Premier Health Plan

POLICY AND PROCEDURE

POLICY TITLE: ***Cystagon and Procysbi (oral cysteamine)***
DEPARTMENT: **Clinical Pharmacy Services- Utilization Management**
ORIGINAL DATE: ***June 2013 (as adopted from UPMC Health Plan)***

Last P & T Committee Approval Date: March *2018*

Product Applicability: *mark all applicable products below:*

COMMERCIAL	[ ] HMO [ ] PPO *Products*: [ ] Small [ ] Indiv. [ ] Large *Exchange*: [ ] Shop [ ] Indiv. [x] All
OTHER	[x] Self-funded/ASO

PURPOSE

The purpose of this policy is to define the prior authorization process for Cystagon and Procysbi (oral cysteamine).

Cysteamine (Cystagon) is indicated for the management of nephropathic cystinosis in children and adults.

Cysteamine delayed-release capsule (Procysbi) is indicated for the treatment of nephropathic cystinosis in adult and pediatric patients 1 year of age and older.

DEFINITIONS

N/A

POLICY

It is the policy of the Health Plan to maintain a prior authorization process that promotes appropriate utilization of specific drugs with potential for misuse or limited indications. This process involves a review using Food and Drug Administration (FDA) criteria to make a determination of Medical Necessity, and approval by the Pharmacy &

Therapeutics Committee of the criteria for prior authorization, as described in RX.002 Pharmacy and Therapeutics Committee and RX.003-Prior Authorization Process.

The drugs, Cystagon and Procysbi (oral cysteamine), are subject to the prior authorization process.

PROCEDURE

Initial Authorization Criteria:

Must meet all of the criteria listed below:

- Must be prescribed by or in consultation with a physician who specializes in the treatment of inherited metabolic disorders
- Must have a diagnosis of nephropathic cystinosis. Chart documentation of a clinical work-up to rule out other diagnoses and clinical rationale for the diagnosis and exclusion of other diagnoses must be provided. The diagnosis must be confirmed by having all of the following (chart documentation is required):
    - Elevated baseline white blood cell (WBC) cystine levels > 2 nmol/1/2cystine/mg protein
    - Laboratory result confirming CTNS gene mutation
    - Clinical symptoms consistent with nephropathic cystinosis including electrolyte imbalances (e.g. Fanconi Syndrome) and polyuria
- For Procysbi:
    - Must use Cystagon as preferred therapy and must provide the following chart documentation when requesting Procysbi:
        - Chart documentation of an adequate trial of immediate-release cysteamine (Cystagon) with an inadequate response despite dose titration and compliance with therapy demonstrated by inadequately controlled WBC cystine levels
        - The physician must provide relevant written documentation of laboratory and/or objective values [e.g., WBC cysteine levels, physician progress notes; or Subjective, Objective, Assessment, and Plan (SOAP note) information representing the physician's interaction with the member] as well as clinical rationale explaining why Cystagon has not produced the same clinical results as would be expected with the use of Procysbi (They are the same chemical entity).

Reauthorization Criteria:

All prior authorization renewals are reviewed on an annual basis to determine the Medical Necessity for continuation of therapy. Authorization may be extended at 1-year intervals based upon:

- Chart documentation that the member's condition has improved based upon the prescriber's assessment while on therapy and reduction in WBC cystine levels since starting treatment with cysteamine (Cystagon) or cysteamine delayed-release capsule (Procysbi).

Limitations:

Length of Authorization (if above criteria met)	
Initial Authorization	Up to 3 months
Reauthorization	Up to 1 year
Quantity Level Limit	
Procysbi	900 capsules per 30 days

If the established criteria are not met, the request is referred to a Medical Director for review.

REFERENCES

1. Procysbi [package insert]. Novato, CA: Raptor Pharmaceuticals, Inc.; December 2017.
2. Cystagon [package insert]. Morgantown, WV: Mylan Pharmaceuticals, Inc.; June 2007.
3. 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 cysteine levels and comparison of safety. Clin J Am Soc Nephrol 2012;7:1112-1120.
4. Nesterova G, Gahl WA. Cystinosis. 2001 Mar 22 [Updated 2012 May 17]. In: Pagon RA, Adam MP, Bird TD, et al., editors. GeneReviews™ [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2013. Available from: http://www.ncbi.nlm.nih.gov/books/NBK1400/
5. Gahl, W, Thoene JG, Schneider JA. Cystinosis. NEJM 2002;2:111-121.
6. American Society of Hospital Pharmacists. ASHP guidelines on adverse drug reaction monitoring and reporting. Am J Health-Syst Pharm. 1995; 52:417-9.

RECORD RETENTION

Records Retention for Evolent Health documents, regardless of medium, are provided within the Evolent Health records retention policy and as indicated in CORP.028.E Records Retention Policy and Procedure.

REVIEW HISTORY

DESCRIPTION OF REVIEW / REVISION	DATE APPROVED
Annual review	*02/16, 02/17, 02/18*
New Indication	*03/18*