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BioCryst Announces Nature Publication Demonstrating Efficacy of BCX4430 in a Non-Human Primate Model of Filovirus Infection

Broad Spectrum Activity Shown Against Multiple RNA Virus Biothreats

RESEARCH TRIANGLE PARK, N.C., March 3, 2014 (GLOBE NEWSWIRE) -- [BioCryst Pharmaceuticals, Inc.](#) (Nasdaq:BCRX) today announced the online publication in the journal [Nature](#) of extensive laboratory and nonclinical characterizations of [BCX4430](#), including efficacy results in animal models of infection with [Marburg virus](#) and [Ebola virus](#), two highly virulent pathogens responsible for viral hemorrhagic fever diseases. The *Nature* online publication, "*Protection against filovirus diseases by a novel broad-spectrum nucleoside analogue BCX4430*," represents the first report of protection of non-human primates from filovirus disease by a small molecule drug, and describes efficacy results generated from an ongoing collaboration between scientists at the [U.S. Army Medical Research Institute of Infectious Diseases](#) (USAMRIID) and BioCryst.

The online publication is available at the following link:

<http://www.nature.com/nature/journal/vaop/ncurrent/full/nature13027.html>

Filoviruses, such as Ebola virus and Marburg virus, are extremely virulent. Case fatality rates associated with filovirus disease outbreaks are the highest reported for any infection, exceeding 90 percent. These pathogens are classified as [Category A Bioterrorism](#) Agents by the Centers for Disease Control and Prevention. BCX4430 completely protected cynomolgus macaques from Marburg virus infection when administered by intramuscular injection 48 hours post-infection. Post-exposure intramuscular administration of BCX4430 also protected rodents against Marburg virus and Ebola virus infections. In addition, BCX4430 was shown to be active in vitro against a broad range of other RNA viruses, including the emerging viral pathogen [Middle East Respiratory Syndrome Coronavirus \(MERS-CoV\)](#).

"Filoviruses, such as Ebola and Marburg virus, constitute serious threats to our national defense," said Colonel Erin P. Edgar, commander of USAMRIID. "Development of cost-effective and versatile treatment options to combat these agents remains an unmet medical need and a high biodefense priority for the U.S. Government."

Developed by BioCryst, BCX4430 has demonstrated antiviral activity in testing conducted at BioCryst, Utah State University/[NIAID](#) and USAMRIID. BCX4430 has been shown to be active against more than 20 RNA viruses in nine different families, including filoviruses, togaviruses, bunyaviruses, arenaviruses, paramyxoviruses, coronaviruses and flaviviruses. In tests conducted at USAMRIID, BCX4430 protected animals against parenteral exposures to Marburg, Ebola and Rift Valley Fever viruses and from exposures to aerosolized Marburg virus, an experimental condition designed to mimic an exposure scenario that could result during a bioterrorist attack.

"With its broad-spectrum antiviral activity, attractive drug-like characteristics and demonstrated efficacy against filoviruses, BCX4430 is well-positioned for continued development as a valuable addition to the nation's arsenal of medical countermeasures," said [Dr. William P. Sheridan, Chief Medical Officer](#) at BioCryst. "A single broad-spectrum agent that treats a range of RNA virus threats, such as BCX4430, presents an efficient one-drug, multi-bug strategic option against high-priority pathogens for the U.S. Government and offers promise as a treatment for patients infected in natural outbreaks."

BCX4430 is being developed as a countermeasure against human filovirus diseases and other viral diseases representing major public health threats. In September 2013, the National Institute of Allergy and Infectious Diseases (NIAID) contracted with BioCryst for the development of BCX4430 as a treatment for Marburg virus disease. In 2013, NIAID awarded funding of \$7.5 million to BioCryst, and total funding of up to \$22.0 million, if all contract options are exercised. The goals of this contract are to file investigational new drug (IND) applications for intravenous and intramuscular BCX4430 for the treatment of Marburg virus disease, and to conduct Phase 1 human clinical trials.

About the BSAV Program & BCX4430

The objective of BioCryst's Broad Spectrum Antiviral (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, which acts by terminating viral RNA synthesis, has demonstrated broad-spectrum activity for multiple viruses and a favorable preliminary preclinical safety profile. BCX4430 has protected animals against viral hemorrhagic fever infections in models of

Marburg virus, Ebola virus and Yellow Fever virus. BioCryst is developing BCX4430 following the Animal Rule regulatory pathway.

This project is being funded in part with Federal funds from the National Institute of Allergy and Infectious Diseases, National Institutes of Health, Department of Health and Human Services, under Contract No. HHSN272201300017C.

About Marburg Virus Disease

Marburg virus is a member of the family *Filoviridae*, along with Ravn virus, Ebola virus, Sudan virus and Bundibugyo virus, all of which cause severe viral hemorrhagic fevers in humans. Reported case fatality rates have been reported to exceed 90 percent. Additional information regarding Marburg virus disease is available online from the Centers for Disease Control and Prevention at:

<http://www.cdc.gov/ncidod/dvrd/spb/mnpages/dispages/marburg/qa.htm>.

About USAMRIID

USAMRIID's mission is to protect the warfighter from biological threats and to be prepared to investigate disease outbreaks or threats to public health. Research conducted at USAMRIID leads to medical solutions-vaccines, drugs, diagnostics and information that benefit both military personnel and civilians. The Institute plays a key role as the lead military medical research laboratory for the Defense Threat Reduction Agency's Joint Science and Technology Office for Chemical and Biological Defense. USAMRIID is a subordinate laboratory of the U.S. Army Medical Research and Materiel Command.

About BioCryst Pharmaceuticals

BioCryst Pharmaceuticals designs, optimizes and develops novel small molecule drugs that block key enzymes involved in infectious and rare diseases, with the goal of addressing unmet medical needs of patients and physicians. BioCryst's core development programs include [BCX4161](#) and two next generation oral inhibitors of plasma kallikrein for hereditary angioedema; [peramivir](#), a viral neuraminidase inhibitor for the treatment of influenza; and [BCX4430](#), a broad spectrum antiviral for hemorrhagic fevers. 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 are 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 or its licensees may not be able to enroll the required number of subjects in planned clinical trials of BCX4430 and that such clinical trials may not be successfully completed; that ongoing and future preclinical and clinical development may not have positive results; that the FDA may require additional studies beyond the studies planned for BCX4430, or may not provide regulatory clearances which may result in delay of planned clinical trials, or may impose a clinical hold upon BCX4430, or withhold market approval for BCX4430; that the Company may not be able to obtain additional funding for BCX4430; that government funding or other contracts for BCX4430 may have certain terms and conditions, including termination provisions, that subject the Company to additional risks and the Company may lose current funding for the program; that the Company may not be able to continue development of BCX4430 for any number of reasons; that the Company may never file an IND for BCX4430; that any product, including peramivir may never be approved for any use by the FDA; that the Company or its licensees may not be able to continue development of ongoing and future development programs; that such development programs may never result in future product, license or royalty payments being received; that the Company 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 financial results may not be consistent with its expectations. 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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