



RATIONALE FOR INCLUSION IN PA PROGRAM

Background

Urea cycle disorders (UCDs) are genetic disorders that involve deficiencies of specific enzymes involved in the urea cycle, a series of biochemical steps normally required to remove ammonia from the blood. When protein is absorbed and broken down by the body, it produces nitrogen as a waste product. The urea cycle removes nitrogen from the blood and converts it to urea, which is removed from the body through urine. In people with UCDs, nitrogen accumulates and remains in the body as ammonia, which can travel to the brain and cause brain damage, or coma (1).

Ravicti, a liquid taken three times a day with meals, helps dispose of ammonia in the body. It is intended for patients whose UCD cannot be managed by a protein-restricted diet or amino acid supplements alone. Ravicti must be used with a protein-restricted diet and, in some cases, dietary supplements (1).

Regulatory Status

FDA-approved indication: Ravicti is indicated for use as a nitrogen-binding agent for chronic management of patients with urea cycle disorders (UCDs) that 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 (e.g., essential amino acids, arginine, citrulline, protein-free calorie supplements) (1).

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 (1).

Summary

Ravicti is indicated for use as a nitrogen-binding agent for chronic management of adult and pediatric patients with urea cycle disorders (UCDs) that 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 (e.g., essential amino acids, arginine,



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**RAVICTI
(glycerol phenylbutyrate)**

citrulline, protein-free calorie supplements). 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 (1).

Prior approval is required to ensure the safe, clinically appropriate, and cost-effective use of Ravicti while maintaining optimal therapeutic outcomes.

References

1. Ravicti [package insert]. Lake Forest, IL: Horizon Therapeutics, LLC.; September 2021.