BioCryst Begins Enrollment in Proof-of-Concept Trial to Confirm Safe, Effective, Once-daily Dose of Oral Factor D Inhibitor, BCX10013

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RESEARCH TRIANGLE PARK, N.C., Oct. 26, 2023 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq: BCRX) today announced the enrollment of the first patient in a proof-of-concept clinical trial evaluating BCX10013, a potential once-daily, oral Factor D inhibitor for the treatment of complement-mediated diseases.

The goal of this proof-of-concept trial is to understand the preliminary efficacy and safety profile of once-daily dosing with BCX10013. If the results in patients living with paroxysmal nocturnal hemoglobinuria (PNH) confirm its potential for a best-in-class profile, the company plans to advance into a pivotal program in patients living with renal complement-mediated diseases, including IgA nephropathy (IgAN).

"BioCryst is committed to developing first-in-class or best-in-class medicines, and an oral Factor D inhibitor with once-daily dosing that has an excellent safety profile and strong efficacy would be differentiated and address significant unmet needs of patients living with complement-mediated diseases. By studying BCX10013 in patients living with PNH, we plan to efficiently evaluate alternative pathway activity with well-defined biomarkers, such as LDH and hemoglobin, to determine if we have a safe, effective once-daily dose that meets our criteria to move forward into a pivotal program," said Dr. Ryan Arnold, chief medical officer of BioCryst.

The trial is an open-label, multicenter dose escalation study to evaluate the safety, tolerability, pharmacokinetics, pharmacodynamics and therapeutic potential of BCX10013 in up to 15 adult patients with PNH and is being conducted in countries that do not have access to standard-of-care complement inhibitor therapy. The trial design employs intra-subject dose escalation approximately every four weeks with the option to continue treatment for up to 24 weeks. The primary endpoints of the trial are incidence of treatment-emergent adverse events and graded laboratory abnormalities, and changes from baseline in laboratory analytes, vital signs, electrocardiograms and physical examination findings. Select secondary endpoints to evaluate efficacy include change from baseline in lactate dehydrogenase (LDH), PNH red blood cell to white blood cell clone size ratio, hemoglobin level and proportion of patients who are transfusion-free, among other biomarkers.

In addition to BCX10013, which is designed to target Factor D in the alternative pathway, BioCryst is pursuing medicines directed at other targets across the classical, lectin and terminal pathways of the complement system. The goal of the company's overall complement program is to advance several potential best-in-class compounds across multiple pathways in the complement system to treat many complement-mediated diseases.

For more information about the trial, visit ClinicalTrials.gov and search NCT number NCT06100900.

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. Oral, once-daily ORLADEYO® (berotralstat) is approved in the United States and many global markets. BioCryst has active programs to develop oral medicines for multiple targets across the complement system, including BCX10013, an oral Factor D inhibitor in clinical development. RAPIVAB® (peramivir injection) is approved in the U.S. and multiple global markets, with post-marketing commitments 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 our plans and expectations for our complement program and other future results. 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, performance 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 BCX10013 and other product candidates may take longer than expected and 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 or other applicable regulatory agency may require additional studies beyond the studies planned for products and 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 products and product candidates, may impose a clinical hold with respect to product candidates, or may withhold, delay or withdraw market approval for products and product candidates; product candidates, if approved, may not achieve market acceptance; BioCryst's ability to successfully 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 revenue, 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 actual results to differ materially from those contained in BioCryst's forward-looking statements.

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