

Dear Healthcare Provider,

Recordati Rare Diseases Inc. has developed a:

**CYSTADROPS® (cysteamine ophthalmic solution) 0.37% Letter of Appeal EXEMPLAR**

In an effort to assist your office and make the Appeal process as smooth as possible, we have developed a Letter of Appeal exemplar for CYSTADROPS.

The appeals process with most insurance plans often requires the submission of a Letter of Appeal. The purpose of this exemplar letter is to assist your office in developing a customized Letter of Appeal, which addresses the reasons CYSTADROPS was denied, as well as outline the medical justification for CYSTADROPS therapy.

Please note - this letter exemplar should only be used as a guide. However, it is suggested that your Letter of Appeal include:

1. The reason(s) CYSTADROPS therapy was denied,
2. Response or rebuttal to each reason CYSTADROPS was denied, and
3. Supporting documentation (such as lab results) justifying the need for CYSTADROPS if needed.

As you know, 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.

For full Prescribing Information and Instructions for Use, please go to [www.cystadrops.com/PI](http://www.cystadrops.com/PI).

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

ON OFFICE LETTERHEAD INCLUDING PROVIDER NAME AND ADDRESS

Name  
Address  
Phone  
Fax

**CYSTADROPS® (cysteamine ophthalmic solution) 0.37%**

**Letter of Appeal**

**EXEMPLAR**

(Date)

(Payer Name)

(Payer Address)

Patient Name: (Patient Name)

Patient Date of Birth: (Patient DOB)

Policy Number: (Policy Number)

Group Number: (Group Number)

Case Number: (Case Number)

Subject: Letter of Appeal regarding CYSTADROPS (cysteamine ophthalmic solution) 0.37%

To Whom It May Concern:

I am writing to request an APPEAL of the decision to deny CYSTADROPS for my patient (**Patient name**). (**Patient name**) has been diagnosed with cystinosis and requires treatment for corneal cystine crystals.

CYSTADROPS (cysteamine ophthalmic solution) 0.37% is a cystine-depleting agent indicated for the treatment of corneal cystine crystal deposits in adults and children with cystinosis.<sup>1</sup>

This patient has been receiving treatment with CYSTADROPS since (**Treatment initiation date**) and had seen the following clinical outcomes (**list outcomes or slit lamp exam results**).

Our office received a denial for CYSTADROPS on (**date**). In that denial, CYSTADROPS was denied due to the following reasons:

- 1.
- 2.
- 3.

I disagree with this decision. In my clinical judgement, treatment with CYSTADROPS is medically necessary due to the following reasons (**answer each reason why CYSTADROPS was denied**):

- 1.
- 2.
- 3.

Cystinosis:

Cystinosis is a rare genetic disorder caused by a mutation in the *CTNS* gene which results in the impaired transport of cystine out of the lysosomes in cells.<sup>2</sup> This leads to the accumulation of cystine crystals in cells, causing damage to organs throughout the body and significant impact on the eyes.<sup>2</sup>

Treatment Plan:

Cystine crystal reduction in the cornea of the eye is the primary objective in the treatment of patients with corneal cystine crystals due to cystinosis.<sup>2</sup> My intended use of CYSTADROPS will be to **<initiate/continue>** treatment four times a day in each eye during waking hours to reduce cystine crystal deposits in the cornea.<sup>1</sup>

Please note that according to the CYSTADROPS Prescribing Information ([www.cystadrops.com/PI](http://www.cystadrops.com/PI)), CYSTADROPS treatment led to the reduction in corneal cystine crystal density as assessed by in vivo confocal microscopy (IVCM). In the CYSTADROPS arm, the trial showed a 40 percent reduction in the IVCM total score across all corneal layers from baseline to 90 days.<sup>1</sup> Another trial showed that treatment with CYSTADROPS resulted in a 30 percent decrease as compared to baseline in IVCM total score, that was maintained for the five-year study period.<sup>1</sup> The most common adverse reactions ( $\geq 10\%$ ) were eye pain, vision blurred, eye irritation, ocular hyperaemia, instillation site discomfort, eye pruritus, lacrimation increased, and ocular deposits.<sup>1</sup>

I would appreciate your reconsideration of this denial and ask that you reverse your decision and approve CYSTADROPS for **(patient name)**.

If you have any questions or wish to conduct a Peer to Peer discussion, feel free to contact me at **(enter phone number)**.

Thank you for your time and consideration.  
**(First and Last name, MD)**

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.