

PHARMACY POLICY STATEMENT

Indiana Medicaid

DRUG NAME	Procysbi and Cystagon (cysteamine bitartrate); Cystaran and Cystadrops (cysteamine hydrochloride solution)
BILLING CODE	Must use valid NDC code
BENEFIT TYPE	Pharmacy
SITE OF SERVICE ALLOWED	Home
COVERAGE REQUIREMENTS	Prior Authorization Required (Non-Preferred Products) QUANTITY LIMIT— See “dosage allowed”
LIST OF DIAGNOSES CONSIDERED NOT MEDICALLY NECESSARY	Click Here

Procysbi and Cystagon (cysteamine bitartrate), Cystaran and Cystadrops (cysteamine hydrochloride solution) are **non-preferred** products and will only be considered for coverage under the **pharmacy** benefit when the following criteria are met:

Members must be clinically diagnosed with one of the following disease states and meet their individual criteria as stated.

NEPHROPATHIC CYSTINOSIS (Procysbi or Cystagon)

For **initial** authorization:

1. Member must be 1 year of age or older if the request is for Procysbi (no limit for Cystagon); AND
2. Medication must be prescribed by or in consultation with a nephrologist; AND
3. Member has a diagnosis of nephropathic cystinosis confirmed by an elevated WBC cystine concentration greater than 2 nmol ½ cystine/mg protein (lab report must include reference values) AND at least **one** of the following:
 - a) CTNS gene mutation;
 - b) Presence of corneal crystals, as shown by slit lamp exam performed by an ophthalmologist; AND
4. If the request is for Procysbi, all the following must also be documented in the chart notes:
 - a) Inability to reach target cystine level despite a minimum of 6 months of compliant therapy with Cystagon at max dose (or highest tolerated dose);
 - b) If requesting switch from Cystagon due to intolerance, member must first attempt to temporarily stop therapy, then re-initiate at a lower dose and gradually increase to the proper dose;²
 - c) If requesting switch from Cystagon due to GI side effects, member must also try taking with a proton pump inhibitor (e.g. omeprazole), in addition to attempting dose adjustment;
 - d) NOTE: Any other rationale for switching from Cystagon (aside from inefficacy or intolerance) will be considered on a case by case basis. In general, CareSource does not recognize frequency of dosing or lack of adherence as being indicative of medical necessity.
5. **Dosage allowed:** Refer to product label for initiation, titration, and adjustment. The max dose is 1.95g/m²/day.

If member meets all the requirements listed above, the medication will be approved for 6 months.

For **reauthorization**:

1. Lab report showing stabilized or decreased cystine levels from baseline; AND
2. Chart notes showing stabilized or improved signs and symptoms of disease or slowed progression.

If member meets all the reauthorization requirements above, the medication will be approved for an additional 12 months.

CYSTINOSIS (Cystaran or Cystadrops)

For **initial** authorization:

1. Medication must be prescribed by or in consultation with a nephrologist or ophthalmologist; AND
2. Member has a diagnosis of cystinosis confirmed by an elevated WBC cystine concentration 1 nmol ½ cystine/mg protein or greater; AND
3. Presence of corneal crystal deposits as evidenced by slit lamp exam.
4. **Dosage allowed:** 1 drop in each eye, every waking hour; (up to 4 times a day for Cystadrops).

If member meets all the requirements listed above, the medication will be approved for 3 months.

For **reauthorization**:

1. Chart notes have been provided that show improvement of signs and symptoms of disease (e.g. reduction of corneal cystine crystal accumulation, decreased severity of photophobia).

If member meets all the reauthorization requirements above, the medication will be approved for an additional 12 months.

CareSource considers Procysbi and Cystagon (cysteamine bitartrate), Cystaran and Cystadrops (cysteamine hydrochloride solution) not medically necessary for the treatment of diseases that are not listed in this document.

DATE	ACTION/DESCRIPTION
10/07/2020	New policy for Procysbi and Cystagon (cysteamine bitartrate), Cystaran and Cystadrops (cysteamine hydrochloride solution) created.

References:

1. Procysbi (cysteamine bitartrate) [package insert]. Lake Forest, IL: Horizon Therapeutics USA, Inc.; 2020.
2. Cystagon (cysteamine bitartrate) [package insert]. Morgantown, WV: Mylan Pharmaceuticals Inc; 2019.
3. Wilmer MJ, Schoeber JP, van den Heuvel LP, Levtchenko EN. Cystinosis: practical tools for diagnosis and treatment. *Pediatr Nephrol*. 2011;26(2):205-215. doi:10.1007/s00467-010-1627-6
4. Elmonem MA, Veys KR, Soliman NA, Dyck MV, Heuvel LPVD, Levtchenko E. Cystinosis: a review. *Orphanet Journal of Rare Diseases*. April 2016. doi:10.1186/s13023-016-0426-y
5. Bäumner S, Weber LT. Nephropathic Cystinosis: Symptoms, Treatment, and Perspectives of a Systemic Disease. *Front Pediatr*. 2018;6:58. Published 2018 Mar 14. doi:10.3389/fped.2018.00058
6. Ahlenstiel-Grunow T, Kanzelmeyer NK, Froede K, et al. Switching from immediate- to extended-release cysteamine in nephropathic cystinosis patients: a retrospective real-life single-center study. *Pediatric Nephrology*. 2016;32(1):91-97. doi:10.1007/s00467-016-3438-x
7. Bäumner S, Weber LT. Conversion from immediate- to extended-release cysteamine may decrease disease control and increase additional side effects. *Pediatric Nephrology*. 2017;32(7):1281-1282. doi:10.1007/s00467-017-3618-3
8. Emma F, Nesterova G, Langman C, et al. Nephropathic cystinosis: an international consensus document. *Nephrol Dial Transplant*. 2014;29 Suppl 4(Suppl 4):iv87-iv94. doi:10.1093/ndt/gfu090

9. Nesterova G, Gahl WA. Cystinosis. 2001 Mar 22 [Updated 2017 Dec 7]. In: Adam MP, Ardinger HH, Pagon RA, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2020. Available from: <https://www.ncbi.nlm.nih.gov/books/NBK1400/>
10. Cystaran (cysteamine hydrochloride solution) [package insert]. Gaithersburg, MD: Leadiant Biosciences, Inc.; 2020.
11. Cystadrops (cysteamine hydrochloride solution) [package insert]. Lebanon, NJ: Recordati Rare Diseases Inc.; 2020.
12. Kaur S, Sarma P, Kaur H, et al. Efficacy and safety of topical cysteamine in corneal cystinosis: a systematic review and meta- analysis. *American Journal of Ophthalmology*. September 2020. doi:10.1016/j.ajo.2020.07.052
13. Biswas S, Gaviria M, Malheiro L, Marques JP, Giordano V, Liang H. 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. doi:10.1007/s40123-018-0146-6.

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