Procysbi (cysteamine bitartrate) Prior Authorization Program Summary

This program applies to KeyRx formulary.

The BCBS MN Step Therapy Supplement also applies to this program for all Commercial/HIM lines of business.

POLICY REVIEW CYCLE

Effective Date	Date of Origin
12/1/2023	10/1/2019

FDA APPROVED 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 Pharma USA, Inc. February 2022.
2	Elmonem MA, Veys KR, Soliman NA, van Dyck M, van den Heuvel LP, Levtchenko E. (2016) Cystinosis: A review. Orphanet J Rare Dis 11:47. 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	KeyRx
Procysbi	cysteamine bitartrate delayed release granules packet	300 MG ; 75 MG	KeyRx

PRIOR AUTHORIZATION CLINICAL CRITERIA FOR APPROVAL

Module	Clinical Criteria for Approval
	Evaluation **Target Agent(s)** will be approved when ALL of the following are met: 1. The patient has an FDA approved indication for the requested agent **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. The prescriber has provided information in support of 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 OR D. The patient is currently being treated with the requested agent as indicated by ALL of the following:

Module	Clinical Criteria for Approval
	1. A statement by the prescriber that the patient is currently taking the requested agent **AND** 2. A statement by the prescriber that the patient is currently receiving a positive therapeutic outcome on requested agent **AND** 3. The prescriber states that a change in therapy is expected to be ineffective or cause harm **OR** E. The prescriber has provided documentation that Cystagon cannot be used due to a documented medical condition or comorbid condition that is likely to cause an adverse reaction, decrease ability of the patient to achieve or maintain reasonable functional ability in performing daily activities or cause physical or mental harm **AND** 4. The patient does NOT have any FDA labeled contraindications to the requested agent **Length of Approval:** 12 months

