# BioCryst Begins Patient Enrollment in RENEW Proof-of-Concept Trial Evaluating BCX9930 for Patients with Renal Complement-mediated Diseases

February 22, 2022

RESEARCH TRIANGLE PARK, N.C., Feb. 22, 2022 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq: BCRX) today announced the enrollment of the first patient in the RENEW proof-of-concept basket study with its oral Factor D inhibitor, BCX9930, in patients with C3 glomerulopathy (C3G), immunoglobulin A nephropathy (IgAN) and primary membranous nephropathy (PMN).

RENEW is an open-label, multicenter, proof-of-concept study designed to evaluate the safety, tolerability and therapeutic potential of BCX9930 (500 mg bid) administered for 24 weeks in approximately 42 adult patients (14 patients in each disease) with either C3G, IgAN or PMN. All patients will be enrolled into one of three parallel study cohorts, based on confirmation of diagnosis and disease activity in a recent kidney biopsy, and will receive BCX9930 for the 24-week treatment period. The primary endpoint of RENEW is percent change from baseline in 24-hour urine protein-to-creatinine ratio (uPCR), as assessed at week 24. For more information about RENEW, visit ClinicalTrials.gov and search NCT number NCT05162066.

“There are currently no targeted treatments available for patients living with these rare renal diseases, which underscores the importance of the RENEW study,” said Dr. William Sheridan, chief medical officer of BioCryst. “We believe an oral Factor D inhibitor could be a groundbreaking therapeutic option for nephritis conditions that are driven by the alternative pathway of complement. Coupled with our recent initiations of the REDEEM-1 and REDEEM-2 pivotal trials in PNH, we are excited to advance our BCX9930 clinical program across four separate complement-mediated diseases.”

BioCryst recently announced it has begun enrolling patients in the REDEEM-1 and REDEEM-2 pivotal trials evaluating BCX9930 for patients with PNH. REDEEM-1 is a randomized, open-label, active comparator-controlled study of the efficacy and safety of oral BCX9930 monotherapy in PNH patients with an inadequate response to a C5 inhibitor. REDEEM-2 is a randomized, placebo-controlled study to evaluate the efficacy and safety of oral BCX9930 as monotherapy versus placebo in PNH patients not currently receiving complement inhibitor therapy.

BioCryst plans to further advance and expand its Factor D program over the next two years by achieving the following:

- Complete and report data from REDEEM-1 and REDEEM-2
- Prepare to submit regulatory approval filings in PNH
- Complete the RENEW PoC basket trial and advance to pivotal trials in C3G, IgAN and PMN
- Commence PoC trials in other complement-mediated diseases

**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, the European Union, Japan, the United Arab Emirates and the United Kingdom. BioCryst has several ongoing development programs including BCX9930, an oral Factor D inhibitor for the treatment of complement-mediated diseases, BCX9250, an ALK-2 inhibitor for the treatment of fibrodysplasia ossificans progressiva, and galidesivir, a potential treatment for Marburg virus disease and Yellow Fever. RAPIVAB® (peramivir injection) has received regulatory approval in the U.S., Canada, Australia, Japan, Taiwan and Korea. 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 BioCryst’s plans and expectations for its BCX9930 program. These statements involve known and unknown risks, uncertainties and other factors which may cause 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 periodically files with the Securities and Exchange Commission; ongoing and future preclinical and clinical development of BCX9930 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 commercialize its products and product candidates, 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, which identify important factors that could cause the actual results to differ materially from those contained in BioCryst’s forward-looking statements.

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