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Section:	Prescription Drugs	**Effective Date:**	July 1, 2024
Subsection:	Genitourinary Agents	**Original Policy Date:**	July 12, 2013
Subject:	Procysbi	**Page:**	1 of 5

Last Review Date: June 13, 2024

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 cysteine-depleting agent indicated for the management of nephropathic cystinosis in adults and pediatric patients 1 year of age 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** if the conditions indicated below are met.

Procysbi may be considered **investigational** 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

Same as above

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

1. Procysbi [package insert]. Lake Forest, IL: Horizon Pharma USA, Inc.; February 2022.

Policy History

Date	Action
June 2013	Addition to PA
December 2013	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.
December 2014	Annual review. Removal of No hypersensitivity to penicillamine
August 2015	Change in age from 6 to 2 years old
September 2016	Annual editorial review and reference update Policy code changed from 5.08.28 to 5.55.01
March 2017	Annual review
March 2018	Annual editorial review Change of the required age from 2 to 1 years of age
March 2019	Annual review
March 2020	Annual review and reference update
December 2020	Annual review and reference update
September 2021	Annual review and reference update
September 2022	Annual review and reference update
June 2023	Annual review
June 2024	Annual review

Keywords

This policy was approved by the FEP® Pharmacy and Medical Policy Committee on June 13, 2024 and is effective on July 1, 2024.