Immedica receives Orphan Drug Designation for pegzilarginase in Japan

STOCKHOLM, Sweden, August 25, 2026 - Immedica announces that Japan's Ministry of Health, Labor and Welfare (MHLW) has granted Orphan Drug Designation (ODD) to pegzilarginase for the treatment of arginase 1 deficiency (ARG1-D), a rare inherited metabolic disorder.

"Receiving Orphan Drug Designation for pegzilarginase in Japan is an important step in our commitment to bringing innovative treatments to patients living with rare diseases worldwide," said Anders Edvell, CEO of Immedica. "We look forward to continuing our engagement with the Japanese regulatory authority as we advance our plans to make pegzilarginase available to patients with ARG1-D in Japan."

Following the Orphan Drug Designation, Immedica is working towards the submission of a Japanese New Drug Application (J-NDA) for pegzilarginase. In Japan, products granted Orphan Drug Designation are eligible for a priority review process, which may result in a shorter regulatory review timeline compared with standard applications.

About ARG1-D
ARG1-D is an ultra-rare, progressive and serious inherited metabolic disorder. The principal defect in ARG1-D leads to accumulation of plasma arginine (hyperargininemia) and its toxic metabolites. Patients are often diagnosed in late infancy or early childhood, and the symptoms include spasticity, seizures, developmental delay, intellectual disability, and early mortality. ARG1-D is one of the eight urea cycle disorder (UCD) subtypes. It shares some overlapping features with other UCDs, including impaired nitrogen excretion. However, in ARG1-D, hyperammonemia is generally less severe.

About pegzilarginase
Pegzilarginase is a novel, recombinant, human arginase-1 enzyme that has been shown to rapidly and sustainably lower levels of the amino acid arginine and its toxic metabolites in plasma, making it the first and only therapy proven to lower plasma arginine. Loargys is approved in the EU, UK, US, Oman and Canada for the treatment of arginase 1 deficiency (ARG1-D), also known as hyperargininemia, in adults, adolescents, and children 2 years and older.

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, RARE endocrinology and specialty care. Immedica was founded in 2018 and employs today around 180 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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