Clinical Policy: Cysteamine bitartrate (Cystagon, Procysbi)

Reference Number: ERX.SPMN.146
Effective Date: 10/16
Last Review Date: 09/16

See Important Reminder at the end of this policy for important regulatory and legal information.

Policy/Criteria
It is the policy of health plans affiliated with Envolve Pharmacy Solutions® that cysteamine bitartrate (Cystagon®, Procysbi®) is medically necessary when one of the following criteria is met:

I. Initial Approval Criteria
   A. Nephropathic Cystinosis (must meet all):
      1. Age ≥ 2 years if request for Procysbi;
      2. Diagnosis of nephropathic cystinosis confirmed by one of the following:
         a. Presence of increased cystine concentration in leukocytes;
         b. DNA testing.

      Approval duration: 6 months

   B. Other diagnoses/indications: Refer to ERX.SPMN.16 - Global Biopharm Policy.

II. Continued Approval
   A. Nephropathic Cystinosis (must meet all):
      1. Currently receiving medication via health plan benefit or member has previously met all initial approval criteria.

      Approval duration: 12 months

   B. Other diagnoses/indications (must meet 1 or 2):
      1. Currently receiving medication via health plan benefit and documentation supports positive response to therapy; or
      2. Refer to ERX.SPMN.16 - Global Biopharm Policy.

Background
Description/Mechanism of Action:
Cysteamine bitartrate is a cystine-depleting agent that lowers the cystine content of cells in patients with nephropathic cystinosis, an inherited defect of lysosomal transport. Cysteamine bitartrate is an aminothiol that participates within lysosomes in a thiol-disulfide interchange reaction converting cystine into cysteine and cysteine-cysteamine mixed disulfide, both of which can exit the lysosome in patients with cystinosis.

FDA Approved Indication(s):
Cystagon is 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 adult and pediatric patients 2 years of age and older.

**Reviews, Revisions, and Approvals**

<table>
<thead>
<tr>
<th>Policy split from USS.SPMN.33 Lysosomal Storage Disorders and converted to new template. Added Cystagon. Removed safety criteria. Lowered age restriction for Procysbi from ≥ 6 years to ≥ 2 years per PI. Modified approval durations to 6 months for initial and 12 months for re-auth.</th>
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**References**


**Important Reminder**

This clinical policy has been developed by appropriately experienced and licensed health care professionals based on a review and consideration of currently available generally accepted standards of medical practice; peer-reviewed medical literature; government agency/program approval status; evidence-based guidelines and positions of leading national health professional organizations; views of physicians practicing in relevant clinical areas affected by this clinical policy; and other available clinical information.

This Clinical Policy is not intended to dictate to providers how to practice medicine, nor does it constitute a contract or guarantee regarding payment or results. Providers are expected to exercise professional medical judgment in providing the most appropriate care, and are solely responsible for the medical advice and treatment of members.

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