December 25, 2024

Announcement Regarding the Designation of Glycerol Phenylbutyrate as an Orphan Drug

OrphanPacific, Inc. (Minato-ku, Tokyo,Japan; President: Megumi Hara; hereinafter referred to as "OrphanPacific") is pleased to announce that on December 25, 2024, glycerol phenylbutyrate (the development code: HPN-100) has been designated as an orphan drug by the Ministry of Health, Labour and Welfare for the expected indication of Urea Cycle Disorder (UCD).

To manage the increase in blood ammonia levels caused by UCD, sodium phenylbutyrate (brand name: Buphenyl®) was designated as an orphan drug in September 2008, and OrphanPacific currently sells tablets and granules in Japan. Glycerol phenylbutyrate is a prodrug and converts to the active substance in the body similar to Buphenyl®. This tasteless and odorless oral liquid formulation is easy to take and provides UCD patients and their families with a new treatment option.

OrphanPacific has been a valued partner to Immedica in Japan, holding exclusive rights to Buphenyl®. This collaboration was further strengthened in May 2022 through an exclusive license agreement, adding glycerol phenylbutyrate to their portfolio for the Japanese market.

OrphanPacific is committed to developing new pharmaceuticals that expand treatment options for diseases with unmet medical needs and strives to deliver glycerol phenylbutyrate to patients with UCD and their families as soon as possible.

■ Overview of the Orphan Drug Designation System

About the Designation System

Orphan drugs, orphan medical devices, and orphan regenerative medical products are designated by the Minister of Health, Labour and Welfare based on Article 77-2 of the Pharmaceuticals and Medical Devices Act. This designation is given to products that meet conditions such as having fewer than 50,000 patients in Japan and being particularly necessary from a medical standpoint, following the opinion of the Pharmaceutical Affairs and Food Sanitation Council.

Designation Criteria

The Minister of Health, Labour and Welfare can designate products as orphan drugs, orphan medical devices, or orphan regenerative medical products based on applications from companies, provided they meet the designation criteria. To be designated as an orphan drug, etc., the product must satisfy criteria related to "number of target patients," "medical necessity," and "development potential."

https://www.mhlw.go.jp/stf/seisakunitsuite/bunya/0000068484.html

(Accessed on December 25, 2024)

■ 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 Immedica Pharma AB

Immedica is a fast-growing private European niche pharma group with excellent proven track record and high performance in international alliances and sales, and has know-how and experience in selling orphan drugs/specialty care products in Europe and the Middle East. Having headquartered in Stockholm, Sweden, Immedica has direct trade areas in Europe and the Middle East, and, in addition, provides some of its products to other parts of the world via a network of regional partners. The company's management has an excellent track record in international alliances and sales of orphan drugs, and Immedica has capabilities to provide optimal access of specialty care medicines to patients with significant medical needs, including key areas such as regulatory affairs, pharmacovigilance, medical affairs, pricing & reimbursement, and product distribution.

https://www.immedica.com/en

■ 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/

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