Clinical Policy Title:	cysteamine bitartrate
Policy Number:	RxA.084
Drug(s) Applied:	Cystagon®, Procysbi®
Original Policy Date:	02/07/2020
Last Review Date:	08/28/2024
Line of Business Policy Applies to:	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. Cystinosin, 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. Auto-approval based on lookback functionality within the past 120 days as a proxy for member responding positively to therapy.

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. Accessed August 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 August 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 August 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: https://www.ncbi.nlm.nih.gov/pmc/articles/PMC1850966/. Accessed August 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 August 28, 2024.
6. National Organization for Rare Disorders (NORD). Cystinosis. Available at: https://rarediseases.org/rare-diseases/cystinosis/. Accessed August 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. 1. Clinical policy title and lines of business updated. 2. Commercial approval duration was updated for initial and Continued approval criteria. 3. Initial criteria for approval and duration of approval updated. 4. Continued therapy criteria II.A.1 was rephrased to "Member is currently receiving medication that has been authorized by RxAdvance…". 5. References updated.	01/21/2021	03/09/2021
Policy was reviewed: 1. References were reviewed and updated.	11/24/2021	01/17/2022
Policy was reviewed: 1. Initial Approval Criteria, approval duration: Updated from 12 months to 6 months. 2. References were reviewed.	10/03/2022	01/17/2023
Policy was reviewed.	10/19/2023	10/19/2023
Policy was reviewed: 1. Removed age restrictions. 2. Removed prescriber restrictions. 3. Removed dose restrictions. 4. Updated Continued therapy approval with auto-approval based on lookback functionality within the past 120 days. 5. Removed reauthorization requirement for positive response to therapy. 6. Updated approval duration verbiage.	08/28/2024	09/13/2024

7. References were reviewed and updated.		