BioCryst Reports New Data from Its Phase 2 Forodesine Study in Patients with Chronic Lymphocytic Leukemia at the 52nd Annual American Society of Hematology Meeting

Study Results Confirm Clinical Activity of Forodesine Against CLL

RESEARCH TRIANGLE PARK, N.C.--(BUSINESS WIRE)-- BioCryst Pharmaceuticals, Inc. (NASDAQ: BCRX) today announced the presentation of new data that confirms forodesine's clinical activity in the treatment of chronic lymphocytic leukemia (CLL) at the 52nd Annual American Society of Hematology (ASH) Meeting & Exposition being held in Orlando, Florida.

In this Phase 2, open-label, single-arm, multi-center study, forodesine was administered orally at 200 mg twice-daily for 28-day cycles in previously treated CLL patients. The primary endpoint of the study was overall response rate. An analysis conducted after all patients were followed through ≥6 months showed that six of 23 response-evaluable patients demonstrated a partial response to forodesine, resulting in a response rate of 26 percent. Forodesine 200 mg orally-administered twice-daily was generally safe and well-tolerated in this study. The pattern, frequencies and severity distribution of adverse events were generally consistent with CLL-associated poor bone marrow function and immunodeficiency, prior therapies and co-morbidities.

"We are encouraged by these results, which support the potential role for forodesine and other purine nucleoside phosphorylase inhibitors in the treatment of patients with hematological malignancies. We believe further evaluation of oral PNP inhibitors in combination with other anti-leukemic agents for CLL is warranted," said Dr. William P. Sheridan, Chief Medical Officer of BioCryst. "BioCryst continues to explore potential for partnering as a possible path forward for the future development of PNP inhibitors for oncology."

The data will be presented during a poster session scheduled for today, December 4, 2010 from 5:30—7:30 p.m. Eastern Time. The poster to be presented during the session is entitled:

- Presentation Number 1397: "Forodesine, a Purine Nucleoside Phosphorylase (PNP) Inhibitor, Shows Clinical Activity in a Phase 2 Trial in Patients with Previously Treated CLL — Interim Analysis".

The abstract is available and can be viewed online through the ASH Web site at www.hematology.org/, and the poster is available on the BioCryst Web site at www.biocryst.com.

About forodesine

Forodesine is an orally-available transition-state analog inhibitor of purine nucleoside phosphorylase (PNP), a purine salvage pathway enzyme that is essential for the proliferation of T-cells and B-cells. Typically, T- and B-cells are an essential part of the body's immune system, but when they multiply uncontrollably they can cause various forms of cancer. Inhibiting PNP produces selective suppression of T- and B-cells, inducing apoptosis in both types of cells. For more information about forodesine, please visit BioCryst's Web site at http://www.biocryst.com/forodesine.

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 in development: 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 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 development and commercialization of forodesine 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 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 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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Source: BioCryst Pharmaceuticals, Inc.