Reference number(s)
2123-A

Specialty Guideline Management
Ravicti

Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

Brand Name	Generic Name
Ravicti	glycerol phenylbutyrate

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.

FDA-approved Indications[1]

Ravicti is indicated for use as a nitrogen-binding agent for chronic management of patients with urea cycle disorders (UCDs) who cannot be managed by dietary protein restriction and/or amino acid supplementation alone. Ravicti must be used with dietary protein restriction and, in some cases, dietary supplements.

Limitations of Use

- Ravicti is not indicated for treatment of acute hyperammonemia in patients with UCDs.
- Safety and efficacy for treatment of N-acetylglutamate synthase (NAGS) deficiency has not been established.

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

Documentation

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

- Initial Requests:
  - Enzyme assay, biochemical, or genetic testing results supporting diagnosis; and
  - Lab results documenting baseline plasma ammonia levels.
- Continuation of therapy requests: lab results documenting a reduction in plasma ammonia levels from baseline.

Prescriber Specialties

This medication must be prescribed by or in consultation with a physician who specializes in the treatment of enzyme or metabolic disorders.

Coverage Criteria

Urea Cycle Disorders (UCDs)[1-5]

Authorization of 12 months may be granted for chronic management of a UCD when both of the following criteria are met:

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

Continuation of Therapy

Authorization of 12 months may be granted for continued treatment in members requesting reauthorization for an indication listed in the coverage criteria section who are experiencing benefit from therapy as evidenced by a reduction in plasma ammonia levels from baseline.

References

1. Ravicti [package insert]. Deerfield, IL: Horizon Therapeutics USA, Inc.; September 2021.
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 June 22, 2017]. 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. Diaz GA, Krivitzky LS, Mokhtarani M, et al. Ammonia control and neurocognitive outcome among urea cycle disorder patients treated with glycerol phenylbutyrate. Hepatology. 2013;57(6):2171-2179.
5. Smith W, Diaz GA, Lichter-Konecki U, et al. Ammonia control in children ages 2 months through 5 years with urea cycle disorders: comparison of sodium phenylbutyrate and glycerol phenylbutyrate. J Pediatr. 2013;162(6):1228-1234.