# Commercial/Healthcare Exchange PA Criteria

***Effective: March 2004***

***Prior Authorization:*** Sodium Phenylbutyrate

***Products Affected:*** Sodium phenylbutyrate oral tablet, Sodium phenylbutyrate powder for oral solution

***Medication Description:***

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 is excreted then 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 used as adjunctive therapy in the chronic management of patients with urea cycle disorders involving deficiencies of carbamoyl phosphate synthetase (CPS), ornithine transcarbamoylase (OTC), or argininosuccinic acid synthetase (AAS); neonatal-onset deficiency (complete enzymatic deficiency, presenting within the first 28 days of life); late-onset disease (partial enzymatic deficiency, presenting after the first month of life) who have a history of hyperammonemic encephalopathy.

***Covered Uses:*** Adjunct therapy to dietary protein restriction and occasionally essential amino acid supplementation in urea cycle disorders with neonatal-onset (complete enzyme deficiency and presents within the first 28 days of life) or with late-onset disease (partial deficiency occurring after the first month of life) who have a history of hyperammonemic encephalopathy.

***Exclusion Criteria:***

1. Acute hyperammonemia

***Required Medical Information:***

1. Diagnosis
2. Medical history

***Age Restrictions:*** N/A

***Prescriber Restrictions:*** N/A

***Coverage Duration:*** 12 months

***Other Criteria:***

1. Patient has a diagnosis of urea cycle disorder (UCD); AND
2. Diagnosis was confirmed by enzymatic, biochemical, or generic testing; AND
3. Patient has UCD that cannot be managed by dietary protein restriction and/or amino acid supplementation alone; AND
4. Sodium phenylbutyrate is being used in combination with dietary restriction.

***References:***


1. Product Information: BUPHENYL(R) oral tablets, powder, sodium phenylbutyrate oral tablets, powder. Ucyclyd Pharma, Inc, Scottsdale, AZ, 2009
2. Caruthers RL and Johnson CE. Stability of Extemporaneously Prepared Sodium Phenylbutyrate Oral Suspensions. *Am J Health Syst Pharm*. 2007;64(14):1513-1515. PubMed
3. Piscitelli SC, Thibault A, Figg WD, et al: Disposition of phenylbutyrate and its metabolites, phenylacetate and phenylacetylglutamine. J Clin Pharmacol 1995; 35:368-373.
4. Tuchman M, Knopman DS, & Shih VE: Episodic hyperammonemia in adult siblings with hyperornithinemia, hyperammonemia, and homocitrullinuria syndrome. Arch Neurol 1990; 47:1134-1137.


***Policy Revision history***

| Rev # | Type of Change | Summary of Change | Sections Affected | Date |
|---|---|---|---|---|
| 1 | New Policy | New Policy | All | 03/10/2016 |
| 2 | Annual Review | Updates to standard of therapy | Other Criteria, | 12/6/2019 |
| 3 | CCI to adopt EH policy | Alignment with enterprise, Replaces CCI Buphenyl policy;<br>CCI P&T Review History: 3/04, 12/05, 12/06, 6/07, 6/08, 9/09, 9/10, 12/11, 10/12, 10/13, 10/14, 11/15, 11/16, 5/17, 5/18, 5/19<br>CCI Revision Record: 5/19 | ALL | 12/12/2019 |