curative

Urea Cycle Disorders Prior Authorization

Drug(s) Applied:	**Sodium phenylbutyrate tablets**

Criteria:
Drug(s) Applied will be approved when the requested medication is being used for an FDA approved indication and all of the following criteria are met:

I. Initial Therapy Criteria
- **A. Urea Cycle Disorder** as indicated by chart notes within past 180 days
  1. Diagnosis of hyperammonemia with ALL of the following:
     a) Elevated ammonia levels according to the patient's age [Neonate: plasma ammonia level 150 µmol/L (greater than 260 µg/dL); Older child or adult: plasma ammonia level greater than 100 µmol/L (175 µg/dL)] **and**
     b) Normal bicarbonate level **and**
     c) Normal anion gap **and**
     d) Normal blood glucose level **and**
  2. Urea cycle disorder of CPSID, OTCD, ASSD, ASLD, HHH, or ARGD is confirmed by enzyme analysis or genetic testing **and**
  3. Patient weighs at least 20 kg **and**
  4. Dose does not exceed 20 g/day **and**
  5. Patient is unable to maintain a plasma ammonia level within the normal range with the use of a protein restricted diet and, when clinically appropriate, essential amino acid supplementation **and**
  6. Patient will be using the requested agent as adjunctive therapy to dietary protein restriction **and**
  7. Chart notes and/or prescriber do not show documentation that the requested drug will be used for treatment of acute hyperammonemia **and**
  8. Chart notes and/or prescriber do not show documentation that patient will be taking the requested agent concurrently with haloperidol or valproic acid or prescriber has provided a statement of benefit over risk **and**
  9. Prescriber is a specialist or has consulted with a specialist in the area of the patient's diagnosis (e.g., metabolic disorders)

Approval Duration: 6 months

II. Continued Therapy Criteria
- **A. Urea Cycle Disorder** as indicated by chart notes within past 12 months

curative

1. Urea cycle disorder of CPSID, OTCD, ASSD, ASLD, HHH, or ARGD is confirmed by enzyme analysis or genetic testing **and**
2. Claims data or provider attestation confirms the patient is adherent to the prescribed regimen (defined as greater than or equal to 80% proportion of days covered) **and**
3. Dose does not exceed 20 g/day **and**
4. Patient has had clinical benefit with the requested agent (e.g., plasma ammonia level within the normal range) **and**
5. Chart notes and/or prescriber do not show documentation that the requested drug is being used for treatment of acute hyperammonemia **and**
6. Patient will be using the requested agent as adjunctive therapy to dietary protein restriction **and**
7. Chart notes and/or prescriber do not show documentation that patient will be taking the requested agent concurrently with haloperidol or valproic acid or prescriber has provided a statement of benefit over risk **and**
8. Prescriber is a specialist or has consulted with a specialist in the area of the patient's diagnosis (e.g., metabolic disorders)

Approval Duration: 12 months

References:

1. Urea Cycle Treatment Guidelines. Urea Cycle Disorders Consortium. Accessed 7/2026. https://ucdc.rarediseasesnetwork.org/resources/researchers-clinicians/treatment-guidelines
2. Häberle J, et al. Suggested guidelines for the diagnosis and management of urea cycle disorders: First revision. J Inherit Metab Dis. 2019 Nov;42(6):1192-1230. Epub 2019 May 15. https://pubmed.ncbi.nlm.nih.gov/30982989/
3. Simpson KL, MacLeod EL, Kakajiwala A, et al. Urea Cycle Disorders Overview. 2003 Apr 29. In: Adam MP, Feldman J, Mirzaa GM, et al., editors. GeneReviews®. Seattle (WA): University of Washington, Seattle; 1993-2025. Updated 2025 July 3. https://www.ncbi.nlm.nih.gov/books/NBK1217/
4. Buphenyl prescribing information. Horizon Therapeutics USA, Inc. October 2024.

Policy Owned by: PBM team