BioCryst Announces Positive Results from Its Ongoing BCX4208 Phase 2B Study in Patients with Gout

- ***Confirmation of favorable BCX4208 safety profile through 24 weeks***
- ***Proportion of patients reaching serum uric acid goal sustained at 24 weeks***
- ***BioCryst to host a conference call & webcast Monday, January 9 at 10:00 a.m. ET***

RESEARCH TRIANGLE PARK, N.C.--(BUSINESS WIRE)-- BioCryst Pharmaceuticals, Inc. (NASDAQ:BCRX) today announced long-term results from the extension phase of its randomized Phase 2b study of BCX4208 added to allopurinol in patients with gout who had failed to reach the serum uric acid (sUA) therapeutic goal of < 6 mg/dL on allopurinol alone. The results of this 24-week, blinded safety extension confirm that BCX4208 was generally safe and well-tolerated, and sustained sUA control over time. Patients generated healthy immune responses to a vaccine challenge at 16 or 20 weeks of BCX4208 treatment. Following the successful outcome of this 24-week analysis, BioCryst is preparing for end of Phase 2 regulatory discussions to take place in the coming months.

In the original 12-week study, 279 patients were randomized and 160 patients entered the extension phase. Patients continued their blinded, randomized therapy of BCX4208 at doses of 5 mg, 10 mg, 20 mg, 40 mg and placebo once-daily. Allopurinol 300 mg once-daily was administered in all study arms.

This longer-term safety profile of BCX4208 is consistent with the 12-week primary analysis results originally reported in October 2011. BCX4208 added to allopurinol was generally safe and well-tolerated at all doses studied, and responses to vaccines indicated healthy immune function. The types and rates of adverse events through 24 weeks, including infections, were similar between the groups treated with BCX4208 and placebo. No opportunistic or unusual infections were observed.

The previously observed lymphocyte plateau reached by 12 weeks of treatment remained unchanged in the 5 mg, 10 mg and 20 mg BCX4208 arms through 24 weeks. The 40 mg study arm met a protocol-defined cohort stopping rule based on the number of withdrawals for CD4+ cell counts, and this arm was discontinued after week 24. No patients from the placebo, 5 mg or 10 mg cohorts discontinued study drug for confirmed reductions of lymphocyte or CD4+ cell counts below certain protocol-specified thresholds; through 24 weeks, a total of four patients were discontinued from the 20 mg group and eleven patients from the 40 mg group for reductions in CD4+ cell counts.

A healthy immune response was seen in all study arms in a vaccine challenge sub-study conducted in 84 patients. The vaccines were administrated at either 16 or 20 weeks of treatment, and responses were assessed by measuring changes in antibody titers 4 weeks later. The response rates to tetanus toxoid (50%-100%) and polyvalent pneumococcal vaccine (64%-67%) in patients treated with BCX4208 were similar to placebo-treated patients who received tetanus toxoid (50%) and pneumococcal vaccine (64%). The response rates for placebo-treated patients are consistent with responses in normal individuals reported in literature.

The approximate doubling of sUA response rates with BCX4208 seen at 12 weeks was sustained through 24 weeks of treatment. After 24 weeks of treatment, BCX4208 doses of 5 mg, 10 mg, 20 mg and 40 mg/day showed response rates of 40%, 50%, 46% and 55% respectively, compared to 25% for placebo. These results are consistent with the previously reported positive findings at the 12-week primary efficacy time point.

There was a low incidence of gout flares in this study. Gout flares over 24 weeks occurred in 5% of placebo-treated patients compared to 7-16% of patients treated with BCX4208.

"We are very pleased to confirm the continued favorable safety profile and sustained efficacy for BCX4208 as an add-on therapy for gout. The sustained efficacy, healthy immune responses to vaccines, and clean safety profile from 900 patient-months of drug exposure in this study provides a robust basis for Phase 3 trials," said Dr. William P. Sheridan, Senior Vice President & Chief Medical Officer of BioCryst Pharmaceuticals. "Based on these results, we have selected the 5, 10 and 20 mg doses of BCX4208 for further evaluation."

Conference Call and Webcast

BioCryst will host a conference call and webcast on Monday, January 9, 2012 at 10:00 a.m. ET to discuss these study results.

To participate in the conference call, please dial 1-877-303-8027 (United States) or 1-760-536-5165 (International). No passcode is needed for the call. The webcast can be accessed by logging onto BioCryst's website at www.BioCryst.com. Please connect to the website at least 15 minutes prior to the start of the conference call to ensure adequate time for any software download that may be necessary.

About BCX4208

BCX4208 is a novel enzyme inhibitor with the potential for once-a-day oral dosing suitable for chronic administration to treat gout. It acts upstream of xanthine oxidase in the purine metabolism pathway to reduce sUA in patients with gout and has a mechanism of action that complements xanthine oxidase inhibitors, such as allopurinol and febuxostat, in reducing uric acid production. With its unique mechanism of action, clinical activity and safety in clinical studies to date, BCX4208 is nearing the end of Phase 2 development as an add-on therapy to xanthine oxidase inhibitors to address unmet medical needs in patients with gout. To date, BCX4208 has been studied in over 500 patients in clinical trials.

About Gout

Gout is a chronic inflammatory arthritis caused by monosodium urate crystal deposits in joints and the kidneys resulting from elevated sUA levels in the blood, a condition known as hyperuricemia. The consequences of gout may include intense, painful flares affecting one or more joints, impaired kidney function and joint destruction. Gout continues to grow in prevalence and severity, affecting over 17 million people in major markets, including 8.3 million in the U.S. A majority of gout patients are also treated to manage other chronic conditions, including hypertension, diabetes and/or high cholesterol. Decreasing sUA to the recommended level (less than 6 mg/dL) can reduce the risk of gout attacks over the long-term. A minority of patients treated with the current standard of care, allopurinol, achieve this therapeutic goal. There is a need for new therapies that effectively and safely get a larger portion of gout suffers to goal without the risk of drug-drug interactions. More information regarding gout and hyperuricemia is available on the CDC website at www.cdc.gov/arthritis/basics/gout.htm.

About BioCryst

BioCryst Pharmaceuticals designs, optimizes and develops novel small-molecule pharmaceuticals that block key enzymes involved in infectious diseases, inflammatory diseases and cancer. BioCryst currently has three novel late-stage compounds in development: peramivir, a neuraminidase inhibitor for the treatment of influenza, BCX4208, a purine nucleoside phosphorylase (PNP) inhibitor for the treatment of gout, and forodesine, an orally-available PNP inhibitor for cancer, which is being developed by Mundipharma under a global license agreement. Utilizing crystallography and structure-based drug design, BioCryst continues to discover additional compounds and to progress others through pre-clinical and early development to address the unmet medical needs of patients and physicians. 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 future results, performance or achievements. These statements involve known and unknown risks, uncertainties and other factors which may cause our actual results, performance or achievements to be materially different from any future results, performances 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 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: that there can be no assurance that our compounds will prove effective in clinical studies; that development and commercialization of our compounds may not be successful; that we or our licensees may not be able to enroll the required number of subjects in planned clinical trials of our product candidates and that such clinical trials may not be successfully completed; that BioCryst or its licensees may not commence as expected additional human clinical trials with our product candidates; that our product candidates may not receive required regulatory clearances from the FDA; that ongoing and future pre-clinical and clinical development may not have positive results; that we or our licensees may not be able to continue future development of our current and future development programs; that our development programs may never result in future product, license or royalty payments being received by BioCryst; that BioCryst may not reach favorable agreements with potential pharmaceutical and biotechnology partners for further development of its product candidates; that our actual cash burn rate may not be consistent with our expectations; that BioCryst may not have sufficient cash to continue funding the development, manufacturing, marketing or distribution of its products and that additional funding, if necessary, may not be available at all or on terms acceptable to BioCryst. 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 our projections and forward-looking statements.

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