BioCryst Reports Initial Clinical Data with Oral Factor D Inhibitor BCX10013 Supporting Development as a Once-daily Treatment for Complement-mediated Diseases

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—Company also expanding its discovery platform in complement-mediated diseases, including potent, selective, oral molecules targeting C2—

RESEARCH TRIANGLE PARK, N.C., Jan. 09, 2023 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq: BCRX) today announced that initial data from ongoing phase 1 single ascending dose (SAD) and multiple ascending dose (MAD) trials in healthy volunteers show rapid, sustained and >97 percent suppression of the alternative pathway (AP) of the complement system 24 hours following a single 110 mg dose. BCX10013 has been safe and generally well-tolerated at all doses studied to date. These data support the development of BCX10013 as a potential best-in-class, once-daily, oral Factor D inhibitor for multiple complement-mediated diseases.

BioCryst has initiated plans to advance BCX10013 into patient studies in mid-2023, including in patients with paroxysmal nocturnal hemoglobinuria (PNH), to evaluate once-daily dosing.

The company expects to confirm the optimal dosing for pivotal trials by the end of the year, move directly into a pivotal trial in patients with immunoglobulin A nephropathy (IgAN), and rapidly expand into pivotal trials in additional indications.

"We remain committed to bringing better options to patients with complement-mediated diseases and we are excited to see the immediate and durable effect of BCX10013 in suppressing the alternative pathway. Our next step is to gather data from a small number of patients, utilizing the excellent biomarkers in PNH, to quickly confirm optimal clinical dosing this year. We then plan to rapidly advance the program into pivotal trials in multiple complement-mediated diseases, beginning next year with IgAN. We believe BCX10013 has the potential to be a best-in-class treatment option with an oral, once-daily profile," said Dr. Helen Thackray, chief research and development officer at BioCryst.

In the SAD assessment to date, cohorts of healthy volunteers received a single dose of 1 mg, 3 mg, 10 mg, 40 mg, 80 mg or 110 mg of oral BCX10013 or placebo. In the MAD assessment to date, cohorts of healthy volunteers received 20 mg, 40 mg or 80 mg of oral BCX10013 or placebo administered once daily for seven days (20 mg cohort) or 14 days (40 mg and 80 mg cohorts).

Following single BCX10013 dose administration, the onset of AP inhibition occurred within one hour and increased in a dose-dependent manner. At 110 mg, the highest dose studied to date, AP activity was suppressed by a mean of 97.8 percent at 24 hours post-dose. Suppression of AP activity by BCX10013 was assessed using the AP Wieslab® assay, which measures functional activity of the complement system. In the MAD studies with once-daily dosing, exposure to BCX10013 was approximately dose proportional over the studied dose range and steady-state was achieved in 7 to 14 days with modest accumulation.

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

Expanding Programs in Complement-mediated Diseases

In addition to BCX10013, which targets Factor D in the alternative pathway of complement, BioCryst is pursuing oral medicines directed at other targets across the classical, lectin and terminal pathways of the complement system, including C2, a critical upstream serine protease enzyme for activation of the classical and lectin pathways. The company has developed potent, selective molecules targeting C2, which are currently in lead optimization.

"We believe that our ability to develop oral medicines as monotherapy against targets across multiple complement pathways, in addition to the alternative pathway, could allow us to help more patients with distinctly different complement-mediated rare diseases. With our oral approach, BioCryst also has the opportunity to develop combination therapies to improve therapeutic options for those diseases affecting multiple pathways of the complement system," Thackray added.

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, 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, 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 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 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 product candidates, may impose a clinical hold with respect to product candidates, or may withhold or delay market approval for 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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Investor Contact:
John Bluth
+1 919 859 7910
jbluth@biocryst.com

Media Contact:
Catherine Collier Kyroulis
+1 917 886 5586
ckyroulis@biocryst.com