# Immedica's partner OrphanPacific initiates phase 3 trial with glycerol phenylbutyrate for treatment of urea cycle disorders in Japan

**Stockholm, April 21 , 2023** - Immedica's partner OrphanPacific Inc. (Tokyo, Japan) has enrolled the first patient in the phase 3 clinical trial with glycerol phenylbutyrate for the treatment of urea cycle disorders (UCD) in Japan.

Immedica's CEO Anders Edvell commented: "There is a significant medical need for better treatment options for people living with urea cycle disorders around the world. The initiation of this trial is a significant step towards expanding access to glycerol phenylbutyrate for patients in Japan."

This phase 3 clinical trial is planned to enroll 15 pediatric and adult patients with UCD in Japan. The primary objective of the study is to evaluate the effectiveness of glycerol phenylbutrate in controlling blood ammonia levels compared with Buphenyl® (sodium phenylbutyrate), which is currently an approved treatment option in Japan. In addition, pharmacokinetics, and safety of glycerol phenylbutrate will be evaluated as well compared with those of Buphenyl® in pediatric and adult patients.

On May 2, 2022, OrphanPacific obtained the exclusive rights to develop, manufacture and sell glycerol phenylbutyrate in Japan from Immedica.

**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, with more severe defects beginning 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 hyper 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 glycerol phenylbutyrate**
Glycerol phenylbutyrate 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. The medicine is used to reduce the amount of ammonia in the blood in order to reduce the risk of neurological consequences.

Glycerol phenylbutyrate is not approved for treatment of UCDs in Japan.

**About Immedica Pharma**
Immedica is 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 and medical affairs as well as market access. 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 by the investment company Impilo and Buy-in-Management. Today Immedica employs more than 90 people across Europe and the Middle East.

For more information visit www.immedica.com

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