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BioCryst Announces Successful Phase 1 Clinical Trial of BCX7353

- *Advancing once-daily BCX7353 into a Phase 2 trial in hereditary angioedema patients*

RESEARCH TRIANGLE PARK, N.C., Oct. 8, 2015 (GLOBE NEWSWIRE) -- [BioCryst Pharmaceuticals, Inc.](http://www.biocryst.com) (NASDAQ:BCRX) today announced that the randomized, placebo-controlled, Phase 1 clinical trial of orally-administered [BCX7353](#) in healthy volunteers successfully met all of its objectives. The safety, tolerability, drug exposure and on-target plasma kallikrein inhibition results strongly support advancing the development program into a Phase 2 study in hereditary angioedema (HAE) patients.

Oral BCX7353 was generally safe and well tolerated at all doses up to 500 mg once-daily for 7 days and 350 mg once-daily for 14 days in healthy volunteers, and no dose-limiting toxicity was identified. There were no serious adverse events (AEs) and most AEs were mild. Two subjects discontinued the study due to moderate gastrointestinal AEs. One subject developed a delayed-type hypersensitivity rash after completing seven days of study drug; the rash resolved quickly with oral and topical steroids. No clinically significant laboratory abnormalities were seen at any dose or duration tested.

BCX7353 plasma levels increased in approximate proportion to dose, and drug exposure was not affected by dosing with food. The half-life of BCX7353 was estimated at 50-60 hours. After daily dosing, blood levels met or exceeded a predicted target therapeutic range throughout the 24 hour dosing interval.

Inhibition of the target enzyme, plasma kallikrein, was measured in a sensitive and specific bioassay. Daily dosing with BCX7353 strongly inhibited plasma kallikrein at all four dose levels; the degree of inhibition was dose-related ($p < 0.0001$) and inhibition was sustained throughout the 24 hour dosing interval. This pharmacodynamic effect correlated strongly to achieved drug concentration ($r = 0.91$, $p < 0.0001$).

"We are very pleased that our first-in-human trial of BCX7353 met all of its objectives. The results show '7353 to be generally safe and well tolerated, and the observed drug exposure and kallikrein inhibition reaffirms our expectation that '7353 has the potential to be a once-daily treatment to wipe out HAE attacks," said Jon Stonehouse, Chief Executive Officer & President of BioCryst. "We are excited to now have the opportunity to test this promising drug in patients with HAE."

A Phase 2, four week dose ranging trial to evaluate the safety, tolerability, pharmacokinetics, pharmacodynamics, and efficacy of BCX7353 as a preventative treatment to reduce the frequency of attacks in HAE patients is expected to begin by late 2015 or early 2016.

About the Study

Overall, 94 healthy volunteers were enrolled, and 92 completed the study. In the single dose part of the study, 34 subjects received a single dose of BCX7353 ranging from 30 mg up to 1000 mg, and 10 subjects received a single dose of placebo. In the daily dosing part of the study, 30 subjects received BCX7353 daily for seven days (10 each at 125 mg, 250 mg, and 500 mg per day), 10 subjects received 350 mg of BCX7353 daily for 14 days, and a total of 20 subjects received daily doses of placebo.

Conference Call and Web Cast

BioCryst's management team will host a conference call and webcast today, October 8th, 2015 at 8:30 a.m. Eastern Time to discuss the results of the BCX7353 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 BCX7353

Discovered by BioCryst, BCX7353 is a novel, once-daily, selective inhibitor of plasma kallikrein in development for the prevention of hereditary angioedema (HAE) 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.

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.

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; [avoralstat](#), 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.

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 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. 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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