Notice of Marketing Authorization for "RAVICTI® Oral Liquid 1.1 g/mL" for Urea Cycle Disorders

Tokyo, Japan — December 22, 2025 — OrphanPacific, Inc. (Head Office: Minato-ku, Tokyo; President and Representative Director: Megumi Hara; hereinafter "OrphanPacific") is pleased to announce that today we obtained marketing authorization in Japan for **"RAVICTI® Oral Liquid 1.1 g/mL"** (generic name: glycerol phenylbutyrate; hereinafter "RAVICTI®"), indicated for **Urea Cycle Disorders (UCD)**.

Ravicti® is a prodrug of phenylbutyrate, formulated as an oral liquid that is easy to administer with minimal taste and odor. Even when the same amount of active ingredient (dose) as **BUPHENYL® Tablets 500 mg** and **Granules 94%** (generic name: sodium phenylbutyrate) is required, Ravicti® allows patients to take a smaller volume of liquid due to its formulation characteristics, while providing comparable and sustained blood ammonia control. These features help reduce the treatment burden and offer a new therapeutic option for patients with UCD and their families.

OrphanPacific believes that RAVICTI® will help address unmet medical needs in UCD treatment. We will continue to provide information for appropriate use and remain committed to the development and supply of medicines for rare diseases, contributing to improved quality of life for patients and their families.

Summary of Approval

Item	Details
Brand Name	RAVICTI® Oral Liquid 1.1 g/mL
Generic Name	Glycerol Phenylbutyrate
Date of Approval	December 22, 2025
Indication	Urea Cycle Disorders
Dosage and Administration	The usual starting dose is 4.5 mL/m² of body surface area per day, divided into 3 to 6 doses, administered orally with or immediately after meals or nutritional supplements. The dose may be adjusted according to the patient's condition, but should not exceed 11.2 mL/m² per day.
Marketing Authorization Holder	OrphanPacific, Inc.

■ About Urea Cycle Disorders (UCD)

The urea cycle, primarily in the liver, converts toxic ammonia produced in the body into non-toxic urea. UCD is a group of disorders caused by congenital defects in enzymes involved in this cycle, leading to hyperammonemia and symptoms such as vomiting, poor feeding, tachypnea, seizures, impaired consciousness, behavioral abnormalities, and developmental delays. In severe cases, it can be life-threatening. Most cases present in infancy, but some are diagnosed in adulthood. The estimated incidence is 1 in 8,000 to 44,000, and UCD is designated as an intractable disease in Japan.

Reference: Nanbyou Information Center

https://www.nanbyou.or.jp/entry/4732

■ About Ravicti® (glycerol phenylbutyrate)

Ravicti is a medicine used to treat patients of all ages with UCDs, including deficiencies of carbamoyl phosphate synthetase I (CPS), ornithine carbamoyltransferase (OTC), argininosuccinate synthetase (ASS), argininosuccinate lyase (ASL), arginase I (ARG) and ornithine translocase deficiency hyperornithinaemia-hyperammonaemia homocitrullinuria syndrome (HHH) 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 (e.g., essential amino acids, arginine, citrulline, protein-free calorie supplements).

The medicine is used to reduce the amount of ammonia in the blood in order to reduce the risk of neurological consequences.

■ About OrphanPacific, Inc.

OrphanPacific is a Japanese pharmaceutical company dedicated to the development, manufacturing, and marketing of drugs for rare diseases, thereby delivering new treatment options to patients with rare diseases. Our mission is “Bringing smiles and happiness to patients with rare diseases and their families.” With a commitment to “Leave No One Behind," we are proactively engaged in the development and provision of therapies for rare diseases with extremely small patient populations.

OrphanPacific is a wholly owned subsidiary of CMIC Holdings (https://www.cmicgroup.com/), a pioneer and leading CRO (Contract Research Organization) in Japan. By leveraging the full breadth of CMIC Group's expertise and experience in pharmaceutical development, manufacturing, and marketing, we strive to ensure that as many patients with rare diseases as possible have access to therapies. https://www.orphanpacific.com/

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