BioCryst Announces Designs for REDEEM-1 and REDEEM-2 Pivotal Trials with BCX9930 as Oral Monotherapy for Patients with PNH

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RESEARCH TRIANGLE PARK, N.C., July 15, 2021 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq: BCRX) today announced the designs for REDEEM-1 and REDEEM-2, two upcoming pivotal trials with its oral Factor D inhibitor, BCX9930, in patients with paroxysmal nocturnal hemoglobinuria (PNH).

REDEEM-1 is 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. 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 as monotherapy for part 2.

REDEEM-2 is a randomized, placebo-controlled trial to evaluate 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 for both trials is the change from baseline in hemoglobin (Hb), assessed at weeks 12 to 24 in REDEEM-1 and at week 12 in REDEEM-2.

Key secondary endpoints for both trials are the proportion of subjects who are transfusion-free, the number of units of packed red blood cells (pRBC) transfused and change from baseline in FACIT-Fatigue score. In REDEEM-2 the percent change from baseline in lactate dehydrogenase also is a key secondary endpoint. Other secondary endpoints in both trials include: percent reduction in the rate of pRBC units transfused, change from baseline in total PNH RBC clone size, ratio of total PNH RBC clone size to PNH white blood cell clone size, reticulocyte count and the proportion of subjects with Hb ≥12 g/dL. In REDEEM-1, reduction in C3 opsonization of red blood cells also is a secondary endpoint.

Trial site start-up activities are now underway at sites around the world and both pivotal trials are expected to begin enrolling patients in the second half of 2021.

In a dose-ranging trial of BCX9930, the company has previously reported that BCX9930 (at doses of 400 mg or 500 mg bid) increased hemoglobin from baseline by a mean of 3.2 g/dL in C5 inadequate response patients and 3.5 g/dL in treatment-naïve patients and reduced or eliminated transfusions in PNH patients. BCX9930 was safe and generally well-tolerated in the trial.

“Based on the excellent control of both intravascular and extravascular hemolysis we have seen with BCX9930 in PNH patients in the clinical program to date, we have advanced rapidly into PNH pivotal trials with BCX9930. Our goal is to achieve a broad indication for BCX9930 as oral monotherapy for patients with PNH,” said Dr. William Sheridan, chief medical officer of BioCryst.

The U.S. Food and Drug Administration has granted both Fast Track status and Orphan Drug Designation to BCX9930 for PNH.

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 and the United Kingdom for the prevention of HAE attacks in adults and pediatric patients 12 years and older. 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), a viral neuraminidase inhibitor for the treatment of influenza, 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,

EMA, 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 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 the actual results to differ materially from those contained in BioCryst's forward-looking statements.

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