

## Pharming Group announces approval of Joenja® (leniolisib) in Japan for the treatment of APDS in patients aged 4 years and older

- First approved treatment in Japan for activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS)
- Approval is based on positive Phase III data from multinational and Japanese studies in patients aged 12 years and older and a multinational pediatric study in children aged 4 to 11 years
- First approval of Joenja covering children aged 4 to 11 with APDS

**Leiden, the Netherlands, March 24, 2026:** Pharming Group N.V. (“Pharming” or “the Company”) (EURONEXT Amsterdam: PHARM/Nasdaq: PHAR) today announced that Japan’s Ministry of Health, Labour and Welfare (MHLW) has approved Joenja® (leniolisib), an oral, selective phosphoinositide 3-kinase (PI3K) delta inhibitor, for the treatment of activated PI3K delta syndrome (APDS) in adult and pediatric patients aged 4 years and older.

With this approval, Joenja becomes the first treatment approved in Japan specifically for APDS and the first approved treatment anywhere globally, for children aged 4 to 11 with the disease. Under an agreement with Pharming, OrphanPacific, Inc. serves as the Marketing Authorization Holder for Joenja in Japan and in collaboration with Pharming, is responsible for supply and distribution of the product. The launch of Joenja is expected following agreement with the MHLW on its National Health Insurance drug price.

**Professor Hirokazu Kanegane, MD, PhD, Department of Child Health and Development at the Graduate School of Medical and Dental Sciences, Institute of Science Tokyo commented:**

*“Patients with APDS in Japan have until now relied largely on supportive care, so this approval marks an important step forward for the APDS community. This approval introduces the first approved targeted treatment option for this rare primary immunodeficiency in Japan, and has the potential to help reduce the burden of disease for patients and their families.”*

**Leverne Marsh, Chief Commercial Officer of Pharming, commented:**

*“The approval of Joenja for patients as young as 4 years of age is an important milestone for the APDS community in Japan. As the first treatment approved specifically for APDS in the country, it is the only targeted treatment option for patients and families affected by this rare and progressive disease. It also marks the first approval globally for children aged 4 to 11 with APDS. Together with OrphanPacific, we look forward to working through the next steps to help make Joenja available to patients in Japan as quickly as possible.”*

The MHLW based the approval on data from Pharming’s Phase III clinical program for leniolisib in APDS, including the global, randomized, placebo-controlled study in patients aged 12 years and

older, in which leniolisib met its co-primary endpoints by significantly reducing lymphadenopathy and significantly increasing the percentage of naïve B cells out of total B cells. This reflects a clinically relevant impact on immune dysregulation and deficiency which are hallmarks of APDS. Leniolisib was generally well tolerated with the most common adverse reactions (>10%) being headache, sinusitis, and atopic dermatitis. Supporting Phase III evidence also included the open-label Japanese study in patients aged 12 years and older, as well as positive data from the open-label, multinational Phase III study in children aged 4 to 11 years, including sites in Japan, which showed improvements over 12 weeks in reduced lymphadenopathy and increased percentage of naïve B cells from total B cells.

The Pharmaceuticals and Medical Devices Agency (PMDA) evaluated the application under the Priority Review pathway following the Orphan Drug Designation granted by the MHLW in May 2023.

Joenja® is approved and marketed in the United States and the United Kingdom for patients 12 years of age and older with APDS.

### **About Activated Phosphoinositide 3-Kinase $\delta$ 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 $\delta$  (phosphoinositide 3-kinase delta) pathway, which causes immune cells to fail to mature and function properly, leading to immunodeficiency and dysregulation<sup>1,2,3</sup> APDS is characterized by a variety of symptoms, including severe, recurrent sinopulmonary infections, lymphoproliferation, autoimmunity, and enteropathy.<sup>4,5</sup> 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.<sup>6</sup> As APDS is a progressive disease, this delay may lead to an accumulation of damage over time, including permanent lung damage and lymphoma.<sup>4,7</sup> A definitive diagnosis can be made through genetic testing. APDS affects approximately 1 to 2 people per million worldwide.<sup>8</sup>

### **About Joenja® (leniolisib)**

Leniolisib is an oral small molecule phosphoinositide 3-kinase delta (PI3K $\delta$ ) inhibitor approved as the first and only targeted treatment of activated phosphoinositide 3-kinase delta (PI3K $\delta$ ) 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.<sup>9,10</sup>

Leniolisib is currently under regulatory review for the treatment of APDS in the European Economic Area, 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 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](http://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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