



BioCryst Begins Proof of Concept Trial in PNH Patients With Oral Factor D Inhibitor, BCX9930

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Data from proof of concept trial expected in 2Q 2020

RESEARCH TRIANGLE PARK, N.C., March 05, 2020 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq: BCRX) today announced the first patients have been dosed with BCX9930 in a proof of concept trial in paroxysmal nocturnal hemoglobinuria (PNH). These patients were naïve to eculizumab and ravulizumab.

BCX9930 is an oral Factor D inhibitor discovered and developed by BioCryst for the treatment of complement-mediated diseases. The company expects to report data from the proof of concept study in PNH patients in the second quarter of 2020.

"In PNH, our goal for BCX9930 is to develop a safe, well-tolerated, oral monotherapy with excellent efficacy, whether or not patients have ever been treated with a C5 inhibitor," said Dr. William Sheridan, chief medical officer of BioCryst.

"Based on the outstanding safety and PK/PD profile we have seen with BCX9930 in healthy subjects, and the historically excellent translation of complement markers into clinical results in PNH patients, we are confident that we will demonstrate proof of concept in PNH, and other alternative pathway complement-mediated diseases," Sheridan added.

Because Factor D is essential for alternative pathway overactivity in PNH, and all other complement diseases of the alternative pathway, successful proof of concept data with BCX9930 would enable BioCryst to advance the program across other target indications.

Study Design

In this proof of concept study of the safety and effectiveness of oral BCX9930, up to 16 PNH patients will receive BCX9930 twice daily (BID) for 28 days. Patients in the first cohort will receive 50 mg BID for 14 days, then 100 mg BID for 14 days, for a total of 28 days. For patients in the second cohort, the planned dosing regimen is 200 mg BID for 14 days, followed by 400 mg BID for 14 days. Each cohort of up to eight patients can enroll up to four treatment-naïve patients and up to four poor responders to eculizumab or ravulizumab. Key markers of effectiveness to be evaluated include levels of LDH, hemoglobin and reticulocytes. BID treatment will continue for subjects who show benefit, such as improvement in LDH levels or hemoglobin. Safety will be monitored clinically and with laboratory tests.

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.

Complement-mediated diseases can cause death or severe morbidity. Patients with these diseases currently have no approved treatments, or are limited to treatment with repeated intravenous infusions.

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 parts 1 and 2 of a Phase 1 SAD/MAD assessment, BCX9930 was safe and generally well tolerated, with no serious adverse events, and no safety signals in routine laboratory monitoring. A benign rash that resolved within a median of five days of onset was observed in the majority of MAD subjects. At 100 mg BID, BCX9930 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.

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 berotralstat (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 BCX9250, an oral ALK-2 inhibitor for the treatment of fibrodysplasia ossificans progressiva (FOP). 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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