Procysbi

Description

Procysbi (cysteamine bitartrate)

Background

Procysbi, is designed to treat nephropathic cystinosis, the most common form of a disease known as cystinosis, in which toxic levels of cystine, a naturally occurring amino acid, build up in the body's cells and organs. Cystinosis may lead to slow body growth and small stature, weak bones and developing and worsening kidney failure. There are three types of cystinosis, the most severe being nephropathic cystinosis, which severely damages the kidneys. The drug works by lowering cystine levels, potentially delaying kidney and other damage (1).

Regulatory Status

FDA-approved indication: Procysbi is a cystine depleting agent indicated for the management of nephropathic cystinosis in adults and children ages 1 year and older (1).

Procysbi should be prescribed by a physician experienced in management of nephropathic cystinosis. Goal of therapy is to maintain a white blood cell (WBC) cystine level < 1 nmol ½ cystine/mg protein or a plasma cysteamine concentration > 0.1 mg/L (1).

Patients should have their WBC cystine levels and/or plasma cysteamine concentration measured in 2 weeks, and quarterly for 6 months then twice yearly at a minimum. If the plasma cysteamine is > 0.1 mg/L, but the WBC cystine is > 1.0 nmol ½ cystine/mg protein, the physician is advised to investigate the following parameters: adherence to dosing interval, adherence to medication, or the relationship between administration of Procysbi and fasted/fed state (1).
The use of Procysbi delayed-release capsules is contraindicated in patients who are hypersensitive to penicillamine (1).

Interrupt Procysbi if patients develop skin or bone lesions. Cysteamine has been associated with reversible leukopenia and elevated alkaline phosphatase levels. Therefore, blood counts and alkaline phosphatase levels should be monitored (1).

Patients receiving immediate-release cysteamine bitartrate have reported central nervous system symptoms such as seizures, lethargy, somnolence, depression, and encephalopathy. Patients have also reported gastrointestinal ulceration and bleeding. Monitor patients and an adjustment in dose may be necessary (1).

Safety and efficacy in pediatric patients under the age of 1 year have not been established (1).

Related policies

Policy

This policy statement applies to clinical review performed for pre-service (Prior Approval, Precertification, Advanced Benefit Determination, etc.) and/or post-service claims.

Procysbi may be considered medically necessary in patients that are 1 year of age and older with a confirmed diagnosis of nephropathic cystinosis and if the conditions below are met.

Procysbi is considered investigational in patients less than 1 year of age and for all other indications.

Prior-Approval Requirements

Age 1 year of age or older

Diagnosis

Patient must have the following:

Nephropathic cystinosis

AND ALL of the following:
1. Inadequate response or intolerance to prior treatment with Cystagon, immediate-release.
2. Diagnosis confirmed by the presence of increased cystine concentration in leukocytes
3. Prescribed by a physician experienced in management of nephropathic cystinosis
   a. Such as an endocrinologist, nephrologist, or urologist
4. Agreement to monitor WBC cystine levels (or plasma cysteamine concentration)
   a. Patients switching from immediate-release: monitor in 2 weeks, then quarterly for 6 months then twice yearly at a minimum

Prior – Approval *Renewal* Requirements

**Age**

1 year of age or older

**Diagnosis**

Patient must have the following:

Nephropathic cystinosis

**AND ALL** of the following:

1. Prescribed by a physician experienced in management of nephropathic cystinosis
   a. Such as an endocrinologist, nephrologist, or urologist
2. Agreement to monitor WBC cystine levels (or plasma cysteamine concentration) twice yearly at a minimum

**Policy Guidelines**

**Pre - PA Allowance**

None

**Prior - Approval Limits**

**Duration**

12 months
Prior – Approval *Renewal* Limits

**Duration** 12 months

**Rationale**

**Summary**

Procysbi is a cystine depleting agent indicated for the management of nephropathic cystinosis in adults and children ages 1 year and older. Procysbi should be prescribed by a physician experienced in management of nephropathic cystinosis. Cystagon is the current standard of care. Procysbi is contraindicated in patients who are hypersensitive to penicillamine. Safety and efficacy in pediatric patients under the age of 1 year have not been established (1).

Prior approval is required to ensure the safe, clinically appropriate and cost effective use of Procysbi while maintaining optimal therapeutic outcomes.

**References**


**Policy History**

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<th>Date</th>
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<tbody>
<tr>
<td>June 2013</td>
<td>Addition to PA</td>
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<tr>
<td>December 2013</td>
<td>Editorial review and update. Addition to criteria of trial and failure of Cystagon, immediate-release product as it is standard of care for first line therapy prior to Procysbi.</td>
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<tr>
<td>December 2014</td>
<td>Annual review. Removal of No hypersensitivity to penicillamine</td>
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<tr>
<td>August 2015</td>
<td>Change in age from 6 to 2 yrs old</td>
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<tr>
<td>September 2016</td>
<td>Annual editorial review and reference update Policy code changed from 5.08.28 to 5.55.01</td>
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<tr>
<td>March 2017</td>
<td>Annual review</td>
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<tr>
<td>March 2018</td>
<td>Annual editorial review</td>
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<tr>
<td>March 2019</td>
<td>Change of the required age from 2 to 1 yrs of age</td>
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**Keywords**
This policy was approved by the FEP® Pharmacy and Medical Policy Committee on March 15, 2019 and is effective on April 1, 2019.