BioCryst Presents New Real-world Evidence Showing Significant Reductions in Healthcare Resource Utilization Among Patients with HAE Following Initiation of ORLADEYO® (berotralstat)

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RESEARCH TRIANGLE PARK, N.C., May 09, 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) after beginning treatment with ORLADEYO.

The study was presented in a poster at the 2024 International Society for Pharmacoeconomics and Outcomes Research conference (ISPOR), which was held in Atlanta from May 5-8, 2024.

"The data presented here represent the first documentation that prescribing ORLADEYO significantly reduces healthcare resource utilization. This outcome provides a compelling argument that ORLADEYO, which is known to be effective in reducing HAE morbidity, is also a cost-effective strategy for patient management. By demonstrating the collective benefit of ORLADEYO therapy for patients and payers alike, this study represents an important step towards the goal of a normal life for patients with 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.

Reductions in Real-World Healthcare Resource Utilization Among United States Hereditary Angioedema (HAE) Patients Following Berotralstat Initiation (Poster #EE477)

The poster detailed findings from a retrospective real-world study that featured analysis of administrative claims data of patients with HAE in the United States. The analysis focused on eligible patients who initiated ORLADEYO between December 2020 and December 2022 who had a baseline of a minimum of six months of continuous health plan enrollment prior to starting ORLADEYO, including commercial and public health plans.

In addition to the overall study population (n=260), results were stratified by two subgroups: patients who had a history of being treated with HAE long-term prophylaxis (LTP) (n=126) and patients who were naïve to LTP but had a history of receiving on-demand treatment (n=67). Average (median) follow-up duration was 12 (13) months for the overall study population, 13 (13) months for LTP treatment-experienced patients, and 13 (14) for LTP treatment-naïve patients with prior on-demand treatment.

- 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 were also observed in both subgroups ($p<0.05$), including in:
  - Angioedema-related hospitalizations (54 percent reduction) and outpatient or emergency room visits (37 percent reduction) among LTP-experienced patients
  - Angioedema-related hospitalizations (62 percent reduction) and outpatient or emergency room visits (45 percent reduction) among LTP-naïve patients with a history of receiving on-demand treatment

"We continue to showcase the value of ORLADEYO with each new real-world study we conduct. Here, we build on previously reported real-world outcomes that have shown patients who are treated with our oral, once-daily prophylactic treatment for HAE can maintain – and improve – control of their disease regardless of various baseline characteristics, including prior prophylaxis. We look forward to reporting additional real-world evidence that highlights the benefits associated with long-term prophylaxis with ORLADEYO," said Dr. William Sheridan, chief development officer and interim chief medical officer of BioCryst.

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 the potential treatment effects, cost savings or other potential benefits of ORLADEYO. 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 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 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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