

Specialty Guideline Management

Cystaran - Cystadrops

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over the counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Cystaran	cysteamine ophthalmic solution
Cystadrops	cysteamine ophthalmic solution

Indications

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-approved Indications¹

Cystaran

Cystaran is indicated for the treatment of corneal cystine crystal accumulation in patients with cystinosis.

Cystadrops

Cystadrops is indicated for the treatment of corneal cystine crystal deposits in adults and children with cystinosis.

All other indications are considered experimental/investigational and not medically necessary.

Reference number(s)
2090-A

Documentation

Submission of the following information is necessary to initiate the prior authorization review:

Initial Requests:

- Assay detecting increased cystine concentration in leukocytes or genetic testing results supporting the diagnosis.
- Chart notes or medical records documenting presence of corneal cystine crystal accumulation at baseline.

Continuation Requests:

- Chart notes or medical records documenting that corneal cystine crystal accumulation has not increased since initiating therapy with the requested medication.

Prescriber Specialties

This medication must be prescribed by or in consultation with an ophthalmologist or a physician who specializes in the treatment of metabolic disease and/or lysosomal storage disorders.

Coverage Criteria

Cystinosis¹⁻³

Authorization of 12 months may be granted for treatment of corneal cystine crystal accumulation when all of the following criteria are met:

- Diagnosis of cystinosis was confirmed by the presence of increased cystine concentration in leukocytes or by genetic testing; and
- Member has corneal cystine crystal accumulation.

Continuation of Therapy

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in the Coverage Criteria section who are responding to therapy met by either of the following criteria:

- Member has experienced a decrease in corneal cystine crystal accumulation; or

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- Member did not experience an increase in corneal cystine crystal accumulation.

References

1. Cystaran [package insert]. Gaithersburg, MD: Leadiant Biosciences, Inc.; April 2025.
2. Cystadrops [package insert]. Lebanon, NJ: Recordati Rare Diseases Inc.; August 2020.
3. Ivanova E, De Leo MG, De Matteis MA, Levtchenko E. Cystinosis: clinical presentation, pathogenesis, and treatment. *Pediatr Endocrinol Rev.* 2014;12(1):176-184.