Early Access to BioCryst's Berotralstat Granted for HAE Patients in France

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For investors and media only

RESEARCH TRIANGLE PARK, N.C., March 11, 2021 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq: BCRX) today announced that the French National Agency for Medicines and Health Products Safety (ANSM) has granted an Autorisation Temporaire d'Utilisation de cohorte (cohort ATU), or Temporary Authorization for Use, for the use of berotralstat to prevent attacks of hereditary angioedema (HAE) in appropriate patients aged 12 and older.

This cohort ATU allows patients with HAE in France to receive treatment with berotralstat before the drug is granted marketing authorization by the European Commmission (EC).

According to the the French Code of Public Health, a cohort ATU is dedicated to high unmet medical needs in serious diseases and covers medicinal products of which the efficacy and safety of use are strongly presumed and intended for a group or sub-group of patients treated and monitored in accordance with criteria defined in a protocol for therapeutic use and collection of information.

"HAE is a debilitating and potentially deadly disease so the early access to a new treatment option for patients is exciting news," said Dr. Laurence Bouillet, head of internal medicine, University Hospital Grenoble, Coordinating Reference Centre for HAE in France (CREAK).

"The cohort ATU represents BioCryst's second early access program approved in Europe and provides patients in France with faster access to treatment," said Jon Stonehouse, chief executive officer of BioCryst.

On February 25, 2021, the company announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has adopted a positive opinion recommending the approval of ORLADEYO™ (berotralstat) for routine prevention of recurrent attacks of hereditary angioedema (HAE) in adult and adolescent patients aged 12 years and older.

The EC will review the CHMP recommendation and a final approval decision from the EC on the marketing authorization application (MAA) for ORLADEYO is expected in the second quarter.

About ORLADEYO ™ (berotralstat)

ORLADEYO™ (berotralstat) is the first and only oral therapy designed specifically to prevent attacks of hereditary angioedema (HAE) in adults 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 and Japan for the prevention of HAE attacks in adults and pediatric patients 12 years and older, and under regulatory review for approval in the European Union and 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), a viral neuraminidase inhibitor for the treatment of influenza, 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 use of ORLADEYO (berotralstat) under the cohort ATU. 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; the commercial viability of ORLADEYO, including its ability to achieve market acceptance; the EMA or other applicable regulatory agency may require additional studies beyond the studies planned for 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 product candidates, may impose a clinical hold with respect to such product candidates, or may withhold market approval for such 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 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, which identify important factors that could cause actual results to differ materially from those contained in BioCryst's forward-looking statements.

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