For media and investors only

Pharming announces marketing authorization in the U.K. for Joenja® (leniolisib)

Indicated for adult and pediatric patients 12 years of age and older with activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS)

Leiden, the Netherlands, September 26, 2024: Pharming Group N.V. ("Pharming" or "the Company") (EURONEXT Amsterdam: PHARM/Nasdaq: PHAR) announces that the United Kingdom (U.K.) Medicines and Healthcare products Regulatory Agency (MHRA) has granted marketing authorization for Joenja® (leniolisib) for the treatment of activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS) in adult and pediatric patients 12 years of age and older.

Leniolisib, an oral, selective PI3Kδ inhibitor, is the first treatment approved in the U.K. specifically for APDS, a rare and progressive primary immunodeficiency. Leniolisib is currently under evaluation by the National Institute for Health and Care Excellence (NICE) regarding reimbursement within the National Health Service (NHS) in England.

Sijmen de Vries, Chief Executive Officer of Pharming, commented:
"The MHRA approval of Joenja®, the first treatment option specifically indicated for APDS, is an important milestone for people in the U.K. living with this debilitating disease. To date, management of APDS has relied on the treatment of the diverse symptoms associated with APDS or for some patients, the need to undergo hematopoietic stem cell transplantation. We are therefore delighted that this product is now approved in the U.K. Today also marks Pharming's third country approval for APDS, bringing us closer to our goal of becoming a leading global rare disease company dedicated to patient communities with unmet medical needs."

The MHRA evaluated the Marketing Authorisation Application (MAA) for leniolisib through the International Recognition Procedure (IRP) pathway on the basis of the U.S. Food and Drug Administration (FDA) approval received in March 2023.

Important Safety Information
The full Summary of Product Characteristics (SPC/SmPC) for Joenja® (leniolisib) will be available on the MHRA website at https://products.mhra.gov.uk/.

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 U.S., the U.K. and Israel as the first and only targeted treatment indicated for 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 III clinical trial demonstrated statistically significant improvement in the coprimary endpoints, reflecting a favorable impact on the immune dysregulation and deficiency seen in 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 the European Economic Area, Canada and Australia, with plans to pursue further regulatory approvals in Japan and South Korea. 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 and biologics. 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 forward-looking statement as a result of new information, future events or other information.

References

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