PRIOR AUTHORIZATION POLICY

POLICY: Metabolic Disorders – Cystaran (cysteamine 0.44% ophthalmic solution – Leadiant Biosciences)

APPROVAL DATE: 03/11/2020

OVERVIEW
Cystaran is a cystine-depleting agent indicated for the treatment of corneal cystine crystal accumulation in patients with cystinosis.[1] The recommended dose is one drop into each every waking hour.

Disease Overview
Cystinosis is a rare autosomal recessive inborn error of metabolism in which the transport of cystine out of lysosomes is abnormal.[2-4] As a result of deficient or absent cystinosin (which normally transports cystine out of the lysosome), cystine accumulates within lysosomes and forms crystals in many tissues, including the kidneys, liver, bone marrow, pancreas, muscle, rectal mucosa, brain and eye. Patients with cystinosis also experience growth failure and rickets, and cystine deposits in the cornea cause photophobia. With time, most organs are damaged. 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.[5] Cystaran is the only approved treatment for corneal cystine crystal accumulation. 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 Cystaran. All approvals are provided for the duration noted below. If prescriber specialty is required, add support for this to policy statement. For example: Because of the specialized skills required for evaluation and diagnosis of patients treated with Cystaran as well as the monitoring required for adverse events and long-term efficacy, approval requires Cystaran to be prescribed by or in consultation with a physician who specializes in the condition being treated.

Automation: None.

RECOMMENDED AUTHORIZATION CRITERIA
Coverage of Cystaran is recommended in those who meet the following criteria:

FDA-Approved Indications

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

CONDITIONS NOT RECOMMENDED FOR APPROVAL

Cystaran has not been shown to be effective, or there are limited or preliminary data or potential safety concerns that are not supportive of general approval for the following conditions.

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. Cystaran [prescribing information]. Gaithersburg, MD: Leadiant Biosciences; May 2018.
2. Wilmer MJ, Schoeber JP, van den Heuvel LP, Levtchenko EN. Cystinosis: practical tools for diagnosis and treatment. *Pediatr Nephrol*. 2011; 26(2): 205–215.
3. Tsilou E, Zhou M, Gahl W, et al. Ophthalmic manifestations and histopathology of infantile nephropathic cystinosis: Report of a case and review of the literature. *Surv Ophthalmol*. 2007;52(1):97–105.
4. Gahl WA, Thoene JG, Schneider JA, et al. NIH Conference. Cystinosis: progress in a prototypic disease. *Ann Int Med*. 1988;109:557-569.
5. 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.