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BioCryst Broad-Spectrum Antiviral BCX4430 Highly Effective against Yellow Fever in a Preclinical Disease Model

Results to be presented at the 2nd Antiviral Congress today

RESEARCH TRIANGLE PARK, N.C.--(BUSINESS WIRE)-- [BioCryst Pharmaceuticals, Inc.](http://www.biocryst.com) (NASDAQ:BCRX) today announced proof-of-principle data demonstrating that BCX4430 is efficacious and well tolerated in a preclinical disease model for evaluating efficacy against yellow fever virus infection. BCX4430 is the lead compound in BioCryst's broad-spectrum antiviral (BSAV) research program. The objective of BioCryst's BSAV program is to develop broad-spectrum parenteral and oral therapeutics for viruses that pose a threat to national health and security.

A presentation entitled "BCX4430, an adenosine analog, with potent activity against yellow fever virus in a hamster model," will be presented by Dr. Justin Julander at the 2nd Antivirals Congress in Cambridge, MA today at 5:10PM E.T.

These studies comprehensively evaluated the efficacious dose, dose duration, and therapeutic use of parenteral BCX4430 in the treatment of yellow fever infection. Syrian Golden Hamsters—a widely accepted model for establishing efficacy against yellow fever—virus were inoculated intraperitoneally with ten times the cell culture infectious dose (CCID₅₀) of yellow fever virus (Jimenez strain) and treated parenterally with BCX4430 administered either once or twice daily for 4 to 7 days. In studies with treatment just prior to infection or delayed up to 4 days after infection, BCX4430 treated animals had significantly ($p < 0.001$) improved survival (80—100%) compared to animals receiving saline placebo (20-30%). It is important to note that in this model, serum viral titers peak at day four after infection, indicating that BCX4430 has the potential to be an effective therapeutic when administered as pre-exposure prophylaxis; as post-exposure prophylaxis during the virus incubation period; and as a treatment during the prodromal period and symptomatic disease. Treated animals also exhibited significantly lower viral titers in serum, improved weight gain, and decreased liver damage as measured by lower levels of alanine transaminase and aspartate transaminase.

In addition, BCX4430 demonstrated a wide therapeutic index of 50 with a minimum effective dose of 4 mg/kg/day ($p < 0.05$ vs control), as measured by survival, and a maximum tolerated dose of 200 mg/kg/day, as measured by percent weight change.

The study was conducted under the National Institute of Allergy and Infectious Diseases' (NIAID's) Animal Models of Infectious Disease Program. Dr. Justin Julander of Utah State University, who coordinates yellow fever testing under the NIAID Program stated, "BCX4430 is an exciting, potential antiviral therapy for the treatment of yellow fever virus infection, for which no approved treatment currently exists. The level of efficacy observed for BCX4430 in these studies compared to other antivirals evaluated against yellow fever is exceptional. BCX4430 warrants further study and development as a potential human therapeutic."

"BioCryst's BSAV research program has generated convincing evidence of efficacy in preclinical studies, not only in the yellow fever model, but also against other viruses studied *in vitro* and *in vivo*. BCX4430 represents an exciting research stage project that could become a breakthrough for the treatment of hemorrhagic fevers caused by flaviviruses and filoviruses. These viruses are viewed by U.S. Government Agencies as high priority targets for development of medical countermeasures," said [Dr. William P. Sheridan, Senior Vice President & Chief Medical Officer](#) of BioCryst Pharmaceuticals. "We are seeking government funding to support development of BCX4430 as a medical countermeasure for the protection of civilian and military populations, and plan to announce additional data from the BSAV program in upcoming publications and at scientific conferences."

About the BSAV Program & BCX4430

The objective of BioCryst's BSAV research program is to develop broad-spectrum parenteral and oral therapeutics for viruses that pose a threat to health and national security. The lead BSAV compound is BCX4430, an RNA dependent-RNA polymerase inhibitor that has demonstrated broad-spectrum activity in multiple viruses and a favorable preliminary preclinical safety profile. BioCryst plans to develop these compounds in collaboration with US Government Agencies following the Animal Rule regulatory pathway.

About BioCryst

BioCryst Pharmaceuticals designs, optimizes and develops novel small molecule drugs that block key enzymes involved in infectious and inflammatory diseases. BioCryst currently has two late-stage development programs: peramivir, a viral neuraminidase inhibitor for the treatment of influenza, and ulodesine (BCX4208), a purine nucleoside phosphorylase (PNP) inhibitor for the treatment of gout. In addition, BioCryst is developing two preclinical compounds: BCX5191, a nucleoside analog inhibitor of HCV RNA polymerase (NS5B) for hepatitis C, and BCX4161, an oral inhibitor of plasma kallikrein for hereditary angioedema. Utilizing state-of-the-art structure-guided drug design and crystallography, BioCryst continues to discover innovative compounds with the goal of addressing unmet medical needs of patients and physicians. For more information, please visit the Company's website 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 BioCryst's 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 BioCryst may not receive government funding to support the further development of BCX4430; that HHS/BARDA may further condition, reduce or eliminate future funding of the peramivir program; that BioCryst or its licensees may not be able to enroll the required number of subjects in planned clinical trials of its product candidates and that such clinical trials may not be successfully completed; that the company or its licensees may not commence as expected additional human clinical trials with product candidates; that the FDA may require additional studies beyond the studies planned for product candidates or may not provide regulatory clearances which may result in delay of planned clinical trials, clinical hold with respect to such product candidate or the lack of market approval for such product candidate; that ongoing and future preclinical and clinical development may not have positive results; that the company or its licensees may not be able to continue future development of current and future development programs; that such development programs may never result in future product, license or royalty payments being received; that the companies may not be able to retain its current pharmaceutical and biotechnology partners for further development of its product candidates or may not reach favorable agreements with potential pharmaceutical and biotechnology partners for further development of product candidates; that its actual cash burn rate may not be consistent with its expectations; that 2012 operating expenses and cash usage will be within management's expected ranges. 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 BioCryst's projections and forward-looking statements.

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