

## **Announcement of Application for Approval of Glycerol Phenylbutyrate for the Treatment of Urea Cycle Disorder**

OrphanPacific, Inc. (Minato-ku, Tokyo, Japan; President: Megumi Hara; hereinafter referred to as "OrphanPacific") is pleased to announce that today, we have submitted an application to the Ministry of Health, Labour and Welfare for the domestic approval of glycerol phenylbutyrate (development code: HPN-100, overseas trade name: RAVICTI<sup>®</sup>) for the treatment of Urea Cycle Disorder (UCD).

OrphanPacific currently offers Buphenyl<sup>®</sup> (sodium phenylbutyrate) in granule and tablet forms for the treatment of UCD. Glycerol phenylbutyrate is a prodrug of phenylbutyrate, which converts to phenylbutyrate in the body similar to Buphenyl<sup>®</sup>. This tasteless and odorless oral liquid formulation is easy to take and provides UCD patients and their families with a new treatment option.

Results from the domestic Phase III clinical trial (HPN-100-J001 study) have shown that glycerol phenylbutyrate can control blood ammonia levels similarly to Buphenyl<sup>®</sup> and has demonstrated favorable safety and tolerability.

OrphanPacific is committed to delivering glycerol phenylbutyrate to patients with UCD and their families as soon as possible.

### **■ About Urea Cycle Disorders (UCD)**

The urea cycle is a metabolic pathway primarily in the liver that converts toxic ammonia produced in the body into harmless urea. Urea Cycle Disorder (UCD) is a group of disorders characterized by congenital abnormalities in the enzymes involved in the urea cycle, leading to hyperammonemia and other symptoms. Symptoms include vomiting, poor feeding, rapid breathing, seizures, altered consciousness, behavioral abnormalities, and developmental retardation. In severe cases, it can be life-threatening. While many cases present in infancy, some individuals are diagnosed in adulthood. The disorder occurs at a frequency of 1 in 8,000 to 44,000 people and is classified as a designated intractable disease.

[Reference] Nanbyo Information Center, Urea Cycle Disorder (Designated Intractable Disease 251, <https://www.nanbyou.or.jp/entry/4732> Accessed on 25 December, 2024)

## ■ About OrphanPacific

OrphanPacific is a Japanese pharmaceutical company dedicated to developing, manufacturing, and marketing treatments for rare diseases. Our mission is to "bring smiles and happiness to patients with rare diseases and their families." With a commitment to "Leave No One Behind," we actively engage in the development and provision of treatments for rare diseases with very few patients.

OrphanPacific is a 100% subsidiary of CMIC Holdings (<https://en.cmicgroup.com/>), a pioneer and leading company in Japan's CRO (Contract Research Organization) industry. We leverage the extensive experience and expertise of the CMIC Group in drug development, manufacturing, and marketing to ensure that as many patients with rare diseases as possible have access to treatments. <https://www.orphanpacific.com/en/>

### < Contact >

OrphanPacific, Inc., Corporate Planning Department.

[info@orphanpacific.com](mailto:info@orphanpacific.com)

Tel.03-6779-8151