# Loargys® (pegzilarginase) receives positive opinion by the CHMP for treatment of arginase 1 deficiency

**Stockholm, October 13, 2023:** Immedica today announces that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency has adopted a positive opinion recommending marketing authorization of Loargys® (pegzilarginase) for the treatment of arginase 1 deficiency (ARG1-D) in patients two years and older. The positive opinion from the CHMP is now referred to the European Commission for a decision.

Anders Edvell, CEO of Immedica commented: “Today’s announcement demonstrates significant progress towards providing the first disease modifying treatment for ARG1-D. The unmet medical need in this vulnerable patient population is huge and we look forward to the decision by the European Commission.”

The opinion 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).

“Immedica would like to acknowledge the collaborative work in the clinical development program, and we would like to thank the investigators and all the staff at their respective sites. We would also like to extend our greatest appreciation to all the patients and caregivers who participated in the clinical trials allowing this break-through in advancement in the treatment of ARG1-D", said Mattias Rudebeck, Global Head of Genetic & Metabolic Diseases.

**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. Pegzilarginase has been developed for the treatment of people with the urea cycle disorder arginase 1 deficiency (ARG1-D).

Pegzilarginase is currently not approved in any market or indication.

**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

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