

## POLICY STATEMENT

etplace

Procysbi and Cystagon (cysteamine bitartrate); Cystaran and Cystadrops (cysteamine hydrochloride solution)

BENEFIT TYPE	Pharmacy
STATUS	Prior Authorization Required

Cysteamine bitartrate, approved by the FDA in 1994 as immediate release Cystagon and in 2013 as extended release Procysbi, is indicated for the treatment of nephropathic cystinosis.

Cystinosis is a rare inherited metabolic disease in which cystine builds up as damaging crystals in organs including the kidneys and eyes. It is caused by a mutation in the CTNS gene, which encodes for a lysosomal transport protein that keeps cystine from effectively accumulating in lysosomes.

Manifestations can include Fanconi syndrome, renal tubular acidosis, cornea, and endocrine disruption such as hypothyroidism.



## References:

1. 2021 Georgia Code Title 33 Insurance Chapter 20A - Managed Health Care Plans Article 2 - Patient's Right to Independent Review § 33-20A-31 Definitions. Justia US Law. Accessed April 25, 2023. <https://law.justia.com/codes/georgia/2021/title-33/chapter-20a/article-2/section-33-20a-31/>.
2. Procysbi (cysteamine bitartrate) [package insert]. Lake Forest, IL: Horizon Therapeutics USA, Inc.; 2022.
3. Cystagon (cysteamine bitartrate) [package insert]. Morgantown, WV: Mylan Pharmaceuticals Inc; 2021.
4. 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
5. 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
6. 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
7. 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
8. 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/>
9. 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