



October 30, 2012

## **BioCryst Provides Update Regarding BCX5191 Development Plan Following Discussion With FDA**

RESEARCH TRIANGLE PARK, N.C.--(BUSINESS WIRE)-- [BioCryst Pharmaceuticals, Inc.](#) (NASDAQ:BCRX) today announced the withdrawal of its Investigational New Drug application (IND) for the antiviral nucleoside, [BCX5191](#), following a discussion with the U.S. Food and Drug Administration (FDA).

The FDA indicated concerns regarding the preclinical toxicity profile of BCX5191 at exposure levels that they believe are likely to be necessary to reduce viral load in patients infected with the hepatitis C virus (HCV). Patient safety remains BioCryst's highest priority. BioCryst continues to believe that BCX5191 may be distinct from other Nucs in exhibiting antiviral potency in man at significantly lower doses than other Nucs in development based on preclinical results, and will therefore conduct additional preclinical studies to determine if low doses—i.e. doses that are not associated with toxicity in animals—exhibit meaningful viral load reductions in HCV infected animals. BioCryst will then determine whether to continue development of BCX5191, based on the results of these studies.

BioCryst agrees with the FDA's cautious approach to the development of nucleoside and nucleotide inhibitors for HCV. Further, BioCryst believes that the recent occurrence of serious adverse events in HCV patients treated with BMS-986094, a nucleotide prodrug previously under clinical development by Bristol-Myers Squibb, has heightened safety concerns regarding this class of HCV inhibitors. FDA has previously placed clinical holds on other nucleotides under development.

### **About BCX5191**

Discovered by BioCryst, BCX5191 is a novel, oral, pan-genotypic adenine nucleoside analog targeting viral RNA polymerase for the potential treatment of patients with HCV. BCX5191 inhibits the viral RNA polymerase enzyme across genotypes 1-4 at sub-micromolar concentrations and it is active in replicon cell assays. In human liver cells, BCX5191 rapidly and efficiently converts into its active triphosphate form. In preclinical models, BCX5191 demonstrates high oral bioavailability and is actively transported into the liver, without requiring prodrug technology. PK modeling supports once-daily dosing in clinical studies and predicts that low doses of BCX5191 will show antiviral activity.

### **About Hepatitis C**

Hepatitis C is a contagious liver disease that results from infection with the hepatitis C virus (HCV), which is the most common virus that infects the liver and can lead to life-threatening liver problems, such as liver damage, cirrhosis, liver failure or liver cancer. There are an estimated 170 million individuals worldwide who are chronically infected with HCV, and about 3 to 4 million people are infected annually. In the United States, there are approximately 4 million people who have chronic hepatitis C.

### **Conference Call and Webcast**

BioCryst's leadership team will host a conference call and webcast Wednesday, October 31, 2012 at 8:30 a.m. Eastern Time. To participate in the conference call, please dial 1-877-303-8027 (United States) or 1-760-536-5165 (International). No passcode is needed for the call. The webcast can be accessed by logging onto [www.BioCryst.com](#). Please connect to the website at least 15 minutes prior to the start of the conference call to ensure adequate time for any software download that may be necessary.

### **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 advancing two preclinical programs: 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](#).

## About the BioCryst-Presidio Merger

BioCryst and privately held [Presidio Pharmaceuticals, Inc.](#) recently announced the signing of a definitive [merger agreement](#) to create a focused, clinical stage biopharmaceutical company with lead programs in high-value infectious and orphan disease indications: specifically HCV and hereditary angioedema (HAE).

## Important Additional Information and Where to Find It

BioCryst intends to file with the Securities and Exchange Commission ("SEC") a registration statement on Form S-4, which will also include a proxy statement and prospectus with respect to its proposed acquisition of Presidio. The final proxy statement/prospectus will be mailed to the stockholders of BioCryst and Presidio. Investors and security holders are urged to read the proxy statement/prospectus regarding the proposed transaction carefully and in its entirety when it becomes available because it will contain important information regarding BioCryst, Presidio and the proposed merger. Investors will be able to obtain a free copy of the proxy statement/prospectus, as well as other filings containing information about BioCryst, without charge, at the SEC's website (<http://www.sec.gov/>). Investors may also obtain these documents, without charge, from BioCryst's website at <http://investor.shareholder.com/biocryst/sec.cfm>.

This communication shall not constitute an offer to sell or the solicitation of an offer to buy any securities in the equity financing connected to the proposed acquisition of Presidio.

## Participants in the Merger Solicitation

BioCryst and its directors, executive officers and other members of management and employees may be deemed to be participants in the solicitation of proxies from shareholders with respect to the transactions contemplated by the definitive merger agreement signed by Presidio. Information regarding BioCryst's directors and executive officers is contained in BioCryst's 2011 Annual Report on Form 10-K filed with the SEC on March 6, 2012 and its definitive proxy statement filed with the SEC on April 9, 2012 in connection with its 2012 meeting of stockholders. Other information regarding the participants in the proxy solicitation and a description of their direct and indirect interests, by security holdings or otherwise, will be contained in the proxy statement/prospectus and other relevant materials to be filed with the SEC when they become available.

## 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 the FDA may require additional studies beyond the studies planned for product candidates or may not provide regulatory clearances (e.g. BCX5191) which may result in delay of planned clinical trials, clinical hold with respect to such product candidate or inability to move forward with development 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 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 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 actual cash burn rate may not be consistent with expectations; that the peramivir interim analysis may not be favorable or that BARDA/HHS may further condition, reduce or eliminate future funding of the peramivir program; that the planned merger with Presidio might not be completed for any number of reasons, most of which are outside of the control of BioCryst; that BioCryst may not be able to obtain the requisite financing to complete the planned merger with Presidio on commercially reasonable terms or that or that the financing may be raised at prices below the currently prevailing price for BioCryst common stock; that integration of BioCryst and Presidio may prove more challenging than anticipated or that anticipated benefits of the merger may not be achieved, or may be achieved less rapidly than anticipated; the outcome of any legal proceedings that may be instituted against BioCryst or Presidio; risks relating to any unforeseen liabilities, future capital expenditures, revenues, expenses, earnings, economic performance, indebtedness, financial condition, losses and future prospects, business and management strategies or the expansion and growth of Presidio's operations; BioCryst's ability to integrate Presidio's business successfully after the closing of the merger agreement; and the risk that disruptions from the merger agreement will harm BioCryst's or Presidio's businesses. There can be no assurance that the proposed merger and financing will in fact be consummated. Other important factors include: that there can be no assurance that BioCryst's or Presidio's compounds will prove effective in clinical trials; that development and commercialization of BioCryst's or Presidio's compounds may not be successful; that BioCryst, Presidio or 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 companies or licensees may not commence as expected additional human clinical trials with product candidates; that 2012 operating expenses and cash usage will be within management's expected ranges; that BioCryst or Presidio may not have

sufficient cash to continue funding the development, manufacturing, marketing or distribution of products and that additional funding, if necessary, may not be available at all or on terms acceptable to BioCryst or Presidio. 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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Source: BioCryst Pharmaceuticals, Inc.

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