



BioCryst Announces Positive Top-Line Results from BCX4208 Phase 2B Gout Study in Patients Not Responding to Allopurinol Alone

- **BioCryst to host a conference call & webcast today at 8:30 AM ET**

RESEARCH TRIANGLE PARK, N.C.--(BUSINESS WIRE)-- [BioCryst Pharmaceuticals, Inc.](http://www.biocryst.com) (NASDAQ: BCRX) today announced positive top-line results from its Phase 2b randomized, double-blind, dose-response study of BCX4208 in gout patients who had failed to reach the clinically important serum uric acid (sUA) goal of <6 mg/dL on allopurinol alone. Detailed study findings will be shared at an upcoming medical meeting.

The study randomized 279 patients to five study arms: BCX4208 at doses of 5 mg, 10 mg, 20 mg, 40 mg and placebo, administered once-daily for 12-weeks. Allopurinol 300 mg once-daily was administered in all study arms. The primary endpoint of the study was the proportion of patients with sUA <6 mg/dL at day 85.

The primary endpoint of the study was successfully achieved. When added to allopurinol 300 mg, BCX4208 was superior to allopurinol plus placebo (p=0.009 overall). BCX4208 doses evaluated in the study showed response rates ranging from 33% to 49%, compared to 18% for placebo (table below).

Top-Line Efficacy Results: BCX4208 Phase 2b Study in Patients Not Responding to Allopurinol Alone

	Placebo	BCX4208 Treatment Groups			
	(N=55)	5 mg (N=56)	10 mg (N=55)	20 mg (N=56)	40 mg (N=53)
Once-daily treatment:					
Primary endpoint: Proportion of patients with uric acid levels <6mg/dL at day 85					
Response Rate (mITT Analysis)	18%	45%	33%	39%	49%
P value vs. placebo		p=0.004*	p=0.125	p=0.021*	p<0.001*

*statistically significant

Adding BCX4208 to allopurinol was generally safe and well-tolerated at all doses studied. Both the frequency and types of adverse events, including infections, were similar between the groups treated with BCX4208 and placebo. No opportunistic or unusual infections were reported in either the BCX4208 treated groups or placebo. As expected, a dose-dependent effect on lymphocyte counts was observed and this effect appeared to plateau within 12 weeks of treatment. No patients from the placebo, 5 mg or 10 mg cohorts discontinued study drug due to confirmed lymphocyte or CD4+ cell counts below certain pre-specified thresholds. Two patients were discontinued from the 20 mg group and eight patients from the 40 mg group due to pre-specified stopping rules based on CD4+ cell counts.

"These positive results in 2nd line treatment are consistent with findings from our prior 1st line combination study, and reinforce BCX4208's potential to safely address the unmet medical need for new treatment options that help gout patients reach their therapeutic goal," said [Dr. William P. Sheridan](#), Senior Vice President & Chief Medical Officer of BioCryst Pharmaceuticals. "We expect to report 6-month results from the ongoing extension study in early 2012, which will provide additional insight into BCX4208's safety and efficacy profile. We are on track to conclude this Phase 2 program during the first half of next year and we look forward to discussing the results in more detail with regulatory authorities and potential partners."

Conference Call and Webcast

BioCryst will host a conference call and webcast today at 8:30 a.m. ET to discuss the study results as well as provide a clinical overview and update for the BCX4208 program. 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 Gout

Gout is a chronic inflammatory arthritis caused by monosodium urate crystal deposits in joints and the kidneys resulting from

elevated serum uric acid (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 sufferers 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 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 subjects in clinical trials.

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: 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 hematological malignancies. 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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