



December 18, 2014

BioCryst Initiates OPuS-2: A Clinical Trial of BCX4161 in Patients With Hereditary Angioedema

RESEARCH TRIANGLE PARK, N.C., Dec. 18, 2014 (GLOBE NEWSWIRE) -- [BioCryst Pharmaceuticals, Inc.](#), (Nasdaq:BCRX) today announced that it has dosed the first patient in [OPuS-2](#) (Oral Prophylaxis-2), a blinded, randomized, placebo-controlled clinical trial of orally-administered [BCX4161](#) in patients with hereditary angioedema (HAE).

OPuS-2 is a 12-week, three-arm, parallel cohort design trial to evaluate the efficacy and safety of two doses of BCX4161, 300 mg and 500 mg, administered three-times daily compared with placebo. This trial, to be conducted in the U.S. and selected European countries, is expected to enroll approximately 100 HAE patients. The primary efficacy endpoint for the trial will be the mean angioedema attack rate for each BCX4161 dose group compared to placebo.

"The OPuS-2 trial will provide important information on the efficacy and safety of 12 weeks of oral BCX4161 for prevention of angioedema attacks in HAE patients. OPuS-2 builds on the positive efficacy, safety, tolerability, drug exposure and kallikrein inhibition results from the [OPuS-1](#) 4-week study," said [Dr. Marc Riedl](#), M.D., M.S., Associate Clinical Professor at the University of California-San Diego School of Medicine, Clinical Director of the U.S. HAEA Angioedema Center and OPuS-2 Principal Investigator. "OPuS-2 has been designed as an adequate and well-controlled study and represents an important next step toward reaching our goal of improving HAE patients' lives using oral kallikrein inhibitors," added [Dr. William P. Sheridan](#), Chief Medical Officer at BioCryst.

In May 2014, BioCryst announced positive results from the OPuS-1 (Oral Prophylaxis-1) proof of concept Phase 2a clinical trial of orally-administered [BCX4161](#) in patients with HAE. The trial met the primary efficacy endpoint, several secondary endpoints and all other objectives established for the trial. The primary efficacy endpoint for the trial was the by-subject difference in mean angioedema attack rate on BCX4161 compared to placebo. Treatment with BCX4161 demonstrated a statistically significant mean attack rate reduction of 0.45 attacks per week versus placebo, $p < 0.001$. The mean attack rate per week was 0.82 on BCX4161 treatment, compared to 1.27 on placebo.

Discovered by BioCryst, BCX4161 is a novel, selective inhibitor of plasma kallikrein in development for prevention of attacks in patients with 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 rare diseases. BioCryst currently has several ongoing development programs: oral inhibitors of plasma kallikrein for hereditary angioedema, including [BCX4161](#) and several second generation compounds; [RAPIVAB](#)TM (peramivir injection), a viral neuraminidase inhibitor for the treatment of influenza, 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 OPuS-2 trial of BCX4161; that the OPuS-2 trial may not have a favorable outcome or may not be successfully completed; that the OPuS-2 trial may cost more or take longer to complete than expected; that the FDA or similar regulatory agency may refuse to approve subsequent HAE studies, or delay approval of clinical studies which may result in a delay of other planned clinical studies and increased development costs of BCX4161; that the FDA may withhold market approval for BCX4161 or 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.

BCRXW

CONTACT: Robert Bennett, BioCryst Pharmaceuticals, +1-919-859-7910



Source: BioCryst Pharmaceuticals

News Provided by Acquire Media