BioCryst Announces FDA Acceptance of NDA for ORLADEYO® (berotralstat) Oral Granules in Patients with Hereditary Angioedema Aged 2 to 11 Years

May 14, 2025

–FDA grants Priority Review of application, with PDUFA target action date of September 12, 2025–

–ORLADEYO would be the first targeted oral prophylactic therapy for patients with HAE under the age of 12, if approved–

RESEARCH TRIANGLE PARK, N.C., May 14, 2025 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq: BCRX) today announced that the U.S. Food and Drug Administration (FDA) has accepted its New Drug Application (NDA) for the use of oral, once-daily ORLADEYO® (berotralstat) in pediatric patients with hereditary angioedema (HAE) aged 2 to 11 years. The FDA also granted Priority Review of the application, with a Prescription Drug User Fee Act (PDUFA) target action date of September 12, 2025.

"We are excited to take another step closer to bringing ORLADEYO to younger pediatric patients with HAE. We consistently hear from patients, caregivers and physicians about their desire for a more convenient therapeutic option to treat young children with HAE, and we now may have the opportunity to bring this to them later this year," said Jon Stonehouse, president and chief executive officer of BioCryst.

The NDA was based on positive interim data from the APeX-P clinical trial, the largest trial evaluating a prophylactic therapy for HAE in patients 2 to 11 years of age. Interim results from APeX-P that were presented at the 2025 American Academy of Allergy, Asthma & Immunology / World Allergy Organization Joint Congress earlier this year showed ORLADEYO was well tolerated and demonstrated a very consistent safety profile across this age group, and resulted in early and sustained reductions in monthly attack rates.

"As detailed in the results from APeX-P, we observed that participants experienced serious HAE attacks at a very early age, with a median age of HAE symptom onset of two years, which suggests there is a larger burden of disease at an earlier age than has been appreciated thus far. If approved, we believe this oral granule formulation of ORLADEYO could help children with HAE and their families better manage their condition and avoid the traumatic experience of acute attacks with emergency care or hospital stays," said Dr. Helen Thackray, chief research and development officer of BioCryst.

ORLADEYO would be the first targeted oral prophylactic therapy for children with HAE under the age of 12, if approved.

BioCryst has also filed its line extension application for the use of ORLADEYO oral granules in patients with HAE aged 2 to 11 years with the European Medicines Agency. Additional regulatory filings are planned in other global territories, including Japan and Canada.

ORLADEYO received FDA approval in December 2020 for prophylaxis to prevent HAE attacks in adult and pediatric patients 12 years and older and is now commercially available in more than 30 countries.

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 12 years and older. One capsule 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 12 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 dosages of ORLADEYO higher than 150 mg once daily are not recommended due to the potential for QT prolongation.

IMPORTANT SAFETY INFORMATION

An increase in QT prolongation was observed at dosages higher than the recommended 150 mg once-daily dosage and was concentration dependent.

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

A reduced dosage of 110 mg taken orally once daily with food is recommended in patients with moderate or severe hepatic impairment (Child-Pugh B or C).

Berotralstat is a substrate of P-glycoprotein (P-gp) and breast cancer resistance protein. P-gp inducers (eg, rifampin, St. John's wort) may decrease berotralstat plasma concentration, leading to reduced efficacy of ORLADEYO. The use of P-gp inducers is not recommended with ORLADEYO.

ORLADEYO at a dose of 150 mg is a moderate inhibitor of CYP2D6 and CYP3A4. For concomitant medications with a narrow therapeutic index that are predominantly metabolized by CYP2D6 or CYP3A4, appropriate monitoring and dose titration is recommended. ORLADEYO at a dose of 300 mg is a P-gp inhibitor. Appropriate monitoring and dose titration is recommended for P-gp substrates (eg, digoxin) when coadministering with ORLADEYO.

The safety and effectiveness of ORLADEYO in pediatric patients <12 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.

Please see full Prescribing Information.

About BioCryst Pharmaceuticals
BioCryst Pharmaceuticals is a global biotechnology company with a deep commitment to improving the lives of people living with hereditary angioedema and other rare diseases. BioCryst leverages its expertise in structure-guided drug design to develop first-in-class or best-in-class small-molecule and protein therapeutics to target difficult-to-treat diseases. BioCryst has commercialized ORLADEYO® (berotralstat), the first oral, once-daily plasma kallikrein inhibitor, and is advancing a pipeline of small-molecule and protein therapies. 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 future results, performance or achievements and statements relating to ORLADEYO performance. These statements involve known and unknown risks, uncertainties and other factors which may cause 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 or maintain its commercialization plans for ORLADEYO; BioCryst's ability to successfully progress its development plans as described herein, including meeting the expected timelines; ongoing and future preclinical and clinical development of product candidates may take longer than expected and may not have positive results; the outcome of preclinical testing and early clinical trials may not be predictive of the success of later clinical trials, and interim results of a clinical trial do not necessarily predict final results; the commercial viability of ORLADEYO for use in pediatric patients with HAE aged 2 to 11 years, including its ability to achieve sustained market acceptance; and the FDA, European Medicines Agency or other applicable regulatory agency may not approve ORLADEYO for use in pediatric patients with HAE aged 2 to 11 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, delay, or withdraw market approval for ORLADEYO. 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 the actual results to differ materially from those contained in BioCryst's forward-looking statements.

BCRXW

Contact:
John Bluth
+1 919 859 7910
jbluth@biocryst.com