Effective Date: 7/1/2020
Last Reviewed: 4/2020, 2/2021, 2/2022 Scope: Medicaid

Sodium Phenylbutyrate oral tablet and powder

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 Indication
Adjunctive treatment to the chronic management of patients with urea cycle disorders (UCDs) involving deficiencies of carbamylphosphate synthetase (CPS), ornithine transcarbamylase (OTC), or argininosuccinic acid synthetase (AS).
Indicated in patients with neonatal-onset deficiency (complete enzymatic deficiency, presenting within the first 28 days of life).
Indicated in patients with late-onset disease (partial enzymatic deficiency, presenting after the first month of life) who have a history of hyperammonemic encephalopathy.

All other indications are considered experimental/investigational and are not a covered benefit.

II. CRITERIA FOR APPROVAL

A. Urea Cycle Disorder involving deficiencies of CPS, OTC, or AS
An authorization may be granted for 6 months when the following criteria are met:

1. Member is at least 20 kg.
2. Documentation of urea cycle disorder as evidenced by a lab value of: fasting ammonia level > 0.5 Upper Limit of Normal OR a glutamine level that is > 1,000 μmol/L.
3. Diagnosis is confirmed by enzymatic, biochemical, or genetic testing
4. Member must have had an inadequate response to and continue to be on a protein restricted diet or amino acid supplementation.
5. Should not be used for the management of acute hyperammonemia.
6. Prescribed dose does not exceed 20 grams per day.

III. CONTINUATION OF THERAPY

Authorization of 6 months may be granted for continued treatment in members requesting reauthorization for chronic management of a urea cycle disorder (UCD), who are experiencing benefit from therapy as evidenced by a reduction in plasma ammonia levels from baseline.

IV. QUANTITY LIMIT

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Sodium Phenylbutyrate has a quantity limit of #1200 tablets per 30 days for the 500mg tablets.

V. REFERENCES

1. Buphenyl (sodium phenylbutyrate). Horizon Therapeutics. Lake Forest, IL. FDA Package Insert. May 2021.

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