# BioCryst to Present New ORLADEYO® (berotralstat) Results from APeX-P Pediatric Trial at 2025 American Academy of Allergy, Asthma & Immunology / World Allergy Organization Joint Congress

February 10, 2025

RESEARCH TRIANGLE PARK, N.C., Feb. 10, 2025 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq: BCRX) today announced that the company will present five abstracts featuring new clinical and real-world outcomes with oral, once-daily ORLADEYO® (berotralstat) for the prophylactic treatment of hereditary angioedema (HAE) at the 2025 American Academy of Allergy, Asthma & Immunology (AAAAI) / World Allergy Organization (WAO) joint congress.

Included among these is a late-breaking abstract that highlights the first presentation of results from the APeX-P trial evaluating oral, once-daily ORLADEYO in pediatric patients with HAE who are 2 to <12 years of age. The congress will take place in San Diego from February 28-March 3, 2025.

BioCryst will present the following five abstracts on Sunday, March 2 from 9:45-10:45 a.m. PT in Hall A at the San Diego Convention Center:

- ***HAE Attack Rates in Pediatric Patients 2 to <12 Years of Age with Prophylactic Berotralstat: Results from Interim Analysis of APeX-P***; Poster #L55
- ***Real-World Attack Rates Before and After Berotralstat Initiation Among Patients with Hereditary Angioedema with C1-Inhibitor Deficiency (Type I/II) Stratified by Monthly Baseline HAE Attack Frequency***; Poster #603
- ***Real-World Attack Rates Before and After Berotralstat Initiation Among Patients with Hereditary Angioedema without C1-Inhibitor Deficiency (HAE-nl-C1-INH) Stratified by Monthly Baseline HAE Attack Frequency***; Poster #607
- ***Exploring the Role of Disease Burden, Treatment Effectiveness, and Administration Preference on Willingness of Patients With HAE to Change Long-Term Prophylaxis***; Poster #608
- ***Patient-Reported Impact of Berotralstat as Long-Term Prophylaxis on Hereditary Angioedema Attack Frequency and Attack Severity***; Poster #655

The abstracts are available to view in an online supplement to *The Journal of Allergy and Clinical Immunology* (JACI) at jacionline.org.

**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, which could take longer or be more expensive than planned; the commercial viability of ORLADEYO, including its ability to achieve sustained market acceptance; the FDA or other applicable regulatory agency may require additional studies beyond the studies planned for products and product candidates, may not provide regulatory clearances which may result in delay of planned clinical trials, may impose certain restrictions, warnings, or other requirements on products and product candidates, may impose a clinical hold with respect to product candidates, or may withhold, delay, or withdraw market approval for products and product candidates; BioCryst's ability to successfully manage its growth and compete effectively; and risks related to the international expansion of BioCryst's business. 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