



BioCryst to Highlight Patient-focused Research at the 2025 US HAEA National Summit

July 9, 2025

RESEARCH TRIANGLE PARK, N.C., July 09, 2025 (GLOBE NEWSWIRE) -- [BioCryst Pharmaceuticals, Inc.](#) (Nasdaq: BCRX) today announced that the company will present new findings from patient-focused research highlighting the burden of current treatments for HAE on pediatric patients under the age of 12 and their caregivers and perspectives on the importance of HAE treatments. The findings will be presented in posters at the 2025 US HAEA National Summit in Baltimore, which is taking place July 10-13, 2025.

"For more than a decade, we have been honored to join the HAE patient community at the US HAEA National Summit. It is an opportunity for us to hear directly from them on their hopes and dreams for a brighter future so we can keep working with them and for them to try to make these dreams a reality. This is what drove us to bring the first oral, once-daily prophylactic therapy for HAE for adults and adolescents and will continue to drive us to try to do even more for this community in the future," said Jon Stonehouse, president and chief executive officer of BioCryst.

BioCryst will present the following three abstracts in a poster session on Friday, July 11 from 12:30-1:30 p.m. ET:

- ***Patient Perspectives on the Importance of HAE Treatments***; Poster #25
- ***Burden of Treatment for Pediatrics Patients (Aged Less Than 12 Years) With Hereditary Angioedema and Their Caregivers***; Poster #4
- ***HAE Attack Rates in Pediatric Patients 2 to <12 Years of Age with Prophylactic Berotralstat: Results from Interim Analysis of APeX-P***; Poster #13 (encore from AAAAI / WAO 2025)

BioCryst President and CEO Jon Stonehouse will also address members of the HAE community in attendance in a corporate presentation during the Summit.

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 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: BioCryst's ability to successfully implement or maintain its commercialization plans for ORLADEYO; the commercial viability of ORLADEYO, including its ability to achieve sustained market acceptance; interim results of a clinical trial do not necessarily predict final results; 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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