

## PRIOR AUTHORIZATION POLICY

**POLICY:** Metabolic Disorders – Betaine Anhydrous Prior Authorization Policy

- Cystadane® (betaine anhydrous powder – Recordati Rare Diseases, generic)

**REVIEW DATE:** 09/11/2024

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### OVERVIEW

Betaine anhydrous powder (Cystadane, generic), a methylating agent, is indicated for the treatment of **homocystinuria** to decrease elevated homocysteine blood concentrations in adults and pediatric patients.<sup>1</sup> Included within the category of homocystinuria are cystathionine beta-synthase deficiency, 5,10-methylenetetrahydrofolate reductase deficiency, and cobalamin cofactor metabolism defect.

### Disease Overview

Homocystinuria is a group of rare, autosomal recessive disorders caused by mutations in specific enzymes that metabolize amino acids.<sup>2,3</sup> Elevated levels of homocysteine can lead to abnormalities in the central nervous system, eye, skeletal system, and vascular system.

### Clinical Efficacy

Clinical and observational studies demonstrated patients with homocystinuria who received betaine anhydrous powder had significant reductions plasma homocystine or homocysteine concentrations.<sup>1</sup> Additionally, improvement in seizures or behavioral and cognitive functioning were reported for many patients. Many of these patients were also taking other therapies such as vitamin B6 (pyridoxine), vitamin B12 (cobalamin), and folate with variable biochemical responses.

### POLICY STATEMENT

Prior Authorization is recommended for prescription benefit coverage of betaine anhydrous powder (Cystadane, generic). All approvals are provided for the duration noted below. Because of the specialized skills required for evaluation and diagnosis of patients treated with betaine anhydrous powder (Cystadane, generic) as well as the monitoring required for adverse events and long-term efficacy, approval requires betaine anhydrous powder (Cystadane, generic) to be prescribed by or in consultation with a physician who specializes in the condition being treated.

**Automation:** None.

### RECOMMENDED AUTHORIZATION CRITERIA

Coverage of betaine anhydrous powder (Cystadane, generic) is recommended in those who meet the following criteria:

### FDA-Approved Indication

1. **Homocystinuria.** Approve for 1 year if the patient meets the following (A, B, and C):
  - A) Patient has a confirmed diagnosis based on genetic testing demonstrating one of the following (i, ii, or iii):
    - i. Cystathionine beta-synthase deficiency; OR
    - ii. 5,10-methylenetetrahydrofolate reductase deficiency; OR

09/11/2024

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- iii. Cobalamin cofactor metabolism defect; AND
- B) Patient has tried or is concurrently receiving vitamin B6 (pyridoxine), vitamin B12 (cobalamin), or folate supplementation; AND
- C) The medication is prescribed by or in consultation with a geneticist, metabolic disease specialist, or a physician who specializes in the management of homocystinuria.

#### **CONDITIONS NOT RECOMMENDED FOR APPROVAL**

Coverage of betaine anhydrous powder (Cystadane, generic) is not recommended in the following situations:

1. Coverage is not recommended for circumstances not listed in the Recommended Authorization Criteria. Criteria will be updated as new published data are available.

#### **REFERENCES**

1. Cystadane® powder [prescribing information]. Lebanon, NJ: Recordati Rare Diseases; October 2019.
2. Truitt C, Hoff WD, Deole R. Health functionalities of betaine in patients with homocystinuria. *Front Nutr.* 2021 Sep 9;8:690359.
3. Morris A, Kožich V, Santra S, et al. Guidelines for the diagnosis and management of cystathionine beta-synthase deficiency. *J Inherit Metab Dis.* 2017 Jan;40(1):49-74.