



July 22, 2013

BioCryst Successfully Completes Its Phase 1 Clinical Trial of BCX4161 for the Treatment of Hereditary Angioedema

- *BioCryst initiating a Phase 2a trial in hereditary angioedema patients in 2013*
- *Management to discuss results during conference call at 8:30 a.m. Eastern Time*

RESEARCH TRIANGLE PARK, N.C.--(BUSINESS WIRE)-- [BioCryst Pharmaceuticals, Inc.](#) (NASDAQ:BCRX) today announced that the randomized, placebo-controlled, Phase 1 clinical trial of orally-administered [BCX4161](#) in healthy volunteers successfully met all of its objectives. The safety, tolerability, drug exposure and on-target kallikrein inhibition results of this Phase 1 trial strongly support advancing the development program into a Phase 2a study in hereditary angioedema (HAE) patients.

Overall, 87 healthy volunteers completed the study; 30 received a single dose of BCX4161 from 50 mg up to 1000 mg, 40 subjects received 100 mg, 200 mg, 400 mg, or 800 mg BCX4161 every eight hours for seven days and 17 received placebo.

Oral administration of BCX4161 was generally safe and well tolerated. There were no serious adverse events and no dose limiting adverse events. Laboratory tests of coagulation remained normal. Drug exposure was dose proportional through 400 mg three times a day. Steady state (day seven) blood levels were 30% higher compared to the first day of dosing. At 400 mg three times a day, pre-dose geometric mean (coefficient of variance, CV) drug levels on day 7 were 28.6 ng/mL (CV 77%) and post-dose maximum drug levels were 152 ng/mL (CV 57%). Kallikrein inhibition was observed throughout the dosing interval, $p < 0.0001$ compared to placebo.

"We are very pleased that this first-in-human trial of BCX4161 met all of its objectives. The safety, tolerability, level and consistency of drug exposure and kallikrein inhibition achieved significantly increases our confidence to move this program forward," said [Dr. William P. Sheridan, Chief Medical Officer](#) at BioCryst. "We look forward to conducting a Phase 2 proof of concept study later this year to evaluate BCX4161's ability to reduce the frequency of edema attacks in HAE patients."

"The successful development of an oral kallikrein inhibitor such as BCX4161 for the prevention of HAE attacks is an exciting advance that has significant implications for HAE treatment," said [Dr. Bruce Zuraw, M.D.](#), Professor, University of California, San Diego and Staff Physician at the V.A. Medical Center, San Diego. "If BCX4161 fulfills the promise shown in this study, it will provide a more effective alternative to androgens, currently the mainstay of oral HAE treatment, with substantially less side effects. BCX4161 has the potential to be an important new treatment for optimally managing patients with HAE."

The Phase 2a clinical trial in patients with HAE is expected to begin in the fourth quarter of 2013. This trial will test 400 mg of BCX4161 administered three times daily for 28 days in a randomized, placebo-controlled, two-period cross-over design. Approximately 25 HAE patients who have a high frequency of attacks (≥ 1 per week) will be enrolled. The main goals for this clinical trial are to evaluate the safety and tolerability of BCX4161 and to estimate the degree of efficacy in reducing the frequency of attacks. This study is designed to provide proof of concept for oral kallikrein inhibition as a treatment strategy for hereditary angioedema.

As a part of BioCryst's strategy to become a leader in the treatment of HAE, we are finalizing our evaluation of multiple potent and specific second generation oral kallikrein inhibitors. Oral bioavailability of these compounds ranges between 20% and 60% in animals. One or more candidates are expected to enter preclinical development by the end of 2013.

Conference Call and Web Cast

BioCryst's management team will host a conference call and webcast today, July 22, 2013 at 8:30 a.m. Eastern Time to discuss the results of the BCX4161 Phase 1 trial and other aspects of BioCryst's HAE development program. 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 <http://www.biocryst.com>. Please connect to the web site 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 BCX4161

Discovered by BioCryst, BCX4161 is a novel, selective inhibitor of plasma kallikrein in development for prevention of attacks in

patients with hereditary angioedema (HAE). By inhibiting plasma kallikrein, BCX4161 suppresses bradykinin production. Bradykinin is the mediator of acute swelling attacks in HAE patients.

About Hereditary Angioedema

HAE is a rare, severely debilitating and potentially fatal genetic condition that occurs in about 1 in 10,000 to 1 in 50,000 people. HAE symptoms include recurrent episodes of edema in various locations, including the hands, feet, face, genitalia and airway. In addition, patients often have bouts of excruciating abdominal pain, nausea and vomiting that are caused by swelling in the intestinal wall. Airway swelling is particularly dangerous and can lead to death by asphyxiation. Further information regarding HAE can be found at www.haea.org.

About BioCryst Pharmaceuticals

BioCryst Pharmaceuticals designs, optimizes and develops novel small molecule drugs that block key enzymes involved in infectious and inflammatory diseases, with the goal of addressing unmet medical needs of patients and physicians. BioCryst currently has two late-stage development programs: [peramivir](#), a viral neuraminidase inhibitor for the treatment of influenza, and [ulodesine](#), a purine nucleoside phosphorylase (PNP) inhibitor for the treatment of gout. In addition, BioCryst has several early-stage programs: BCX4161 and a next generation oral inhibitor of plasma kallikrein for hereditary angioedema 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 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 be able to enroll the required number of subjects in the Phase 2a clinical trial of BCX4161; that the Phase 2a trial of BCX4161 may not have a favorable outcome or may not be successfully completed; that the FDA or similar regulatory agency may refuse to approve subsequent studies, or delay approval of clinical studies which may result in a delay of planned clinical studies and increase development costs of a product candidate; that the FDA may withhold market approval for product candidates; that ongoing and future preclinical and clinical development of HAE second generation candidates 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 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. 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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