BioCryst Presents Data Showing Sustained Attack Rate Reductions, Improved Patient Satisfaction and Quality of Life for HAE Patients Taking Berotralstat in APeX-2 Trial

November 13, 2020

—Data presented at 2020 Annual Scientific Meeting of the American College of Allergy, Asthma & Immunology (ACAAI)—

RESEARCH TRIANGLE PARK, N.C., Nov. 13, 2020 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq:BCRX) today presented new clinical data that further evaluates the attack rate reductions, patient satisfaction and quality of life of hereditary angioedema (HAE) patients in the APeX-2 trial over 48 weeks. Berotralstat is an investigational treatment for the prevention of attacks in patients with HAE.

The data from three abstracts, including a Distinguished Industry Oral Abstract, are being presented at the 2020 Annual Scientific Meeting of the American College of Allergy, Asthma & Immunology (ACAAI), which is being conducted virtually from November 13-15.

“Presenting all available treatment options to patients is an important part of HAE clinical management. These data continue to demonstrate the potential of berotralstat as a prophylactic medication, if approved by the FDA, with sustained reduction in attacks and meaningful improvements in quality of life seen over 48 weeks of treatment. With its oral, once-daily administration, berotralstat would offer patients a therapeutic alternative for managing this chronic disease,” said H. James Wedner, M.D., the Dr. Phillip and Arleen Korenblat Professor of Medicine at Washington University School of Medicine in St. Louis, and lead author of the Distinguished Industry Oral Abstract.

Following is a brief description of the clinical data posters being presented at ACAAI.

***Berotralstat Reduces Attacks in Patients with Hereditary Angioedema (HAE): APeX-2 Trial 48 Week Results*;** Distinguished Industry Oral Abstract, Session A, Friday, November 13, 4:30-5:30 p.m. CT

Patients treated with oral, once-daily berotralstat 150 mg for 48 weeks experienced a sustained reduction in mean investigator confirmed HAE attack rates through month 12.

In patients re-randomized to berotralstat 150 mg after 24 weeks on placebo, there was a marked reduction in investigator-confirmed HAE attack rates over 24 weeks of treatment. These patients had a mean attack rate per month of 2.5 at baseline, 1.7 at month six (while on placebo), 0.6 at month seven (one month after starting berotralstat 150 mg) and 0.6 at month 12 (six months after starting berotralstat 150 mg).

Berotralstat was generally well-tolerated in APeX-2 through 48 weeks. The safety profile observed from weeks 24 to 48 was consistent with the data observed through the first 24 weeks. The most commonly reported treatment-related adverse events were upper respiratory tract infection, abdominal pain, diarrhea and vomiting.

***Berotralstat Positively Impacts Patient-Reported Satisfaction: Results from the Phase 3 APeX-2 trial*;** Poster #158

Patient satisfaction with treatment was assessed using the validated Treatment Satisfaction Questionnaire for Medicine (TSQM), which is comprised of three specific scales (side effects, effectiveness and convenience) and is scored on a global satisfaction scale from 0-100.

HAE patients who transitioned from placebo to berotralstat 150 mg at week 24 reported improved overall treatment satisfaction and effectiveness. These patients experienced statistically significant improvements from weeks 24 to 48, with a mean global satisfaction increase of 26 points ($p=0.005$) and a mean effectiveness increase of 29.6 points ($p<0.001$). Convenience scores remained high through week 48, reflecting the positive experiences patients had taking an oral medication.

Berotralstat Improves Patient-Reported Quality of Life Through 48 Weeks in the Phase 3 APeX-2 Trial; Poster #154

Quality of life was assessed with the Angioedema Quality-of-Life (AE-QoL) questionnaire, a validated tool to measure impairment of QoL based on a total and domain (functioning, fatigue/mood, fear/shame and nutrition) scores. The minimal clinically important difference (MCID) is defined as an improvement of six points.

Clinically meaningful improvements in mean AE-QoL total scores were observed as early as week four, with a mean improvement from baseline of 15 points at week 24. This improvement was sustained through 48 weeks of treatment with berotralstat 150 mg.

Improvements were observed in all four domains (functioning, fatigue/mood, fear/shame, nutrition) through week 48. Notably, 77 percent of patients exceeded the MCID in total AE-QoL total scores at 48 weeks, indicating the reduction in attacks following berotralstat therapy appears to have a positive impact on patients’ quality of life.

About the APeX-2 Trial
In APeX-2, 121 eligible HAE type 1 or type 2 patients were randomized 1:1:1 to oral, once daily berotralstat 110 mg or 150 mg or placebo for 24 weeks. At 24 weeks, patients initially randomized to 110 mg or 150 mg of berotralstat continued on that dose. Patients who initially received placebo were re-randomized 1:1 at week 24 to receive either berotralstat 110 mg or 150 mg.

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. BioCryst has several ongoing development programs including ORLADEYO ™

(berotralstat), an oral treatment for hereditary angioedema, BCX9930, an oral Factor D inhibitor for the treatment of complement-mediated diseases, galidesivir, a potential treatment for COVID-19, Marburg virus disease and Yellow Fever, and BCX9250, an ALK-2 inhibitor for the treatment of fibrodysplasia ossificans progressiva. RAPIVAB® (peramivir injection), a viral neuraminidase inhibitor for the treatment of influenza, is BioCryst's first approved product and has received regulatory approval in the U.S., Canada, Australia, Japan, Taiwan, Korea and the European Union. 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 BioCryst's actual results, performance or achievements to be materially different from any future results, performances 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 files periodically with the Securities and Exchange Commission; developing and commercializing ORLADEYO or any HAE product candidate may take longer or may be more expensive than planned; BioCryst may not be able to enroll the required number of subjects in planned clinical trials of product candidates; BioCryst may not advance human clinical trials with product candidates as expected; the FDA, EMA, PMDA 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 product candidates; product candidates, if approved, may not achieve market acceptance; BioCryst's ability to successfully commercialize its product candidates, 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 2020 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.

BCRXW

Contact:
John Bluth
+1 919 859 7910
jbluth@biocryst.com

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