cysteamine ophthalmic solution 0.37% (Cystadrops®)

Policy # 00737
Original Effective Date: 03/08/2021
Current Effective Date: 03/10/2025

Applies to all products administered or underwritten by Blue Cross and Blue Shield of Louisiana and its subsidiary, HMO Louisiana, Inc.(collectively referred to as the "Company"), unless otherwise provided in the applicable contract. Medical technology is constantly evolving, and we reserve the right to review and update Medical Policy periodically.

When Services May Be Eligible for Coverage

Coverage for eligible medical treatments or procedures, drugs, devices or biological products may be provided only if:

- *Benefits are available in the member's contract/certificate, and*
- *Medical necessity criteria and guidelines are met.*

Based on review of available data, the Company may consider cysteamine ophthalmic solution 0.37% (Cystadrops®)‡ for the treatment of corneal cystine crystal deposits to be **eligible for coverage.****

Patient Selection Criteria
Coverage eligibility for cysteamine ophthalmic solution 0.37% (Cystadrops) will be considered when the following criteria are met:

- Patient has a diagnosis of cystinosis; AND
- Patient has corneal cystine crystal deposits.

When Services Are Considered Investigational

Coverage is not available for investigational medical treatments or procedures, drugs, devices or biological products.

Based on review of available data, the Company considers the use of cysteamine ophthalmic solution 0.37% (Cystadrops) in patients WITHOUT a diagnosis of cystinosis OR WITHOUT corneal cystine crystal deposits to be **investigational.***

Background/Overview

Cystadrops is a cystine-depleting agent indicated for the treatment of corneal cystine deposits in adults and children with cystinosis. The dosing of Cystadrops is one drop in each eye, 4 times a day during waking hours. Cystinosis is a lysosomal storage disease characterized by an accumulation of cystine in different organs and tissues, such as the cornea. In cystinosis, cystine builds up in the lysosomes because there is a genetic defect in the gene that codes for the cystine transportation out of the lysosome. Given cystine's poor solubility, it forms crystals as the concentration increases. Another product available for the treatment of this condition is Cystaran™‡, which is available in a 0.44% concentration. Cystaran is administered every waking hour.

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FDA or Other Governmental Regulatory Approval

U.S. Food and Drug Administration (FDA)

Cystadrops was approved in late 2020 and is a cystine-depleting agent indicated for the treatment of corneal cystine deposits in adults and children with cystinosis.

Rationale/Source

This medical policy was developed through consideration of peer-reviewed medical literature generally recognized by the relevant medical community, U.S. Food and Drug Administration approval status, nationally accepted standards of medical practice and accepted standards of medical practice in this community, technology evaluation centers, reference to federal regulations, other plan medical policies, and accredited national guidelines.

The safety and efficacy of Cystadrops were assessed in two studies: a single-arm study conducted for 5 years (OCT-1) and a randomized controlled study conducted for 90 days (CHOC). In the OCT-1 study, 8 patients with cystinosis (2 males and 6 females) with a mean age of 12.1 ± 4.6 (range: 7.0 – 21.0) were enrolled and received a median of 4 drops/eye/day of Cystadrops. In CHOC study, 32 patients with cystinosis (15 males and 17 females) with a mean age of 17.1 ± 13.0 (range: 2.9 – 62.6) were enrolled and received a median of 4 drops/eye/day. Fifteen patients were exposed to Cystadrops and 16 were exposed to cysteamine hydrochloride 0.1% (control arm).

Efficacy was assessed with In-Vivo Confocal Microscopy total score (IVCM score) by quantifying the cystine crystals in the cornea. A decrease in IVCM total score from baseline indicated a reduction in corneal crystals. In the CHOC study, after 30 and 90 days of treatment with Cystadrops, 12% and 40% reduction in the total IVCM total score across all corneal layers was observed from baseline, respectively. Cystadrops demonstrated greater reduction compared to the control arm at 90 days. The average reduction in IVCM total score was 4.6 in the Cystadrops arm and 0.5 in the control arm, mean difference 3.8 [95% CI: (2.1, 5.6)]. In the OCT-1 study, a mean decrease in corneal cystine crystal deposits of 30%, in comparison with baseline, was maintained over the 60 month period of the study.

References

1. Cystadrops [package insert]. Recordati Rare Diseases, Inc. Lebanon, New Jersey. Updated September 2020.
2. Cystinosis. UpToDate. Accessed January 11, 2021.

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02/04/2021	Medical Policy Committee review
02/10/2021	Medical Policy Implementation Committee approval. New policy.
02/03/2022	Medical Policy Committee review
02/09/2022	Medical Policy Implementation Committee approval. No change to coverage.
02/02/2023	Medical Policy Committee review
02/08/2023	Medical Policy Implementation Committee approval. No change to coverage.
02/01/2024	Medical Policy Committee review
02/14/2024	Medical Policy Implementation Committee approval. Coverage eligibility unchanged.
02/06/2025	Medical Policy Committee review
02/12/2025	Medical Policy Implementation Committee approval. Coverage eligibility unchanged.

Next Scheduled Review Date: 02/2026

*Investigational – A medical treatment, procedure, drug, device, or biological product is Investigational if the effectiveness has not been clearly tested and it has not been incorporated into standard medical practice. Any determination we make that a medical treatment, procedure, drug, device, or biological product is Investigational will be based on a consideration of the following:

A. Whether the medical treatment, procedure, drug, device, or biological product can be lawfully marketed without approval of the U.S. Food and Drug Administration (FDA) and whether such approval has been granted at the time the medical treatment, procedure, drug, device, or biological product is sought to be furnished; or
B. Whether the medical treatment, procedure, drug, device, or biological product requires further studies or clinical trials to determine its maximum tolerated dose, toxicity, safety, effectiveness, or effectiveness as compared with the standard means of treatment or diagnosis, must improve health outcomes, according to the consensus of opinion among experts as shown by reliable evidence, including:
    1. Consultation with technology evaluation center(s);
    2. Credible scientific evidence published in peer-reviewed medical literature generally recognized by the relevant medical community; or
    3. Reference to federal regulations.

**Medically Necessary (or "Medical Necessity") - Health care services, treatment, procedures, equipment, drugs, devices, items or supplies that a Provider, exercising prudent clinical judgment, would provide to a patient for the purpose of preventing, evaluating, diagnosing or treating an illness, injury, disease or its symptoms, and that are:

A. In accordance with nationally accepted standards of medical practice;

B. Clinically appropriate, in terms of type, frequency, extent, level of care, site and duration, and considered effective for the patient's illness, injury or disease; and
C. Not primarily for the personal comfort or convenience of the patient, physician or other health care provider, and not more costly than an alternative service or sequence of services at least as likely to produce equivalent therapeutic or diagnostic results as to the diagnosis or treatment of that patient's illness, injury or disease.

For these purposes, "nationally accepted standards of medical practice" means standards that are based on credible scientific evidence published in peer-reviewed medical literature generally recognized by the relevant medical community, Physician Specialty Society recommendations and the views of Physicians practicing in relevant clinical areas and any other relevant factors.
‡ Indicated trademarks are the registered trademarks of their respective owners.

NOTICE: If the Patient's health insurance contract contains language that differs from the BCBSLA Medical Policy definition noted above, the definition in the health insurance contract will be relied upon for specific coverage determinations.

NOTICE: Medical Policies are scientific based opinions, provided solely for coverage and informational purposes. Medical Policies should not be construed to suggest that the Company recommends, advocates, requires, encourages, or discourages any particular treatment, procedure, or service, or any particular course of treatment, procedure, or service.

NOTICE: Federal and State law, as well as contract language, including definitions and specific contract provisions/exclusions, take precedence over Medical Policy and must be considered first in determining eligibility for coverage.