



## BioCryst's Oral Factor D Inhibitor, BCX9930, Shows Clinical Benefit as Monotherapy Through 400 mg bid in Treatment-naïve PNH Patients

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RESEARCH TRIANGLE PARK, N.C., Sept. 30, 2020 (GLOBE NEWSWIRE) -- [BioCryst Pharmaceuticals, Inc.](http://www.biocryst.com) (Nasdaq:BCRX) today announced new data from treatment-naïve (no prior treatment with C5 inhibitors) paroxysmal nocturnal hemoglobinuria (PNH) patients receiving doses through 400 mg bid of its oral Factor D inhibitor, BCX9930, as monotherapy in an ongoing dose-ranging trial.

Oral BCX9930 is driving rapid and dose-dependent reductions in key biomarkers, including LDH, and increasing hemoglobin levels in all PNH patients in the trial. Increases in hemoglobin levels were maintained without transfusions.

BCX9930 has been safe and well tolerated at all doses in the trial. No drug-related serious adverse events have been reported.

"We are thrilled with the clinical benefits and safety profile of oral BCX9930 monotherapy that we continue to see in PNH patients with dosing up to 400 mg bid. With these excellent results, we plan to initiate advanced development trials next year in multiple complement-mediated hematology and nephrology diseases," said Dr. William Sheridan, chief medical officer of BioCryst.

The FDA has granted both Fast Track status and Orphan Drug Designation to BCX9930 for PNH. BioCryst has confirmed meetings with regulators in the 4<sup>th</sup> quarter of 2020 to discuss the advanced development program for BCX9930.

### **Updated Data Through 400 mg bid**

- All seven PNH patients in the trial were severely ill, with pre-treatment LDH from 3.8 to 11 × ULN, indicating active hemolysis. Four patients had a history of compromised bone marrow function, and two had thrombotic, lung or kidney complications from PNH.
- New data from the four treatment-naïve PNH patients who have received more than six weeks of therapy at 400 mg bid show significant clinical benefits for these patients.
  - Hemoglobin levels increased by a mean of 3.8 g/dL from baseline. These increases are being maintained without transfusions.
  - Three of four patients have responded with hemoglobin levels >11 g/dL to date. Hemoglobin in the fourth patient, who has compromised bone marrow function from aplastic anemia PNH, responded with an increase from 6 g/dL at baseline to 9.5 g/dL on treatment.
  - In all four patients, the size of the PNH red blood cell clone approached that of the PNH granulocyte clone, indicating near-complete control of complement-mediated hemolysis. The mean relative (red blood cells/granulocytes) PNH red blood cell clone size increased from 48 percent at baseline to 94 percent on treatment.
  - All four patients have shown reductions in LDH. Three of four patients show average serial LDH of <1.5 × ULN. In the fourth patient, the LDH has decreased from a pretreatment baseline of 11 × ULN to 2.2 × ULN on treatment to date.
  - All four patients have continued on therapy with BCX9930 based on the investigators' assessment of clinical benefit.
- The most common adverse event was mild to moderate headache lasting one to three days. One patient had a mild rash that resolved after continued BCX9930 dosing at 100 mg bid. One patient had a mild rash at 200 mg bid that is resolving during uninterrupted dosing after dose escalation to 400 mg bid.

In addition to the ongoing dose-ranging trial in treatment-naïve PNH patients, the company plans to report data from PNH patients with an inadequate response to C5 inhibitors receiving at least 400 mg bid of BCX9930 by the end of 2020.

Additional details can be found on slides, which can be accessed at the Investors' section of BioCryst's website at <http://www.biocryst.com>.

### **Conference Call and Webcast**

BioCryst management will host a conference call and webcast at 8:30 a.m. ET today to discuss the new data. The live call may be accessed by dialing 877-303-8027 for domestic callers and 760-536-5165 for international callers and using conference ID # 6798951. A live webcast of the call and any slides will be available online at the investors section of the company website at [www.biocryst.com](http://www.biocryst.com). A telephone replay of the call will be available by

dialing 855-859-2056 for domestic callers or 404-537-3406 for international callers and entering the conference ID #6798951.

### **About the Alternative Pathway and Complement-Mediated Diseases**

The complement system is part of the body's natural immune system and is responsible for helping the body eliminate microbes and damaged cells. Once activated, the complement system stimulates inflammation, phagocytosis and cell lysis. Excessive or uncontrolled activation of the complement system can cause severe, and potentially fatal, immune and inflammatory disorders. Patients with these diseases currently have no approved treatments or are limited to treatment with repeated intravenous infusions.

The alternative pathway is constantly active and provides a critical amplification loop for all three pathways (alternative, lectin, classical) of the complement system, regardless of the initiating mechanism. Factor D is an essential enzyme, and the first enzyme, in the alternative pathway, making Factor D an attractive target to address complement-mediated diseases.

### **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. 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 ALK2 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](http://www.BioCryst.com).

### **Forward-Looking Statements**

This press release contains forward-looking statements, including statements regarding BioCryst's plans for its BCX9930 program. These statements involve known and unknown risks, uncertainties and other factors which may cause actual results and developments of such program 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 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; development may be more expensive than expected; and the FDA 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 a clinical hold with respect to such product candidates, or may withhold market approval for product candidates. 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 and developments to differ materially from those contained in BioCryst's forward-looking statements.

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