Pharming announces first patient dosed in pediatric clinical trial for children aged 1 to 6 years for leniolisib

The multinational Phase III study is evaluating a new pediatric granulated formulation of leniolisib in children aged 1 to 6 years with APDS, a rare primary immunodeficiency

Leiden, The Netherlands, November 21, 2023: Pharming Group N.V. ("Pharming" or "the Company") (EURONEXT Amsterdam: PHARM)/(Nasdaq: PHAR) announces that the first patient has been dosed in its Phase III pediatric clinical trial evaluating a new pediatric formulation of the investigational drug leniolisib, an oral, selective phosphoinositide 3-kinase delta (PI3Kδ) inhibitor, in children ages one to six years, with activated phosphoinositide 3-kinase delta syndrome (APDS).

At sites in the U.S., Japan, and the EU, the single-arm, open-label, multinational clinical trial will evaluate the safety, tolerability, and efficacy of leniolisib in 15 children, one to six years of age, who have a confirmed APDS diagnosis. These patients will receive a specific, pediatric granulated formulation of leniolisib. The study's primary efficacy endpoints and secondary endpoints mirror those used to evaluate the clinical outcomes in previous leniolisib Phase II/III APDS trials for patients aged 12 and older.

Dr. Eveline Wu, MD, MSCR, Division Chief, Paediatric Rheumatology & Associate Professor of Paediatric Rheumatology and Allergy/Immunology at The University of North Carolina School of Medicine, commented:

"As a physician who has seen how significantly APDS can affect children's lives, I'm excited that a targeted treatment may soon become available for even younger patients. While standard supportive therapies can ease some of the progressive, immune-related symptoms that characterize APDS, leniolisib is designed to help prevent those symptoms from arising. This could improve the standard of care for these children with the hope of a more full and enjoyable participation in the typical activities of childhood."

Anurag Relan, MD, MPH, Chief Medical Officer of Pharming, commented:

"With the first patient dosed in this pediatric trial of leniolisib, using a new, granulated formulation, we are continuing to demonstrate Pharming's ongoing commitment to providing new treatment options for younger pediatric patients with APDS. We look forward to generating the necessary supporting data to facilitate additional regulatory filings, thereby ensuring that patients from as broad an age range as possible can potentially benefit from this disease-modifying treatment."

This is the second pediatric clinical trial launched by Pharming this year for the study of leniolisib in pediatric patients with APDS. The first pediatric study, announced in February 2023, is ongoing and will evaluate 15 children, four to 11 years of age, receiving leniolisib's tablet formulation as an investigational treatment for APDS.

Leniolisib, marketed under the brand name Joenja® in the U.S., received approval from the US Food and Drug Administration (FDA) for the treatment of APDS in adult and pediatric patients 12 years of age and older in March 2023.

About Activated Phosphoinositide 3-Kinase δ Syndrome (APDS)

APDS is a rare primary immunodeficiency that was first characterized in 2013. APDS is caused by variants in either one of two identified genes known as *PIK3CD* or *PIK3R1,* which are vital to the development and function of immune cells in the body. Variants of these genes lead to hyperactivity of the PI3Kδ (phosphoinositide 3-kinase delta) pathway, which causes immune cells to fail to mature and function properly, leading to immunodeficiency and dysregulation.[1,2,3] APDS is characterized by a variety of symptoms, including 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, it has been reported that 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] A definitive diagnosis can be made through genetic testing. APDS affects approximately 1 to 2 people per million worldwide.

About leniolisib

Leniolisib is an oral small molecule phosphoinositide 3-kinase delta (PI3Kδ) inhibitor approved in the US as the first and only targeted treatment of activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS) in adult and pediatric patients 12 years of age and older. Leniolisib inhibits the production of phosphatidylinositol-3-4-5-trisphosphate, which serves as an important cellular messenger and regulates a multitude of cell functions such as proliferation, differentiation, cytokine production, cell survival, angiogenesis, and metabolism. Results from a randomized, placebo-controlled Phase II/III clinical trial demonstrated clinical efficacy of leniolisib in the coprimary endpoints; demonstrating statistically significant impact on immune dysregulation and normalization of immunophenotype within these patients, and interim open label extension data has supported the safety and tolerability of long-term leniolisib administration.[8] Leniolisib is currently under regulatory review by the European Medicines Agency, with plans to pursue further regulatory approvals in the UK, Canada, Australia and Japan. Leniolisib is also being evaluated in a Phase III clinical trial in children aged 4 to 11 with APDS, with a further trial planned in children aged 1 to 6 years with APDS.

About Pharming Group N.V.

Pharming Group N.V. (EURONEXT Amsterdam: PHARM/Nasdaq: PHAR) is a global biopharmaceutical company dedicated to transforming the lives of patients with rare, debilitating, and life-threatening diseases. Pharming is commercializing and developing an innovative portfolio of protein replacement therapies and precision medicines, including small molecules, biologics, and gene therapies that are in early to late-stage development. Pharming is headquartered in Leiden,

Netherlands, and has employees around the globe who serve patients in over 30 markets in North America, Europe, the Middle East, Africa, and Asia-Pacific.

For more information, visit www.pharming.com and find us on LinkedIn.

Forward-looking Statements

This press release may contain forward-looking statements. Forward-looking statements are statements of future expectations that are based on management's current expectations and assumptions and involve known and unknown risks and uncertainties that could cause actual results, performance, or events to differ materially from those expressed or implied in these statements. These forward-looking statements are identified by their use of terms and phrases such as "aim", "ambition", ''anticipate'', ''believe'', ''could'', ''estimate'', ''expect'', ''goals'', ''intend'', ''may'', "milestones", ''objectives'', ''outlook'', ''plan'', ''probably'', ''project'', ''risks'', "schedule", ''seek'', ''should'', ''target'', ''will'' and similar terms and phrases. Examples of forward-looking statements may include statements with respect to timing and progress of Pharming's preclinical studies and clinical trials of its product candidates, Pharming's clinical and commercial prospects, and Pharming's expectations regarding its projected working capital requirements and cash resources, which 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 2022 Annual Report and the Annual Report on Form 20-F for the year ended December 31, 2022, 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. All forward-looking statements contained in this press release are expressly qualified in their entirety by the cautionary statements contained or referred to in this section. Readers should not place undue reliance on forward-looking statements. 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. Pharming does not undertake any obligation to publicly update or revise any.

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.

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