

## PHARMACY POLICY STATEMENT

### Georgia 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 <b>NOT</b> MEDICALLY NECESSARY	<a href="#">Click Here</a>

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:

- Member must be 1 year of age or older if the request is for Procysbi (no limit for Cystagon); AND
- Medication must be prescribed by or in consultation with a nephrologist; AND
- 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:
  - CTNS gene mutation;
  - Presence of corneal crystals, as shown by slit lamp exam performed by an ophthalmologist; AND
- If the request is for Procysbi, all the following must also be documented in the chart notes:
  - Inability to reach target cystine level despite a minimum of 6 months of compliant therapy with Cystagon at max dose (or highest tolerated dose);
  - 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;<sup>2</sup>
  - 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;
  - 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.
- Dosage allowed:** Refer to product label for initiation, titration, and adjustment. The max dose is 1.95g/m<sup>2</sup>/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.
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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
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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

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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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