April 13, 2023

Announcement for Initiation of Phase III Trial of Glycerol Phenylbutyrate in Urea Cycle Disorders in Japan

OrphanPacific, Inc. (Minato-ku, Tokyo, Japan; hereinafter referred to as “OrphanPacific”) announces that the first patient has been successfully enrolled in the phase III clinical trial of glycerol phenylbutyrate (the development code: HPN-100; the brand name in the United States and Europe: Ravicti®) in Japanese patients with Urea Cycle Disorders (UCD).

In Japan, both Buphenyl® tablets and granules are currently being prescribed to control the increase in blood ammonia levels caused by UCD, while 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 a highly convenient formulation, especially for infants and pediatric patients; therefore, it has been awaited to become available in Japan as well.

OrphanPacific obtained exclusive rights to develop, manufacture and sell Ravicti® in Japan on May 2, 2022, from Immedica Pharma AB (Stockholm, Sweden).

This phase III clinical trial is planned to enroll 15 pediatric and adult patients with UCD in Japan. The primary objective of this study is to evaluate the effectiveness of glycerol phenylbutyrate in controlling blood ammonia levels compared with that of Buphenyl®. In addition, pharmacokinetics and safety of glycerol phenylbutyrate will be evaluated as well compared with those of Buphenyl® in pediatric and adult patients.

For detailed information on this study (jRCT2071220110), please visit Japan Registry of Clinical Traials.

OrphanPacific will strive to deliver Ravicti® as quickly as possible for patients who are fighting this intractable disease, UCD and their families.

■ 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 per 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 (written in Japanese; access as of 10 April. 2023)

■ 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 development, manufacturing and sales of drugs, the company aims to enable as many patients with rare diseases as possible to have access to therapeutic drugs.

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