BioCryst Presents New Clinical Data and Real-World Evidence Demonstrating Impact of HAE Portfolio at the 2026 European Academy of Allergy and Clinical Immunology Annual Meeting

June 12, 2026

— Growing body of ORLADEYO® clinical data and real-world evidence demonstrates consistent reductions in HAE attack burden and healthcare utilization across diverse patient populations

— New post hoc analysis of Phase 1b/2 ALPHA-STAR study of investigational navenibart demonstrates consistent reductions in HAE attack rates across patient subgroups, supporting ongoing Phase 3 evaluation as a potential long-acting therapeutic option for the broad HAE population

RESEARCH TRIANGLE PARK, N.C., June 12, 2026 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq: BCRX) today announced new clinical data and real-world evidence for ORLADEYO® (berotralstat), the first and only targeted oral prophylactic therapy for patients with hereditary angioedema (HAE) aged 2 and older, in addition to new data from the Phase 1b/2 multicenter, dose-ranging, open-label ALPHA-STAR study of navenibart, an investigational, long-acting, monoclonal antibody plasma kallikrein inhibitor for prophylaxis to prevent attacks of HAE. These data will be featured across multiple poster presentations during the European Academy of Allergy and Clinical Immunology (EAACI) Annual Meeting in Istanbul, Turkey, from June 12-15.

"Together, these data reinforce the strength of our HAE portfolio, demonstrating consistent real-world impact for children and adults living with HAE today, while advancing next-generation treatment approaches to further improve outcomes, address ongoing clinical unmet need, and align with patient treatment preferences," said Sandeep Menon, Chief Research and Development Officer of BioCryst.

New HAE Portfolio Clinical Data

Updated analysis of 48-week data from the ongoing APeX-P study, the largest trial of long-term prophylaxis (LTP) in pediatric patients with HAE, evaluating once-daily ORLADEYO in HAE patients aged 2 to <12 years will be featured in a poster presentation (Poster D2.498).

Analysis of 48-week trial data showed that treatment with ORLADEYO was associated with early and sustained reductions in rate and number of HAE attacks requiring on-demand treatment and professional care, with results as follows:

- The median (range) adjusted HAE attack rate requiring on-demand treatment decreased from 0.691 attacks/month (0-5.03) during the 12-week standard of care (SOC) period to 0.169 attacks/month (0-1.75) during the 48-week ORLADEYO treatment period.
- The number of HAE attacks requiring professional care decreased from 22 during the 12-week SOC period to 3 over 12 weeks of ORLADEYO treatment; this was sustained throughout the treatment period, with a trended decrease in level of professional care required from emergency department or urgent care treatment to physicians' office, and further reduction to 0 attacks during Week 37-48.
- No significant safety concerns were identified over the 48-week treatment period.

Additionally, a new post hoc analysis of the Phase 1b/2 ALPHA-STAR study of navenibart evaluating clinical outcomes across patient subgroups defined by baseline attack rate, body mass index (BMI), and age will be featured as a poster presentation (Poster D3.438).

The analysis demonstrated that investigational navenibart consistently reduced HAE attacks, with results as follows:

- Reductions in overall HAE attack rate were observed across subgroups defined by baseline attack rate, BMI, and age.
- Following treatment, reductions in clinically relevant HAE outcomes were observed across analyzed subgroups, including reductions in moderate or severe attacks, and in baseline attack rate subgroup analyses, and reduced on-demand medication use.
    - Reductions in the number of moderate or severe attacks were also observed with treatment across all BMI subgroups.
- Navenibart was previously shown to be well tolerated with no severe or serious treatment-emergent adverse events (TEAEs) reported and few injection site reactions. The most common TEAEs were headache, nasopharyngitis, and urinary tract infection.

Together with the primary findings from Phase 1b/2 ALPHA-STAR, this analysis supports the ongoing Phase 3 evaluation of navenibart in the ALPHA-ORBIT trial as a potential long-acting therapeutic option for the broad HAE population.

Real-World Impact of ORLADEYO

Through a comprehensive real-world evidence (RWE) generation program, BioCryst continues its commitment to generating meaningful, practice-informing research to demonstrate the value of long-term prophylaxis with ORLADEYO in routine clinical practice across the diverse HAE patient community. In addition to clinical data generated in clinical trials, RWE across multiple studies demonstrated ORLADEYO's ability to achieve sustained reductions in HAE burden. Benefits were observed in adolescent and adult patients, including those switching to ORLADEYO from other LTP

therapies, and translated into fewer HAE attacks, reduced healthcare resource utilization, and high patient satisfaction.

This RWE will be presented in the following posters:

- ***Real-World Patient Characterization, Prior Long-Term Prophylactic Prescribing Patterns, and Treatment Outcomes for Adults on Berotralstat with Hereditary Angioedema in Japan***; poster D1.407; Friday, June 12, 12:00–13:00 p.m. (TRT)
- ***Reductions in Hereditary Angioedema Attacks among Patients with C1 Esterase Inhibitor Deficiency Who Switched from Another Long-Term Prophylaxis to Berotralstat***; poster D2. 360; Saturday, June 13, 12:00–13:00 p.m. (TRT)
- ***Hereditary Angioedema Attack Rates among Patients with Normal C1 Esterase Inhibitor Before and After Switching from Another Long-Term Prophylaxis to Berotralstat***; poster D2. 357; Saturday, June 13, 12:00–13:00 p.m. (TRT)
- ***Reductions in Healthcare Resource Utilization in Adolescents with Hereditary Angioedema on Berotralstat;*** poster D2.361; Saturday, June 13, 12:00–13:00 p.m. (TRT)
- ***Hereditary Angioedema Attack Frequency and Severity According to Individuals Taking Berotralstat for Long-Term Prophylaxis***; poster D3.324; Sunday, June 14, 12:15–13:15 p.m. (TRT)

Visit www.ORLADEYO.com for more information.

About APeX-P
APeX-P is an ongoing, open-label study evaluating the pharmacokinetics, safety, and efficacy of ORLADEYO in patients aged 2 to <12 years with HAE due to C1-inhibitor deficiency. Before ORLADEYO initiation, patients received SOC for 12 weeks. The rates of HAE attacks requiring on-demand treatment and number of attacks requiring professional care were compared between 12 weeks of SOC and 48 weeks of ORLADEYO treatment. Participants (n=29) were placed in one of four cohorts by body weight at baseline.

About ALPHA-STAR
ALPHA-STAR is a Phase 1b/2, multicenter, dose-ranging, proof-of-concept, open-label trial that assessed the safety and clinical activity of single- and multiple-dose navenibart in adults aged 18 years and older with HAE due to C1-inhibitor deficiency. Eligible participants were those who experienced at least two HAE attacks during the 8-week trial run-in period. Participants (n=29) were placed in three navenibart dose cohorts and were pooled for the purpose of this post hoc analysis. Post hoc outcomes included the overall change from baseline in monthly HAE attack rate, the rate of attacks treated with on-demand medication, and the rate of moderate or severe attacks. Outcome measures were assessed for 6 months after the last dose. Due to small sample size and short-term study design, these data may not fully represent the broader population of patients 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 2 years and older. One dose of ORLADEYO per day works to prevent HAE attacks by decreasing the activity of plasma kallikrein.

About navenibart
Navenibart is an investigational YTE-modified monoclonal antibody inhibitor of plasma kallikrein, an established and safe mechanism, currently being evaluated in clinical trials for long-term prevention of HAE attacks with potential best-in-class dosing every 3 or 6 months.

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 2 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 doses of ORLADEYO higher than the prescribed once-daily dose are not recommended due to the potential for QTc interval prolongation.

IMPORTANT SAFETY INFORMATION
An increase in QTc interval was observed in adults at dosages higher than 150 mg once daily and was concentration dependent.

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

In adult and pediatric patients aged 12 years and older with moderate or severe hepatic impairment (Child-Pugh B or C), the recommended dosage of ORLADEYO capsules is 110 mg once daily with food. In pediatric patients aged 2 to <12 years with moderate or severe hepatic impairment (Child-Pugh B or C), avoid use of ORLADEYO.

Berotralstat is a substrate of P-glycoprotein (P-gp) and breast cancer resistance protein. P-gp inducers may decrease berotralstat plasma concentration, leading to reduced efficacy of ORLADEYO. Avoid concomitant use of P-gp inducers with ORLADEYO.

ORLADEYO at a dose of 150 mg is a moderate inhibitor of CYP2D6 and CYP3A4. Concomitant use of ORLADEYO with CYP2D6 or CYP3A4 substrates can increase exposure of the CYP2D6 or CYP3A4 substrates and may increase the risk of adverse reactions associated with the substrates. If ORLADEYO is concomitantly used with CYP2D6 or CYP3A4 substrates where minimal increases in the concentration of the substrates may lead to serious adverse reactions, closely monitor or modify the dosage of the CYP2D6 or CYP3A4 substrate.

The safety and effectiveness of ORLADEYO in pediatric patients <2 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.

About BioCryst Pharmaceuticals
BioCryst is a global biotechnology company focused on developing and commercializing medicines for hereditary angioedema ("HAE") and other rare diseases, driven by its deep commitment to improving the lives of people living with these conditions. BioCryst has commercialized ORLADEYO® (berotralstat), the first oral, once-daily plasma kallikrein inhibitor, and is advancing a pipeline of potential first-in-class or best-in-class oral small-molecule and injectable protein therapeutics for a range of rare diseases. 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 new clinical trial data, real-world outcomes and expectations with respect to BioCryst's HAE portfolio and the potential dosing profile, competitive positioning, and expectations for navenibart. 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: risks related to the development and interpretation of clinical and real-world data; BioCryst's ability to successfully implement or maintain its commercialization plans for ORLADEYO and successfully commercialize future products; BioCryst's ability to successfully progress its development plans for navenibart; 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; ongoing and future clinical development of product candidates, including navenibart, may take longer than expected and may not have positive results; the FDA or other applicable regulatory agencies may require additional studies beyond the studies planned for navenibart, may not provide regulatory clearances which may result in delay of planned clinical trials, may not review regulatory filings on our expected timeline, may impose certain restrictions, warnings, or other requirements, may impose a clinical hold, or may withhold, delay or withdraw market approval, 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, or may impose certain restrictions, warnings, or other requirements; and risks related to the expected dosing or best-in-class profile of navenibart. This list is not exclusive. To see a more comprehensive set of risks, 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.

BCRXW

Contact:

Investors:
investorrelations@biocryst.com

Media:
media@biocryst.com