BioCryst's Oral Factor D Inhibitor (BCX9930) Shows High Potency and Specificity for Alternative Pathway of Complement

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—Data presented at the 62nd American Society of Hematology Annual Meeting—

RESEARCH TRIANGLE PARK, N.C., Dec. 06, 2020 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq: BCRX) today announced that preclinical data on BCX9930, an oral Factor D inhibitor under development as monotherapy for paroxysmal nocturnal hemoglobinuria (PNH) and other complement-mediated diseases, showed complete in vitro blockade of both hemolysis of PNH erythrocytes and accumulation of C3 fragments on PNH erythrocytes, indicating that BCX9930 monotherapy has the potential to inhibit both intravascular and extravascular hemolysis.

In the study, BCX9930 was highly specific for the alternative pathway and, after oral dosing of BCX9930 in primates, alternative pathway activity was completely suppressed.

The data were presented at the 62nd American Society of Hematology (ASH) Annual Meeting being held as a virtual event, December 5-8, 2020.

"These data demonstrate that BCX9930 is a highly potent and specific orally bioavailable Factor D inhibitor with potential for treatment of patients with PNH and other alternative pathway mediated diseases," said Dr. William Sheridan, chief medical officer of BioCryst.

"The preclinical results have translated as expected into the clinic, where we have seen oral monotherapy with BCX9930 drive rapid and dose-dependent reductions in key biomarkers, including LDH, and increase hemoglobin levels in PNH patients. We look forward to completing our proof of concept study in PNH and progressing into advanced development next year across multiple hematology and nephrology complement-mediated diseases," Sheridan added.

About BCX9930
Discovered by BioCryst, BCX9930 is a novel, oral, potent and selective small molecule inhibitor of Factor D currently in Phase 1 clinical development for the treatment of complement-mediated diseases. The U.S. Food and Drug Administration (FDA) has granted both Fast Track status and Orphan Drug designation for BCX9930 in PNH. In an ongoing dose ranging trial of BCX9930 in patients with PNH, BCX9930 was safe and well tolerated, with no drug-related serious adverse events. As a Factor D inhibitor, BCX9930 is designed as an oral monotherapy that can address both intravascular and extravascular hemolysis in PNH patients. Treatment-naïve PNH patients who have received more than six weeks of therapy at a monotherapy dose of 400 mg bid showed rapid and dose-dependent reductions in key biomarkers, including LDH, and increases in hemoglobin levels that were maintained without transfusions.

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 for the prevention of HAE attacks in adults and pediatric patients 12 years and older, and under regulatory review for approval in Japan and the European Union. BioCryst has several ongoing development programs including, 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, 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; ongoing and future preclinical and clinical development of BCX9930 may not have positive results; 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.

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