BioCryst's CTCL Pivotal Study Achieves Enrollment Target

BIRMINGHAM, Ala., Jan 15, 2010 /PRNewswire via COMTEX News Network/ -- BioCryst Pharmaceuticals, Inc. (Nasdaq: BCRX) today announced that it has achieved its protocol-specified objective of enrolling 100 late-stage patients (Stage IIB to IVA) in its pivotal study for forodesine in the treatment of cutaneous T-cell lymphoma (CTCL). Top-line data is expected in the second half of 2010. Additionally, BioCryst's exploratory Phase 2 study for forodesine in subjects with chronic lymphocytic leukemia (CLL) is continuing to progress and has enrolled over half of its targeted number of patients.

"Reaching this milestone for the CTCL pivotal study is an important event for the development of forodesine, and will enable a complete analysis of the trial results once protocol-specified patient follow-up has been concluded," said Dr. William P. Sheridan, Chief Medical Officer at BioCryst. "Also, we are pleased with progress in the CLL Phase 2 study and look forward to completing enrollment."

Currently, 143 patients are enrolled in the CTCL study. Eligible patients are those with CTCL of stages IB through IVA who have disease that is persistent, progressive or recurrent during or after treatment with at least three systemic therapies. The study is a multinational, non-randomized, open-label, single-arm trial that is evaluating 200 mg once-daily oral forodesine treatment. The study will examine the rate of objective responses in patients enrolled at sites in North America, Europe and Australia. The study's primary endpoint is objective response rate, defined as either complete response or partial cutaneous response that is sustained for at least 28 days. This trial is being conducted under a Special Protocol Assessment (SPA) agreement negotiated with the U.S. Food and Drug Administration (FDA) and will serve as a basis for a new drug application (NDA) to the FDA. Further details regarding the study design are available at the following clinicaltrials.gov link:

http://clinicaltrials.gov/ct2/show/NCT00501735.

BioCryst's Phase 2 study for forodesine in subjects with CLL has enrolled 15 of the targeted 26 patients, with 12 patients currently still on treatment. The primary purpose of the study is to evaluate the effectiveness and safety of oral forodesine administered as monotherapy at a dose of 200 mg twice-daily in relapsed CLL patients. Previous clinical trial data indicated that forodesine demonstrated clinical activity in CLL patients at a dose of 200 mg once-daily, and was generally safe and well-tolerated. The current trial is testing the benefit and safety of increasing forodesine drug exposure with twice-daily dosing. Top-line study results are expected in the second half 2010. Further details regarding the study design are available at the following clinicaltrials.gov link:

http://clinicaltrials.gov/ct2/show/NCT00640523.

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-cells 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-cells and B-cells, inducing apoptosis in both types of cells.

About cutaneous T-cell lymphoma (CTCL)

CTCL affects approximately 18,000 people in the U.S. CTCL is a primary skin neoplasm and accounts for nearly 50% of all T-cell malignancies. Unlike most cancer patients, CTCL patients are treated chronically and could benefit from an oral agent that is well-tolerated like forodesine. Long term treatment (>12 months) of CTCL with forodesine in the supportive Phase 1/2 study suggests a favorable safety profile. This data was presented at ASCO in 2009.

For more information on CTCL please visit the National Cancer Institute at the following link: http://www.cancer.gov/cancertopics/pdq/treatment/mycosisfungoides/Patient/page1.

About chronic lymphocytic leukemia (CLL)

Lymphoma is a general term for a group of cancers that originate in the lymphatic system. About 58,870 Americans will be diagnosed with a non-Hodgkin's lymphoma in 2006 and approximately 15% of these will be considered T-cell lymphomas. T-cell lymphoma results when a T-lymphocyte (a type of white blood cell) undergoes a malignant change and begins to multiply,

eventually crowding out healthy cells and creating tumors, which enlarge the lymph nodes and invade other sites in the body.

For more information on CLL please visit the National Cancer Institute at the following link: http://www.cancer.gov/cancertopics/pdq/treatment/CLL.

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 forcutaneous T-cell lymphoma (CTCL). BioCryst is collaborating to develop and commercialize forodesine with Mundipharma International Holdings, Ltd., which obtained rights to forodesine in markets across Europe, Asia and Australasia in 2006. 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 the U.S. government and ex-U.S. governments may choose not to issue additional orders for peramivir and such orders, if any, may not be profitable for BioCryst; that to the extent peramivir is used as a treatment for H1N1 flu (or other strains of flu), there can be no assurance that it will prove effective; that HHS may further condition, reduce or eliminate future funding of the peramivir program; that ongoing peramivir clinical trials or our peramivir program in general may not be successful; 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 and partnerships 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, most recent 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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