Application to the Ministry of Health in Japan for Ravicti® (Glycerol Phenylbutyrate) submitted

Stockholm, December 27, 2024 – Immedica is pleased to announce that its partner OrphanPacific, Inc has submitted an application to the Ministry of Health, Labour and Welfare in Japan for the approval of Ravicti® (glycerol phenylbutyrate) for the treatment of Urea Cycle Disorders (UCD). The submission follows the approval of the orphan drug designation of Ravicti on December 25.

"This submission marks a significant step toward bringing this important treatment addition to patients with UCD in Japan. We are pleased to see this progress together with our partner OrphanPacific, as we continue our commitment to addressing unmet medical needs in rare diseases" says Anders Edvell, CEO of Immedica.

An orphan drug designation is handled by the Ministry of Health, Labour and Welfare based on Article 77-2 of the Pharmaceuticals and Medical Devices Act. The 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 an opinion of the Pharmaceutical Affairs and Food Sanitation Council.

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

About Urea Cycle Disorders (UCD)
Urea cycle disorders are a group of metabolic diseases that affect a specific enzyme or transporter in the urea cycle leading to elevated ammonia or glutamine levels in the circulation. Symptoms of the disorder can begin at any age, where more severe defects are seen with an onset of the disease early in life. UCD patients may experience episodes, called hyperammonemic crises, when ammonia levels in the blood become excessively high, which can result in irreversible brain damage, coma, or death. Beyond hyperammonemic crises there are also more subtle symptoms including vomiting, refusal to feed, irritability, muscular hypotonia as well as delayed motor and psychointellectual development. As a group, these disorders occur in 1 in 35,000 newborns.

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
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.

http://www.orphanpacific.com/

About Immedica
Immedica is a pharmaceutical company, headquartered in Stockholm, Sweden, focused on the commercialization of medicines for rare diseases and specialty care products. Immedica's capabilities cover marketing and sales, compliance, pharmacovigilance, quality assurance, regulatory, medical affairs and market access, as well as a global distribution network serving patients in more than 50 countries. Immedica is fully dedicated to helping those living with diseases which have a large unmet medical need. Immedica's therapeutic areas are within genetic & metabolic diseases, hematology & oncology and specialty care.

Immedica was founded in 2018 and employs today around 130 people across Europe, the Middle East and the U.S. The main owners are the investment firms KKR and Impilo.

For more information visit www.immedica.com

Immedica contact:
Linda Holmström
Head of Communication
linda.holmstrom@immedica.com
+ 46 708 73 40 95