Reference number(s)
2089-A

Specialty Guideline Management Cystagon

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Cystagon	cysteamine bitartrate

Indications

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-approved Indications[1]

Cystagon is indicated for the management of nephropathic cystinosis in children and adults.

All other indications are considered experimental/investigational and not medically necessary.

Documentation

Submission of the following information is necessary to initiate the prior authorization review:

Initial Requests:

- Assay detecting increased cystine concentration in leukocytes or genetic testing results supporting diagnosis.

- Chart notes or medial records documenting clinical signs and symptoms of disease at baseline (e.g., growth deficiency; vomiting/feeding difficulties; severe polyuria, polydipsia, and dehydration; progressive rachitic skeletal changes; failure to walk at a normal age; tetany; corneal crystals).

Continuation Requests:

- Lab results or chart notes documenting a positive response to therapy (e.g., improvement, stabilization, or slowing of disease progression for serum creatinine, calculated creatinine clearance, leukocyte cystine concentration, or maintained growth [height]).

Prescriber Specialties

This medication must be prescribed by or in consultation with a physician who specializes in the treatment of metabolic disease and/or lysosomal storage disorders.

Coverage Criteria

Nephropathic Cystinosis[1,2]

Authorization of 12 months may be granted for treatment of nephropathic cystinosis when all of the following criteria are met:

- Diagnosis of cystinosis was confirmed by the presence of increased cystine concentration in leukocytes or by genetic testing.
- Member exhibits clinical signs and symptoms of disease at baseline (e.g., growth deficiency; vomiting/feeding difficulties; severe polyuria, polydipsia, and dehydration; progressive rachitic skeletal changes; failure to walk at a normal age; tetany; corneal crystals).
- Member will not use Cystagon in combination with Procysbi.

Continuation of Therapy

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in the Coverage Criteria section who are responding to therapy (e.g., improvement, stabilization, or slowing of disease progression for serum creatinine, calculated creatinine clearance, leukocyte cystine concentration, or maintained growth [height]).

References

1. Cystagon [package insert]. Morgantown, WV: Mylan Pharmaceuticals Inc.; August 2021.
2. Nesterova G, Gahl WA. Cystinosis. 2001 Mar 22 [Updated 2025 Aug 14]. In: Adam MP, Bick S, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2026. Available from: https://www.ncbi.nlm.nih.gov/books/NBK1400/