

Pharming announces U.S. FDA acceptance of sNDA resubmission for Joenja® (leniolisib) to treat children aged 4 to 11 years with APDS

- If approved, Joenja will be the first approved treatment in the U.S. for children with activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS), a rare primary immunodeficiency
- Supplemental New Drug Application (sNDA) resubmission seeks approval for 40 mg and 50 mg twice-daily dosing
- Separate sNDA for lower weight pediatric patients planned for second half 2026

Leiden, the Netherlands, June 4, 2026: Pharming Group (Euronext: PHARM; Nasdaq: PHAR) today announced that the U.S. Food and Drug Administration (FDA) has accepted its resubmitted supplemental New Drug Application (sNDA) seeking approval 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. Following the Complete Response Letter (CRL) received on January 30, 2026, and a subsequent Type A meeting with the FDA on March 26, 2026, the resubmission seeks approval of 40 mg and 50 mg twice-daily dosing for pediatric patients weighing 27 kg or more, who represent a meaningful proportion of the identified pediatric patient population. The resubmission includes additional data requested by the FDA on analytical methods used for production batch testing. The FDA has assigned a Prescription Drug User Fee Act (PDUFA) target action date of October 24, 2026.

A separate sNDA seeking approval for lower doses for patients weighing less than 27 kg is planned for this summer.

Fabrice Chouraqui, Chief Executive Officer of Pharming, commented:

“We are pleased that the FDA has accepted our resubmission, bringing us one step closer to potentially making Joenja available to younger patients living with APDS. These young patients and their families continue to face the significant burden of this rare disease, and there is a clear need for an approved targeted treatment. We look forward to the continued collaboration with the FDA to help bring Joenja to the broader pediatric APDS population as quickly as possible.”

The resubmission is supported by 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 improvements in lymphoproliferation and immunophenotype correction were seen across all 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.

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 PI3K δ (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.⁸

About leniolisib

Leniolisib is an oral small molecule phosphoinositide 3-kinase delta (PI3K δ) inhibitor approved as the first and only targeted treatment of activated phosphoinositide 3-kinase delta (PI3K δ) syndrome (APDS) in the U.S., U.K., Australia and Israel in adult and pediatric patients 12 years of age and older and in Japan for patients 4 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 open label extension data has supported the safety and tolerability of long-term leniolisib administration.^{9,10}

Leniolisib is currently under regulatory review for the treatment of APDS in Canada and several other countries. Leniolisib is also being evaluated 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 develop and commercialize a portfolio of innovative medicines, including small molecules and biologics. Pharming is headquartered in Leiden, the Netherlands, with U.S. and European operations.

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 2025 Annual Report and the Annual Report on Form 20-F for the year ended December 31, 2025, 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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