PLUVIA

Pluvia Biotech Presents Preclinical Data of potential PKU treatment PBAS499 at SSIEM Annual Symposium

Bergen, Norway, 4 September 2024 – Pluvia Biotech (Pluvia), a company dedicated to developing pharmacological chaperones for the treatment of Phenylketonuria (PKU), today announced the presentation of preclinical data showing efficacy, safety and broad applicability for the company's lead compound PBAS499 at the Society for the Study of Inborn Errors of Metabolism (SSIEM) Annual Symposium 2024 (Porto, Portugal).

Based on decades of research on protein structure and protein misfolding by Prof. Aurora Martinez and her team, Pluvia has developed small molecule pharmacological chaperones with the potential to stabilize the deficient PAH enzyme, the underlying cause of PKU. Based on extensive efficacy and safety studies, the company carefully selected PBAS499 as its lead compound for future clinical development. Today represents the first time that PBAS499 data will be shared with the scientific community during the SSIEM conference, where rare genetic disease expert clinicians and scientists from all over the world will come together.

Ann Kari Grindheim, PhD, Director of Preclinical Development and presenter of the data, says "We are proud to share recent data that show the ability of our lead compound PBAS499 to stabilize a majority of the most frequent missense variants of the PAH enzyme. Furthermore, PBAS499 shows convincing efficacy in a PKU mouse model, as well as a clean safety profile, both in vitro and in vivo."

"We are excited about the potential broad applicability of PBAS499 in these recent in vitro studies." adds Pluvia's CEO, Willem van Weperen, "Moreover, progress made together with our CMC partner shows a straightforward manufacturing process of this oral small molecule. Based on these positive developments, we look forward to move PBAS499 to clinical Proof of Concept with the ultimate goal to make a difference in the lives of many PKU patients."

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