Announcement of exclusive license agreement for development, manufacturing and sale of glycerol phenylbutyrate in Japan

OrphanPacific, Inc. (hereinafter referred to as “OrphanPacific”) and Immedica Pharma AB (hereinafter referred to as “Immedica”) announced that on May 2, 2022, both companies signed an exclusive license agreement for the following rights in Japan:

- Development, manufacturing and sale of glycerol phenylbutyrate (the brand name in the United States and Europe: Ravicti®) for the treatment of Urea Cycle Disorders (UCD)

OrphanPacific has already manufactured and sold Buphenyl® (sodium phenylbutyrate) that has been supplied by Immedica to Japan. This time, Immedica has further granted OrphanPacific the rights within Japan to develop, manufacture and sell glycerol phenylbutyrate that has already been approved and sold for the treatment of UCD in the United States and Europe,

In Japan, both Buphenyl® tablets and granules are currently prescribed to control the increase in blood ammonia levels caused by UCD. In the United States and Europe, Ravicti®, has been widely used instead of Buphenyl® for the management of UCD patients. Ravicti® is an oral liquid containing glycerol phenylbutyrate that is tasteless, odorless and easy to take, and it is a highly convenient formulation, especially for infants and pediatric patients. Accordingly, medical professionals have been expecting that Ravicti® will become available in Japan as well.

OrphanPacific is going to start a clinical trial for obtaining approval for glycerol phenylbutyrate in Japan.

■ About Urea Cycle Disorders (UCD)

The Urea Cycle is a metabolic pathway that converts toxic ammonia (NH_3) generated in the body into non-toxic urea mainly in the liver. The UCD is a group of diseases that present with hyperammonemia due to congenital abnormalities in enzymes involved in the Urea Cycle. Patients with UCD may experience vomiting, poor feeding, tachypnea, convulsions, impaired consciousness, behavioral disorders, developmental disorders, and sometimes life-threatening conditions. Most of UCD patients develop in infancy, but some of them are diagnosed only in adulthood. The UCD occurs in 1 in 8,000 to 44,000 people and is one of the designated intractable diseases in Japan.
[Reference] Intractable Disease Information Center; Urea Cycle Disorders (Designated Intractable Disease 251)
https://www.nanbyou.or.jp/entry/4732 (Access as of 18 May 2022, in Japanese)

■ About OrphanPacific

OrphanPacific is a Japanese pharmaceutical company that brings new therapeutic drugs to patients with rare diseases through the development, manufacturing and sale of orphan drugs. The company’s mission is to "deliver smiles and happiness to patients with rare diseases and their families." With the determination of "Leave No One Behind", OrphanPacific is actively

working on the development and distribution of drugs for the treatment of rare diseases with a very small number of patients. OrphanPacific is a wholly owned subsidiary of CMIC Holdings, a pioneer and leading CRO (Contract Research Organization) in Japan. Making the best use of the CMIC Group's experience, and know-how of drug development, manufacturing and sales, the company aims to enable as many patients with rare diseases as possible to access to therapeutic drugs.

■ 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.

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