

Clinical Policy: Urea Cycle Disorder Agents

Reference Number: PHW.PDL.733

Effective Date: 01/01/2020

Last Review Date: 11/2024

Policy/Criteria

Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

It is the policy of PA Health & Wellness® that Urea Cycle Disorder Agents is **medically necessary** when the following criteria are met:

I. Requirements for Prior Authorization of Urea Cycle Disorder Agents

A. Prescriptions That Require Prior Authorization

Prescriptions for Urea Cycle Disorder Agents that meet any of the following conditions must be prior authorized:

1. A non-preferred Urea Cycle Disorder Agent.
2. A Urea Cycle Disorder Agent with a prescribed quantity that exceeds the quantity limit.

B. Review of Documentation for Medical Necessity

In evaluating a request for prior authorization of a prescription for a Urea Cycle Disorder Agent, the determination of whether the requested prescription is medically necessary will take into account whether the member:

1. Is prescribed the Urea Cycle Disorder Agent by or in consultation with a physician who specializes in treating metabolic disorders; **AND**
2. Is being treated for a diagnosis that is indicated in the U.S. Food and Drug Administration (FDA)-approved package labeling OR a medically accepted indication; **AND**
3. Has chart documentation supporting the diagnosis (e.g., ammonia levels, genetic testing, enzyme assays, plasma amino acid/urine orotic acid analyses, progress notes); **AND**
4. Is prescribed a dose and duration of therapy that is consistent with FDA-approved package labeling, nationally recognized compendia, or peer-reviewed medical literature; **AND**
5. For a non-preferred Urea Cycle Disorder Agent, has a documented history of

therapeutic failure, contraindication, or intolerance to the preferred Urea Cycle Disorder Agent; **AND**

6. If a prescription for a Urea Cycle Disorder Agent is in a quantity that exceeds the quantity limit, the determination of whether the prescription is medically necessary will also take into account the guidelines set forth in PA.CP.PMN.59 Quantity Limit Override.

NOTE: If the member does not meet the clinical review guidelines above but, in the professional judgement of the physician reviewer, the services are medically necessary to meet the medical needs of the member, the request for prior authorization will be approved

FOR RENEWALS OF PRESCRIPTIONS FOR UREA CYCLE DISORDER

AGENTS: The determination of medical necessity of a request for renewal of a prior authorization for a Urea Cycle Disorder Agent that was previously approved will take into account whether the member:

1. Has documentation from the prescribing provider that the member had a positive clinical response to therapy; **AND**
2. Is prescribed the Urea Cycle Disorder Agent by or in consultation with a physician who specializes in treating metabolic disorders; **AND**
3. Is prescribed a dose and duration of therapy that is consistent with FDA-approved package labeling, nationally recognized compendia, or peer-reviewed medical literature; **AND**
4. If a prescription for a Urea Cycle Disorder Agent is in a quantity that exceeds the quantity limit, the determination of whether the prescription is medically necessary will also take into account the guidelines set forth in PA.CP.PMN.59 Quantity Limit Override.

NOTE: If the member does not meet the clinical review guidelines above but, in the professional judgement of the physician reviewer, the services are medically necessary to meet the medical needs of the member, the request for prior authorization will be approved.

C. Clinical Review Process

Prior authorization personnel will review the request for prior authorization and apply the clinical guidelines in Section B. above to assess the medical necessity of a prescription for a Urea Cycle Disorder Agent. If the guidelines in Section B. are met, the reviewer will prior authorize the prescription. If the guidelines are not met, the prior authorization request will be referred to a physician reviewer for a medical necessity determination. Such a request for prior authorization will be approved when, in the professional

judgment of the physician reviewer, the services are medically necessary to meet the medical needs of the member.

D. Approval Duration:

- **New Request: 6 months**
- **Renewal Request: 12 months**

E. References

1. Haberle J, Boddaert N, et.al. Suggested guidelines for the diagnosis and management of urea cycle disorders. *Orphanet Journal of Rare Diseases*. 2012, 7:32.
2. Diaz G.A, Krivitzy L.S, et.al. Ammonia Control and Neurocognitive Outcome Among Urea Cycle Disorder Patients Treated with Glycerol Phenylbutyrate. *Hepatology*. 2013 June; 57(6): 2171–2179.
3. Smith W, Diaz G.A, et al. Ammonia control in children ages 2 months through 5 years with urea cycle disorders: comparison of sodium phenylbutyrate and glycerol phenylbutyrate. *Journal of Pediatrics*. 2013 June; 162(6): 1228–1234.
4. Ravicti Prescribing Information. Lake Forest, IL. Horizon Therapeutics, LLC.
5. Buphenyl Prescribing Information. Scottsdale, AZ: Ucyglyd Pharma Inc.; April 2009.

Reviews, Revisions, and Approvals	Date
Policy created	01/01/2020
Q3 2020 annual review: no changes.	07/2020
Q1 2021 annual review: no changes.	01/2021
Q1 2022 annual review: no changes.	11/2021
Q1 2023 annual review: no changes.	11/2022
Q1 2024 annual review: no changes.	11/2023
Q1 2025 annual review: no changes.	11/2024