BioCryst Announces Peramivir Phase 3 Safety & Virology Study Results and Provides a Clinical Program Update

RESEARCH TRIANGLE PARK, N.C.--(BUSINESS WIRE)-- BioCryst Pharmaceuticals, Inc. (NASDAQ:BCRX) today announced top-line results from one of its two Phase 3 studies of intravenous (i.v.) peramivir for the treatment of patients hospitalized with influenza, and provided an update regarding its peramivir program.

The completed Phase 3 safety and virology study ("303") was an open-label, randomized trial of the anti-viral activity, safety and tolerability of i.v. peramivir administered either as a once-daily infusion of 600 mg or a twice-daily infusion of 300 mg to adult and adolescent subjects hospitalized with confirmed or suspected influenza infection. Treatment was planned for 5 days with an extension to 10 days in patients who needed additional treatment.

"Physicians would like to have additional treatments approved for seriously-ill patients with influenza who need hospital care, including an intravenous anti-viral drug," said the study's Principal Investigator, Dr. Michael G. Ison, Assistant Professor, Divisions of Infectious Diseases and Organ Transplantation at Northwestern University Feinberg School of Medicine. "This study is the largest, prospective study of an i.v. influenza anti-viral in the hospital setting completed to date, and significantly expands our knowledge on the anti-viral activity, safety and tolerability of i.v. peramivir in patients hospitalized with influenza."

The study enrolled 234 patients aged 14 to 92 years during the 2009-2010 H1N1 pandemic of whom 200 patients (85%) had a duration of illness of more than 48 hours. Peramivir was administered to 230 patients; 170 patients (74%) had received prior treatment with oseltamivir. At study entry 158 patients (69%) needed supplemental oxygen and 39 patients (17%) were in intensive care. The median duration of peramivir treatment was five days (range, 1-11 days). The intent to treat infected (ITTI) population consisted of 127 patients with influenza confirmed by RT-PCR, viral culture, or serology.

The primary endpoint of the study was the change in influenza virus titer in nasopharyngeal samples, measured by $\log_{10}$ tissue culture infective dose$_{50}$ ($TCID_{50}$). Forty-four patients had a positive baseline culture, 20 for the 300 mg twice-daily group and 24 for the 600 mg once-daily group. Similar reductions in $\log_{10}$ $TCID_{50}$ viral titer were observed over the first 48 hours in the two treatment groups, -1.66 (95% CI -2.32, -0.61) for 300 mg peramivir twice-daily and -1.47 (95% CI -1.89, -0.75) for peramivir 600 mg once-daily.

Both dose regimens of i.v. peramivir were generally safe and well-tolerated. The frequency and severity of adverse events was similar in the two groups, and was consistent with the profile of influenza patients hospitalized during the 2009-2010 pandemic. Serious adverse events (SAE's) were reported in 20 percent of patients. Of the total SAEs reported, one case of elevated liver enzymes was attributed to the study drug and all other SAE's were attributed to other factors. The most common SAE's reported were respiratory failure, acute respiratory distress syndrome (ARDS), septic shock and acute renal failure. Overall mortality within 28 days of initial peramivir treatment was 8.7 percent; no deaths were attributed to study drug. No safety signals were identified.

The analysis of the combined ITTI population showed median time to resolution of fever was 25.3 hours; time to clinical resolution, 92.0 hours; time to alleviation of symptoms, 145 hours; and time to resumption of usual activities, 26.8 days. Further analyses of the data are ongoing, and the Company will submit detailed analyses for presentation at an upcoming medical meeting.

"The successful completion of this safety and virology study is an important step in peramivir development, which is continuing to progress with the ongoing phase 3 efficacy study in hospitalized influenza," said Dr. William P. Sheridan, Chief Medical Officer at BioCryst. "This study's protocol included broad eligibility criteria and allowed enrollment of patients who were in the ICU or who failed oseltamivir, unlike our prior Phase 2 study in patients hospitalized with flu. We are pleased with peramivir's safety profile in this seriously-ill population."

Peramivir Clinical Program Update

BioCryst's other peramivir Phase 3 study ("301") is an ongoing, multicenter, randomized, double-blind, controlled study to evaluate the efficacy and safety of 600 mg i.v. peramivir administered once-daily for five days in addition to standard of care (SOC), compared to SOC alone, in adults and adolescents who are hospitalized due to serious influenza. Based on recent discussions between the U.S. Department of Health & Human Services (HHS) and the FDA, HHS/Biomedical Advanced Research & Development Authority (BARDA) has asked the Company to focus on completing the ongoing Phase 3 efficacy

study, and the Company has identified changes to the current study design that could increase the likelihood of a favorable clinical outcome.

BioCryst has submitted a revised contract proposal to HHS seeking additional funding to enable completion of the Phase 3 development plan for i.v. peramivir, with modifications in the conduct of study 301 that include:

- Revision of the primary efficacy analysis, to focus on the subset of patients not treated with neuraminidase inhibitors as SOC, in order to provide the greatest opportunity to demonstrate a statistically significant treatment effect
- Increasing the total sample size, with further expansion of the number of sites
- Expansion to additional geographical regions
- Extending the timeline to complete enrollment beyond the end of 2011

These changes are expected to increase the amount of time required to complete enrollment in this ongoing study, and the Company believes it is unlikely that the study will reach its enrollment goal before the end of 2011. The actual time to reach completion of enrollment will depend on the 301 study sample size and number of investigation sites, as well as the prevalence and severity of influenza.

About peramivir

Peramivir is a potent, intravenously administered investigational anti-viral agent that rapidly delivers high plasma concentrations to the sites of infection. Discovered by BioCryst, peramivir inhibits the interactions of influenza neuraminidase, an enzyme which is critical to the spread of influenza within a host. In laboratory tests, peramivir has shown activity against multiple influenza strains, including pandemic H1N1 swine origin flu viral strains. In January 2010, Shionogi & Co., Ltd. launched intravenous (i.v.) peramivir in Japan under the name RAPIACTA® to treat patients with influenza and in August 2010, Green Cross Corporation announced that it had received marketing and manufacturing authorization for i.v. peramivir in Korea to treat patients with influenza A & B viruses, including H1N1 and avian influenza. For more information about peramivir please visit BioCryst's Web site at http://www.biocryst.com/peramivir.

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 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 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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