Procysbi (cysteamine bitartrate) Prior Authorization Program Summary

POLICY REVIEW CYCLE

Effective Date	Date of Origin
02-01-2025	

FDA LABELED INDICATIONS AND DOSAGE

Agent(s)	FDA Indication(s)	Notes	Ref#
Procysbi® (cysteamine bitartrate delayed release) Oral capsule Oral granules	Treatment of nephropathic cystinosis in adults and pediatric patients 1 year of age and older		1

See package insert for FDA prescribing information: https://dailymed.nlm.nih.gov/dailymed/index.cfm

CLINICAL RATIONALE

Cystinosis	Cystinosis is a rare autosomal recessive lysosomal storage disorder in which cystine accumulates in lysosomes of cells. It is a systemic disease wherein cystine crystals accumulate in all body cells and tissues. Cystinosis has three major clinical presentations depending on the severity of mutations affecting the CTNS gene: the infantile nephropathic form, the juvenile nephropathic form, and the adult (non-nephropathic ocular) form. The infantile nephropathic form is the most common and severe form, occurring in over 95% of patients, with consecutively progressive loss of glomerular function leading to end-stage renal disease (ESRD). ESRD usually develops by the end of the first or second decade of life. Nearly all nephropathic cystinosis patients will develop major extra-renal symptoms including retinal, endocrine, and neuromuscular complications by 30 years of age if cystine depletion therapy is not initiated early.(2) Cysteamine, in combination with symptomatic care, is the standard of care for patients with cystinosis. Cysteamine can deplete the intralysosomal cystine through the reduction of cystine, and the formation of cysteine and a cysteamine-cysteine mixed disulfide which exits the lysosome via the cationic amino acid transporter PQLC2, thus bypassing the original genetic and biochemical defects of the disease. Treatment with cysteamine improves overall prognosis by delaying progression to ESRD, preventing hypothyroidism and extra-renal complications, and improves growth in affected children. Therefore, cysteamine treatment should be started as soon as possible and needs to be continued lifelong.(2)
Efficacy	A multicenter, open-label, randomized clinical trial was completed comparing Procysbi to immediate-release cysteamine bitartrate (Cystagon). All patients were required to

	be on a stable dose of immediate-release cysteamine bitartrate prior to randomization. The study demonstrated that Procysbi administered every 12 hours was non-inferior to immediate-release cysteamine bitartrate dosed every 6 hours.(1)
Safety	Procysbi is contraindicated in patients with a serious hypersensitivity reaction, including anaphylaxis, to penicillamine or cysteamine.(1)

REFERENCES

Number	Reference
1	Procysbi prescribing information. Horizon Therapeutics USA, Inc. February 2022.
2	Elmonem MA, Veys KR, Soliman NA, Van Dyck M, Van Den Heuvel LP, Levtchenko E. Cystinosis: a review. *Orphanet Journal of Rare Diseases*. 2016;11(1). doi:10.1186/s13023-016-0426-y

POLICY AGENT SUMMARY PRIOR AUTHORIZATION

Target Brand Agent(s)	Target Generic Agent(s)	Strength	Targeted MSC	Available MSC	Final Age Limit	Preferred Status
Procysbi	cysteamine bitartrate cap delayed release	25 MG ; 75 MG	M ; N ; O ; Y	N		
Procysbi	cysteamine bitartrate delayed release granules packet	300 MG ; 75 MG	M ; N ; O ; Y	N		

CLIENT SUMMARY – PRIOR AUTHORIZATION

Target Brand Agent Name(s)	Target Generic Agent Name(s)	Strength	Client Formulary
Procysbi	cysteamine bitartrate cap delayed release	25 MG ; 75 MG	Commercial ; HIM ; ResultsRx
Procysbi	cysteamine bitartrate delayed release granules packet	300 MG ; 75 MG	Commercial ; HIM ; ResultsRx

PRIOR AUTHORIZATION CLINICAL CRITERIA FOR APPROVAL

Module	Clinical Criteria for Approval
PA	**Target Agent(s)** will be approved when ALL of the following are met: 1. ONE of the following: A. The patient has a diagnosis of nephropathic cystinosis **OR** B. The patient has another FDA labeled indication for the requested agent and route of administration **OR** C. The patient has an indication that is supported in compendia for the requested agent and route of administration **AND** 2. If the patient has an FDA labeled indication, then ONE of the following: A. The patient's age is within FDA labeling for the requested indication **OR** B. There is support for using the requested agent for the patient's age for the requested indication **AND** 3. ONE of the following: A. The patient has tried and had an inadequate response to Cystagon (immediate release cysteamine) **OR** B. The patient has an intolerance or hypersensitivity to Cystagon that is not expected to occur with the requested agent **OR** C. The patient has an FDA labeled contraindication to Cystagon that is not expected to occur with the requested agent **AND**

Module	Clinical Criteria for Approval
	4. The prescriber is a specialist in the area of the patient's diagnosis (e.g., nephrologist), or the prescriber has consulted with a specialist in the area of the patient's diagnosis **AND** 5. The patient does NOT have any FDA labeled contraindications to the requested agent **Compendia Allowed:** AHFS or DrugDex 1 or 2a level of evidence **Length of Approval:** 12 months

