Pharming to be honored as Industry Innovator at National Organization for Rare Disorders (NORD®) 2024 Rare Impact Awards

Recognition given for achievement in commercializing Joenja® (leniolisib), a first-in-class medication brought to market 10 years after disease state, APDS, was first characterized

Leiden, the Netherlands, April 24, 2024: Pharming Group N.V. ("Pharming" or "the Company") (EURONEXT Amsterdam: PHARM/Nasdaq: PHAR) is pleased to announce its recognition as an Industry Innovator by the National Organization for Rare Disorders (NORD®) at the Rare Impact Awards on June 8, 2024. The Rare Impact Awards honor individuals, groups and organizations who are making exceptional strides to improve the lives of people living with rare diseases.

Pharming will accept the award in recognition of the U.S. Food and Drug Administration (FDA) approval and commercialization of Joenja® (leniolisib), an oral, selective PI3Kδ inhibitor. Joenja® is the first and only treatment approved in the U.S. for activated phosphoinositide 3-kinase delta syndrome (APDS) in adult and pediatric patients 12 years of age and older. Approximately two weeks after Joenja® was approved for use on March 24, 2023, Pharming announced that the first shipments to patients in the U.S. were delivered.

Peter Saltonstall, President and CEO of NORD, commented:
"NORD is very pleased to recognize Pharming for the approval of Joenja® in the treatment of activated phosphoinositide 3-kinase delta syndrome (APDS). APDS is a rare inborn error of immunity characterized primarily by frequent infections, lymphoproliferation and autoimmunity for which there was not a targeted therapy until FDA approval of Joenja® for both adults and pediatrics 12 years of age and older. On behalf of the approximately 500 individuals in the US with this rare condition, NORD is thrilled to see such innovation taking place at Pharming. With 95% of rare diseases not having therapies available, the approval of Joenja® for APDS is worthy of the NORD Industry Innovation Award. NORD congratulates Pharming for this achievement."

Stephen Toor, Chief Commercial Officer of Pharming, commented:
"We are honored to be recognized by NORD® as an industry leader making a difference in the lives of those living with and caring for someone living with a rare disease. Of the more than 7,000 rare diseases that have been identified, very few have an FDA-approved therapy. Pharming is proud to provide a treatment option for those living with APDS. This is a significant milestone for the scientists who developed Joenja and the patients we serve. We look forward to commemorating this achievement alongside members of the APDS community later this year."

For more information about the Rare Impact Awards and to view the full list of honorees, please click here.

About the National Organization for Rare Disorders (NORD)

With a 40-year history of advancing care, treatments and policy, the National Organization for Rare Disorders (NORD) is the leading and longest-standing patient advocacy group for the more than 25 million Americans living with a rare disease. NORD, a 501(c)(3) nonprofit, is dedicated to individuals with rare diseases and the organizations that serve them. NORD, along with its more than 330 patient organization members, is committed to improving the health and well-being of people with rare diseases by driving advances in care, research and policy. For more information, please visit https://rarediseases.org/.

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.[8,9] Leniolisib is currently under regulatory review in Europe, Canada and Australia, and a regulatory submission has been made in the U.K. with plans to pursue regulatory approval in Japan. Leniolisib is also being evaluated in two Phase III clinical trials in children-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, the 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, commercial, competitive and technical developments. In light of these risks and uncertainties, and other risks and uncertainties that are described in Pharming's 2023 Annual Report and the Annual Report on Form 20-F for the year ended December 31, 2023, 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.

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