BioCryst Resumes Enrollment in BCX9930 Clinical Program

August 4, 2022

—U.S. Food and Drug Administration lifts partial clinical hold—

RESEARCH TRIANGLE PARK, N.C., Aug. 04, 2022 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq:BCRX) today announced that the U.S. Food and Drug Administration (FDA) has lifted its partial clinical hold on the BCX9930 program. The company will resume enrollment in global clinical trials under revised protocols at a reduced dose of 400 mg twice daily of BCX9930. This includes the REDEEM-1 and REDEEM-2 pivotal trials in patients with paroxysmal nocturnal hemoglobinuria (PNH) and the RENEW proof-of-concept trial in patients with C3 glomerulopathy (C3G), immunoglobulin A nephropathy (IgAN) and primary membranous nephropathy (PMN).

Clinical evidence and recent laboratory studies have informed the company's hypothesis that crystals form in the kidneys of some patients. The company believes that lowering the dose to 400 mg and ensuring adequate hydration will dilute the concentration of drug in the urine below the threshold where crystals can form.

On April 8, 2022, BioCryst announced that the company had voluntarily paused enrollment in BCX9930 clinical trials while it investigated observed elevations in serum creatinine (SCr) seen in some patients at the 500 mg twice daily dose. The FDA subsequently placed the program on a partial clinical hold. Patients already enrolled in the trials and demonstrating clinical benefit were able to continue on therapy.

"Now that the FDA has lifted the partial clinical hold, we can take the next step toward our goal of finding a safe and effective dose for BCX9930. We expect this can be accomplished in a reasonable time frame after resuming enrollment, in a relatively small number of patients given the rate and timing of the SCr rises in patients prior to the enrollment pause. If we are successful, we will invest more significantly in BCX9930 to tap the full potential of reaching many patients suffering from a number of alternative pathway diseases, and, if we are not successful, we will stop investment in BCX9930 and move on to other molecules in our pipeline," said Jon Stonehouse, president and chief executive officer of BioCryst.

PNH patients naive to C5 inhibitor therapy who received 400 mg twice daily in the Phase 1 clinical program (n=7) experienced a mean change from baseline increase in hemoglobin of 4.3 g/dL and required no transfusions, and no patients experienced an elevation in their SCr during dose-ranging at the 400 mg twice daily dose. All observed cases of SCr elevations occurred at 500 mg twice daily.

About the BCRX9930 Clinical Trial Program

REDEEM-1 is a randomized, open-label, active comparator-controlled comparison of the efficacy and safety of BCX9930 monotherapy in approximately 81 PNH patients with an inadequate response to a C5 inhibitor. In part 1 of this trial, patients who have not had an adequate response to a C5 inhibitor will be randomized 2:1 to discontinue their C5 inhibitor and receive BCX9930 as monotherapy or to continue receiving their C5 inhibitor for 24 weeks. All patients will receive BCX9930 in part 2 (weeks 25-52) to assess the long-term safety, tolerability and effectiveness of BCX9930. Patients who are randomized to C5 inhibitor therapy in part 1 will discontinue that therapy at the week 24 visit and start BCX9930 for part 2. The primary endpoint of REDEEM-1 is change from baseline in hemoglobin, as assessed at weeks 12 to 24.

REDEEM-2 is a randomized, placebo-controlled trial evaluating the efficacy and safety of BCX9930 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.

RENEW is an open-label, multicenter, proof-of-concept study designed to evaluate the safety, tolerability and therapeutic potential of BCX9930 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.

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 multiple global markets. 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) 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.

Conference Call and Webcast

As previously announced, BioCryst management will host a conference call and webcast at 8:30 a.m. ET today to discuss its second quarter financial results and provide a corporate update, including updates on the BCX9930 clinical program. The live call may be accessed by dialing 866-374-5140 for domestic callers and 404-400-0571 for international callers and using conference ID 68509725#. A live webcast of the call and any slides will be available online at the investors section of the company website at www.biocryst.com. A replay of the call will be available on the company website.

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 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 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; and 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 or market approval for product candidates. 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.

BCRXW

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

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