



BioCryst to Present Data at the 2020 ASH Annual Meeting

November 12, 2020

RESEARCH TRIANGLE PARK, N.C., Nov. 12, 2020 (GLOBE NEWSWIRE) -- [BioCryst Pharmaceuticals, Inc.](https://www.biocryst.com) (Nasdaq: BCRX) today announced that preclinical data on BCX9930, an oral Factor D inhibitor, will be presented at the 62nd American Society of Hematology (ASH) Annual Meeting being held as a virtual event, December 5-8, 2020.

The abstract is available on the ASH website at www.hematology.org.

- *Preclinical Characterization of BCX9930, a Potent Oral Complement Factor D Inhibitor, Targeting Alternative Pathway-Mediated Diseases Including Paroxysmal Nocturnal Hemoglobinuria (PNH)*; Poster #1680, Sunday, December 6, 2020, 10:00 a.m. – 6:30 p.m. ET

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. 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; 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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