Pharming Announces Presentation of Positive Results of Phase III Leniolisib Trial in APDS at Clinical Immunology Society 2022 Annual Meeting

Principal Investigator Dr. V. Koneti Rao Will Share Data Supporting the Investigational Treatment for Activated PI3K Delta Syndrome (APDS)

Leiden, The Netherlands, March 28, 2022: Pharming Group N.V. ("Pharming" or "the Company") (Euronext Amsterdam: PHARM/NASDAQ: PHAR) announces that Principal Investigator V. Koneti Rao, MD, FRCPA, a staff physician in the Primary Immune Deficiency Clinic at the National Institutes of Health in Bethesda, Maryland, will present positive findings from the Phase III pivotal clinical trial of leniolisib for patients with activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS) at the Clinical Immunology Society (CIS) 2022 Annual Meeting in Charlotte, North Carolina.

The presentation for healthcare practitioners and other experts will take place on April 1, 2022, from 11:30 to 11:45 ET/17:30 to 17:45 CET and will be accessible on site and virtually. To attend, please register here:

https://cis.clinimmsoc.org/education/meetings/am22/program/amprogram

Leniolisib is being developed for the targeted treatment of APDS, a rare primary immunodeficiency caused by genetic variants that currently has no approved therapy. On February 2, 2022, Pharming announced that the Phase III trial of leniolisib, sponsored by Novartis, has met both of its co-primary endpoints by demonstrating improvements in lymphoproliferation and immunophenotype correction, and that the treatment was well tolerated by patients.

Pharming plans to begin submitting global regulatory filings for leniolisib, a small-molecule PI3Kδ inhibitor, in the first half of 2022 and, subject to approval, launching the treatment in the U.S. in the first quarter of 2023 and starting a series of European launches in the second half of 2023.

Nicholas Hartog, MD, FAAAAI, FACAAI, a specialist in allergy and immunology at Spectrum Health Helen Devos Children's Hospital in Grand Rapids, Michigan, said:

"I'm eager to learn more about the positive results of this Phase III study of leniolisib in patients with APDS. A promising therapy for this rare and challenging disease sparks hope in physicians like me, who are dedicated to improving care for affected patients and reducing their symptoms. I could not be more excited about the opportunity for a personalized and precision-based therapy on the horizon for this patient population."

Anurag Relan, Chief Medical Officer of Pharming, commented:

"Pharming is committed to bringing new therapies to people with rare diseases and will work closely with regulatory authorities across the globe in an effort to make this innovative option available to physicians who care for patients with primary immunodeficiencies. We are excited to be pursuing a development program that aims to bring patients the first targeted therapy option for APDS, as this disease significantly impacts their lives, yet treatment has been limited to supportive therapies such as antibiotics and immunoglobulin replacement therapy."

In his presentation during a plenary session titled “Precision Medicine for Hyperinflammatory Disorders,” Dr. Rao will explain the study’s design, share its primary and secondary findings, and detail patient disposition and safety results. The annual CIS meeting will be dedicated to exploring immune deficiency and dysregulation.

About Activated Phosphoinositide 3-Kinase δ Syndrome (APDS)

APDS is a rare primary immunodeficiency that affects approximately one to two people per million. Also known as PASLI, it is caused by variants in either of two genes, PIK3CD or PIK3R1, that regulate maturation of white blood cells. Variants of these genes lead to hyperactivity of the PI3Kδ (phosphoinositide 3-kinase delta) pathway.[1,2] Balanced signaling in the PI3Kδ pathway is essential for physiological immune function. When this pathway is hyperactive, immune cells fail to mature and function properly, leading to immunodeficiency and dysregulation.[1,3] APDS is characterized by severe, recurrent sinopulmonary infections, lymphoproliferation, autoimmunity, and enteropathy.[4,5] Because these symptoms can be associated with a variety of conditions, including other primary immunodeficiencies, people with APDS are frequently misdiagnosed and suffer a median 7-year diagnostic delay.[6] As APDS is a progressive disease, this delay may lead to an accumulation of damage over time, including permanent lung damage and lymphoma.[4-7] The only way to definitively diagnose this condition is through genetic testing.

About leniolisib

Leniolisib is a small-molecule inhibitor of the delta isoform of the 110 kDa catalytic subunit of class IA PI3K with immunomodulating and potentially anti-neoplastic activities. Leniolisib inhibits the production of phosphatidylinositol-3-4-5-trisphosphate (PIP3). PIP3 serves as an important cellular messenger specifically activating AKT (via PDK1) and regulates a multitude of cell functions such as proliferation, differentiation, cytokine production, cell survival, angiogenesis, and metabolism. Unlike PI3Kα and PI3Kβ, which are ubiquitously expressed, PI3Kδ and PI3Kγ are expressed primarily in cells of hematopoietic origin. The central role of PI3Kδ in regulating numerous cellular functions of the adaptive immune system (B-cells and, to a lesser extent, T cells) as well as the innate immune system (neutrophils, mast cells, and macrophages) strongly indicates that PI3Kδ is a valid and potentially effective therapeutic target for several immune diseases.

To date, leniolisib has been well tolerated during both the Phase 1 first-in-human trial in healthy subjects and the Phase II/III registration-enabling study.

About the Phase II/III leniolisib Trial

Sponsored by Novartis, the Phase II/III registration-enabling study was composed of two parts, the first being a 12-week open-label dose escalation part that included six patients with APDS and determined the dose of leniolisib to be given in the Phase III part.

The Phase III part consisted of a randomized, placebo-controlled, blinded trial of leniolisib that enrolled 31 patients who had APDS and were age 12 or older. The patients were randomly assigned according to a 2:1 ratio to receive either leniolisib 70mg twice daily or placebo for 12 weeks. The co-primary endpoints of the randomized study evaluated reduction in lymph node size and correction of immunodeficiency as shown by an increase in naïve B cells. Following study treatment, patients were permitted to roll over to an open-label extension study evaluating long-term safety, tolerability, and efficacy.

About Pharming Group N.V.

Pharming Group N.V. is a global, commercial stage biopharmaceutical company developing innovative protein replacement therapies and precision medicines for the treatment of rare diseases and unmet medical needs.

The flagship of our portfolio is our recombinant human C1 esterase inhibitor (rhC1INH) franchise. C1INH is a naturally occurring protein that down regulates the complement and contact cascades in order to control inflammation in affected tissues.

Our lead product, RUCONEST®, is the first and only plasma-free rhC1INH protein replacement therapy. It is approved for the treatment of acute hereditary angioedema (HAE) attacks. We are commercializing RUCONEST® in the United States, the European Union and the United Kingdom through our own sales and marketing organization, and the rest of the world through our distribution network.

In addition, we are investigating the clinical efficacy of rhC1INH in the treatment of further indications, including pre-eclampsia, acute kidney injury and severe pneumonia as a result of COVID-19 infections.

We are also studying our oral precision medicine, leniolisib (a phosphoinositide 3-kinase delta, or PI3K delta, inhibitor), for the treatment of activated PI3K delta syndrome, or APDS. World-wide rights for leniolisib were licensed from Novartis AG in 2019. Leniolisib met both of its primary end points in a registration enabling Phase 2/3 study in the United States and Europe. We are targeting global regulatory filings for leniolisib from Q2 2022 onwards.

Additionally, we entered into a strategic collaboration with Orchard Therapeutics to research, develop, manufacture and commercialize OTL-105, a newly disclosed investigational ex-vivo autologous hematopoietic stem cell (HSC) gene therapy for the treatment of HAE.

Furthermore, we are leveraging our transgenic manufacturing technology to develop next-generation protein replacement therapies, most notably for Pompe disease, which is currently in preclinical development.

Forward-looking Statements

This press release contains forward-looking statements, including with respect to timing and progress of Pharming's preclinical studies and clinical trials of its product candidates, Pharming's clinical and commercial prospects, Pharming's ability to overcome the challenges posed by the COVID-19 pandemic to the conduct of its business, and Pharming's expectations regarding its projected working capital requirements and cash resources. These statements are subject to a number of risks, uncertainties and assumptions, including but not limited to: the scope, progress and expansion of Pharming's clinical trials and ramifications for the cost thereof; and clinical, scientific, regulatory and technical developments. In light of these risks and uncertainties, and other risks and uncertainties that are described in Pharming's 2020 Annual Report and the Annual Report on Form 20-F for the year ended December 31, 2020 filed with the U.S. Securities and Exchange Commission, the events and circumstances discussed in such forward-looking statements may not occur, and Pharming's actual results could differ materially and adversely from those anticipated or implied thereby. Any forward-looking statements speak only as of the date of this press release and are based on information available to Pharming as of the date of this release.

Inside Information

This press release relates to the disclosure of information that qualifies, or may have qualified, as inside information within the meaning of Article 7(1) of the EU Market Abuse Regulation.

References:

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