PLUVIA

Pluvia Biotech Receives Orphan Drug and Rare Pediatric Disease Designations for Its PKU Program from FDA

Bergen, Norway, 5 January 2024 – Pluvia Biotech (Pluvia), a company dedicated to developing pharmacological chaperones for the treatment of Phenylketonuria (PKU), today announced that the U.S. Food and Drug Administration (FDA) has granted both the Orphan Drug Designation (ODD) and the Rare Pediatric Disease Designation (RPDD) for the company's lead compound PBAS499.

Pluvia has developed oral small molecule chaperones to stabilize the deficient enzyme in PKU that causes accumulation of the amino acid phenylalanine, which becomes toxic to the brain of PKU patients. Recently, Pluvia selected PBAS499 as lead compound based on strong pre-clinical Proof of Concept data in PKU models and a clean preliminary safety profile. These positive results now allow Pluvia to take PBAS499 through Phase 1 in 2025 and into Phase 2 in 2026 to demonstrate clinical Proof of Concept.

Lisa Milberg, Executive Director of the National PKU Alliance says: "The PKU community has a high need for additional treatment options to alleviate PKU patients from the burden of dietary restrictions. We are grateful for the ODD and RPDD incentives from the FDA to support companies like Pluvia to develop therapies to address this."

Pluvia's CEO, Willem van Weperen adds: "We are excited about these valuable designations by the FDA to support Pluvia's mission to develop a new oral therapy option for PKU patients. We look forward to continue our momentum to realize a Series A round early 2024 and to move PBAS499 to clinical Proof of Concept."

Benefits of ODD and RPDD

The FDA grants ODD to support development of medicines for rare diseases or conditions that affect fewer than 200,000 people in the U.S. Potential benefits of the ODD include market exclusivity for the first ODD drug for an approved indication within the ODD for a seven-year period upon FDA approval, federal tax credits for qualified clinical research expenses incurred in the U.S., and a waiver of Prescription Drug User Fee Act (PDUFA) fees.

The RPDD acknowledges therapies under investigation for rare pediatric diseases affecting less than 200,000 people in the U.S. with serious or life-threatening manifestations primarily affecting individuals up to 18 years of age. RPDD provides priority review of the marketing application, and, if approved for marketing, grants that sponsor a priority review voucher (with a market value in excess of $100M), which can be transferred or sold to another sponsor.

About PKU and Pharmacological Chaperones

Phenylketonuria (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 phenylalanine hydroxylase (PAH). 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. Pluvia aims to develop pharmacological chaperones for oral use to stabilize the naturally occurring PAH variants, thereby allowing the breakdown of phenylalanine and a potential normalization of food intake for PKU patients.

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 Phenylketonuria (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