

FOR IMMEDIATE RELEASE

Tokyo, August 26, 2026

OrphanPacific, Inc.

Manufacturing and Marketing Approval Granted in Japan for ORLADEYO® for Pediatric Patients with Hereditary Angioedema**— A new oral treatment option for children aged 2 to under 12 years weighing 15 kg or more —**

OrphanPacific, Inc. (head office: Minato-ku, Tokyo; President and Representative Director: Megumi Hara; “OrphanPacific”) announced today that manufacturing and marketing approval has been granted in Japan for ORLADEYO® Granule Sachet 72 mg, 96 mg, 108 mg and 132 mg (generic name: berotralstat hydrochloride; “ORLADEYO® Granule Sachet”), a plasma kallikrein inhibitor.

ORLADEYO® Granule Sachet is a once-daily oral granular formulation indicated for prophylactic therapy in pediatric patients with hereditary angioedema (HAE) aged 2 to under 12 years who weigh 15 kg or more.

ORLADEYO® is an orally administered plasma kallikrein inhibitor discovered by BioCryst Pharmaceuticals, Inc. (“BioCryst”). In Japan, OrphanPacific holds the manufacturing and marketing approval.

In Japan, ORLADEYO® capsules (150 mg) received manufacturing and marketing approval in January 2021 for prophylactic treatment of hereditary angioedema (HAE) in adults and pediatric patients 12 years and older. The newly approved ORLADEYO® Granule Sachets are available in four strengths, containing 72 mg, 96 mg, 108 mg, and 132 mg of berotralstat per sachet. This approval provides a new once-daily oral treatment option for pediatric patients in Japan aged 2 to under 12 years who weigh 15 kg or more.

HAE is a rare disease characterized by unpredictable, recurrent swelling attacks affecting various parts of the body, including the skin, gastrointestinal tract and larynx. The disease may manifest during childhood, and anxiety about sudden attacks and recurrence can have a substantial impact not only on a child’s school and daily life, but also on the lives of family members.

OrphanPacific believes this approval is an important step in providing a new oral treatment option to pediatric patients aged 2 to under 12 years who weigh 15 kg or more and who were previously not eligible for treatment with ORLADEYO® in Japan.

The launch of ORLADEYO® for children in Japan will follow completion of the Japanese National Health Insurance System (NHI) reimbursement pricing process.

APPROVAL SUMMARY

Product Names	ORLADEYO® Granule Sachet 72 mg ORLADEYO® Granule Sachet 96 mg ORLADEYO® Granule Sachet 108 mg ORLADEYO® Granule Sachet 132 mg
Generic Name	Berotralstat hydrochloride
Indication	Suppression of acute attacks of hereditary angioedema
Dosage and Administration	For pediatric patients from 2 years to less than 12 years of age, the following weight-based doses of berotralstat should be administered orally once daily, after meals or with meals. 15 kg to less than 24 kg: 72 mg of berotralstat 24 kg to less than 32 kg: 96 mg of berotralstat 32 kg to less than 40 kg: 108 mg of berotralstat 40 kg and above: 132 mg of berotralstat
Formulation and Strength	72 mg: 81.33 mg of berotralstat hydrochloride per sachet (72 mg as berotralstat) 96 mg: 108.44 mg of berotralstat hydrochloride per sachet (96 mg as berotralstat) 108 mg: 122.00 mg of berotralstat hydrochloride per sachet (108 mg as berotralstat) 132 mg: 149.11 mg of berotralstat hydrochloride per sachet (132 mg as berotralstat)
Marketing Authorization Holder	OrphanPacific, Inc.

ABOUT ORLADEYO®

ORLADEYO® (berotralstat) is the first and only oral therapy designed specifically to prevent attacks of hereditary angioedema (HAE) in adult and pediatric patients 2 years and older. One dose of ORLADEYO® per day works to prevent HAE attacks by decreasing the activity of plasma kallikrein.

ABOUT HEREDITARY ANGIOEDEMA (HAE)

HAE is a rare inherited disease caused primarily by excessive production of bradykinin, which increases vascular permeability, due to deficiency or impaired function of C1 inhibitor.

HAE causes sudden swelling in various parts of the body, including the extremities, face, gastrointestinal tract and larynx. Gastrointestinal swelling may be accompanied by severe abdominal pain, nausea and vomiting, while laryngeal swelling can obstruct the

airway and may be life-threatening. Because attacks are unpredictable, HAE can have a significant impact on patients' school life, employment, social activities and psychological well-being.

ABOUT ORPHANPACIFIC, INC.

OrphanPacific is a Japan-based pharmaceutical company that provides new treatments for people living with rare diseases through the development, manufacturing and marketing of rare-disease medicines.

Guided by its mission, "Bringing smiles and happiness to patients with rare diseases and their families," and its commitment to "Leave No One Behind," OrphanPacific actively develops and supplies treatments for ultra-rare diseases affecting very small patient populations.

OrphanPacific is a wholly owned subsidiary of CMIC Holdings Co., Ltd., a pioneer and leading contract research organization in Japan. (<https://en.cmicgroup.com/>) By leveraging the CMIC Group's experience and expertise in pharmaceutical development, manufacturing and commercialization, OrphanPacific aims to help more people with rare diseases gain access to needed treatments. (<https://www.orphanpacific.com/en/>)

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