



## **BIOCRYST INITIATES INTRAVENOUS PHASE I/II CLINICAL TRIAL OF FODOSINE™ IN PATIENTS WITH B-CELL ACUTE LYMPHOBLASTIC LEUKEMIA**

Birmingham, Alabama – December 22, 2005 – BioCryst Pharmaceuticals, Inc. (Nasdaq: BCRX) today announced that it has initiated a Phase I/II clinical trial of Fodosine™ (forodesine hydrochloride), the company's lead anticancer compound, to determine the safety of repeat doses of an intravenous (IV) formulation of the drug in patients with B-cell acute lymphoblastic leukemia (B-ALL). Fodosine™ is a purine nucleoside phosphorylase (PNP) inhibitor, which functions by blocking the DNA synthesis machinery of the body's T-cells. This small molecule drug is being developed for treatment of T-cell malignancies, and has been designated an "Orphan Drug" for broader indications, including cutaneous T-cell lymphoma (CTCL), chronic lymphocytic leukemia (CLL) and acute lymphoblastic leukemia (ALL), by the FDA. The design of this B-ALL trial was based on data from ex vivo studies on cells from B-ALL patients at M.D. Anderson Cancer Center and by preliminary patient data from Cornell Medical Center. The latter study concluded that Fodosine™ could represent an important breakthrough in the development of a more effective and less toxic treatment for ALL.

This multicenter, open-label, repeat-dose study is scheduled to enroll 30 patients and consists of two periods, an Initial Treatment Period and an Extended Treatment Period. The Initial Treatment Period will last four weeks, during which patients will receive single daily infusions of 80mg/m<sup>2</sup> of Fodosine™ for five consecutive days each week with at least two non-treatment days per week. This regimen will be extended by four additional weeks in the Extended Treatment Period if the patient continues to show benefit from the treatment. Additionally, patients who are responding to treatment with Fodosine™ at the end of the Extended Treatment Period may continue receiving the drug under compassionate use guidelines.

"This study will allow us to continue the optimization of Fodosine™ in the treatment of B-cell ALL," stated Dr. Charles E. Bugg, Chairman and CEO of BioCryst. "Additionally, we will be monitoring these patients for evidence of efficacy, which was suggested from the encouraging clinical responses observed in the earlier Cornell trial."

### **About BioCryst**

BioCryst Pharmaceuticals, Inc. designs, optimizes and develops novel drugs that block key enzymes involved in cancer, cardiovascular diseases, autoimmune diseases, and viral infections. BioCryst integrates the necessary disciplines of biology, crystallography, medicinal chemistry and computer modeling to effectively use structure-based drug design to discover and develop small molecule pharmaceuticals.

BioCryst's lead product candidate, Fodosine™, is a transition state analog inhibitor of the target enzyme purine nucleoside phosphorylase (PNP). The drug is currently in a Phase IIa trial for patients with T-cell leukemia and a combination IV and oral Phase I pharmacokinetic trial in healthy volunteers. Results of the Phase IIa and the Phase I pharmacokinetic trial will assist in the design of a planned combination IV and oral Phase IIb pivotal clinical trial in patients with T-cell leukemia. The Company has requested a Special Protocol Assessment from the FDA for this planned trial. Additionally, Fodosine™ is currently being studied in a Phase I trial with an oral formulation in cutaneous T-cell lymphoma (CTCL), a Phase II trial in chronic lymphocytic leukemia (CLL) and a Phase I/II trial in B-cell acute lymphoblastic leukemia. Fodosine™ has been granted Orphan Drug status by the U.S. Food and Drug Administration for three indications: T-cell non-Hodgkin's lymphoma, including CTCL; CLL and related leukemias including T-cell prolymphocytic leukemia, adult T-cell leukemia, and hairy cell leukemia; and for treatment of B-cell acute lymphoblastic leukemia (ALL). Additionally the FDA has granted "fast track" status to the development of Fodosine™ for the treatment of relapsed or refractory T-cell leukemia.

In August, 2005, BioCryst initiated a Phase Ib study with its second-generation PNP inhibitor, BCX-4208, to evaluate the safety, tolerability and pharmacokinetics of multiple oral doses of BCX-4208. In November, 2005 BioCryst announced it had entered into an exclusive licensing agreement with Roche to develop and commercialize BCX-4208 for the prevention of acute rejection in transplantation and for the treatment of autoimmune diseases.

Additionally, BioCryst has re-initiated clinical development of peramivir, an inhibitor of influenza neuraminidase, with a focus on intravenous and intramuscular delivery. Also, BioCryst has identified a clinical candidate, BCX-4678, in its hepatitis C polymerase inhibitor program, and is advancing this compound through preclinical testing with the goal of filing an IND in early 2006. For more information about BioCryst, please visit the company's web site at <http://www.biocryst.com>.

### **Forward-looking statements**

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 clinical trials of Fodosine™ or BCX-4208, that each of the Phase IIa trial for patients with T-cell leukemia, Phase I trial of BCX-4208, the Phase I trial of Fodosine™ for treatment of patients with cutaneous-T-cell lymphoma, the Phase I/II trial of Fodosine™ for treatment of patients with B-cell ALL and the Phase II trial of Fodosine™ for advanced fludarabine-refractory CLL may not be successfully completed, that BioCryst or its licensees may not commence as expected additional trials with Fodosine™ and with BGX208 or planned human trials with peramivir or BCX-4678, that Fodosine™, BCX208, peramivir, BCX-4678 or any of our other product candidates may not receive required regulatory clearances from the FDA, that clinical trials of Fodosine™ may not show the drug is effective over the initial treatment period, that ongoing and future clinical trials may not have positive results, that we may not be able to obtain a Special Protocol Assessment or otherwise be able to complete successfully the Phase IIb trial that is currently planned to be pivotal, that we or our licensees may not be able to continue future development of Fodosine™, BCX208, peramivir, BCX-4678 or any of our other current development programs including tissue factor/factor VIIa, that Fodosine™, BCX208, peramivir, BCX-4678 or our other 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 biotech 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, current reports on Form 8-K which identify important factors that could cause the actual results to differ materially from those contained in the projections or forward-looking statements.

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