Pharming Group receives Complete Response Letter from U.S. FDA for sNDA for Joenja® (leniolisib) in children aged 4 to 11 years with APDS

Leiden, the Netherlands, February 1, 2026: Pharming Group (Euronext: PHARM; Nasdaq: PHAR) today announced that the U.S. Food and Drug Administration (FDA) has issued a Complete Response Letter (CRL) to its supplemental New Drug Application (sNDA) for Joenja® (leniolisib), an oral, selective phosphoinositide 3-kinase delta (PI3Kδ) inhibitor, as a treatment for children aged 4 to 11 years with activated phosphoinositide 3-kinase delta syndrome (APDS), a rare primary immunodeficiency.

The FDA raised an issue with the potential for underexposure in lower weight pediatric patients. As a result, the FDA has requested additional pediatric pharmacokinetic data to reassess the proposed pediatric doses and confirm that children in the lower weight dose groups can achieve exposure levels comparable to the approved adult and adolescent regimen. The letter also identified an issue with one of the analytical methods used for production batch testing, and the FDA requested additional data and clarification on this point.

We believe we can address the clinical pharmacology and batch testing methodology issues outlined in the letter, and we plan to work closely with the FDA to meet the Agency's requirements and determine next steps for resubmission. We plan to request a Type A meeting with the FDA.

Joenja's U.S. FDA approval for the treatment of APDS in patients aged 12 years of age and older is unaffected by this regulatory action.

Fabrice Chouraqui, Chief Executive Officer of Pharming, commented:

"While we are disappointed in the FDA's response, we remain dedicated to making Joenja available to pediatric patients aged 4-11 with APDS. Joenja has the potential to address the immune dysregulation and deficiency that drive APDS and significantly impact the long-term course of disease in this population, for whom there is currently no approved targeted treatment. We are going to work closely with the FDA to provide the necessary information and determine the best and most effective path forward."

Pharming submitted the sNDA to the FDA based on positive data from the open-label, multinational, single-arm Phase III study in children aged 4 to 11 years, which showed improvements over 12 weeks in two clinically relevant hallmarks of APDS, reduced lymphadenopathy and increased naïve B cells, together indicating a correction of the underlying immune defect. The submission also included safety data from 8 months of treatment. The improvements in lymphoproliferation and immunophenotype correction were seen across the four dose levels investigated and were consistent with the improvements previously reported in

adolescent and adult patients. All treatment emergent adverse events were reported to be mild to moderate in nature. There were no drug related serious adverse events, and all patients completed the 12-week treatment period.

In October 2025, the FDA granted the application Priority Review[1] based on its guidelines stating the medicine would offer significant improvements in effectiveness or safety of the treatment, prevention, or diagnosis of serious conditions. Currently, there are no approved treatments for children with APDS under the age of 12 years globally. Joenja received approval from the 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[2,3,4] APDS is characterized by a variety of symptoms, including severe, recurrent sinopulmonary infections, lymphoproliferation, autoimmunity, and enteropathy.[5,6] 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.[7] As APDS is a progressive disease, this delay may lead to an accumulation of damage over time, including permanent lung damage and lymphoma.[5-8] 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., U.K., Australia and Israel 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 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.[9,10] Leniolisib is currently under regulatory review in the European Economic Area, Japan, Canada and several other countries for APDS. Leniolisib is also being evaluated in two Phase III clinical trials in children with APDS and in two Phase II clinical trials in primary immunodeficiencies (PIDs) with immune dysregulation. The safety and efficacy of leniolisib has not been established for PIDs with immune dysregulation beyond 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. We are developing and commercializing a portfolio of innovative medicines, including small molecules and biologics. Pharming is headquartered in Leiden, the Netherlands, with a significant proportion of its employees based in the U.S.

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 2024 Annual Report and the Annual Report on Form 20-F for the year ended December 31, 2024, 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.

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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