Pharming announces commercial launch of Joenja (leniolisib) in Japan for adult and pediatric patients with APDS aged 4 and older

- ***Launch marks first commercial availability of Joenja® (leniolisib) for children aged 4 to 11 worldwide***
- ***OrphanPacific, Inc. serves as Marketing Authorization holder and is responsible for supply and distribution in Japan***

Leiden, the Netherlands, August 13, 2026: Pharming Group (Euronext Amsterdam: PHARM/Nasdaq: PHAR), today announced the commercial launch of Joenja (leniolisib) in Japan for adult and pediatric patients with activated PI3K delta syndrome (APDS) aged 4 years and older, following its inclusion on the Japan National Health Insurance (NHI) drug price list. With this launch, Japan is now the first market where Joenja is commercially available for children aged 4 to 11.

"This launch is an important milestone in Pharming's global expansion and underscores our commitment to bringing innovative medicines to rare disease communities worldwide," said ***Leverne Marsh***, ***Chief Commercial Officer*** of Pharming. "For eligible patients and their families in Japan, who until now have had limited treatment options beyond supportive care, this launch represents meaningful progress in addressing a significant unmet need. We are grateful to OrphanPacific for its partnership and close collaboration in helping make this treatment available."

The launch of Joenja in Japan follows its approval by Japan's Ministry of Health, Labour and Welfare (MHLW) in March of this year as the first treatment specifically for APDS. 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.

Joenja® is a registered trademark owned by Pharming Group N.V. or its affiliates.

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 a treatment of activated phosphoinositide 3-kinase delta (PI3Kδ) syndrome (APDS) in the U.S., U.K., the European Union, Australia, Israel, Canada, South Korea and Saudi Arabia 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 pediatric patients in several 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 have 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.

About OrphanPacific, Inc.

OrphanPacific is a Japanese pharmaceutical company dedicated to bringing new treatment options to patients with rare diseases through the development, manufacturing, and commercialization of orphan drugs. The company is actively engaged in delivering treatments for rare and ultra-rare diseases in Japan.

For more information, visit https://www.orphanpacific.com/en/

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.

References

1. Lucas CL, et al. Nat Immunol. 2014;15(1):88-97.
2. Elkaim E, et al. J Allergy Clin Immunol. 2016;138(1):210-218.
3. Nunes-Santos C, Uzel G, Rosenzweig SD. J Allergy Clin Immunol. 2019;143(5):1676-1687.
4. Coulter TI, et al. J Allergy Clin Immunol. 2017;139(2):597-606.
5. Maccari ME, et al. Front Immunol. 2018;9:543.
6. Jamee M, et al. Clin Rev Allergy Immunol. 2020 Dec;59(3):323-333.
7. Condliffe AM, Chandra A. Front Immunol. 2018;9:338.

8. Vanselow S, et al. Frontiers in Immunology. 2023;14:1208567.
9. Rao VK, et al Blood. 2023 Mar 2;141(9):971-983.
10. Rao VK, et al. J Allergy Clin Immunol 2024;153:265-74.

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