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BioCryst Announces Initiation of a Phase 1 Clinical Trial of BCX4161 for the Treatment of Hereditary Angioedema

RESEARCH TRIANGLE PARK, N.C.--(BUSINESS WIRE)-- [BioCryst Pharmaceuticals, Inc.](#) (NASDAQ:BCRX) today announced the initiation of a Phase 1 clinical trial to evaluate the safety, pharmacokinetics and pharmacodynamics of orally-administered [BCX4161](#) in healthy volunteers. Discovered by BioCryst, BCX4161 is a novel, selective inhibitor of plasma kallikrein in development for prevention of attacks in patients with hereditary angioedema (HAE).

"Daily, oral administration of a safe and efficacious prophylactic drug would revolutionize treatment for patients suffering from this serious condition," said [Jon P. Stonehouse, President & Chief Executive Officer](#) of BioCryst. "BCX4161 has the potential to be the first oral treatment developed specifically for the prevention of HAE attacks."

BioCryst has successfully completed nonclinical safety studies, as well as *in vitro* and *in vivo* studies in which BCX4161 exhibited potent and selective inhibition of plasma kallikrein and a favorable safety profile. A poster titled "[BCX4161, A Small Molecule Orally Bioavailable Plasma Kallikrein Inhibitor, for the Treatment of Hereditary Angioedema](#)" was presented at the 2013 American Academy of Allergy, Asthma & Immunology (AAAAI) Annual Meeting in February.

The main goals of the Phase 1 clinical trial are to assess safety, characterize plasma drug levels, and estimate the extent of kallikrein inhibition achieved after oral dosing of BCX4161. This clinical trial is being conducted at Quotient Clinical in the United Kingdom utilizing an integrated Translational Pharmaceuticals™ platform and the results are expected to be announced in 2013. If the Phase 1 program achieves its goals, BioCryst plans to initiate a Phase 2 program 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 clinical trial for BCX4161, that the BCX4161 trial may not have a favorable outcome or may not be successfully completed; that the Company may not commence additional human clinical trials for BCX4161; that the FDA or similar regulatory agency may refuse to approve subsequent studies, may impose a clinical hold for BCX4161 or other product candidates, 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 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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