



BioCryst to Present New Pediatric HAE Data at the American College of Allergy, Asthma & Immunology 2025 Annual Scientific Meeting

October 23, 2025

RESEARCH TRIANGLE PARK, N.C., Oct. 23, 2025 (GLOBE NEWSWIRE) -- [BioCryst Pharmaceuticals, Inc.](#) (Nasdaq: BCRX) today announced that the company will present four abstracts at the Annual Scientific Meeting of the American College of Allergy, Asthma & Immunology (ACAAI) taking place in Orlando, Fla., from November 6-10, 2025. The data will include results from a second interim analysis of the APeX-P trial evaluating long-term prophylaxis with an oral granule formulation of once-daily ORLADEYO[®] (berotralstat) in pediatric patients with hereditary angioedema (HAE) aged 2 to <12 years as well as findings on the psychosocial impact of HAE on young patients and their caregivers.

BioCryst poster presentations include:

- **Hereditary Angioedema Attack Rates in Children 2-12 with Oral Prophylactic Berotralstat Over One Year;** ePoster #R089; Friday, November 7, 2:50–3:05 p.m. ET; Monitor #15, West Exhibit Hall
- **Psychosocial Impact of Hereditary Angioedema on Young Patients and Their Caregivers;** ePoster #R102; Friday, November 7, 2:50–3:05 p.m. ET; Monitor #16, West Exhibit Hall
- **Burden of Attack for Young Patients with Hereditary Angioedema and Their Caregivers;** ePoster #R109; Saturday, November 8, 11:30–11:45 a.m. ET; Monitor #13, West Exhibit Hall
- **Emergency Department Experiences of Young Patients with Hereditary Angioedema and Their Caregivers;** ePoster #R117; Sunday, November 9, 12:00–12:15 p.m. ET; Monitor #13, West Exhibit Hall

In addition to displaying in the exhibit hall at the noted times, ePosters will be accessible online and on demand to registered attendees on Thursday, November 6, 2025, beginning at 8:00 a.m. ET on ACAAI's website.

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 ($\geq 10\%$ 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 oral 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 BioCryst's expectations relating to pediatric patients with HAE under the age of 12. 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: interim results of a clinical trial do not necessarily predict final results; and the FDA 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 actual results to differ materially from those contained in BioCryst's forward-looking statements.

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