Clinical Policy: Sodium phenylbutyrate (Buphenyl)

Reference Number: PA.CP.PHAR.208
Effective Date: 01/18
Last Review Date: 07/18

Coding Implications
Revision Log

Description
The intent of the criteria is to ensure that patients follow selection elements established by Pennsylvania Health and Wellness® clinical policy for sodium phenylbutyrate (Buphenyl®).

FDA Approved Indication(s)
Buphenyl is indicated as adjunctive therapy in the chronic management of patients with urea cycle disorders (UCDs) involving deficiencies of carbamylphosphate synthetase (CPS), ornithine transcarbamylase (OTC), or argininosuccinic acid synthetase (ASS).

Limitation(s) of use: Buphenyl should not be used to manage acute hyperammonemia, which is a medical emergency.

Policy/Criteria
It is the policy of Pennsylvania Health and Wellness that Buphenyl is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Urea Cycle Disorder: CPS, OTC, AS (must meet all):

1. Prescribed by or in consultation with a physician experienced in treating metabolic disorder;
2. Diagnosis of one of the following urea cycle disorders (UCDs) confirmed by enzymatic, biochemical or genetic analysis:
   a. Carbamylphosphate synthetase (CPS) deficiency;
   b. Ornithine transcarbamylase (OTC) deficiency;
   c. Argininosuccinic acid synthetase (AS) deficiency;
3. Prescribed dose does not exceed 20 grams of sodium phenylbutyrate per day.

Approval duration: 6 months

B. Other diagnoses/indications: Refer to PA.CP.PMN.53

II. Continued Approval

A. Urea Cycle Disorder: CPS, OTC, AS (must meet all)**:**

1. Currently receiving medication via Pennsylvania Health and Wellness benefit or member has previously met all initial approval criteria; or the Continuity of Care policy (PA.LTSS.PHAR.01) applies;
2. Member is responding positively to therapy;
3. Prescribed dose does not exceed 20 grams of sodium phenylbutyrate per day;

Approval duration: 12 months

B. Other diagnoses/indications (1 or 2)**:**

1. Currently receiving medication via Pennsylvania Health and Wellness benefit and documentation supports positive response to therapy; or the Continuity of Care policy (PA.LTSS.PHAR.01) applies;
   Approval duration: Duration of request or 6 months (whichever is less); or

2. Refer to PA.CP.PMN.53

Background

Description/Mechanism of Action:

Sodium phenylbutyrate is a pro-drug and is rapidly metabolized to phenylacetate. Phenylacetate is a metabolically-active compound that conjugates with glutamine via acetylation to form phenylacetylglutamine. Phenylacetylglutamine then is excreted by the kidneys. On a molar basis, it is comparable to urea (each containing two moles of nitrogen). Therefore, phenylacetylglutamine provides an alternate vehicle for waste nitrogen excretion. Sodium phenylbutyrate is an oral product that provides an alternative vehicle for nitrogen waste removal in patients with UCDs.

Formulations:

Buphenyl is supplied as

- Oral tablets containing 500 mg of sodium phenylbutyrate;
- Oral powder containing 3.0 grams of sodium phenylbutyrate per level teaspoon.

Sodium phenylbutyrate is supplied as

- Oral powder containing 3.0 grams of sodium phenylbutyrate per level teaspoon.

Appendices

Appendix A: Abbreviation Key

AS: argininosuccinate synthetase
CPS: carbamylphosphate synthetase
OTC: ornithine transcarbamylase
UCD: urea cycle disorder

Coding Implications

Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
N/A	

Reviews, Revisions, and Approvals	Date	Approval Date
Removed dietary protein restriction requirements as this cannot be confirmed. References reviewed and updated	02/18	

References

1. Buphenyl Prescribing Information. Lake Forest, IL: Horizon Pharma USA, Inc.; April 2016. Available at http://www.horizonpharma.com/wp-content/uploads/2016/06/BUPHENYL_PI_April-2016.pdf. Accessed November 15, 2017.