PLUVIA

# Pluvia Biotech Receives European Orphan Drug Designation for Its Lead Compound for PKU

Bergen, Norway, 30 May 2024 – Pluvia Biotech (Pluvia), a company dedicated to developing pharmacological chaperones for the treatment of Phenylketonuria (PKU), today announced that the European Medicines Agency (EMA) Committee for Orphan Medicinal Products (COMP) has granted Orphan Drug Designation (ODD) for the company's lead compound PBAS499, as confirmed by the European Commission Decision of 24 May 2024. The designation covers the treatment of hyperphenylalaninaemia, a slightly broader definition of the disease which includes PKU as a more severe subset of patients.

PKU is caused by a deficiency of the liver enzyme Phenylalanine Hydroxylase (PAH) leading to high levels of the amino acid phenylalanine in the blood stream, which is toxic to the brain. Since phenylalanine is part of almost all protein containing foods, PKU patients are held to a strict diet throughout their lives.

Pluvia aims to treat the underlying cause of PKU by developing oral small molecule chaperones to stabilize the deficient PAH enzyme. The company's lead compound PBAS499 has shown consistent pre-clinical Proof of Concept data in PKU models, a clean preliminary safety profile and a straightforward manufacturing process. These data provide the basis for further development of PBAS499 through Phase 1 and Phase 2 clinical studies to demonstrate clinical Proof of Concept.

"Dietary treatment in patients with PKU could seem theoretically easy, but in day-to-day practice trying to keep natural protein intake as low as 5 gram daily is very difficult, considering that we normally can eat more than 100 grams of natural protein per day. Therefore, we need additional treatment options to control their disease and to allow for a more normal protein intake." says Prof. Francjan van Spronsen, MD, PhD, Prof. of Pediatric Metabolic Diseases, University Medical Center Groningen, The Netherlands. "The direct burden of the disease, as well as the burden of the diet, can have significant medical, psychological and social impacts on these patients. Pluvia's approach to treat the underlying cause of PKU by stabilizing the deficient PAH enzyme is a promising way to address this."

Pluvia's CEO, Willem van Weperen adds: "Obtaining the EU Orphan Drug Designation from the EMA is a key next step for Pluvia, confirming the ability of our team to execute our plans. This designation follows the confirmation by the US Food and Drug Administration (FDA), who awarded the US Orphan Drug Designation and Rare Pediatric Disease Designation for PBAS499 late last year. We look forward to continue our momentum to move PBAS499 to clinical Proof of Concept."

**About the EU ODD**

The European Commission grants ODD to products that treat a life-threatening or chronically debilitating condition affecting no more than 5 in 10,000 people in the EU and where no satisfactory treatment is available. Products receiving Orphan Drug Designation are eligible to receive market exclusivity for a period of up to 10 years in the EU upon approval, as well as eligibility for protocol assistance, reduced fees and access to the EU's centralized marketing authorization procedure. PKU affects between 1 to 2 in 10,000 in the EU, which amounts to 45,000 to 90,000 EU citizens.

**About PKU**

PKU, also known as Føllings disease (named after the Norwegian physician Ivar Asbjørn Følling, the first to identify the disease in 1934), is a rare genetic disorder characterized by the body's inability to break down the amino acid phenylalanine due to a deficiency in the enzyme PAH, caused by genetic variants. This results in the accumulation of phenylalanine in the bloodstream, leading to intellectual disabilities and other neurological problems if left untreated. Patients need a strict lifelong diet without regular protein intake to prevent cognitive impairment.

**About Pluvia Biotech**

Pluvia Biotech is a spin-out from the University of Bergen (Norway) dedicated to addressing orphan diseases from protein misfolding with a high unmet medical need. The company's lead program is focused on developing a "first in class" oral pharmacological chaperone treatment of PKU. Through pioneering research and development, Pluvia aims to provide PKU patients with the opportunity to live life without the dietary constraints imposed by the condition. Pluvia's progress is supported by investors Sarsia, Trond Mohn Foundation and Investinor.

For more information about Pluvia Biotech and its innovative work in PKU treatment development, please visit www.pluviabiotech.com. For media inquiries and additional information, please contact info@pluviabiotech.com
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