BioCryst Begins Patient Enrollment in REDEEM-2 Pivotal Trial Evaluating BCX9930 as Oral Monotherapy for Patients with PNH

November 29, 2021

RESEARCH TRIANGLE PARK, N.C., Nov. 29, 2021 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq: BCRX) today announced the enrollment of the first patient in the REDEEM-2 pivotal trial with its oral Factor D inhibitor, BCX9930, in patients with paroxysmal nocturnal hemoglobinuria (PNH).

REDEEM-2 is a randomized, placebo-controlled trial evaluating the efficacy and safety of BCX9930 (500 mg bid) as monotherapy versus placebo in approximately 57 PNH patients not currently receiving complement inhibitor therapy. In part 1 of this trial, patients will be randomized 2:1 to receive BCX9930 or placebo under double-blind conditions for 12 weeks. All patients will receive BCX9930 in part 2 (weeks 13-52) to assess the long-term safety, tolerability and effectiveness of BCX9930, with patients randomized to placebo in part 1 switching to BCX9930 at the week 12 visit. The primary endpoint of REDEEM-2 is change from baseline in hemoglobin, as assessed at week 12.

“There are important unmet needs among patients living with PNH based on the current standard of care, specifically related to efficacy and burden of therapy. As an oral monotherapy with proof-of-concept data showing control of both intravascular and extravascular hemolysis, BCX9930 has the potential to help patients live their lives with more freedom from their disease,” said Dr. William Sheridan, chief medical officer of BioCryst.

“Beginning patient enrollment in our REDEEM-2 pivotal trial marks a critical step forward as BCX9930 advances closer to registration for patients living with PNH,” Sheridan added.

REDEEM-2 is powered at 90 percent to detect a difference in mean change from baseline of hemoglobin of ≥ 2.15 g/dL at 12 weeks. In a dose-ranging trial of BCX9930 in treatment-naïve patients, the company previously reported that BCX9930 (at doses of 400 mg or 500 mg bid) increased hemoglobin from baseline by a mean of 3.7 g/dL at week 12 and eliminated transfusions. BCX9930 was safe and generally well-tolerated in the trial.

BioCryst also is preparing to enroll patients in the REDEEM-1 pivotal trial, a randomized, open-label, active, comparator-controlled comparison of the efficacy and safety of BCX9930 (500 mg bid) monotherapy in approximately 81 PNH patients with an inadequate response to a C5 inhibitor. Additionally, the company is initiating a proof-of-concept trial of BCX9930 in renal complement-mediated diseases.

The U.S. Food and Drug Administration has granted both Fast Track status and Orphan Drug Designation to BCX9930 for PNH. For more information about REDEEM-2, visit ClinicalTrials.gov and search NCT number NCT05116787.

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.

BCRXW

Investor Contact:
John Bluth
+1 919 859 7910
jbluth@biocryst.com

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