Clinical Policy: Cysteamine ophthalmic (Cystaran, Cystadrops)

Reference Number: PA.CP.PMN.130
Effective Date: 04/2019
Last Review Date: 04/2026

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.

It is the policy of PA Health & Wellness® 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 cystine 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 (1 bottle per week).

Approval duration: 12 months

B. Other diagnoses/indications

1. Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): PA.CP.PMN.53.

II. Continued Therapy

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

1. Currently receiving medication via PA Health & Wellness benefit or member has previously met all initial approval criteria or the Continuity of Care Policy (PA.PHARM.01) applies;
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 (1 bottle per week).

Approval duration: 12 months

B. Other diagnoses/indications (must meet 1 or 2)**:**
1. Currently receiving medication via PA Health & Wellness benefit or member has previously met all initial approval criteria or the Continuity of Care Policy (PA.PHARM.01) applies.
   Approval duration: Duration of request or 12 months (whichever is less); or
2. Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized PA.CP.PMN.53.

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 – PA.CP.PMN.53 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

Indication	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. Rockville, MD: Leadiant Biosciences, Inc., May 2023. Available at: http://www.cystaran.com/. Accessed February 12, 2026.
2. Cystadrops Prescribing Information. Bridgewater, NJ: Recordati Rare Diseases Inc.; August 2020. Available at: https://www.cystadrops.com. Accessed February 12, 2026.
3. Cystinosis. National Organization for Rare Disorders website. https://rarediseases.org/rare-diseases/cystinosis/. Updated February 14, 2024. Accessed February 12, 2026.
4. Elmonem MA, Veys KR, Soliman NA, et. al. Cystinosis: a review. *Orphanet J Rare Dis*. 2016 Apr 22; 11: 47.

Reviews, Revisions, and Approvals	Date
Policy created.	04/2019
2Q 2020 annual review: added appendix C; references reviewed and updated	04/2020
2Q 2021 annual review: added Cystadrops to policy; references reviewed and updated.	04/2021
2Q 2022 annual review: references reviewed and updated.	04/2022
2Q 2023 annual review: no significant changes; references reviewed and updated.	04/2023
2Q 2024 annual review: no significant changes; references reviewed and updated.	04/2024
2Q 2025 annual review: no significant changes; references reviewed and updated.	04/2025
2Q 2026 annual review: extended initial approval duration from 6 months to 12 months for this maintenance medication for a chronic condition; references reviewed and updated.	04/2026