



BioCryst Reports Data From Phase 1 Trial of BCX9930 and Announces Plans to Advance Program Into Proof of Concept Study in PNH Patients

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—Phase 1 data showed rapid, sustained and >95% inhibition of alternative pathway of complement system with 100 mg of oral BCX9930 BID—

—BCX9930 safe and generally well tolerated—

—Part 1 SAD and part 2 MAD data support advancement of program into part 3 proof of concept (PoC) study in PNH patients; data from PNH PoC study expected in 1H 2020—

—Company to host conference call at 8:30 a.m. ET—

RESEARCH TRIANGLE PARK, N.C., Oct. 28, 2019 (GLOBE NEWSWIRE) -- [BioCryst Pharmaceuticals, Inc.](http://www.biocryst.com) (Nasdaq: BCRX) today announced results from an ongoing three part Phase 1 trial of BCX9930, an oral Factor D inhibitor discovered and developed by BioCryst for the treatment of complement-mediated diseases, in 72 healthy volunteers. BCX9930 was safe and generally well tolerated, and showed rapid, sustained and >95% suppression of the alternative pathway (AP) of the complement system at 100 mg every 12 hours, as measured by the AP Wieslab[®] assay.

In part 1 of the trial, a single ascending dose (SAD) assessment, six cohorts of healthy volunteers received a single dose of 10 mg, 30 mg, 100 mg, 300 mg, 600 mg or 1200 mg of oral BCX9930 or placebo (each SAD cohort randomized 6:2). In part 2 of the trial, the multiple ascending dose (MAD) assessment, two cohorts of healthy volunteers received 50 mg or 100 mg of oral BCX9930 or placebo (each MAD cohort randomized 10:2) administered every 12 hours for seven days. Healthy volunteers in the MAD cohorts were prophylactically dosed with the broad-spectrum antibiotic, amoxicillin/clavulanate.

BCX9930 was safe and generally well tolerated at all doses studied. There were no serious adverse events. A clinically benign rash was observed in some healthy volunteers in the MAD (two in the 50 mg cohort, seven in the 100 mg cohort), which was self-limited and resolved in 4-8 days after onset. There were no discontinuations from the trial.

Based on the safety, tolerability, PK and PD dose-response results from parts 1 and 2 of the Phase 1 trial, the company plans to complete additional MAD dosing cohorts and advance to part 3 of the trial, a proof of concept (PoC) study of BCX9930 in paroxysmal nocturnal hemoglobinuria (PNH) patients who are poor responders to eculizumab or ravulizumab, and treatment-naïve patients. The company expects to report data from the PoC study in PNH patients in the first half of 2020.

Additional data from the Phase 1 trial of BCX9930 can be found in slides at the investors section of the company website at www.biocryst.com.

"We are excited that BioCryst's oral Factor-D inhibitor, BCX9930, was safe, generally well-tolerated and demonstrated rapid, strong, and sustained suppression of the alternative pathway. Based on these excellent data from the SAD/MAD cohorts, we look forward to advancing to part 3 of the trial, a proof of concept study in PNH patients, and reporting data in the first half of 2020," said Dr. William Sheridan, chief medical officer of BioCryst.

About Complement-Mediated Diseases

The complement system is part of the body's natural immune system and is responsible for helping the body eliminate microbes (including viral and bacterial infections) and damaged cells. It is comprised of proteins which are primarily produced in the liver and circulate in the blood. 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.

The complement system comprises biological cascades of amplifying enzyme cleavages involving more than 30 proteins and protein fragments, and may be activated through three pathways: the classical pathway (initiated by antibody-antigen complexes), the lectin pathway (initiated by lectin binding) and the alternative pathway (initiated by microbial surfaces).

The alternative pathway also provides a critical amplification loop for all three pathways, regardless of the initiating mechanism. Factor D is an essential enzyme in the alternative pathway, thus 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. Patients with complement-mediated diseases, many of which can cause death or severe morbidity, currently either have no treatments available, or are limited to repeated intravenous infusion treatments. In parts 1 and 2 of a Phase 1 SAD/MAD assessment, BCX9930 was safe and generally well tolerated and, at 100 mg BID, showed rapid, sustained and >95% suppression of the alternative pathway of the complement system. Preclinical data showed that BCX9930 was a potent and specific inhibitor of Factor D. Based on results from parts 1 and 2 of the Phase 1 trial in healthy volunteers, the company plans to complete additional multiple ascending dose cohorts while advancing to part 3 of the trial, a proof of concept study in PNH patients, which the company plans to report data from in the first half of 2020.

Conference Call and Webcast

BioCryst management will host a conference call and webcast at 8:30 a.m. ET today to discuss the Phase 1 trial of BCX9930, and next steps for the program. 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 # 1691557. 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. 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 # 1691557.

About BioCryst Pharmaceuticals

BioCryst 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 BCX7353, an oral treatment for hereditary angioedema; BCX9930, an oral Factor D inhibitor for the treatment of complement-mediated diseases; galidesivir, a potential treatment for Marburg virus disease and Yellow Fever, and a preclinical program to develop oral ALK-2 inhibitors 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: that the development of BCX9930 may take longer or may be more expensive than planned; that ongoing and future preclinical and clinical development of BCX9930 may not advance as expected, enroll the required number of subjects or have positive results; that further analysis of the current data or from additional data from the study may yield results which are different from our current view, that the FDA, EMA or other applicable regulatory agency may not agree with our interpretation, may require additional studies beyond the studies planned, may not provide regulatory clearances, may impose a clinical hold or may withhold market approval with respect to BCX9930. 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 projections and forward-looking statements.

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