

## SPECIALTY GUIDELINE MANAGEMENT

### BUPHENYL (sodium phenylbutyrate) sodium phenylbutyrate (generic)

#### 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.

###### A. FDA-Approved Indication

Buphenyl is indicated as adjunctive therapy in the chronic management of patients with urea cycle disorders involving deficiencies of carbamylphosphate synthetase (CPS), ornithine transcarbamylase (OTC), or argininosuccinic acid synthetase (AS). It is indicated in all patients with neonatal-onset deficiency (complete enzymatic deficiency, presenting within the first 28 days of life). It is also indicated in patients with late-onset disease (partial enzymatic deficiency, presenting after the first month of life) who have a history of hyperammonemic encephalopathy. It is important that the diagnosis be made early and treatment initiated immediately to improve survival. Any episode of acute hyperammonemia should be treated as a life-threatening emergency.

###### B. Compendial Use

Arginase deficiency

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

##### II. DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:

###### A. Initial requests:

1. Enzyme assay, biochemical, or genetic testing results supporting diagnosis; and
2. Lab results documenting baseline plasma ammonia levels.

###### B. Continuation of therapy requests: lab results documenting a reduction in plasma ammonia levels from baseline.

##### III. CRITERIA FOR INITIAL APPROVAL

Authorization of 12 months may be granted for chronic management of urea cycle disorder (UCD) including arginase deficiency, when both of the following criteria are met:

1. The diagnosis is confirmed by enzymatic, biochemical, or genetic testing.
2. The member has elevated plasma ammonia levels at baseline.

##### IV. CONTINUATION OF THERAPY

Reference number(s)
2121-A

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for chronic management of a urea cycle disorder (UCD) including arginase deficiency, who are experiencing benefit from therapy as evidenced by a reduction in plasma ammonia levels from baseline.

## V. REFERENCES

1. Buphenyl [package insert]. Lake Forest, IL: Horizon Pharma USA, Inc.; March 2020.
2. Mew NA, Lanpher BC. Urea Cycle Disorders Overview. In: Pagon RA, Adam MP, Ardinger HH, et. al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2017 [updated April 9, 2015]. Available at: <https://www.ncbi.nlm.nih.gov/books/NBK1217/?report=printable>.
3. Häberle J, Boddaert N, Burlina A, et al. Suggested guidelines for the diagnosis and management of urea cycle disorders. *J Inherit Metab Dis*. 2019;42(6):1192-1230.
4. Wong D, Cederbaum S, Crombez, E. Arginase Deficiency. *GeneReviews*. August 2014; <http://www.ncbi.nlm.nih.gov/books/NBK1159/>.