I. Medication Description

Cysteamine is an aminothiol that participates within lysosomes in a thiol-disulfide interchange reaction, converting cystine into cysteine and cysteine-cysteamine, both of which can exit the lysosome of the cystinosis patient. This results in long-term depletion of lysosomal cystine. The administration of cysteamine early in life slows the progression of renal failure, improves growth, decreases the need for thyroid hormone replacement and decreases corneal cystine deposits. Procysbi® is a delayed release formulation of cysteamine bitartrate that allows for twice daily dosing. The previously marketed formulation, Cystagon®, an immediate release product, requires every 6 hour, around the clock dosing for optimal response.

II. Position Statement

Coverage is determined through a prior authorization process with supporting clinical documentation for every request.

III. Policy

Coverage of Procysbi® will be provided when:

- Member has a confirmed diagnosis of nephropathic cystinosis AND
- Therapy is requested by a nephrologist or other specialist experienced in the management of nephropathic cystinosis AND
- Member has been unable to comply with the recommended dosing regimen of plan-preferred medication (Cystagon) OR the following criteria have been met:
  - When requesting coverage of a brand medication for which an A/B rated generic is available, there is sufficient evidence that the use of the A/B rated generic equivalent has resulted in inadequate results AND
  - At least one of the following is met:
    - The plan-preferred medications are contraindicated or will likely cause an adverse reaction by or physical or mental harm to the member.
    - The plan-preferred medications are expected to be ineffective based on the known clinical history and conditions of the member and the member’s prescription drug regimen.
    - The member has tried the plan-preferred medications or another prescription drug in the same pharmacologic class or with the same mechanism of action and such prescription drug was discontinued due to lack of efficacy or effectiveness, diminished effect, or an adverse event.
    - The member is stable on the medication selected by their healthcare professional for the medical condition under consideration (where “stable” is defined as receiving the medication for an adequate period of time, have achieved optimal response, and...
continued favorable outcomes are expected UNLESS the medication was initially selected due to the availability of a drug sample or a coupon card).

- The plan-preferred medication is not in the best interest of the member because it will likely cause a significant barrier to the member’s adherence or to compliance with the member’s plan of care, will likely worsen a comorbid condition of the member, or will likely decrease the member’s ability to achieve or maintain reasonable functional ability in performing daily activities.

IV. **Quantity Limitations**

Coverage will be provided for a quantity sufficient to provide dosing up to a maximum of 1.95 grams/ m²/day.

V. **Coverage Duration**

Coverage will be granted indefinitely through the life of this policy once the initial criteria are met.

VI. **Coverage Renewal Criteria**

n/a

VII. **Billing/Coding Information**

Available as 25mg, 75mg delayed release capsules

VIII. **Summary of Policy Changes**

- 9/15/13: new policy
- 9/15/14: no changes
- 7/1/15: formulary distinctions made
- 3/15/16: no policy changes
- 1/1/17: no policy changes
- 5/1/17: step therapy criteria added
- 1/1/18: no policy changes

IX. **References**


The Plan fully expects that only appropriate and medically necessary services will be rendered. The Plan reserves the right to conduct pre-payment and post-payment reviews to assess the medical appropriateness of the above-referenced therapies.

The preceding policy applies only to members for whom the above named pharmacy benefit medications are included on their covered formulary. Members with closed formulary benefits are subject to trying all appropriate formulary alternatives before a coverage exception for a non-formulary medication will be considered.

The preceding policy is a guideline to allow for coverage of the pertinent medication/product, and is not meant to serve as a clinical practice guideline.