



BioCryst Announces U.S. FDA Approval of ORLADEYO® 150 mg Oral Pellets for Adults and Adolescents with HAE

October 21, 2026

—Expands BioCryst portfolio of targeted oral prophylactic therapy options to meet patients' individual needs and preferences—

—ORLADEYO is the first and only targeted oral prophylactic therapy with options for HAE patients aged 2 and above—

RESEARCH TRIANGLE PARK, N.C., Oct. 21, 2026 (GLOBE NEWSWIRE) -- [BioCryst Pharmaceuticals, Inc.](#) (Nasdaq: BCRX) today announced the U.S. Food and Drug Administration (FDA) has approved its supplemental New Drug Application (sNDA) for use of the oral pellet formulation of once-daily ORLADEYO® (berotralstat) for prophylactic therapy in adults and adolescents aged 12 years and older. The new 150 mg ORLADEYO pellet formulation provides an alternative administration option for adults and adolescents who may have difficulty swallowing, prefer not to take a capsule or choose to remain on a pellet formulation beyond age 12.

With this approval, prescribers can now provide their adult and adolescent patients with the option to take once-daily, orally administered ORLADEYO for prophylaxis to prevent attacks of HAE in either capsule or pellet formulation. The ORLADEYO pellet formulation 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.

"We are focused on advancing HAE attack prevention through a portfolio of therapies designed to support the diverse needs and preferences of patients," said Charlie Gayer, President and Chief Executive Officer of BioCryst. "The approval of ORLADEYO pellets for adults and adolescents strengthens our ability to provide personalized treatment choice by offering the proven efficacy of ORLADEYO in a new formulation to make administration easier for patients who have difficulty swallowing or prefer not to take a capsule. This milestone reflects our commitment to optimizing the management of HAE with proven therapies that help address the evolving needs of patients across the treatment landscape."

ORLADEYO is the first and only targeted oral prophylactic therapy for patients with HAE aged 2 and above. A capsule formulation of ORLADEYO received 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 was approved by FDA for prophylactic therapy in pediatric patients in the U.S. with HAE aged 2 to <12 years.

Across two clinical studies, ORLADEYO capsules (150 mg) resulted in sustained attack prevention in patients 12 years and older with a mean attack-rate reduction of 91% at 96 weeks and an 88% reduction in rescue medication use¹. ORLADEYO was safe and well tolerated in both trials. The most frequently reported adverse reactions in patients receiving ORLADEYO compared with placebo were gastrointestinal reactions and back pain. These gastrointestinal reactions generally occurred early after initiation of treatment with ORLADEYO, became less frequent with time, and typically self-resolved.

The FDA approval was based on chemistry, manufacturing, and controls information and data demonstrating the safety and tolerability of the ORLADEYO pellet formulation compared to ORLADEYO capsules.

BioCryst is committed to providing support every step of the way and offers Empower Patient Services, a single stop for all patient and HCP ORLADEYO needs. It combines a specialty pharmacy and patient support program with a dedicated team of experts who provide individualized and streamlined support navigating the insurance process, affordability, adherence, and related needs.

To download multimedia: <https://www.globenewswire.com/NewsRoom/AttachmentNg/91e297e1-8672-4a7b-aa7a-ad4bfe93ea47>

Visit www.ORLADEYO.com for more information.

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.

Berotrastat is a substrate of P-glycoprotein (P-gp) and breast cancer resistance protein. P-gp inducers may decrease berotrastat 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 berotrastat 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® (berotrastat), 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 oral pellet formulation of ORLADEYO for patients with HAE. 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 pellet formulation; the commercial viability of ORLADEYO oral pellets, including their ability to achieve market acceptance; 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; and the FDA 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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¹ Kiani-Alikhan S, Gower R, Craig T, et al. Once-daily oral berotrastat for long-term prophylaxis of hereditary angioedema: the open-label extension of the APeX-2 randomized trial. *J Allergy Clin Immunol Pract.* 2024;12(3):733-743.e10.