NICE Recommends BioCryst's ORLADEYO® (berotralstat), the First Oral, Once-daily Therapy, to Prevent Attacks in HAE Patients in the UK

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RESEARCH TRIANGLE PARK, N.C., Sept. 15, 2021 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq: BCRX) today announced that the United Kingdom's (UK) National Institute for Health and Care Excellence (NICE) has recommended ORLADEYO ® (berotralstat) for preventing recurrent attacks of hereditary angioedema (HAE) in eligible patients 12 years and older if they have at least two attacks per month. With this recommendation, HAE patients in England, Wales and Northern Ireland will have access to the first oral, once-daily therapy for routine prevention of recurrent HAE attacks.

"HAE is a very rare genetic condition which is at best painful and debilitating, and can be fatal if left untreated. The unpredictability of the condition severely affects quality of life for patients and their families," said Laura Szutowicz, chief executive officer of HAE UK. "HAE UK welcomes the NICE decision on berotralstat, which means that eligible patients and clinicians have another choice of treatment for this lifelong condition."

"We are excited for HAE patients that this recommendation from NICE provides access to the first oral, once-daily treatment for UK patients to achieve symptom control and experience relief from the burdens of HAE. The positive NICE recommendation also expands access to modern prophylaxis with ORLADEYO, compared to the attack frequency requirements from NICE for injectable options," said Charlie Gayer, chief commercial officer of BioCryst.

The decision follows European Commission (EC) marketing authorization of ORLADEYO in April 2021 and approval from the UK's Medicines and Healthcare products Regulatory Agency (MHRA) in May 2021. A decision from the Scottish Medicines Consortium (SMC) for use of ORLADEYO for HAE patients in Scotland under the UK's National Health Service (NHS) is anticipated in the first half of 2022.

The NICE recommendation was based on findings from the Phase 3 APeX-2 trial, in which ORLADEYO met its primary endpoint, significantly reducing HAE attacks vs placebo at 24 weeks. This reduction was sustained through 96 weeks, with an 80 percent average reduction in patients' mean attack rate per month during weeks 25-96 of the trial, compared to baseline, as demonstrated in long-term data presented at the European Academy of Allergy and Clinical Immunology (EAACI) Hybrid Congress 2021. ORLADEYO was generally well-tolerated during the treatment period with fewer drug-related adverse events reported in part 3 (weeks 49-96) as compared to part 1 (weeks 0-24) and part 2 (weeks 25-48).

"The impact of HAE on patients goes beyond the potentially life-threatening swellings. It can also have a long-term effect on patients' self-esteem and quality of life," said Dr. Sorena Kiani, consultant immunologist at Barts Health NHS Trust. "The NICE recommendation of berotralstat is great news for clinicians and eligible patients who now have access to the first oral preventive treatment for HAE that could significantly reduce the number of attacks and may improve quality of life."

The UK Summary of Product Characteristics (SPC) and Patient Information Leaflet (PIL) for ORLADEYO are available from the MHRA website at https://products.mhra.gov.uk/.

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) and in patients taking chronically administered P-glycoprotein (P-gp) or breast cancer resistance protein (BCRP) inhibitors (eg, cyclosporine).

Berotralstat is a substrate of P-gp and BCRP. 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 discovers novel, oral, small-molecule medicines that treat rare diseases in which significant unmet medical needs exist and an enzyme plays a key role in the biological pathway of the disease. Oral, once-daily ORLADEYO® (berotralstat) is approved in the United States, the European Union, Japan, the United Arab Emirates and the United Kingdom. BioCryst has several ongoing development programs including BCX9930, an oral Factor D inhibitor for the treatment of complement-mediated diseases, BCX9250, an ALK-2 inhibitor for the treatment of fibrodysplasia ossificans progressiva, and galidesivir, a potential treatment for Marburg virus disease and Yellow Fever. RAPIVAB® (peramivir injection) has received regulatory approval in the U.S., Canada, Australia, Japan, Taiwan and Korea. Post-marketing commitments for RAPIVAB are ongoing. For more information, please visit the company's website at www.biocryst.com.

Forward-Looking Statements
This press release contains forward-looking statements, including statements regarding BioCryst's plans and expectations for 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 those 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: the ongoing COVID-19 pandemic, which could create challenges in all aspects of BioCryst's business, including without limitation delays, stoppages, difficulties, and increased expenses with respect to BioCryst's and its partners' development, regulatory processes and supply chains, negatively impact BioCryst's ability to access the capital or credit markets to finance its operations, or have the effect of heightening many of the risks described below or in the documents BioCryst files periodically with the Securities and Exchange Commission; BioCryst's ability to successfully implement its commercialization plans for, and to commercialize, ORLADEYO, which could take longer or be more expensive than planned; risks relating to government actions, including that decisions and other actions relating to pricing and reimbursements may not be taken when expected or at all, or that the outcomes of such decisions and other actions may not be in line with BioCryst's current expectations; the commercial viability of ORLADEYO, including its ability to achieve market acceptance; the FDA, EMA, MHRA 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; risks related to the international expansion of BioCryst's business; and actual financial results may not be consistent with expectations, including that revenue, operating expenses and cash usage may not be within management's expected ranges. 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, all of 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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Investors:
John Bluth
+1 919 859 7910
jbluth@biocryst.com

Media:
Catherine Collier Kyroulis
+1 917 886 5586
ckyroulis@biocryst.com