Immedica initiates a phase 3 pediatric study for Loargys® (pegzilarginase) in arginase 1 deficiency

Stockholm, September 18, 2024: Immedica announces today that the first subject has been dosed in the phase 3 pediatric study (CAEB1102-301A) for Loargys® (pegzilarginase) in arginase 1 deficiency (ARG1-D).

Loargys is approved in the EU and Great Britain for the treatment of ARG1-D in adults, adolescents and children aged 2 years and older. The initiation of a clinical study in children below 2 years of age is of high importance in this progressive disease exposing patients to elevated toxic levels of arginine from birth.

Study CAEB1102-301A is a phase 3, open-label, single-arm, non-controlled, repeat dosing, international, multicenter study to evaluate the safety, pharmacokinetics, and activity (pharmacodynamics) of weekly subcutaneous administration of pegzilarginase over 12 weeks in subjects with ARG1-D who are < 24 months of age.

“The approval of Loargys for patients with ARG1-D is addressing a high unmet medical need in this disease, providing the first disease-modifying treatment. However, Immedica is committed to serve all patients with the disease and to investigate the ability to treat patients as early as possible, before any effects of the disease in the crucial first years of life, a particularly important period of child development”, says Mattias Rudebeck, Head of Global Integrated Evidence Generation and Global Medical Head for Genetic and Metabolic Diseases at Immedica.

Dr Arunabha Ghosh at Bradford Royal Infirmary in the United Kingdom and the international coordinating investigator for the study says: “As an experienced clinical study center for the previous pivotal study (PEACE) with pegzilarginase we have earned great insights in the efficacy and safety data for pegzilarginase in ARG1-D. Data enabling the initiation of treatment already in the first years of life could have substantial impact on the course of the disease in the future”.

“Immedica is a fast-growing pharmaceutical company, and with the increased opportunities in our pipeline, our organization is evolving with the building of our new scientific function with senior experts in data generation strategies and clinical studies,” says Anders Edvell, Chief Executive Officer. He continues: “Over the last years, our company has entered into new types of product licensing and acquisition, and clinical studies can provide further product development and growth. In addition, real-world data is increasingly important, and a clinical research function will help provide balanced scientific data that can improve clinical care and meet the needs of regulators, payers, clinicians, and allied care professionals to make good healthcare decisions.”

About CAEB1102-301A
Study CAEB1102-301A is a phase 3, open-label, single-arm, non-controlled, repeat dosing, international, multicentre study to evaluate the safety, pharmacokinetics, and activity (pharmacodynamics) of weekly subcutaneous administration of pegzilarginase over 12 weeks in subjects with ARG1-D who are < 24 months of age. The study is part of the Paediatric Investigation Plan (PIP) for Loargys as agreed with the Paediatric Committee (PDCO) at the

European Medicines Agency (EMA) and is planned to be performed in the United Kingdom, Austria and Portugal.

About Loargys®
Loargys (pegzilarginase) is a novel recombinant human enzyme and has been shown to rapidly and sustainably lower levels of the amino acid arginine and its toxic metabolites in plasma accompanied by improvements in clinical outcomes. Loargys is approved in the EU and Great Britain for the treatment of ARG1-D, also known as hyperargininaemia, in adults, adolescents and children aged 2 years and older, and is the first and only disease modifying treatment for ARG1-D. The EU product information for Loargys is found here.

About ARG1-D
ARG1-D is one of the eight urea cycle disorder (UCD) subtypes. It shares overlapping features with other UCDs and the most prominent is the impairment in excreting nitrogen. However, in ARG1-D, hyperammonemia is generally less severe and instead these patients show spasticity, which other subtypes do not. The principal defect in ARG1-D leads to accumulation of plasma arginine and its toxic metabolites, which occurs in almost all patients with this disorder. Patients are often diagnosed in late infancy or early childhood and the symptoms include spasticity, seizures, developmental delay, intellectual disability, and early mortality.

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 by the investment company Impilo and Buy-in-Management. Today Immedica employs more than 120 people across Europe, the Middle East and the US.

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