Policy and Procedure	
PHARMACY PRIOR AUTHORIZATION POLICY AND CRITERIA ORPTCOTH008.1024	**MISCELLANEOUS PRODUCTS** **PROCYSBI®** (cysteamine delayed release capsules & granules)
Effective Date: 1/1/2025	**Review/Revised Date**: 10/13, 04/14, 04/15, 03/16, 03/17, 03/18, 02/19, 09/1, 08/20, 08/21, 09/22, 09/23, 10/24 (ZJN)
Original Effective Date: 11/13	**P&T Committee Meeting Date**: 10/13, 04/14, 04/15, 04/17, 04/18, 04/19, 10/19, 10/20, 10/21, 10/22, 10/23, 10/24
Approved by: Oregon Region Pharmacy and Therapeutics Committee	

SCOPE:
Providence Health Plan and Providence Health Assurance as applicable (referred to individually as "Company" and collectively as "Companies").

APPLIES TO:
Commercial
Medicaid

POLICY CRITERIA:

COVERED USES:
All Food and Drug Administration (FDA) approved indications not otherwise excluded from the benefit.

REQUIRED MEDICAL INFORMATION:
Initial Authorization:
All of the following:
1. Confirmed diagnosis of nephropathic cystinosis as evidenced by measuring leukocyte cystine levels (LCL) or genetic analysis of the CTNS gene (gene that encodes cystinosin)
2. Documentation of trial and failure, contraindication or intolerance to immediate release cysteamine capsules (Cystagon®).

EXCLUSION CRITERIA: N/A

AGE RESTRICTIONS:
One year of age and older

PRESCRIBER RESTRICTIONS: N/A

COVERAGE DURATION:
Authorization may be reviewed annually to assess continued medical necessity and effectiveness of the medication.

Requests for indications that were approved by the FDA within the previous six (6) months may not have been reviewed by the health plan for safety and effectiveness and inclusion on this policy document. These requests will be reviewed using the New Drug and or Indication Awaiting P&T Review; Prior Authorization Request ORPTCOPS047.

Requests for a non-FDA approved (off-label) indication requires the proposed indication be listed in either the American Hospital Formulary System (AHFS), Drugdex, or the National Comprehensive Cancer Network (NCCN) and is considered subject to evaluation of the prescriber's medical rationale, formulary alternatives, the available published evidence-based research and whether the proposed use is determined to be experimental/investigational.

Coverage for Medicaid is limited to a condition that has been designated a covered line item number by the Oregon Health Services Commission listed on the Prioritized List of Health Care Services.

Coverage decisions are made on the basis of individualized determinations of medical necessity and the experimental or investigational character of the treatment in the individual case.

INTRODUCTION:
Procysbi® (cysteamine bitartrate) is indicated for the management of nephropathic cystinosis in adults and children aged one year and older. Cysteamine is a cystine-depleting agent. It converts cystine to cysteine and cysteine-cysteamine mixed disulfides, thereby reducing cystine crystal accumulation. Procysbi® is available as delayed release oral capsules and delayed release oral granules. Dosing of oral cysteamine is based upon weight and leukocyte cystine levels.

FDA APPROVED INDICATIONS:
Management of nephropathic cystinosis in adults and children aged one year and older.

POSITION STATEMENT:
Nephropathic cystinosis is a rare autosomal recessive lysosomal storage disease caused by genetic mutations on chromosome 17. This mutation causes cysteine to accumulate as crystals which causes progressive organ dysfunction including kidneys, thyroid, testis, pancreas, muscle, brain, and eyes. Approximately 1 in 100, 000 to 1 in 200, 000 individuals have cystinosis. There are variations of this disorder depending on which organ is affected. Nephropathic syndrome results in impaired glomerular function, growth retardation, hypophosphatemic rickets, and hypothyroidism. Ocular nonnephropathic cystinosis patients do not accumulate crystals in their kidneys, but they do have crystal accumulation in their cornea and bone marrow. The primary symptom is photophobia, but ocular cysteine crystal accumulation can progress to blindness. Oral cysteamine is not well absorbed into the ophthalmic area.

Procysbi® offers an advantage over the immediate release formulation of cysteamine (Cystagon®) because they only have to be taken twice daily instead of four times a day. In order to be effective in reducing cysteine levels, Cystagon® must be taken every six hours around the clock. Improving adherence with a twice daily regimen has been demonstrated to enhance quality of life in cystinosis patients.

REFERENCE/RESOURCES:

1. Procysbi® (cysteamine bitartrate) delayed-release capsules and delayed release oral granules package insert. Novato, CA: Raptor Pharmaceuticals Inc., 2022 Feb.
2. Langman CB, Greenbaum LA, Sarwal M, et al. A randomized controlled crossover trial with delayed-release cysteamine bitartrate in nephropathic cystinosis: effectiveness on white blood cell cystine levels and comparison of safety. I. 2012; 7(7):1112-1120. PMID: 22554716
3. National Institutes of Health. National Center for Advancing Translational Sciences. Genetic and Rare Diseases Information Center. Cystinosis (updated 2021 Nov.). https://rarediseases.info.nih.gov/diseases/6236/cystinosis#ref_15544 (accessed 09/2024).