March 24, 2026

OrphanPacific Obtains Marketing Approval in Japan forJoenja® (leniolisib) for the Treatment of APDS for adults and pediatric patients 4 years of age and older

OrphanPacific, Inc. (Minato-ku, Tokyo, Japan; President and Representative Director: Megumi HARA, hereinafter "OrphanPacific") and Pharming Group N.V. (headquartered in the Netherlands , hereinafter “Pharming”) announce that OrphanPacific obtained marketing approval in Japan on March 23, 2026, for Joenja® (leniolisib), an oral, selective PI3Kδ inhibitor indicated for the treatment of activated phosphoinositide 3-kinase delta syndrome (APDS) in adult and pediatric patients ages 4 years and older.
Under an agreement with Pharming, OrphanPacific, Inc. is the Marketing Authorization Holder for Joenja in Japan and, in collaboration with Pharming, is responsible for the product’s supply and distribution.

APDS is a rare genetic primary immunodeficiency disorder caused by variants in genes regulating the PI3Kδ（phosphoinositide 3-kinase delta）signaling pathway, leading to immune dysregulation and recurrent infections. It affects approximately 1 to 2 people per million worldwide. Leniolisib targets the underlying cause of the disease by selectively inhibiting PI3Kδ and has the potential to significantly improve the management of patients with APDS.

OrphanPacific will work closely with Pharming to ensure that patients with APDS in Japan, their families, and the healthcare professionals supporting them gain access to this treatment as soon as possible.

Joenja® was designated as an Orphan Drug by Japan’s Ministry of Health, Labour and Welfare (MHLW) in May 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 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 wide range of symptoms, including severe and recurrent sinopulmonary infections, lymphoproliferation, autoimmunity, and enteropathy. 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 (PI3Kδ) inhibitor approved as the first and only targeted treatment of activated phosphoinositide 3-kinase delta (PI3Kδ) 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 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 or LinkedIn.

■ About OrphanPacific

OrphanPacific is a Japanese pharmaceutical company that brings new therapeutic drugs to patients with rare diseases through the development, manufacturing and sale of orphan drugs. The company’s mission is to "deliver smiles and happiness to patients with rare diseases and their families." With the determination of "Leave No One Behind", OrphanPacific is actively working on the development and distribution of drugs for the treatment of rare diseases with a very small number of patients (ultra-orphan medicines). OrphanPacific is a wholly-owned subsidiary of CMIC Holdings (https://www.cmicgroup.com/), a pioneer and leading CRO (Contract Research

Organization) in Japan. Making the best use of the CMIC Group's experience and know-how of development, manufacturing and sales of drugs, OrphanPacific aims to enable as many patients with rare diseases as possible to have access to therapeutic drugs.
For more information, visit https://www.orphanpacific.com/.

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