Loargys® (pegzilarginase) approved in the EU for treatment of arginase 1 deficiency (ARG1-D)

Stockholm, December 18, 2023: Immedica today announces that the European Commission has granted marketing authorization of Loargys® (pegzilarginase) for the treatment of arginase 1 deficiency (ARG1-D), also known as hyperargininemia, in adults, adolescents and children aged 2 years and older. The approval follows the positive opinion from the European Medicines Agency's Committee for Medicinal Products for Human Use on October 12.

Anders Edvell, CEO of Immedica comments: “Today’s approval marks a major milestone for Immedica, but an even greater one for the people living with ARG1-D and their families. Being the first and only disease modifying treatment, Loargys offers health care professionals a treatment option specifically targeted for this ultra rare disease.”

He continues: “This approval would not have been possible without an outstanding work by all the engaged physicians, patients and families being part of the clinical trials as well as the dedicated Immedica employees. Our focus will now be on ensuring timely and sustainable access to Loargys throughout Europe, with Germany being the first market. At the same time, we continue to pursue regulatory pathways for other territories”.

The approval is based on data from the clinical development program of pegzilarginase for ARG1-D providing evidence of clinically relevant outcomes and balanced safety profile, including the phase 3 randomized, double-blind, placebo-controlled study named PEACE (CAEB1102-300A), supported by the phase 2 open-label long-term study (CAEB1102-102A).

Dr Anaïs Brassier, PEACE Principal Investigator at Necker Hospital, France comments: “The unmet medical need within ARG1-D is huge and better treatment options for this disease has been desperately needed for very long. Loargys has truly given hope to the patients and their families, who live with a daily burden of managing the progressive manifestations of the disease.”

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. It is the first and only disease modifying treatment.

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 100 people across Europe and the Middle East.

For more information visit www.immedica.com

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

Immedica Pharma AB
Solnavägen 3H
SE-113 63 Stockholm