



Dear Healthcare Provider,

Recordati Rare Diseases Inc. has developed an:

CYSTADROPS® (cysteamine ophthalmic solution) 0.37% Letter of Medical Necessity and Intent to Treat TEMPLATE

The purpose of this template letter is to assist your office in developing a customized Letter of Medical Necessity which outlines the medical justification for Cystadrops® therapy. Often, by submitting a Letter of Medical Necessity tailored around the history and current treatment needs of your patient, insurance plans may better understand the reasoning for Cystadrops®.

Please note - this letter template should only be used as a guide. Each patient will have their own unique and specific reasons for needing Cystadrops® therapy. In addition, each insurance plan may have their own rules and guidelines for approving Cystadrops®.

This sample letter and related information are provided for informational purposes only. It is the responsibility of the HCP and/or their office staff, as appropriate, to determine the correct diagnosis, treatment protocol, and content of all such letters and related forms for each individual patient. Recordati Rare Diseases (RRD) does not guarantee coverage or reimbursement for the product. There is no requirement that any patient or healthcare provider use any RRD product in exchange for this information, and this template is not meant to substitute for a prescriber's independent medical decision-making.

For full Prescribing Information and Instructions for Use, please go to www.CYSTADROPS.com/PI.

Sincerely,

The Cystadrops Team
Phone: (866) 925-6212
Fax: (855) 813-2039

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[ON OFFICE LETTERHEAD INCLUDING PROVIDER NAME AND ADDRESS]

Cystadrops® (cysteamine ophthalmic solution) 0.37%

Letter of Medical Necessity and Intent to Treat

TEMPLATE

[Date]

[Insurance Name]

[Insurance Address]

Patient Name: **[Patient Name]**

Patient Date of Birth: **[Patient DOB]**

Policy Number: **[Policy Number]**

Group Number: **[Group Number]**

Subject: Intent to Treat with Cystadrops® (cysteamine ophthalmic solution) 0.37%

To Whom It May Concern:

I am writing on behalf of my patient **[Patient Name]**, who has been diagnosed with cystinosis and requires treatment for corneal cystine crystals. I am writing to support the treatment of **[Patient Name]** with Cystadrops® (cysteamine ophthalmic solution) 0.37%. Cystadrops® is a cystine-depleting agent indicated for the treatment of corneal cystine crystal deposits in adults and children with cystinosis.¹

Cystinosis:

Cystinosis is a rare genetic disorder caused by a mutation in the *CTNS* gene that results in impaired transport of the amino acid cystine out of lysosomes in cells.² This leads to formation and accumulation of cystine crystals in cells, causing damage to organs throughout the body and significant impact on the eyes.² The cornea is the part of the eye that may be most affected.³ As the disease progresses, ocular symptoms increase in number and intensity, daily activities become more difficult to carry out, and severe complications may develop including visual impairment.³ Oral treatments for cystinosis do not affect cystine crystals accumulation in the cornea.²

Summary of Patient's Diagnosis:

- **[Description of genetic test that supports diagnosis of cystinosis]**
- **[Slit lamp exam or ophthalmologic assessment reports that demonstrate corneal cystine crystals]**

Summary of Patient's History:

- **[Description of symptoms related to the cornea]**
- **[Description of other symptoms related to cystinosis that may be relevant for treatment choice]**
- **[If patient has previously received Cystadrops, treatment initiation date and response]**
- **[List of previous prescription medications related to corneal cystine crystals and response]**
- **[If not previously mentioned, rationale for not using prescription medications that are requested by insurance plan]**

Rationale for Treatment:

It is my medical opinion that **[initiating/continuing to treat with]** Cystadrops® for **[patient's name]** is appropriate and medically necessary at this time. My intended use of Cystadrops® will be to initiate treatment four times daily in each eye during waking hours to deplete cystine crystal deposits in the cornea. In the clinical trial, Cystadrops® treatment led to the reduction in corneal cystine crystal density as assessed by in vivo confocal microscopy (IVCM). From baseline to 90 days, Cystadrops® showed a 40 percent reduction in the IVCM total score across all corneal layers.¹ A second study showed that treatment with Cystadrops® resulted in a 30 percent decrease in IVCM total score that was maintained for the five-year study period.¹ The most common adverse reactions (≥ 10%) were eye pain, vision blurred, eye irritation, ocular hyperaemia, instillation site discomfort, eye pruritus, lacrimation increased, and ocular deposits.¹

I would appreciate your evaluation of this request and ask that you approve Cystadrops®. If you have any questions or wish to conduct a Peer to Peer discussion, feel free to contact me at **[phone number]**.

Sincerely,

[HCP Name and participating provider number]

Enclosures: **[List of documentation described in above letter]**

References:

1. Cystadrops® (cysteamine ophthalmic solution) 0.37% [prescribing information]. Bridgewater, NJ: Recordati Rare Diseases Inc.; 2020.
2. Gahl, W et al. Cystinosis. *New England Journal of Medicine*. 2002; 347(2): 111-121.
3. Elmonem et al. Cystinosis: a review. *Orphanet Journal of Rare Diseases*. 2016; 11:47.