Premier Health Plan

POLICY AND PROCEDURE

POLICY TITLE: ***Cystadane (betaine anhydrous)***
DEPARTMENT: **Clinical Pharmacy Services- Utilization Management**
ORIGINAL DATE: ***September 2013 (as adopted from UPMC Health Plan)***

Last P & T Committee Approval Date: *February 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 Cystadane (betaine anhydrous).

Cystadane (betaine anhydrous) is indicated for the treatment of homocystinuria to decrease elevated homocysteine blood levels. Included within this category of homocystinuria are:

- Cystathionine beta-synthase (CBS) deficiency
- 5, 10-methylenetetrahydrofolate reductase (MTHFR) deficiency
- Cobalamin cofactor metabolism (cbl) defect

DEFINITIONS

Homocystinuria – an inherited disorder of the metabolism of the amino acid methionine, which is broken down into to homocysteine, which is in turn converted back to methionine. Homocystinuria results in severely elevated levels of homocysteine which manifests as detrimental effects in organ systems such as the eyes, bones, vascular and neurological systems.

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 drug, Cystadane (betaine anhydrous), is 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 homocystinuria. Chart documentation describing how diagnosis was confirmed (e.g. genetic testing results, plasma and urine levels of homocysteine and of methionine, liver biopsy and enzyme assays, progress notes, etc.) is required.

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 from the prescriber that the member's condition has improved based upon the prescriber's assessment while on therapy.

Limitations:

Length of Authorization (if above criteria met)	
Initial Authorization	Up to 3 months
Reauthorization	Up to 1 year

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

REFERENCES

1. Cystadane [prescribing information]. Franklin, NJ: Rare Disease Therapeutics, Inc.; October 2010
2. Singh RH, Kruger WD, Wang L, et al. Cystathionine β-synthase deficiency: effects of betaine supplementation after methionine restriction in B6-nonresponsive homocystinuria. *Genet Med* 2004;6(2):90-95
3. Lawson-Yuen A, Levy HL. The use of betaine in the treatment of elevated homocysteine. *Molecular Genetics and Metabolism* 2006;88:201-207
4. Schiff M, Blom HJ. Treatment of inherited homocystinurias. *Neuropediatrics* 2012;43:295-304

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	*02/17, 02/18*