Olpruva™ (sodium phenylbutyrate) – New drug approval

- On December 27, 2022, Acer Therapeutics and Relief Therapeutics announced the FDA approval of Olpruva (sodium phenylbutyrate) oral suspension, as adjunctive therapy to standard of care, which includes dietary management, for the chronic management of adult and pediatric patients weighing 20 kg or greater and with a body surface area of 1.2 m^2 or greater, with urea cycle disorders involving deficiencies of carbamylphosphate synthetase, ornithine transcarbamylase, or argininosuccinic acid synthetase.
    - Olpruva is not indicated for the treatment of acute hyperammonemia.
- Acer cited preclinical and clinical safety and efficacy data from the reference listed drug, Buphenyl® (sodium phenylbutyrate) powder. Acer also provided additional data including studies that evaluated the bioavailability and bioequivalence of Olpruva compared to Buphenyl powder.
- Warnings and precautions for Olpruva include neurotoxicity of phenylacetate, hypokalemia, and conditions associated with edema.
- The most common adverse reactions (≥ 3%) with Olpruva use were menstrual dysfunction, decreased appetite, body odor and bad taste or taste aversion.
- The recommended dose of Olpruva is 9.9 −13 g/m^2/day orally. The calculated total daily dose should be administered as three to six divided doses and taken with food.
    - Each individual dose of Olpruva should be rounded to the nearest available dosage strength.
    - The maximum dosage is 20 grams per day.
    - The Olpruva dosage should be adjusted to maintain the plasma ammonia level within the normal range for the patient's age, taking into consideration their clinical condition (eg, nutritional requirements, protein intake, growth parameters, etc.).
    - Olpruva should be combined with dietary protein restriction and, in some cases, amino acid supplementation (eg, essential amino acids, arginine, citrulline, and protein-free calorie supplements).
- Acer and Relief's launch plans for Olpruva are pending. Olpruva will be available as 2 g, 3 g, 4 g, 5 g, 6 g, and 6.67 g pellets for oral suspension.