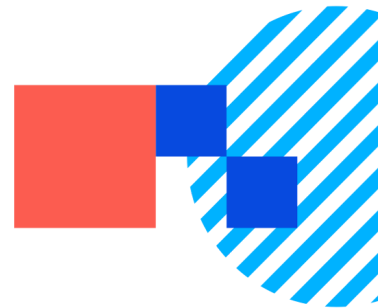




Urea Cycle Disorders Prior Authorization

Drug(s) Applied:	Sodium phenylbutyrate tablets
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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 $\mu\text{mol/L}$ (greater than 260 $\mu\text{g/dL}$); Older child or adult: plasma ammonia level greater than 100 $\mu\text{mol/L}$ (175 $\mu\text{g/dL}$)] **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



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