BioCryst Announces Approval of ORLADEYO® for Pediatric HAE Patients in Japan

August 25, 2026

— Expands availability of the first and only oral prophylactic therapy for patients with HAE ages 2 to <12 years in Japan —

RESEARCH TRIANGLE PARK, N.C., Aug. 25, 2026 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq: BCRX) today announced that Japan's Ministry of Health, Labor and Welfare (MHLW) has granted marketing approval for once-daily ORLADEYO ® (berotralstat) for prophylactic therapy in pediatric patients with hereditary angioedema (HAE) aged 2 to <12 years.

"The approval of ORLADEYO for children in Japan marks an important step toward our goal of bringing a once-daily oral treatment to people living with HAE around the world," said Charlie Gayer, President and Chief Executive Officer of BioCryst. "We're proud to advance an oral prophylactic option in Japan that can help reduce treatment burden for children and provide greater convenience for families managing this lifelong disease."

For children with HAE, treatment has traditionally required injections, creating additional burden for patients and caregivers. Developed with the unique administration needs of children in mind, the ORLADEYO granule formulation (known as pellet formulation in the U.S.) is sprinkle-like in appearance and size and can be poured directly into the mouth and swallowed immediately with water or milk, or sprinkled over a spoonful of soft, non-acidic food.

This approval is based on positive interim results from the APeX-P clinical trial, the largest trial of long-term prophylaxis (LTP) in pediatric patients with HAE, evaluating the pharmacokinetics, safety and efficacy of ORLADEYO in patients aged 2 to <12 years with HAE due to C1-inhibitor deficiency. Interim results from APeX-P presented at multiple allergy and immunology-focused congresses and published in the *Annals of Allergy, Asthma & Immunology* showed ORLADEYO was generally well tolerated, demonstrated a consistent safety profile across this age group, and resulted in early and sustained reductions in monthly attack rates with no new safety signals identified beyond those previously described in prior adult and adolescent trials. The most commonly reported treatment-emergent adverse event (TEAE) was nasopharyngitis.

BioCryst will launch ORLADEYO for children in Japan following completion of the Japanese National Health Insurance System (NHI) reimbursement pricing process. OrphanPacific, Inc. is BioCryst's representative partner in Japan and holds the marketing authorization.

ORLADEYO is the first and only targeted oral prophylactic therapy for patients with HAE aged 2 and above. A capsule formulation of ORLADEYO received U.S. Food and Drug Administration (FDA) approval for prophylaxis to prevent HAE attacks in adult and pediatric patients 12 years and older in December 2020 and is now approved in more than 45 countries around the world. In December 2025, the oral pellet formulation of ORLADEYO (known as granule formulation in Japan) was approved by FDA for prophylactic therapy in pediatric patients in the U.S. with HAE aged 2 to <12 years.

Today's milestone represents the second regulatory approval for ORLADEYO in Japan. The capsule formulation of ORLADEYO was approved in January 2021 by the MHLW in Japan for prophylactic treatment of HAE in adults and pediatric patients 12 years and older.

BioCryst has also filed its application for the use of ORLADEYO in patients with HAE aged 2 to <12 years with the European Medicines Agency and Health Canada. Additional regulatory filings are planned in other global territories.

Visit www.ORLADEYO.com for more information.

About APeX-P
APeX-P is an ongoing, open-label study evaluating the pharmacokinetics, safety and efficacy of ORLADEYO in patients aged 2 to <12 years with HAE due to C1-inhibitor deficiency. Before ORLADEYO initiation, patients received SOC for 12 weeks. The rates of HAE attacks requiring on-demand treatment and number of attacks requiring professional care were compared between 12 weeks of SOC and 48 weeks of ORLADEYO treatment. Participants (n=29) were placed in one of four cohorts by body weight at baseline.

About ORLADEYO® (berotralstat)
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.

U.S. Indication and Important Safety Information

INDICATION
ORLADEYO® (berotralstat) is a plasma kallikrein inhibitor indicated for prophylaxis to prevent attacks of hereditary angioedema (HAE) in adults and pediatric patients 2 years and older.

Limitations of use
The safety and effectiveness of ORLADEYO for the treatment of acute HAE attacks have not been established. ORLADEYO should not be used for the treatment of acute HAE attacks. Additional doses or doses of ORLADEYO higher than the prescribed once-daily dose are not recommended due to the potential for QTc interval prolongation.

IMPORTANT SAFETY INFORMATION
An increase in QTc interval was observed in adults at dosages higher than 150 mg once daily and was concentration dependent.

The most common adverse reactions (≥10%) in patients receiving ORLADEYO were abdominal pain, vomiting, diarrhea, back pain and gastroesophageal reflux disease.

In adult and pediatric patients aged 12 years and older with moderate or severe hepatic impairment (Child-Pugh B or C), the recommended dosage of ORLADEYO capsules is 110 mg once daily with food. In pediatric patients aged 2 to <12 years with moderate or severe hepatic impairment (Child-Pugh B or C), avoid use of ORLADEYO.

Berotralstat is a substrate of P-glycoprotein (P-gp) and breast cancer resistance protein. P-gp inducers may decrease berotralstat plasma concentration, leading to reduced efficacy of ORLADEYO. Avoid concomitant use of P-gp inducers with ORLADEYO.

ORLADEYO at a dose of 150 mg is a moderate inhibitor of CYP2D6 and CYP3A4. Concomitant use of ORLADEYO with CYP2D6 or CYP3A4 substrates can increase exposure of the CYP2D6 or CYP3A4 substrates and may increase the risk of adverse reactions associated with the substrates. If ORLADEYO is concomitantly used with CYP2D6 or CYP3A4 substrates where minimal increases in the concentration of the substrates may lead to serious adverse reactions, closely monitor or modify the dosage of the CYP2D6 or CYP3A4 substrate.

The safety and effectiveness of ORLADEYO in pediatric patients <2 years of age have not been established.

There are insufficient data available to inform drug-related risks with ORLADEYO use in pregnancy. There are no data on the presence of berotralstat in human milk, its effects on the breastfed infant, or its effects on milk production.

To report SUSPECTED ADVERSE REACTIONS, contact BioCryst Pharmaceuticals, Inc. at 1-833-633-2279 or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

About BioCryst Pharmaceuticals
BioCryst is a global biotechnology company focused on developing and commercializing medicines for hereditary angioedema (“HAE”) and other rare diseases, driven by its deep commitment to improving the lives of people living with these conditions. BioCryst has commercialized ORLADEYO® (berotralstat), the first oral, once-daily plasma kallikrein inhibitor and is advancing a pipeline of potential first-in-class or best-in-class therapeutics for rare diseases. For more information, please visit www.biocryst.com or follow us on LinkedIn.

Forward-Looking Statements
This press release contains forward-looking statements, including statements regarding BioCryst’s expectations relating to the use of ORLADEYO for pediatric patients with HAE aged 2 to <12 years, including with respect to regulatory filings, commercialization and potential benefits. These statements involve known and unknown risks, uncertainties and other factors which may cause BioCryst’s actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements. These statements reflect our current views with respect to future events and are based on assumptions and are subject to risks and uncertainties. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Some of the factors that could affect the forward-looking statements contained herein include: BioCryst’s ability to successfully implement its commercialization plans for ORLADEYO, including the once-daily oral granule formulation; the commercial viability of ORLADEYO oral granules; interim results of a clinical trial do not necessarily predict final results; risks related to government actions, including that decisions and other actions related to pricing and reimbursement may not be taken when expected or at all, or that the outcomes of such decisions and other actions may not be in line with BioCryst’s current expectations; other applicable regulatory agencies may not approve ORLADEYO for use in pediatric patients with HAE aged 2 to <12 years within the timeframe expected, or at all, may ultimately determine that there are deficiencies in the development program or execution thereof, may require additional information or studies, may disagree with our safety and efficacy conclusions, may impose certain restrictions, warnings, or other requirements, may impose a clinical hold with respect to ORLADEYO, or may withhold or delay market approval for ORLADEYO; and once approved, the FDA, MHLW and other applicable regulatory agencies may withdraw market approval for ORLADEYO. This list is not exclusive. To see a more comprehensive list of risks, please refer to the documents BioCryst files periodically with the Securities and Exchange Commission, specifically BioCryst’s most recent Annual Report on Form 10-K, Quarterly Reports on Form 10-Q and Current Reports on Form 8-K, which identify important factors that could cause actual results to differ materially from those contained in BioCryst’s forward-looking statements.

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