

<b>Clinical Policy Title:</b>	cysteamine bitartrate
<b>Policy Number:</b>	RxA.084
<b>Drug(s) Applied:</b>	Cystagon®, Procysbi®
<b>Original Policy Date:</b>	02/07/2020
<b>Last Review Date:</b>	12/05/2024
<b>Line of Business Policy Applies to:</b>	All lines of business (except Medicare)

## Criteria

### I. Initial Approval Criteria

#### A. Nephropathic Cystinosis (must meet all):

1. Diagnosis of nephropathic cystinosis confirmed by one of the following (a, b, or c):
  - a. Increased leukocyte cystine concentration (normal concentration is less than 0.2 nmol half-cystine/mg protein);
  - b. Cystinosis, lysosomal cystine transporter gene mutation;
  - c. Corneal crystals on slit lamp examination;
2. If Procysbi® is requested, medical justification supports inability to use Cystagon® (e.g., contraindication to excipients in Cystagon®);
3. Cystagon® and Procysbi® are not being used concomitantly.

#### Approval Duration

**All Lines of Business (except Medicare):** 6 months

### II. Continued Therapy Approval

#### A. Nephropathic Cystinosis (must meet all):

1. Member is currently receiving medication that has been authorized by RxAdvance or the member has met initial approval criteria.

#### Approval Duration

**All Lines of Business (except Medicare):** 12 months

## References

1. Kleta R, Kaskel F, Dohil R, et al. First NIH/Office of Rare Diseases conference on cystinosis: past, present, and future. *Pediatr Nephrol.* 2005; 20: 452-454. Available at: [https://cystinosis.org/wp-content/uploads/2019/01/ConsensusStatement\\_FirstNIHRareDiseaseConf-1.pdf](https://cystinosis.org/wp-content/uploads/2019/01/ConsensusStatement_FirstNIHRareDiseaseConf-1.pdf). Accessed November 28, 2024.
2. Bendavid C, Kleta R, Long R, et al. FISH diagnosis of the common 57-kb deletion in CTNS causing cystinosis. *Hum Genet.* November 2004; 115(6): 501-514. Available at: <https://pubmed.ncbi.nlm.nih.gov/15365816/>. Accessed November 28, 2024.
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. Available at: <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC3016220/>. Accessed November 28, 2024.
4. Tsilou E, Zhou M, Gahl W, et al. Ophthalmic manifestations and histopathology of infantile nephropathic cystinosis: Report of a case and review of the literature. *Surv Ophthalmol.* 2007; 52(1): 97–105. Available at:

This clinical policy has been developed to authorize, modify, or determine coverage for individuals with similar conditions. Specific care and treatment may vary depending on individual need and benefits covered by the plan. This policy is not intended to dictate to providers how to practice medicine, nor does it constitute a contract or guarantee regarding payment or results. This document may contain prescription brand name drugs that are trademarks of pharmaceutical manufacturers that are not affiliated with RxAdvance.

<https://www.ncbi.nlm.nih.gov/pmc/articles/PMC1850966/>. Accessed November 28, 2024.

5. Gahl WA, Thoene JG, Schneider JA, et al. NIH Conference. Cystinosis: progress in a prototypic disease. *Ann Int Med.* 1988; 109: 557-569. Available at: <https://pubmed.ncbi.nlm.nih.gov/3048161/>. Accessed November 28, 2024.
6. National Organization for Rare Disorders (NORD). Cystinosis. Available at: <https://rarediseases.org/rare-diseases/cystinosis/>. Accessed November 28, 2024.

Review/Revision History	Review/Revision Date	P&T Approval Date
Policy was established.	01/2020	02/07/2020
Updated line of business, drug availability, and removed old policy reference	4/2020	05/20/2020
Policy was reviewed and updated. <ol style="list-style-type: none"> <li>1. Clinical policy title and lines of business updated.</li> <li>2. Commercial approval duration was updated for initial and Continued approval criteria.</li> <li>3. Initial criteria for approval and duration of approval updated.</li> <li>4. Continued therapy criteria II.A.1 was rephrased to "Member is currently receiving medication that has been authorized by RxAdvance...".</li> <li>5. References updated.</li> </ol>	01/21/2021	03/09/2021
Policy was reviewed: <ol style="list-style-type: none"> <li>1. References were reviewed and updated.</li> </ol>	11/24/2021	01/17/2022
Policy was reviewed: <ol style="list-style-type: none"> <li>1. Initial Approval Criteria, approval duration: Updated from 12 months to 6 months.</li> <li>2. References were reviewed.</li> </ol>	10/03/2022	01/17/2023
Policy was reviewed.	10/19/2023	10/19/2023
Policy was reviewed: <ol style="list-style-type: none"> <li>1. Removed age restrictions.</li> <li>2. Removed prescriber restrictions.</li> <li>3. Removed dose restrictions.</li> <li>4. Updated Continued therapy approval with auto-approval based on lookback functionality within the past 120 days.</li> <li>5. Removed reauthorization requirement for positive response to therapy.</li> </ol>	08/28/2024	09/13/2024

<p>6. Updated approval duration verbiage.</p> <p>7. References were reviewed and updated.</p>		
<p>Updated Continued therapy approval to “Member is currently receiving medication that has been authorized..”</p>	<p>11/28/2024</p>	<p>12/05/2024</p>