dosing Regimen	Maximum Dose
Cystaran (cysteamine)	1 drop in each eye every waking hour	1 drop/eye/hour during waking hours
Cystadrops (cysteamine)	1 drop in each eye, 4 times a day during waking hours	See dosing regimen

VI. Product Availability

Drug Name	Availability
Cystaran (cysteamine)	Ophthalmic solution: 6.5 mg/mL of cysteamine hydrochloride equivalent to 4.4 mg/mL of cysteamine (0.44%)
Cystadrops (cysteamine)	Ophthalmic solution containing 3.8 mg/mL of cysteamine (0.37%) in 5 mL bottle

VII. References

1. Cystaran Prescribing Information. Gaithersburg, MD: Leadiant Biosciences, Inc., May 2018. Available at http://www.cystaran.com/. Accessed February 7, 2020.
2. Cystadrops Prescribing Information. Lebanon, NJ: Recordati Rare Diseases Inc.; August 2020. Available at: https://www.accessdata.fda.gov/drugsatfda_docs/label/2020/211302s000lbl.pdf. Accessed October 22, 2020.
3. Cystinosis. National Organization for Rare Disorders website. https://rarediseases.org/rare-diseases/cystinosis/. Published 1986. Updated 2017. Accessed February 7, 2020.

Reviews, Revisions, and Approvals	Date	P&T Approval Date
Policy created	02.08.19	05.19
2Q 2020 annual review: no significant changes; references reviewed and updated.	02.07.20	05.20
RT4: added Cystadrops to policy.	10.22.20	

Important Reminder

This clinical policy has been developed by appropriately experienced and licensed health care professionals based on a review and consideration of currently available generally accepted standards of medical practice; peer-reviewed medical literature; government agency/program approval status; evidence-based guidelines and positions of leading national health professional organizations; views of

physicians practicing in relevant clinical areas affected by this clinical policy; and other available clinical information.

This Clinical Policy is not intended to dictate to providers how to practice medicine, nor does it constitute a contract or guarantee regarding payment or results. Providers are expected to exercise professional medical judgment in providing the most appropriate care, and are solely responsible for the medical advice and treatment of members.