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BioCryst Announces Positive Results From OPuS-1, a Phase 2 Trial of BCX4161 for the Prophylactic Treatment of Hereditary Angioedema

Significant reduction in HAE attacks by 0.45 per week versus placebo (p < 0.001)

RESEARCH TRIANGLE PARK, N.C., May 27, 2014 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc., (Nasdaq:BCRX) today announced preliminary results from its OPuS-1 (**O**ral **P**rophylaxi**S-1**) proof of concept Phase 2a clinical trial of orally-administered BCX4161 in patients with hereditary angioedema (HAE). The trial met the primary efficacy endpoint, several secondary endpoints and all other objectives established for the trial.

OPuS-1 evaluated 400 mg of BCX4161 administered three times a day for 28 days in HAE patients with a high attack frequency (≥ 1 per week), in a randomized, placebo-controlled, two-period cross-over design. The primary goals for the trial were to estimate the degree of efficacy of BCX4161 in reducing the frequency of angioedema attacks, and to evaluate the safety and tolerability of 28 days of BCX4161 treatment.

Twenty-four patients received study drug, and all completed the study. 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.

Oral administration of BCX4161 was generally safe and well tolerated, with an adverse event profile similar to that observed for placebo. There was one serious adverse event reported, an abdominal HAE attack during the placebo period. Patient dosing compliance was 98 percent.

The mean number of attack-free days during each treatment period improved from 19 for placebo to 22 for BCX4161, p=0.008. Three subjects were attack-free during the BCX4161 period, compared to none during the placebo period. Quality of life was measured by the Angioedema Quality of Life questionnaire, AeQoL, and disease activity by the Angioedema Activity Score, AAS28. For BCX4161, the mean total AeQoL score improved by 8.4 units from baseline compared to 0.5 for placebo, p=0.004, and the AAS28 was 21.4 for BCX4161 compared to 28.8 for placebo, p=0.022.

Plasma drug concentrations and the degree of plasma kallikrein inhibition achieved after oral dosing with BCX4161 in OPuS-1 HAE patients were similar to those seen in healthy subjects in the Phase 1 trial. In OPuS-1, higher drug exposure was associated with a better clinical outcome.

"OPuS-1 represents a milestone study in establishing the proof of concept that prophylaxis with an oral kallikrein inhibitor can effectively reduce attacks for patients living with HAE," said Marcus Maurer MD, Professor of Dermatology and Allergy, Charité-Universitätsmedizin, Berlin, and the principal investigator for the study. "Existing therapies for prophylaxis of attacks in patients with HAE require frequent i.v. infusions, or the use of oral androgens that have significant long term side effects. The OPuS-1 results open up the possibility of an exciting new treatment option for this challenging disease."

"The efficacy and safety profile of BCX4161 seen in the OPuS-1 trial strongly support its continued development," said Dr. William P. Sheridan, Chief Medical Officer at BioCryst. "We look forward to working with clinical investigators, the HAE community and regulatory authorities in advancing BCX4161 to the next stage and starting the OPuS-2, 12-week trial later this year."

Conference Call and Web Cast

BioCryst's management team will host a conference call and webcast today, May 27, 2014 at 8:30 a.m. Eastern Time to discuss the results of the BCX4161 OPuS-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 BCX4161

Discovered by BioCryst, BCX4161 is a novel, selective inhibitor of plasma kallikrein in development for prevention of attacks in patients with hereditary angioedema (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 infectious and rare 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 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 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.

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