August 13, 2026

OrphanPacific Announces the Launch of Joenja® (leniolisib) in Japan for the Treatment of APDS in 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 Joenja® Tablets 70 mg, 30 mg and 10 mg (generic name: leniolisib), an oral, selective PI3Kδ inhibitor indicated for the treatment of activated phosphoinositide 3-kinase delta syndrome (APDS) in adult and pediatric patients 4 years of age and older, was listed on Japan's National Health Insurance Drug Price List on August 13, 2026.

Joenja was approved in Japan by the Ministry of Health, Labour and Welfare in March 2026 and was designated as an Orphan Drug in May 2023.

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 signaling pathway. The disease leads to immune dysregulation, recurrent infections and a broad range of other clinical manifestations. APDS affects approximately 1 to 2 people per million worldwide.

Leniolisib selectively inhibits the PI3Kδ pathway and targets the underlying disease mechanism of APDS. The launch of Joenja will provide patients with APDS in Japan with a new targeted treatment option that has the potential to improve disease management.

Megumi HARA, President and Representative Director of OrphanPacific, said:

"We are very pleased to make Joenja available to patients with APDS in Japan. APDS is an extremely rare and progressive disease, and patients may experience a prolonged journey before receiving an accurate diagnosis. We hope that the availability of Joenja will provide a meaningful new treatment option for patients and their families. OrphanPacific will continue to work closely with Pharming and healthcare professionals to ensure the stable supply and appropriate use of Joenja in Japan."

OrphanPacific is committed to its mission of delivering smiles and happiness to patients with rare diseases and their families. With its determination to "Leave No One Behind," OrphanPacific will

continue working to ensure that patients with APDS in Japan, their families and the healthcare professionals supporting them have appropriate and reliable access to Joenja.

■ Product Overview

<table>
<tr><td>Brand name</td><td>Joenja® Tablets 70 mg
Joenja® Tablets 30 mg
Joenja® Tablets 10 mg</td></tr>
<tr><td>Generic name</td><td>leniolisib</td></tr>
<tr><td>Indication</td><td>Activated phosphoinositide 3-kinase delta syndrome</td></tr>
<tr><td>Dosage and administration</td><td>For adults and pediatric patients aged 4 years and older, administer leniolisib orally twice daily, approximately every 12 hours, according to body weight, at the following single doses.

<table><tr><th>body weight</th><th>Single dose</th></tr><tr><td>13 kg to less than 19 kg</td><td>20 mg</td></tr><tr><td>19 kg to less than 27 kg</td><td>30 mg</td></tr><tr><td>27 kg to less than 38 kg</td><td>40 mg</td></tr><tr><td>38 kg to less than 45 kg</td><td>50 mg</td></tr><tr><td>45 kg or more</td><td>70 mg</td></tr></table></td></tr>
<tr><td>National Health Insurance drug price</td><td>Joenja® Tablets 70 mg: JPY 133,176.50 per tablet
Joenja® Tablets 30 mg: JPY 102,115.70 per tablet
Joenja® Tablets 10 mg: JPY 27,077.90 per tablet</td></tr>
<tr><td>Date of National Health Insurance Drug Price Listing</td><td>August 13, 2026</td></tr>
<tr><td>Marketing Authorization Holder</td><td>OrphanPacific, Inc.</td></tr>
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■ About Activated Phosphoinositide 3-Kinase δ Syndrome (APDS)

APDS is a rare primary immunodeficiency that was first characterized in 2013. It is caused by variants in either PIK3CD or PIK3R1, two genes that are vital to the development and function of immune cells.

Variants in these genes lead to hyperactivity of the PI3Kδ signaling pathway, causing immune cells to fail to mature and function properly and resulting in immunodeficiency and immune dysregulation.[1,2,3]

APDS is characterized by a broad range of symptoms, including severe and recurrent sinopulmonary infections, bronchiectasis, lymphoproliferation, autoimmune complications and

enteropathy. Because these manifestations are also associated with other conditions, including other primary immunodeficiencies, patients with APDS may be misdiagnosed, and a median diagnostic delay of seven years has been reported.[6]

APDS is a progressive disease. Delayed diagnosis and treatment may result in the 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 for the treatment of activated phosphoinositide 3-kinase delta syndrome (APDS). In Japan, Joenja is approved for adult and pediatric patients 4 years of age and older.

Joenja inhibits the production of phosphatidylinositol-3-4-5-trisphosphate, an important cellular messenger involved in the regulation of multiple cellular functions, including proliferation, differentiation, cytokine production, cell survival, angiogenesis and metabolism.

Results from a randomized, placebo-controlled Phase III clinical trial demonstrated statistically significant improvements in the two coprimary endpoints, reflecting a favorable effect on the immune dysregulation and immunodeficiency observed in patients with APDS. The safety and tolerability of long-term leniolisib administration were further evaluated in an open-label extension study.[9,10]

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. Pharming develops and commercializes 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 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 to "Leave No One Behind," OrphanPacific is actively engaged in the development and distribution of treatments for rare diseases affecting very small patient populations, including ultra-orphan medicines.

OrphanPacific is a wholly owned subsidiary of CMIC Holdings Co., Ltd., a pioneer and leading contract research organization in Japan. By making full use of the CMIC Group's experience and expertise in pharmaceutical development, manufacturing and commercialization, OrphanPacific aims to enable as many patients with rare diseases as possible to gain access to appropriate therapies.

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

■ References

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