FDA approves Pharming's Joenja® as first treatment for children with APDS in the U.S.

- **FDA approval expands Joenja indication to include children with activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS) ages 4 to 11 who weigh at least 27kg**
- **Newly approved Joenja doses are expected to be available in October**

Leiden, the Netherlands, September 11, 2026: Pharming (Euronext Amsterdam: PHARM/Nasdaq: PHAR), a global biotechnology company focused on rare immune and genetic diseases, today announced that the U.S. Food and Drug Administration (FDA) has approved its supplemental New Drug Application (sNDA) for 40 mg and 50 mg twice-daily dosing of Joenja® (leniolisib), an oral, selective phosphoinositide 3-kinase delta (PI3Kδ) inhibitor, as a treatment for children aged 4 to 11 years weighing at least 27 kg with activated phosphoinositide 3-kinase delta syndrome (APDS), a rare primary immunodeficiency. With this approval, Joenja becomes the first FDA-approved treatment for children aged 4 to 11 years with APDS in the U.S. The newly approved Joenja doses are expected to be available to eligible pediatric patients in the U.S. in October through Pharming's established specialty distribution network and patient-support infrastructure.

On July 30, Pharming also submitted a separate sNDA seeking approval of lower Joenja doses for pediatric APDS patients aged 4 to11 years who weigh 13 kg to less than 27 kg.

"To give children living with APDS the best chance at a healthier future, early disease intervention is critical," commented Eveline Wu, M.D., MSCR, Associate Professor of Pediatrics at the University of North Carolina at Chapel Hill. *"Previously, our best approach for children aged 4 to 11 years with APDS was to provide an accurate diagnosis and a treatment plan to manage the symptoms of APDS. With the approval of Joenja for this pediatric population, we can now take that support even further to address the underlying pathway of disease, with the goal of reducing symptoms and preventing the irreversible complications that often arise with APDS."*

"APDS is not a disease that waits. It can affect important aspects of childhood, potentially impacting time in school, time with friends, and other activities of daily life. This approval is a meaningful step forward because it gives younger patients and their physicians another much-needed option earlier in the disease journey," said Vanessa Tenembaum, CEO of the Jeffrey Modell Foundation, an international, non-profit, organization dedicated to helping individuals and family members affected by primary immunodeficiency disorders. *"For families living with APDS, it means more than progress; it means renewed hope, greater possibility and a clearer path forward."*

APDS typically manifests in early childhood and is characterized by significant immune dysregulation and recurrent sinopulmonary infections, which over time can lead to permanent lung damage and other serious complications. For children living with APDS, the burden can extend beyond clinical symptoms. Recurrent infections, medical appointments and treatment demands may disrupt school attendance, learning and social development, adding to the daily impact on patients and families.

"Today's approval marks an important achievement in our efforts to expand the availability of Joenja to more individuals living with APDS. We are grateful to the FDA, trial participants, caregivers, investigators, healthcare providers and the APDS community whose partnership helped make this milestone possible," said Leverne Marsh, Chief Commercial Officer of Pharming. *"Our immediate priority is to support physicians and families in navigating the access pathway so treatment can start as quickly and responsibly as possible."*

Joenja 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. Today's approval is supported by data from a multinational, open-label, single-arm Phase III study in children aged 4 to 11 years with APDS. The study demonstrated improvements over 12 weeks in two clinically relevant hallmarks of the condition, reduced lymphadenopathy and increased naïve B cells, together indicating an improvement in the underlying immune defect. The safety profile was consistent with prior Joenja experience; all treatment emergent adverse events were mild to moderate in nature, and no drug related serious adverse events were observed.

US Important Safety Information for Joenja® (leniolisib)

INDICATIONS AND USAGE

Joenja® (leniolisib) is a kinase inhibitor indicated for the treatment of activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS) in adult and pediatric patients 4 years of age and older who weigh 27 kg or greater.

IMPORTANT SAFETY INFORMATION

Verify pregnancy status in females of reproductive potential prior to initiating treatment with Joenja.

Joenja may cause fetal harm when administered to a pregnant woman. Advise patients of the potential risk to a fetus and to use highly effective methods of contraception during treatment with Joenja and for 1 week after the last dose. Additionally, advise women not to breastfeed during treatment with Joenja and for 1 week after the last dose.

Live, attenuated vaccinations may be less effective if administered during Joenja treatment.

Joenja may cause hypersensitivity reaction(s), including anaphylaxis. Advise patients to discontinue Joenja and to seek immediate medical attention if they develop any signs and symptoms of serious allergic reactions.

Use of Joenja in patients with moderate to severe hepatic impairment is not recommended. There is no recommended dosage for patients under 4 years of age or weighing less than 27 kg.

Avoid co-administration of Joenja with other medications known to be strong CYP3A4 inhibitors, strong or moderate CYP3A4 inducers, or BCRP, OATP1B1, and OATP1B3 substrates.

Most common adverse reaction (incidence >10%):

- **Adult and pediatric patients 12 years of age and older:** headache, sinusitis, atopic dermatitis, and weight gain.
- **Pediatric patients 4 to <12 years of age who weigh 27 kg or greater:** abdominal pain, cough, and respiratory tract infection.

Seven (33%) patients 12 years and older (n=21) and 5 (63%) patients 4 to 12 years of age and weighing more than 27 kg or greater (n=8) developed an absolute neutrophil count (ANC) between 500 and 1500 cells/microL after receiving Joenja. No patients developed an ANC <500 cells/microL and there were no reports of infection associated with neutropenia.

Before prescribing Joenja, please read the full Prescribing Information.

Joenja is available in 40 mg, 50 mg, and 70 mg tablets.

Based on PI Approved: 09/2026

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®

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, Canada 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

Pharming Group N.V. (Euronext Amsterdam: PHARM/Nasdaq: PHAR) is a global biotechnology company that develops and commercializes innovative medicines for people living with rare immune and genetic diseases.

We combine specialized scientific, medical, regulatory and commercial expertise to advance a focused portfolio of approved medicines and development programs that address significant unmet medical needs. Guided by insights from patients and the wider rare disease community, we are dedicated to delivering innovative therapies for some of the most challenging rare diseases.

Pharming is headquartered in Leiden, the Netherlands, with operations in the United States and Europe.

For more information, visit **www.pharming.com** and follow 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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