Immedica presents new data highlighting treatment benefits of Loargys® (pegzilarginase) in arginase 1 deficiency at the SSIEM congress

Stockholm, September 3, 2024: Immedica announces today that new scientific data on Loargys® (pegzilarginase), the first disease modifying treatment in arginase 1 deficiency, has been accepted at the 2024 Annual symposium of the Society for the Study of Inborn Errors of Metabolism (SSIEM) in Porto, Portugal on 3-6 September.

The poster presentations will feature data on the long-term outcomes of treatment with pegzilarginase from the Phase 3 PEACE clinical trial, highlighting sustained and increasing efficacy over time. Additionally, a separate poster will present an analysis of long-term dietary intake before and during pegzilarginase treatment from the same study, indicating that increased natural protein intake and reduced essential amino acid supplementation could be achieved without compromising the blood arginine-lowering effect. Furthermore, the first real-world clinical data will be presented, involving 14 patients across 6 clinical sites in France, where early access to pegzilarginase was granted by authorities starting in July 2022. This real-world data not only confirms the biochemical results observed in the clinical trial setting but also suggests further opportunities for diet liberalization during pegzilarginase treatment.

“These data are of high importance for Loargys as a new treatment option in arginase 1 deficiency providing long-term and real-world outcomes data”, says Mattias Rudebeck, Head of Global Integrated Evidence Generation and Global Medical Head for Genetic and Metabolic Diseases at Immedica.

He continues: “The sustainability of mobility improvement long-term is highly clinically relevant in this chronic, progressive disease where a gradual decline of motor function would be expected, and the indications on potential for diet modification could promote normal growth and minimize burden of a restricted diet for patients and their families.”

In addition to the above presentations, data on the validation of the Immedica-manufactured blood collection tubes containing the inhibitor nor-NOHA, which are required during monitoring of patients under treatment with Loargys, will be presented.

The following poster presentations will be made during the SSIEM congress:

Improved clinically meaningful treatment outcomes of pegzilarginase during longer-term treatment: Final functional mobility analysis from the Phase 3 PEACE trial (EP-121)

Authors: Frank Rutsch, Markey McNutt, Spyros Batzios, Anaïs Brassier, Greg Enns, Serena Gasperini, Rossana Sanchez Russo, Mattias Rudebeck

Experience of pegzilarginase for the treatment of arginase 1 deficiency outside clinical trial setting (PO-613)

Authors: Jean-Baptiste Arnoux, Alina Arion, Magalie Barth, Anaïs Brassier, Dries Dobbelaere, Magali Gorce, Cécile Laroche, Manuel Schiff, Raphaële Dion, Åsa Jansson, Olivia Rime, Mattias Rudebeck

Pegzilarginase treatment in arginase 1 deficiency allows for liberalization of protein restriction (EP-120)

Authors: Rani Singh, Markey McNutt, Marjorie Dixon, Frank Rutsch, Mattias Rudebeck

Validation of nor-NOHA containing blood collection tubes for accurate arginine measurement in pegzilarginase-treated patients (PO-643)

Authors: Diran Herebian, Joseph P. Campbell, Jörg van den Boom, Mattias Rudebeck

About SSIEM
The Society for the Study of Inborn Errors of Metabolism Society (SSIEM), founded in 1963, exists to promote exchange of ideas between professional workers in different disciplines who are interested in inherited metabolic disease. The aim of the SSIEM is to foster the study of inherited metabolic disorders and related topics.

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 for the treatment of arginase 1 deficiency, also known as hyperargininemia, in adults, adolescents and children aged 2 years and older, and is the first and only disease modifying treatment for ARG1-D. EMA CHMP summary of opinion is available 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 100 people across Europe and the Middle East.

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