



December 18, 2013

BioCryst Advances Second Generation Oral Plasma Kallikrein Inhibitors for Hereditary Angioedema into Preclinical Development

RESEARCH TRIANGLE PARK, N.C.--(BUSINESS WIRE)-- [BioCryst Pharmaceuticals, Inc.](#) (NASDAQ:BCRX) today announced that it has selected two optimized plasma kallikrein inhibitors to advance into preclinical development as potential once-daily, oral treatments for the prevention of hereditary angioedema (HAE) attacks.

The second generation discovery program's goals of improving selectivity and bioavailability compared to [BCX4161](#) were both met, with no effect on prothrombin time at high concentrations (> 50 micromolar), and oral fraction absorbed exceeding 25%. Similar to BCX4161, these BioCryst discovered compounds demonstrate sub-nanomolar potency on the isolated enzyme and single digit nanomolar potency in suppressing kallikrein activity in an ex vivo activated human plasma kallikrein inhibition (aPKI) assay. Plasma concentrations of each of the optimized compounds exceeded the aPKI assay EC₈₀ concentration at 24 hours after a single oral dose of 10 mg/kg in rats, indicating suitability for once-daily dosing.

"We are pleased with the outcome of our discovery program for second-generation plasma kallikrein inhibitors," said [Yarlagadda S. Babu, Ph.D., Senior Vice President, Drug Discovery](#) at BioCryst. "We look forward to evaluating nonclinical safety in the preclinical development phase, and to providing updates regarding our progress towards our goal of developing a once-daily prophylactic therapy for hereditary angioedema."

About BCX4161

Discovered by BioCryst, BCX4161 is a novel, selective inhibitor of plasma kallikrein in Phase 2 development for prevention of attacks in patients with hereditary angioedema. 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 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 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 ongoing and future preclinical and clinical development of BCX4161 and HAE second generation candidates may not have positive results; that either or both second generation candidates may not advance beyond preclinical development; 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 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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Source: BioCryst Pharmaceuticals, Inc.

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