

## PRIOR AUTHORIZATION POLICY

- POLICY:** Metabolic Disorders – Cysteamine Ophthalmic Solution Prior Authorization Policy
- Cystadrops® (cysteamine 0.37% ophthalmic solution – Recordati Rare Diseases)
  - Cystaran® (cysteamine 0.44% ophthalmic solution – Leadiant Biosciences)

**REVIEW DATE:** 03/20/2024

---

### OVERVIEW

Cysteamine ophthalmic solution is a cystine-depleting agent indicated for the treatment of **corneal cystine crystal accumulation in patients with cystinosis**.<sup>1,2</sup>

### Disease Overview

Cystinosis is a rare autosomal recessive inborn error of metabolism in which cystine accumulates within lysosomes and forms crystals in many tissues, including the kidneys, liver, bone marrow, pancreas, muscle, rectal mucosa, brain, and eye.<sup>3</sup> Cystine deposits in the cornea cause photophobia. Patients may present only with corneal crystal deposition but no associated systemic manifestations; the kidney, retina, and other organs are free of cystine accumulation in these patients. In patients without systemic symptoms, diagnosis of ocular cystinosis is often in adulthood when corneal crystal deposits are noted on ocular examination.<sup>4</sup> Of note, with oral cysteamine the concentration obtained in corneal tissue is inadequate and does not affect corneal cystine crystals. Topical treatment is required to dissolve existing cystine crystals.

### POLICY STATEMENT

Prior Authorization is recommended for prescription benefit coverage of cysteamine ophthalmic solution. All approvals are provided for the duration noted below. Because of the specialized skills required for evaluation and diagnosis of patients treated with cysteamine ophthalmic solution as well as the monitoring required for adverse events and long-term efficacy, approval requires cysteamine ophthalmic solution to be prescribed by or in consultation with a physician who specializes in the condition being treated.

**Automation:** None.

### RECOMMENDED AUTHORIZATION CRITERIA

Coverage of cysteamine ophthalmic solution is recommended in those who meet the following criteria:

#### FDA-Approved Indication

- 1. Cystinosis, Corneal Cysteine Crystal Deposits.** Approve for 1 year if the patient meets the following (A and B):
  - A)** Patient has corneal cysteine crystal deposits confirmed by slit-lamp examination; **AND**
  - B)** The medication is prescribed by or in consultation with an ophthalmologist or a metabolic disease specialist (or specialist who focuses in the treatment of metabolic diseases).

03/20/2024

© 2024. All Rights Reserved.

This document is confidential and proprietary. Unauthorized use and distribution are prohibited.

### **CONDITIONS NOT RECOMMENDED FOR APPROVAL**

Coverage of cysteamine ophthalmic solution is not recommended in the following situations:

1. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

### **REFERENCES**

1. Cystadrops® ophthalmic solution [prescribing information]. Lebanon, NJ: Recordati Rare Diseases; August 2020.
2. Cystaran® ophthalmic solution [prescribing information]. Gaithersburg, MD: Leadiant Biosciences; March 2022.
3. Wilmer MJ, Schoeber JP, van den Heuvel LP, Levtchenko EN. Cystinosis: practical tools for diagnosis and treatment. *Pediatr Nephrol.* 2011; 26(2): 205–215.
4. Biswas S, Gaviria M, Malheiro L, et al. Latest clinical approaches in the ocular management of cystinosis: a review of current practice and opinion from the ophthalmology cystinosis forum. *Ophthalmol Ther.* 2018;7(2):307-322.