BioCryst Reports 96-week Data from APeX-2 Showing ORLADEYO®(berotralstat) Reduced HAE Attack Rate by 80 Percent from Baseline

July 10, 2021

—Multiple data presentations at the European Academy of Allergy and Clinical Immunology (EAACI) Hybrid Congress 2021 highlight durability of effect and long-term safety of ORLADEYO—

RESEARCH TRIANGLE PARK, N.C., July 10, 2021 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq: BCRX) today announced that hereditary angioedema (HAE) patients who were randomized to receive 150 mg of oral, once-daily ORLADEYO® (berotralstat) at the start of the APeX-2 trial had an 80 percent average reduction in their mean attack rate per month during weeks 25-96 of the trial, compared to baseline. Median attack rates also decreased from 2.7 attacks/month at baseline to 0.0 attacks per month in 16 of 17 months through the same period.

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). Eighty-one percent of the patients who entered part 3 completed the trial.

“The long-term data we now see from two years of therapy in the clinical program reinforces the substantial, sustained reduction in HAE attacks patients experienced with ORLADEYO. These results are consistent with the experience many patients are having in the real world since we launched ORLADEYO, which is a key driver for the strong patient demand we are seeing as patients switch from injectable prophylactic agents and injectable acute-only therapies to oral, once-daily ORLADEYO for control of their HAE attacks,” said Dr. William Sheridan, chief medical officer of BioCryst.

The data are scheduled to be presented in an oral presentation on Monday, July 12, 2021 at 2:15pm CET at the EAACI Hybrid Congress 2021.

Additional Presentations of New ORLADEYO Data at EAACI

On-demand medication use was reduced in HAE patients treated with ORLADEYO (150 mg) in APeX-2

- In HAE patients taking oral, once-daily ORLADEYO 150 mg who had a ≥50 percent reduction in their rate of investigator-confirmed attacks relative to their baseline attack rate, there was a 78 percent reduction in the use of on-demand medication (doses/month) from baseline to week 24, leading to 2.1 fewer doses of on-demand medication per month. In patients who had a ≥70 percent reduction in their rate of investigator-confirmed attacks relative to their baseline attack rate, there was an 85 percent reduction in the use of on-demand medication (doses/month) from baseline to week 24, leading to 2.2 fewer doses of on-demand medication per month.
- The data were presented in poster #250 at the EAACI Hybrid Congress 2021.

ORLADEYO demonstrated consistently low attack rates in adolescent patients in APeX-S

- In an analysis of adolescent patients (ages 12-17) treated with oral, once-daily ORLADEYO 150 mg in the open-label safety study, APeX-S, the mean (SEM) attack rate at week 4 was 0.4 attacks/month, which was generally sustained through week 48.
- Median attack rates in these adolescents were 0.0 attacks/month throughout the 48 weeks of treatment.
- Greater than 70 percent of patients were attack-free in weeks 4 to 48.
- ORLADEYO was generally well-tolerated in APeX-S.
- The data were presented in poster #336 at the EAACI Hybrid Congress 2021.

ORLADEYO demonstrated consistently low HAE attack rates during COVID-19

- Stress is a documented trigger for HAE attacks and recently published physician and patient survey data shows an increase in patient-reported HAE attack rates due to an increase in stress related to the COVID-19 pandemic (pre-COVID: 1.5 attacks/3-months vs during COVID: 4.4 attacks/3-months).[1]
- In an analysis of HAE patients taking oral, once-daily ORLADEYO 150 mg in the APeX-S trial, monthly HAE attack rates, pre-COVID and during COVID, remained consistently low, <1 attack/month, for patients treated with ORLADEYO. Patients receiving ORLADEYO maintained low attack rates during this time of high societal stress and disruption.
- The data were presented in poster #166 at the EAACI Hybrid Congress 2021.

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, the European Union, Japan and the United Kingdom for the prevention of HAE attacks in adults and pediatric patients 12 years and older. 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 future results, performance or achievements. 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: 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 periodically files 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 FDA, EMA, PMDA 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 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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[1] Soteres DF, et al. Update on the impact of COVID-19 in the care of patients with Hereditary Angioedema (HAE): Results of a Patient and HCP Survey. Presented at Academy of Managed Care Pharmacy; April 12-16, 2021; Virtual. Abstract D21.