



Biocryst Begins Enrollment of Phase 1 Trial of BCX9930, an Oral Factor D Inhibitor for Complement-Mediated Diseases

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—Data expected in Q4 2019—

—BCX9930 showed high potency, specificity, suppression of hemolysis and wide safety margin in preclinical studies—

RESEARCH TRIANGLE PARK, N.C., June 27, 2019 (GLOBE NEWSWIRE) -- [BioCryst Pharmaceuticals, Inc.](#) (Nasdaq: BCRX) today announced that the company has begun enrollment of a Phase 1 trial of BCX9930, an oral Factor D inhibitor discovered and developed by BioCryst, for the treatment of complement-mediated diseases.

The objectives of the trial are to evaluate the safety and tolerability of single and multiple ascending doses of BCX9930 in healthy subjects and to characterize the pharmacokinetic and pharmacodynamic profiles of BCX9930 in single and multiple ascending doses of BCX9930 in healthy subjects.

The company expects to report data from the trial in the fourth quarter of 2019.

"An oral Factor D inhibitor would meet a significant unmet medical need for patients with many complement-mediated diseases. The preclinical profile of BCX9930 showed high potency, excellent specificity for Factor D, complete suppression of complement-mediated hemolysis after oral dosing, and a wide safety margin, so we look forward to seeing the clinical results from this Phase 1 trial," said Dr. William Sheridan, chief medical officer of BioCryst.

"We are very excited to begin enrollment of our Phase 1 trial of BCX9930, another proprietary BioCryst oral medicine for rare diseases. With complement-mediated diseases, we can rapidly gather important data from our Phase 1 trial regarding the effect on the complement system, and we will use these results to direct our plans to generate proof of concept data in PNH patients next year," said Jon Stonehouse, chief executive officer of BioCryst.

Preclinical Profile of BCX9930

- In preclinical oral dosing studies, BCX9930 completely suppressed complement-mediated hemolysis in laboratory assays of complement activity.
- In preclinical in-vitro studies of red blood cells from patients with paroxysmal nocturnal hemoglobinuria (PNH), BCX9930 completely suppressed complement-mediated hemolysis, and completely blocks the deposition of C3 fragments on PNH red blood cells.
- In preclinical in-vitro studies, BCX9930 demonstrated significant activity at low drug concentrations in several well-established complement activity assays.
- In preclinical in-vitro assays, BCX9930 was more potent on Factor D by approximately 200-fold to more than 3000-fold compared with other serine protease enzymes outside the complement pathway.
- In preclinical oral dosing studies, drug exposure increased in proportion to dose, and high drug levels were achieved.
- In preclinical in-vivo safety pharmacology and toxicology studies, drug concentrations at the no observed adverse event level (NOAEL) doses of BCX9930 were more than 500-fold greater than the estimated therapeutic target level.

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. The company plans to report results from the ongoing Phase 1 trial in the fourth quarter of 2019.

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 developing 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 the FDA, EMA or other applicable regulatory agency 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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