Immedica Pharma AB submits Marketing Authorisation Application to the European Medicines Agency seeking approval of pegzilarginase for the treatment of Arginase 1 Deficiency (ARG1-D)

Immedica Pharma AB announced today that the company's Marketing Authorisation Application for pegzilarginase has been successfully validated by the European Medicines Agency (EMA) for the treatment of patients with Arginase 1 Deficiency (ARG1-D).

The application is supported by positive results from the international phase 3 study (CAEB1102-300A, also named PEACE), investigating the safety and efficacy of pegzilarginase.

"I am pleased that our application for pegzilarginase has been validated by the EMA. The clinical data on efficacy and safety for pegzilarginase provide opportunities for improved treatment options in patients with the severe and debilitating disease ARG1-D" says Anders Edvell, CEO at Immedica Pharma AB.

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. Pegzilarginase has been developed for the treatment of people with the Urea Cycle Disorder ARG1-D, a rare debilitating and progressive disease with a high unmet medical need characterized by the accumulation of arginine. ARG1-D presents in early childhood and patients experience spasticity, seizures, developmental delay, intellectual disability and early mortality.

"Today's announcement marks an important milestone in Immedica's ambition to bring new innovative medicines for people with Urea Cycle Disorders, and especially for ARG1-D where a disease modifying treatment is currently lacking" says Anders Edvell.

Pegzilarginase has been developed by Immedica´s valued partner Aeglea BioTherapeutics Inc, a US based clinical-stage biotechnology company redefining the potential of human enzyme therapeutics to benefit people with rare metabolic diseases with limited treatment options.

About the phase 3 study PEACE

PEACE (Pegzilarginase Effect on Arginase 1 deficiency Clinical Endpoints) is an international, randomized, double-blind, placebo-controlled trial that enrolled 32 patients aged 2 years and older with arginase 1 deficiency in the United States, Canada and Europe (NCT03921541). PEACE was designed to assess the effects of treatment with pegzilarginase versus placebo over 24 weeks with a primary endpoint of plasma arginine reduction from baseline. Secondary endpoints include clinical outcome assessments focused on multiple mobility assessments, in addition to safety and pharmacokinetics. Patients were randomized on a two-to-one basis to receive weekly infusions of pegzilarginase or placebo for the double-blind 24-week treatment period.

About arginase 1 deficiency (ARG1-D)

ARG1-D is a rare, progressive and debilitating disease characterized by high levels of the amino acid arginine and its metabolites. It is a metabolic, autosomal recessive disease caused by a deficiency in the arginase 1 enzyme which is active in the urea cycle. The disease manifestations include spasticity, developmental delay, intellectual disability and seizures and causes significant reductions in quality of life, increased morbidity, and premature mortality[1,2,3,4]. There are currently no approved pharmacological treatments that address elevated arginine, the key driver of ARG1-D manifestations and progression[4,5,6]. Published non-clinical and clinical data demonstrate that lowering arginine is associated with slower disease progression and/or disease improvement[5,7,8,9,10,11,12]. Current standard of care includes severe dietary protein restriction and essential amino acid supplementation, which does not effectively or sustainably reduce high arginine levels causing continued disease progression[2].

About Immedica Pharma

Immedica Pharma is a fast-growing private European niche pharma company with the headquarter based in Stockholm, Sweden. Today, the company has strong direct pan-European and Middle East commercial coverage. In addition, Immedica provides some of its products to other parts of the world via a network of regional partners.

Immedica provides significant know-how and experience from commercialization of niche/specialty care products across Europe and the Middle East, and the company's management team has an outstanding track record of partnering and operating niche pharma products internationally. Immedica has capabilities to provide optimal access of specialty care medicines to patients with significant medical needs, including key areas such as regulatory affairs, pharmacovigilance, medical affairs, pricing & reimbursement, and product distribution.

References

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