



BioCryst Highlights Real-world Data Showing ORLADEYO® (berotralstat) Consistently Reduces HAE Attacks Across All Ages

May 30, 2025

New data shared at the 14th C1-inhibitor Deficiency & Angioedema Workshop supports berotralstat use at all stages of life

RESEARCH TRIANGLE PARK, N.C., May 30, 2025 (GLOBE NEWSWIRE) -- [BioCryst Pharmaceuticals, Inc.](https://www.biocryst.com) (Nasdaq: BCRX) today announced new data which highlights the reduction in the percentage of days with hereditary angioedema (HAE) symptoms among young children initiating berotralstat in the APeX-P trial, and the broad safety and efficacy outcomes observed across all age groups of patients taking ORLADEYO to prevent HAE attacks.

"These data underscore the potential of our oral, once-daily prophylactic treatment to deliver consistent, meaningful benefits for people living with HAE across all stages of life. From young children to adolescents and adults, ORLADEYO continues to show strong efficacy and tolerability, helping patients experience more days free from attacks. These clinical and real-world results give me confidence that we can continue to have a very positive impact on advancing care for all those living with HAE, regardless of their age," said Helen Thackray, chief research and development officer at BioCryst.

The following three studies are being presented at the 14th C1-Inhibitor Deficiency & Angioedema Workshop in Budapest, Hungary from May 29–June 1, 2025.

- **Reduction in Percentage of Days with Angioedema Symptoms Among Young Children Initiating Berotralstat – Interim Results from ApeX-P**

Oral Presentation - Session IV - Saturday, May 31 - 10:30 – 12:45 (12:00-12:15)

The ongoing open-label APeX-P study is evaluating berotralstat in children aged 2 to 11 years with HAE. There were four weight-based cohorts. Cohort 1 from these interim results received 150 mg capsules daily, while cohorts 2–4 received daily oral granule doses of 108, 96, and 78 mg, respectively. The median age was 8 years (range: 3–11), with disease onset typically between ages 2–6 years. Prior to enrollment, patients reported a mean (SD) of 18.0 (20.5) missed school days annually due to HAE. While on berotralstat, at least half of patients were attack-free each month. The percentage of days with angioedema symptoms dropped from a mean (SD) of 11 percent (9.4 percent) during the standard-of-care period, measured pre-study, to 4.0 percent (4.7 percent) over 12 weeks, with this improvement sustained up to 48 weeks. The most common treatment emergent adverse events (TEAEs) were nasopharyngitis, upper respiratory tract infection and headache. These interim findings suggest berotralstat may help children with HAE experience fewer symptoms and a more normal daily life.

- **Berotralstat for the Prevention of Hereditary Angioedema Attacks: Results from the Italian Expanded Access Program**

Oral Presentation - Session VI- Sunday, June 1 - 08:00 – 09:30 (09:00-09:15)

In the Italian expanded access program (EAP), 22 patients received berotralstat free of charge until commercial availability, with an additional 12 weeks of treatment to ensure continuity. Safety was monitored throughout, and data on breakthrough attacks, disease control, and quality of life (QoL) were collected.

Among 12 patients with available data, median (mean) Angioedema Control Test (AECT) scores improved from 11.5 (12.8) at baseline to 13.0 (14.0) at month three and 16.0 (14.8) at month six, indicating better disease control. Median (mean) AE-QoL total scores decreased from 45.0 (42.3) at baseline to 40.0 (37.6) at month three and 36.2 (34.0) at month six, reflecting clinically meaningful improvements in QoL.

All 15 patients still enrolled at program closure transitioned to commercially available ORLADEYO. The treatment was associated with a consistently low HAE attack rate, improved disease control and a safety profile consistent with clinical trial findings.

- **Tolerability and Effectiveness of Berotralstat for Prophylaxis in Adolescents with Hereditary Angioedema: subgroup analysis of the Berolife Study**

Oral Presentation - Session VI - Sunday June 1 – 08:00-9:30 (08:30-08:45)

The Berolife study, a real-world observational study in France, assessed the tolerability and effectiveness of berotralstat in patients with HAE, including a subgroup of adolescents (aged 12–17) who received 150 mg once daily.

The mean (SD) baseline attack rate was 1.8 (1.1) attacks/month (median: 1.8), based on the six months prior to enrolment. After six months of treatment, the median monthly attack rate decreased to 0.55 (from 2.25 at baseline), with similar reductions sustained at 12 and 18 months. TEAEs in adolescents were consistent with those seen in the overall Berolife population and in the APeX-2 and APeX-S clinical trials.

These findings support berotralstat as an effective and well-tolerated long-term prophylactic option for adolescents with HAE.

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 future results, performance or achievements and statements relating to ORLADEYO performance and effectiveness. 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; interim results of a clinical trial do not necessarily predict final results; 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; and BioCryst's ability to successfully manage its growth and compete effectively. 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.

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