BioCryst Reports Positive Results From A Phase 2 Study Of BCX4208 Combined with Allopurinol in Patients with Gout

Top-line Results to be Discussed During Investor Day Meeting Today

BIRMINGHAM, Ala., Sep 16, 2010 (BUSINESS WIRE) -- BioCryst Pharmaceuticals, Inc. (NASDAQ: BCRX) today announced positive top-line results from its randomized, double-blind, multi-center, placebo-controlled Phase 2 study designed to evaluate the urate-lowering activity and safety of several doses of BCX4208 alone and in combination with selected doses of allopurinol administered once-daily.

The study utilized a factorial design. The primary endpoint was change in serum uric acid concentration (sUA) after 21 days of treatment compared to baseline concentration prior to treatment. Eighty-seven gout patients with sUA concentrations greater than or equal to 8 mg/dL were randomized to receive BCX4208 at daily doses of 20 mg, 40 mg and 80 mg administered orally as monotherapy or in combination with allopurinol at daily doses of 100 mg, 200 mg and 300 mg administered orally.

A dose-response was demonstrated for both BCX4208 and allopurinol, and the combination of BCX4208 and allopurinol was shown to be superior to either drug alone in sUA reduction. The mean reduction in sUA from baseline observed in the nine groups of patients receiving combinations of BCX4208 and allopurinol ranged from 2.4 to 5.6 mg/dL. In five of these nine combination groups, 80 percent or more of the patients achieved a sUA concentration of less than 6 mg/dL. Combinations of lower doses of BCX4208 with allopurinol showed synergistic effects in sUA reduction.

These doses of BCX4208 alone and in combination with allopurinol were generally safe and well-tolerated. Consistent with prior BCX4208 clinical studies, reductions in peripheral blood lymphocytes were observed in patients treated with BCX4208. The protocol included stopping rules for CD4+ cell counts below certain thresholds; no subjects were discontinued for this reason.

"This successful study demonstrates that BCX4208 and allopurinol in combination may synergistically reduce uric acid levels in the blood and supports its continued evaluation as a potential treatment for patients with gout," said Dr. William P. Sheridan, Chief Medical Officer at BioCryst. "We are now finalizing plans for a 12 week, randomized, controlled study of BCX4208 as add-on therapy in gout patients who have failed to adequately respond to allopurinol. We look forward to starting this study in early 2011 and plan to enroll over 200 patients."

Investor Day Web Cast

BioCryst management will discuss the results of this BCX4208 gout study and provide other program updates during a previously announced Investor Day meeting scheduled for today, Thursday, September 16, 2010 from 9 a.m. to 12 p.m. Eastern Time. Links to a live audio webcast and replay of the presentation may be accessed on the BioCryst Web site at www.biocryst.com.

About BCX4208

BCX4208 is a next generation purine nucleoside phosphorylase (PNP) inhibitor with the potential for once-a-day dosing suitable for chronic administration. Previous studies have shown that BCX4208 may have utility in diseases dependent on T-cells, B-cells or uric acid. With its novel mechanism of action, BCX4208 has the potential to address unmet medical needs across a broad spectrum of inflammatory diseases, including gout.

About Gout

Gout is an inflammatory arthritis that affects up to five million people in the U.S. Gout is caused by higher-than-normal uric acid in the blood, (a condition known as hyperuricemia) that may lead to the buildup of uric acid in synovial fluid around joints and the formation of monosodium urate crystals that result in painful joint inflammation. More information regarding gout and hyperuricemia is available at: http://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, cancer and inflammatory diseases. BioCryst has progressed two novel compounds into late-

stage pivotal clinical trials; peramivir, an anti-viral for influenza, and forodesine, a purine nucleoside phosphorylase (PNP) inhibitor for cutaneous T-cell lymphoma (CTCL). Additionally, BioCryst has a third product candidate, BCX4208--a next generation PNP inhibitor--in mid-stage trials for the treatment of gout. 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 Web site 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 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 our product candidates may not receive required regulatory clearances from the FDA; that ongoing and future preclinical or clinical development and commercialization of our product candidates, including peramivir, forodesine, BCX4208 and other PNP inhibitor and hepatitis C development programs, 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 be able to retain its current pharmaceutical and biotechnology partners for further development of its product candidates or it may not reach favorable agreements with potential pharmaceutical and biotechnology partners for further development of its product candidates; 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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SOURCE: BioCryst Pharmaceuticals, Inc.

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