



## **BIOCRYST RECEIVES ORPHAN DRUG DESIGNATION FOR BCX-1777 IN TWO ADDITIONAL CANCER INDICATIONS**

Birmingham, Alabama - August 24, 2004 - BioCryst Pharmaceuticals, Inc. (Nasdaq NM: BCRX) today announced that forodesine hydrochloride, its lead anti-cancer compound formerly known as BCX-1777 (forodesine), has been granted designation as an "Orphan Drug" for two additional cancer indications by the U.S. Food and Drug Administration (FDA). The Company is developing forodesine for treatment of T-cell mediated cancers, and a Phase IIa clinical trial in patients with T-cell leukemia is in progress. Forodesine, a purine nucleoside phosphorylase (PNP) inhibitor which functions by blocking the T-cell's ability to synthesize DNA, was granted orphan status for treatment of T-cell non-Hodgkin's lymphoma, including cutaneous T-cell lymphoma, in February, 2004. The two additional indications are for treatment of chronic lymphocytic leukemia (CLL) and related leukemias to include polymorphocytic leukemia, adult T-cell leukemia, and hairy cell leukemia; and for treatment of acute lymphoblastic leukemia (ALL).

"We are delighted to have received orphan drug designation from the FDA for these two additional indications," said Charles E. Bugg, Chairman and Chief Executive Officer of BioCryst. "BioCryst continues to gain recognition for its very novel approach to developing a treatment for T-cell cancers. This milestone supports our belief that forodesine has the potential to help patients who are suffering from several rare and devastating cancers, and will enhance our efforts to bring this promising new treatment to market as quickly as possible."

Orphan drug classification is awarded to select approaches that offer potential therapeutic value in the treatment of rare diseases and conditions. The granting of orphan drug status to forodesine hydrochloride enables BioCryst to receive pre-filing regulatory guidance as well as reduced filing fees, and would provide for market exclusivity in the U.S. for a period of seven years if forodesine receives market approval by the FDA.

### **About BioCryst**

BioCryst Pharmaceuticals, Inc. designs, optimizes and develops novel drugs that block key enzymes involved in cancer, cardiovascular and 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, forodesine hydrochloride (BCX-1777, or forodesine), an inhibitor of purine nucleoside phosphorylase (PNP), is currently in a Phase IIa trial for patients with T-cell malignancies with additional Phase I/II trials planned for hematologic malignancies, and for cutaneous T-cell lymphoma (CTCL). BioCryst plans to initiate a Phase I study with a second generation PNP inhibitor, BCX-4208, during the second half of 2004 for use in autoimmune diseases such as psoriasis. In addition, BioCryst has several new enzyme targets in drug discovery including tissue factor/factor VIIa and hepatitis C polymerase. For more information about BioCryst, please visit the company's web site at [www.biocryst.com](http://www.biocryst.com).

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 may not be able to enroll the required number of subjects in clinical trials of forodesine, that the Phase I/II trials of forodesine for treatment of patients with cutaneous T-cell lymphoma and hematologic malignancies may not be successfully completed, that BioCryst may not commence as expected additional Phase II trials with forodesine and Phase I studies with BCX 4208, that forodesine or any of our other product candidates may not receive required regulatory clearances from the FDA, that Phase IIa clinical trials of forodesine may not show the drug is effective over the 6-week period, that we may not be able to obtain a Special Protocol Assessment or otherwise be able to complete successfully the Phase IIb that is currently planned, that we may not be able to continue future development of forodesine or any of our other current development programs including BCX-4208, tissue factor/factor VIIa and hepatitis C polymerase, that forodesine or our other development programs may never result in future product, license or royalty payments being received by BioCryst, 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, which identify important factors that could cause the actual results to differ materially from those contained in the projections or forward-looking statements.