European Commission grants marketing authorization to Pharming's Joenja® (leniolisib) – the first approved treatment for APDS in the European Union

- **Joenja® (leniolisib) is first approved treatment in the European Union for activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS), a rare primary immunodeficiency, in adult and pediatric patients aged 12 years and older**
- **First European launch expected in Germany in Q3 2026**
- **Approval adds to Joenja® approvals in the United States, United Kingdom, Japan, Australia and Israel, supporting Pharming's global expansion strategy**

Leiden, the Netherlands, May 22, 2026: Pharming Group ("Pharming" or "the Company") (EURONEXT Amsterdam: PHARM/Nasdaq: PHAR) today announced that the European Commission (EC) has granted marketing authorization for Joenja® (leniolisib), an oral, selective phosphoinositide 3-kinase (PI3K) delta inhibitor, for the treatment of activated PI3K delta syndrome (APDS), a rare primary immunodeficiency, in adult and pediatric patients 12 years of age and older. Joenja is the first and only approved treatment for APDS in the European Union (EU). The first European launch is expected in Germany in Q3 2026, with additional launches anticipated pending completion of national reimbursement negotiations.

Leverne Marsh, Chief Commercial Officer of Pharming, commented:
"APDS is a progressive disease that can have a profound, lifelong impact on those living with the condition and their families, significantly affecting quality of life and underscoring the need for targeted treatment options. Today's approval marks an important milestone for the European APDS community, making Joenja the first therapy in Europe specifically indicated to treat this disease.

This milestone reinforces our commitment to tackling rare disease challenges where new options are urgently needed. Joenja has the potential to meaningfully change how this complex condition is managed. We are proud to work with healthcare providers, patient communities and reimbursement authorities to support timely availability for eligible individuals across Europe."

The EC approval is based on results from a multinational, triple-blind, placebo-controlled, randomized Phase II/III clinical trial, evaluating leniolisib in 31 patients with APDS aged 12 years and older. The study demonstrated statistically significant improvements in markers of immune dysregulation and immunodeficiency. The application also included long-term, open-label extension data from 37 patients who received leniolisib for a median of three years.

Prof. Ulrich Baumann, MD, Managing Senior Physician, Department of Pediatric Pneumology, Allergology and Neonatology, Hannover Medical School, Germany commented:

"The available clinical data on leniolisib consistently demonstrated a significant improvement in key parameters of immune dysregulation in patients with APDS. Of particular note is its targeted approach, which directly targets the underlying pathophysiology. Against the background of previously limited therapeutic options, this approval represents an important advancement in the management of this patient population."

The approval is valid across all 27 EU Member States, as well as Norway, Iceland and Liechtenstein. Joenja is currently marketed in the United States and the United Kingdom for patients aged 12 years and older with APDS.

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.[8]

About Joenja® (leniolisib)

Joenja® (leniolisib) is an oral small molecule phosphoinositide 3-kinase delta (PI3Kδ) inhibitor approved as the first and only treatment of activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS) in the U.S., U.K., Australia, Israel and the European Union in adult and pediatric patients 12 years of age and older and in Japan for patients 4 years of age and older. Joenja® 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 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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