Pharming Group announces U.S. FDA acceptance and Priority Review of supplemental New Drug Application for leniolisib in children with APDS aged 4 to 11 years

- ***If approved, leniolisib will be first and only treatment indicated for children with activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS), a rare primary immunodeficiency***
- ***Decision based on positive data from multinational Phase III study in children aged 4 to 11 years with APDS***
- ***PDUFA target action date of January 31, 2026***

Leiden, the Netherlands, October 1, 2025: Pharming Group N.V. ("Pharming" or "the Company") (Euronext: PHARM; Nasdaq: PHAR) today announced that the U.S. Food and Drug Administration (FDA) has accepted its supplemental New Drug Application (sNDA) seeking approval for 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 application has been granted Priority Review and assigned a Prescription Drug User Fee Act (PDUFA) target action date of January 31, 2026.

The sNDA submitted to the FDA is based on positive data from the 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 the condition, 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 FDA grants Priority Review to applications for medicines that, if approved, would offer significant improvements in effectiveness or safety of the treatment, prevention, or diagnosis of serious conditions.[1] There are no approved treatments for children with APDS under the age of 12 years globally.

Fabrice Chouraqui, Chief Executive Officer of Pharming, commented:

"APDS is a rare, complex, and progressive primary immunodeficiency. Typically, it begins in early childhood causing immune dysregulation, recurrent infections and potentially permanent lung damage and lymphoma. Early access to targeted therapies has the potential to change the trajectory of the disease for young patients. Today's Priority Review designation marks a significant step for children aged 4-11 in the U.S. living with APDS."

Leniolisib, marketed under the brand name Joenja® in the U.S., received approval from the U.S. 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.

References

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For further public information, contact:

Pharming Group, Leiden, the Netherlands
Michael Levitan, VP Investor Relations & Corporate Communications
T: +1 (908) 705 1696

Saskia Mehring, Corporate Communications Manager
T: +31 6 28 32 60 41
E: investor@pharming.com

LifeSpring Life Sciences Communication, Amsterdam, the Netherlands
Leon Melens
T: +31 6 53 81 64 27
E: pharming@lifespring.nl

US PR
Ethan Metelenis
T: +1 (917) 882-9038
E: Ethan.Metelenis@precisionaq.com