Reference number(s)
2988-A

SPECIALTY GUIDELINE MANAGEMENT

CYSTADANE (betaine anhydrous)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indications
Cystadane is indicated for the treatment of homocystinuria to decrease the elevated homocysteine blood concentrations in pediatric and adult patients. Included within the category of homocystinuria are:
A. Cystathionine beta-synthase (CBS) deficiency
B. 5,10-methylenetetrahydrofolate reductase (MTHFR) deficiency
C. Cobalamin cofactor metabolism (cbl) defect

All other indications are considered experimental/investigational and not medically necessary.

II. REQUIRED DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:
A. For cystathionine beta-synthase (CBS) deficiency, enzyme analysis of CBS activity or genetic testing results
B. For 5,10-methylenetetrahydrofolate reductase (MTHFR) deficiency, enzyme analysis of MTHFR activity or genetic testing results
C. For cobalamin cofactor metabolism (cbl) defect, genetic testing results

III. CRITERIA FOR INITIAL APPROVAL

Homocystinuria
Authorization of 12 months may be granted for treatment of homocystinuria to decrease elevated homocysteine blood levels when all of the following criteria are met:
A. The member has one of the following types of homocystinuria and the diagnosis was confirmed by enzyme assay or genetic testing:
    1. Cystathionine beta-synthase (CBS) deficiency
    2. 5,10-methylenetetrahydrofolate reductase (MTHFR) deficiency
    3. Cobalamin cofactor metabolism (cbl) defect
B. If the member has CBS deficiency, plasma methionine concentrations will be monitored and kept below 1,000 micromol/L through dietary modification, and if necessary, a reduction in Cystadane dose.

IV. CONTINUATION OF THERAPY

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for homocystinuria when both of the following criteria are met:

1. The total homocysteine level is undetectable or present only in small amounts, OR there is a substantial decrease in homocysteine levels and the dose will be increased until maximum tolerability or plasma total homocysteine is undetectable or present in only small amounts.
2. If the member has CBS deficiency, plasma methionine concentrations will be monitored and kept below 1,000 micromol/L through dietary modification, and if necessary, a reduction in Cystadane dose.

V. REFERENCES

1. Cystadane [package insert]. Lebanon, NJ: Recordati Rare Diseases, Inc.; October 2019.