Ravicti® (glycerol phenylbutyrate) – Expanded indication

- On May 1, 2017, Horizon Pharma announced the FDA approval of Ravicti (glycerol phenylbutyrate) oral liquid, for chronic management of patients 2 months of age and older with urea cycle disorders (UCDs) who cannot be managed by dietary protein restriction and/or amino acid supplementation alone.
    - Previously, Ravicti was approved for people 2 years of age and older.
    - Ravicti must be used with dietary protein restriction and, in some cases, dietary supplements.
    - Ravicti is not indicated for treatment of acute hyperammonemia in patients with UCDs because more rapidly acting interventions are essential to reduce plasma ammonia levels.
    - Safety and efficacy for treatment of N-acetylglutamate synthase deficiency have not been established.

- UCD is a rare genetic disorder that affects an estimated 1 in 35,000 live births in the U.S. It is caused by an enzyme deficiency in the urea cycle, a process that is responsible for converting excess ammonia from the bloodstream and ultimately removing it from the body. Elevated ammonia levels in the blood can reach the brain causing irreversible brain damage, coma or death.

- The expanded indication for Ravicti was approved based on 3 open-label studies conducted in 17 pediatric patients between 2 months and < 2 years of age with UCDs. The studies assessed monthly ammonia control and hyperammonemic crises.
    - Ravicti-treated patients maintained stable ammonia levels relative to their pre-study enrollment.

- The most common adverse reactions (≥ 10%) with Ravicti use in patients 2 months to < 2 years of age were neutropenia, vomiting, diarrhea, pyrexia, hypophagia, cough, nasal congestion, rhinorrhea, rash and papule.

- The recommended dose of Ravicti is based on body surface area. In patients 2 months to < 2 years of age, Ravicti should be given in 3 or more equally divided dosages, each rounded up to the nearest 0.1 mL.