BioCryst to Present New Real-World Evidence Showing Significant Reductions in Medical Visits and Hospitalizations After Starting ORLADEYO® (berotralstat)

October 14, 2024

RESEARCH TRIANGLE PARK, N.C., Oct. 14, 2024 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq: BCRX) today announced new real-world evidence on the use of oral, once-daily ORLADEYO® (berotralstat) demonstrating that patients with hereditary angioedema (HAE) in the United States experience significant reductions in healthcare resource utilization (HRU), including significant reductions in hospitalizations, emergency room visits and use of on-demand therapies, after beginning treatment with ORLADEYO.

The study will be presented in a poster at the Academy of Managed Care Pharmacy (AMCP) Nexus 2024 national meeting, which is being held in Las Vegas from October 14-17, 2024.

“Our interrogation of claims data builds upon previously reported findings demonstrating that ORLADEYO not only has a favorable impact on the lives of patients with HAE – but the broader healthcare system, as well. Here, our analysis reveals that significant reductions in healthcare resource utilization are achieved across multiple outcomes – such as reductions in hospitalizations and medical visits, including those related to HAE attacks. Decreases in on-demand treatment were also observed. Taken together, our investigation provides promising real-world evidence which supports ORLADEYO’s clinical and financial value as a prophylactic therapy for HAE,” said Sandra Christiansen, MD, professor of medicine and director of translational research at the US HAEA Angioedema Center at the University of California, San Diego.

The poster *Healthcare Resource Utilization among Patients Initiating Berotralstat for the Long Term Prophylaxis of Hereditary Angioedema in the United States* (#D20) detailed findings from a retrospective pre-post study that featured analysis of administrative U.S. claims data of patients with HAE in the United States. The analysis focused on eligible patients enrolled in commercial and public health plans who initiated ORLADEYO between December 2020 and December 2022 who had a baseline of at least six months of continuous health plan enrollment prior to starting ORLADEYO (n=260).

- Significant reductions in HRU were observed in the overall study population following initiation of ORLADEYO ($p<0.05$), including in:
    - All-cause hospitalizations (34 percent reduction) and outpatient or emergency room visits (14 percent reduction).
    - Angioedema-related hospitalizations (52 percent reduction) and outpatient or emergency room visits (44 percent reduction).
- Significant reductions in HAE attack-related HRU were also observed ($p<0.05$), including in:
    - HAE attack-related visits (51 percent reduction), driven by significant decreases in hospitalization (60 percent) and outpatient or emergency room visits (50 percent).
    - Reduction in HAE attack-related visits were observed when stratified by body location of the attack, including in the head and upper airways (48 percent reduction), gastrointestinal system (58 percent reduction) and unspecified locations (52 percent reduction).
    - Additionally, a decrease in use of on-demand therapies administered by a healthcare professional was observed (39 percent reduction) among patients who previously received on-demand treatment.

The poster will be on display in the expo hall of the MGM Grand Hotel and Convention Center on Tuesday, October 15, from 5:00-7:00 pm PT and will be presented on Wednesday, October 16, from 11:30 am-1:00 pm PT.

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 complement-mediated 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 future results, performance or achievements and statements relating to ORLADEYO performance, including with respect to ORLADEYO's impact on healthcare resource utilization. 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.

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