

**Protocol for Cysteamine Products
Approved April 2022**

Cystagon® (cysteamine bitartrate) [immediate-release capsules]

Procysbi® (cysteamine bitartrate) [delayed-release capsules]

Background:

Nephropathic cystinosis is a rare, inherited autosomal recessive disease caused by cystine accumulation in lysosomes.

***Cystagon** is a cystine-depleting agent indicated for the management of nephropathic cystinosis in children and adults.*

***Procysbi** is a cystine-depleting agent indicated for the treatment of nephropathic cystinosis in adults and pediatric patients 1 year of age and older*

Criteria for approval:

1. Diagnosis of nephropathic cystinosis confirmed by at least one of the following:
 - a. A white blood cell (WBC) cystine level >2 nmol half cystine/mg protein
 - b. Mutations in the CTNS gene
2. If **Procysbi** is requested, the patient has a documented failure of, intolerance to, or contraindication to immediate release cysteamine bitartrate (**Cystagon**)
3. Medication is prescribed by or in consultation with a nephrologist or ophthalmologist
4. Patient will not use Cystagon in combination with Procysbi
5. Patient has no contraindications to treatment
6. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs (Lexicomp), national guidelines, or other peer-reviewed evidence

Initial Approval: 3 months

Continuation of therapy:

1. Patient is responding to therapy (e.g., improvement, stabilization, or slowing of disease progression for serum creatinine, calculated creatinine clearance, or leukocyte cystine concentration).
2. Medication is prescribed in accordance with Food and Drug Administration (FDA) established indication and dosing regimens or in accordance with medically appropriate off-label indication and dosing according to American Hospital Formulary Service, Micromedex, Clinical Pharmacology, Wolters Kluwer Lexi-Drugs (Lexicomp), national guidelines, or other peer-reviewed evidence

Renewal Approval: 6 months

References:

1. Cystagon [prescribing information]. Mylan Pharmaceuticals Inc; Morgantown, WV, 2019
2. Procysbi [prescribing information]. Horizon Therapeutics USA, Inc. Lake Forest, IL: 2020
3. Clinical Pharmacology® Gold Standard Series [Internet database]. Tampa FL. Elsevier 2019. Updated periodically
4. Bäumner S, and Weber L. Nephropathic Cystinosis: Symptoms, Treatment, and Perspectives of a Systemic Disease. *Frontiers in Pediatrics*; 2018; 6: 58.