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## BioCryst Announces Initiation of the APeX-1 Clinical Trial of BCX7353 for Hereditary Angioedema

RESEARCH TRIANGLE PARK, N.C., Aug. 11, 2016 (GLOBE NEWSWIRE) -- [BioCryst Pharmaceuticals, Inc.](http://www.biocryst.com) (NASDAQ:BCRX) today announced that it has dosed the first subject in the APeX-1 clinical trial of BCX7353 for the oral treatment of hereditary angioedema (HAE).

"We are very pleased that the adaptively-designed APeX-1 trial is now under way, and look forward to reporting Part 1 results around the end of 2016," said [William P. Sheridan, SVP](#) & Chief Medical Officer at BioCryst. "Results of the phase 1 study of '7353 support its potential to provide a normal life to HAE patients as a once-daily oral treatment by increasing kallikrein inhibition to normal levels."

APeX-1 is a two part, Phase 2, randomized, double-blind, placebo-controlled dose ranging trial to evaluate the safety, tolerability, pharmacokinetics, pharmacodynamics and efficacy of BCX7353 as a preventative treatment to eliminate or reduce the frequency of angioedema attacks in HAE patients. Up to approximately 50 eligible subjects with HAE will be enrolled in the study.

"The APeX-1 trial is an exciting opportunity for the HAE patient community," said [Dr. Emel Aygören-Pürsün, M.D.](#), Head of Angioedema Clinic, Goethe University Hospital Frankfurt/Main Pediatric Clinic, Frankfurt, Germany, and Principal Investigator for APeX-1. "The value of an effective & well tolerated oral preventive treatment for HAE patients cannot be overestimated."

### About APeX-1

In part 1 of APeX-1, subjects with HAE will be randomized in a 1:1 ratio to receive an oral dose of either 350 mg of BCX7353 once daily or placebo once daily for four weeks. An interim analysis will be conducted after the first 24 subjects have completed treatment through study day 28. If a robust treatment effect is observed at the interim analysis, Part 2 of the study will be initiated. In the event the treatment effect is not well characterized with 24 subjects, a total of up to approximately 36 subjects will be enrolled in part 1. The sample size in Part 1 was kept flexible to cover a range of response options that would achieve 90% power with an alpha of 0.05, based on reduction of attack rate of at least 70% on BCX7353, placebo response rate of approximately 30%, and standard deviation of approximately 0.45 attacks per week.

To characterize dose-response in part 2 of APeX-1, 14 additional subjects with HAE will be randomized to 250mg of BCX7353 once daily (n=6), 125mg of BCX7353 once daily (n=6) or placebo (n=2).

The primary efficacy endpoint of APeX-1 is the number of angioedema attacks; attack rate per week, counts of attacks, proportion of subjects with no attacks, and number of attack-free days will be analyzed. Efficacy analyses will be conducted for HAE attacks reported over the entire dosing interval (Days 1 through 28) and during the dosing period in which plasma concentrations of BCX7353 should be at steady-state conditions (Days 8 through 28). Secondary efficacy endpoints include severity and duration of angioedema attacks, and measures of health-related quality of life. Safety will be characterized through evaluation of adverse events and laboratory testing. Pharmacokinetics and pharmacodynamic effects will be assessed through measurement of plasma drug levels and kallikrein inhibition.

Additional details regarding the APeX-1 trial design will be posted to [www.clinicaltrials.gov](http://www.clinicaltrials.gov)

### About BCX7353

Discovered by BioCryst, BCX7353 is a novel, once-daily, selective inhibitor of plasma kallikrein in development for the prevention of angioedema attacks in patients diagnosed with HAE. By inhibiting plasma kallikrein, BCX7353 suppresses bradykinin production. Bradykinin is the mediator of acute swelling attacks in HAE patients. BCX7353 has been generally safe and well tolerated in clinical pharmacology studies that have enrolled 117 healthy volunteers, 46 receiving single doses of up to 1000 mg, and 71 receiving once-daily doses of up to 500 mg for 7 days and 350 mg for 14 days. In the second week of study, approximately 5% of healthy volunteers administered daily doses of '7353 for at least 7 days developed a drug-related skin rash that resolved within a few days.

## About Hereditary Angioedema

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

## About BioCryst Pharmaceuticals

BioCryst Pharmaceuticals designs, optimizes and develops novel small molecule drugs that block key enzymes involved in rare diseases. BioCryst's ongoing development programs include oral plasma kallikrein inhibitors for hereditary angioedema; BCX7353 and other second generation compounds, and [BCX4430](#), a broad spectrum viral RNA polymerase inhibitor. For more information, please visit the Company's website at [www.BioCryst.com](http://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 2 clinical trial of BCX7353; that the Phase 2 trial of BCX7353 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, delay approval of clinical studies or require other changes to our development plan, which may result in a delay of planned clinical studies and increase development costs of a product candidate, including BCX7353; that the FDA may withhold market approval for BCX7353; that ongoing and future preclinical and clinical development of second generation kallikrein inhibitor 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. 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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