Reference number(s)
2095-A

Specialty Guideline Management
Procysbi

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
Procysbi	cysteamine bitartrate delayed-release

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]

Procysbi is indicated for the treatment of nephropathic cystinosis in adults and pediatric patients 1 year of age and older.

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 medical 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[1-3]

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

- Member is 1 year of age or older.
- 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 Procysbi in combination with Cystagon.

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. Procysbi [package insert]. Deerfield, IL: Horizon Therapeutics USA, Inc.; December 2024.
2. Ivanova E, De Leo MG, De Matteis MA, Levtchenko E. Cystinosis: clinical presentation, pathogenesis, and treatment. Pediatr Endocrinol Rev. 2014;12(1):176-184.
3. 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/