July 24, 2012

BioCryst Announces Positive Results from Two Ulodesine Phase 2 Trials in Patients with Gout

- ***Confirmation of favorable ulodesine safety profile through 52 weeks***
- ***Proportion of patients reaching serum uric acid goal sustained at 52 weeks***
- ***Management to discuss during conference call/webcast August 2, 2012 at 11 a.m. ET***

RESEARCH TRIANGLE PARK, N.C.--(BUSINESS WIRE)-- BioCryst Pharmaceuticals, Inc. (NASDAQ:BCRX) today announced favorable 52-week safety results and sustained efficacy from the extension phase of its randomized Phase 2b trial of ulodesine (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, as well as positive Phase 2 safety results in patients with mild to moderate renal impairment.

In the original 12-week study, 279 patients were randomized. The extension to 52 weeks, in which 119 patients entered the final extension phase, concludes the Phase 2b trial and Phase 2 development program for ulodesine. Patients continued their blinded, randomized therapy of ulodesine at doses of 5 mg, 10 mg and 20 mg and placebo once-daily. Allopurinol 300 mg once-daily was administered in all study arms.

The results of the 52-week, blinded Phase 2b safety extension trial confirm that ulodesine continues to be generally safe and well-tolerated in gout patients who inadequately responded to allopurinol alone, many of which had multiple co-morbidities. No clinical adverse event signals were observed that distinguished ulodesine from placebo, either by type or by rate at the doses tested. No opportunistic or unusual infections were observed and no signal for other organ toxicities was detected. No fatal or life-threatening adverse events were observed in any of the treatment groups. It was previously reported that eligible patients responded favorably to a vaccine challenge at 16 or 20 weeks of ulodesine treatment, confirming maintenance of a healthy immune response. The previously observed lymphocyte plateau reached by 12 weeks of treatment remained unchanged in the 5 mg, 10 mg and 20 mg ulodesine arms through 52 weeks. No patients from the placebo or 5 mg discontinued study drug for confirmed reductions of lymphocyte or CD4+ cell counts below certain protocol-specified thresholds; through 52 weeks, one patient was discontinued from the 10 mg group, four patients from the 20 mg group and twelve patients from the 40 mg group for reductions in CD4+ cell counts. The 40 mg arm was discontinued after the 24-week analysis.

The approximate doubling of sUA response rates with ulodesine seen at 12 weeks was sustained through 52 weeks of treatment. After 52 weeks of treatment, ulodesine doses of 5 mg, 10 mg, and 20 mg/day showed response rates of 45%, 47% and 64% respectively, compared to 19% 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 the Phase 2b study. Gout flares over 52 weeks occurred in 7% of placebo-treated patients compared to 9-21% of patients treated with ulodesine.

"Over 115 patient-years of drug exposure solidifies our confidence in the long-term safety of ulodesine and its ability to provide sustained sUA control over time. These comprehensive results reduce the risk of ulodesine Phase 3 development. Ulodesine has demonstrated clinical safety and efficacy in a wide variety of gout patients, including those with mild to moderate renal impairment, as well as those with a risk of kidney stones," said Dr. William P. Sheridan, Senior Vice President & Chief Medical Officer of BioCryst Pharmaceuticals. "We are sharing these long-term Phase 2 results with potential partners who are evaluating licensing ulodesine for Phase 3 development and commercialization. We remain focused on concluding our partnering discussions, while preparing materials and protocols for Phase 3 trials that would enable a partner to quickly initiate the pivotal program."

In addition to the Phase 2b trial, BioCryst completed a small Phase 2 trial in patients with moderate renal impairment. In this 12-week trial, 20 patients were randomized to placebo (n=4), 5 mg (n=8) or 10 mg (n=8) in combination with 200 mg allopurinol per day. A total of 118 patients with mild to moderate renal impairment, based on the body surface area adjustment of the Cockroft-Gault formula, have been evaluated on study drug across the Phase 2 program. These trials confirmed that ulodesine can be used safely in patients with mild to moderate renal impairment, a common co-morbidity in gout patients.

About Ulodesine

Ulodesine 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 (XO) in the purine metabolism pathway to reduce new formation of uric acid in patients with gout. It has a mechanism of action that complements XO inhibitors, such as allopurinol and febuxostat. With its unique mechanism of action, clinical activity and safety in clinical studies to date, ulodesine is a Phase-3-ready asset in development as an add-on therapy to XO inhibitors to address unmet medical needs in patients with gout. To date, ulodesine 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 drugs that block key enzymes involved in infectious and inflammatory diseases. BioCryst currently has two late-stage development programs: peramivir, a viral neuraminidase inhibitor for the treatment of influenza, and ulodesine (BCX4208), a purine nucleoside phosphorylase (PNP) inhibitor for the treatment of gout. In addition, BioCryst is advancing two preclinical programs towards IND filings: BCX5191, a nucleoside analog inhibitor of HCV RNA polymerase (NS5B) for hepatitis C, and BCX4161, an oral inhibitor of plasma kallikrein for hereditary angioedema. Utilizing state-of-the-art structure-guided drug design and crystallography, BioCryst continues to discover innovative compounds with the goal of addressing 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 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 ulodesine; 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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BioCryst Pharmaceuticals
Investors:
Robert Bennett, +1-919-859-7910
or
WCG
Media:
Catherine Kyroulis, +1-212-301-7174

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