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BioCryst Initiates OPuS-1: a Phase 2a Clinical Trial of BCX4161 in Patients with Hereditary Angioedema

RESEARCH TRIANGLE PARK, N.C.--(BUSINESS WIRE)-- [BioCryst Pharmaceuticals, Inc.](#) (NASDAQ:BCRX) today announced that it has dosed the first subject in OPuS-1 (**O**ral **P**rophylaxis-1), a Phase 2a proof of concept clinical trial of orally-administered [BCX4161](#) in patients with hereditary angioedema (HAE).

The OPuS-1 trial will test 400 mg of BCX4161 administered three times daily for 28 days in up to 25 HAE patients who have a high frequency of attacks (≥ 1 per week), in a randomized, placebo-controlled, two-period cross-over design. The main goals for the OPuS-1 trial are to estimate BCX4161's degree of efficacy in reducing the frequency of angioedema attacks, and to evaluate the safety and tolerability of 28 days of BCX4161 treatment.

"We look forward to obtaining the results of OPuS-1, the first trial of an oral kallikrein inhibitor in HAE patients. The safety, tolerability, drug exposure and kallikrein inhibition achieved in the Phase 1 healthy volunteer trial of BCX4161 strongly support its further evaluation in HAE patients," said [Dr. William P. Sheridan, Chief Medical Officer](#) at BioCryst. "OPuS-1 is another step toward our goal of improving HAE patients' lives by dramatically changing the management of this disease."

The Phase 2a OPuS-1 trial is being conducted at up to four centers in Germany and will evaluate the efficacy, safety, tolerability, pharmacokinetics and pharmacodynamics of BCX4161. Each subject will receive BCX4161 and placebo in two separate 28-day periods, with the order of therapy randomized (placebo followed by BCX4161 or BCX4161 followed by placebo). The primary efficacy endpoint for the OPuS-1 trial is the mean attack frequency in each period. Other efficacy measures include average severity of attacks, the number of attack-free days and quality of life.

As a part of BioCryst's strategy to become a leader in the treatment of HAE, its scientists are finalizing the evaluation of multiple potent and specific second generation oral kallikrein inhibitors, with the goal of developing compounds with potential for once daily dosing. One to three candidates are expected to enter preclinical development before the end of 2013.

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's core development programs include BCX4161 and a next generation oral inhibitor 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 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 Phase 2a trial may cost more or take longer to complete than expected; 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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BioCryst Pharmaceuticals
Robert Bennett, 919-859-7910

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