Immedica announces positive results from phase 3 study of Loargys® (pegzilarginase) in patients below 2 years

Stockholm, Sweden, Februari 2, 2026 – Immedica today announces the results from a phase 3 clinical study Loargys® in children below 2 years of age with arginase 1 deficiency (ARG1-D).

ARG1-D is an autosomal recessive urea cycle disorder (UCD) characterized by chronic hyperargininemia, leading to progressive spasticity, developmental delay, and seizures. Available treatment before Loargys involved a strict protein-restricted diet, essential amino acid supplementation, and symptom management. Despite these interventions, maintaining normal plasma arginine levels and slowing disease progression remained challenging. Early interventions to prevent disease onset and potentially irreversible damage is of importance.

The completed study was an open-label, multicenter study to evaluate the safety, pharmacokinetics (PK), and activity (pharmacodynamics) of weekly subcutaneous administration of Loargys in patients with ARG1-D who were <2 years of age. The study consisted of a 12-week treatment period, and a safety follow-up period of 8 weeks. The same dosage as approved for older patients was administer in this study.

This study further provides new data by complementing existing data with tolerability data on treatment initiation by the subcutaneous route.

The study demonstrated that initiating Loargys treatment subcutaneously was well tolerated and after 12 weeks of weekly Loargys treatment, a clinically meaningful reduction in plasma arginine levels was demonstrated. The PK profile was similar and as expected to that of patients ≥2 years of age. The reported treatment-emergent adverse events are consistent with the observed safety profile across the Loargys development program.

"The completion of the pediatric study on Loargys underscore our commitment to addressing unmet needs in rare metabolic diseases," said Dr. Mattias Rudebeck, Head of Global Integrated Evidence Generation and Global Medical Head for RARE Metabolic diseases at Immedica. "Early initiation of a disease-modifying treatment in ARG1-D is a high unmet medical need and not only has the potential to alter the trajectory of disease, but also to give families greater confidence about the future."

"Everyday matters when a child is living with a serious progressive condition," said Dr. Arunabha Ghosh at Bradford Royal Infirmary in the United Kingdom and the international coordinating investigator for the study. "For patients with ARG1-D, early treatment may delay or halt disease progression, providing the opportunity to preserve function and improve long-term outcomes."

About the Study
Study CAEB1102-301A (NCT06582524, EU CT No 2024-510797-25) was a phase 3, multicentre, open-label study of safety, pharmacokinetics, and activity of weekly subcutaneous pegzilarginase in subjects <24 months old with ARG1-D. The study enrolled 3 patients in agreement with the Paediatric Investigational Plan (PIP) as agreed with the EMA Paediatric

Committee (PDCO).

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, UK and Oman for the treatment of ARG1-D, also known as hyperargininemia, in adults, adolescents and children aged 2 years and older. It is the first and only disease-modifying treatment for ARG1-D. In regions such as the U.S. where pegzilarginase is not approved, pegzilarginase is an investigational product and health authorities have not established the safety and efficacy of pegzilarginase.

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 RARE metabolic, RARE hematology & oncology, RARE neurology and specialty care. Immedica was founded in 2018 and employs today around 160 people across Europe, the Middle East and the United States. Immedica is backed by the investment firms KKR and Impilo.

For more information visit www.immedica.com.

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