

Pheburane[®] (sodium phenylbutyrate) – New orphan drug approval

- On June 17, 2022, the [FDA approved](#) Medunik's [Pheburane \(sodium phenylbutyrate\)](#) oral pellets, as adjunctive therapy to standard of care, which includes dietary management, for the chronic management of adult and pediatric patients with urea cycle disorders (UCDs), involving deficiencies of carbamylphosphate synthetase (CPS), ornithine transcarbamylase (OTC) or argininosuccinic acid synthetase (AS).
 - Episodes of acute hyperammonemia may occur in patients while on Pheburane. Pheburane is not indicated for the treatment of acute hyperammonemia, which can be a life-threatening medical emergency that requires rapid acting interventions to reduce plasma ammonia levels.
- Sodium phenylbutyrate is also available generically as an [oral tablet](#) and a [powder for oral solution](#). Both of these formulations carry similar indications as Pheburane.
- Warnings and precautions for Pheburane include neurotoxicity of phenylacetate; hypokalemia; conditions associated with edema; and diabetes mellitus, hereditary fructose intolerance, glucose galactose malabsorption or sucrase-isomaltase insufficiency.
- The most common adverse reactions ($\geq 3\%$) with Pheburane use were menstrual dysfunction, decreased appetite, body odor and bad taste or taste aversion.
- The recommended dosage of Pheburane is:
 - Patients weighing less than 20 kg: 450 to 600 mg/kg/day of sodium phenylbutyrate orally. The calculated total daily dose should be divided into three to six doses.
 - Patients weighing greater than or equal to 20 kg: 9.9 to 13 g/m²/day of sodium phenylbutyrate orally. The calculated total daily dose should be divided into three to six doses.
 - The maximum dosage is 20 grams per day. Pheburane 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).
- Pheburane treatment should be supervised by a healthcare provider experienced in the treatment of UCDs.
- Medunik's launch plans for Pheburane are pending. Pheburane oral pellets will be available as 84 g of sodium phenylbutyrate per Pheburane bottle.