## Drug Therapy Guidelines

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### 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 is one year of age or older **AND**
- 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**
- When requesting coverage of a brand medication for which a plan-preferred 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**
- Member has been unable to comply with the recommended dosing regimen of plan-preferred medication (Cystagon) first OR when at least ONE of the following criteria have been 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 solely due to the
availability of a drug sample or a coupon card and the member does not otherwise meet the
definition of “stable”).
   o 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
• 2/15/19: added minimum member age to coverage criteria

IX. References

3. Langman CB, et al. A randomized controlled crossover trial of delayed-release cysteamine bitartrate in
nephropathic cystinosis: effectiveness on white blood cell cystine levels and comparison of safety. Clin J Am
May; 156(5): 823-7.

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.