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BioCryst Receives Orphan Drug Designation for BCX4161 for the Treatment of Hereditary Angioedema

RESEARCH TRIANGLE PARK, N.C., Dec. 23, 2014 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc., (Nasdaq:BCRX) today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation to BCX4161 for the prevention of acute attacks of angioedema in patients with hereditary angioedema (HAE).

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.

Orphan drug designation is granted by the FDA Office of Orphan Products Development to novel drugs intended for the safe and effective treatment of a rare disease or condition that affects fewer than 200,000 patients in the U.S. This designation provides certain incentives, including federal grants, tax credits, waiver of PDUFA filing fees and a seven-year marketing exclusivity period against competition, once the product is approved.

The approval of an orphan drug designation request does not alter the standard regulatory requirements and processes for obtaining marketing approval of an investigational drug. Sponsors must establish safety and efficacy of a compound in the treatment of a disease through adequate and well-controlled studies.

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 patient-week versus placebo, $p < 0.001$. The mean attack rate per patient-week was 0.82 on BCX4161 treatment, compared to 1.27 on placebo. On December 17, 2014, the first patient was dosed in the OPuS-2 trial, a double-blind, randomized, placebo controlled trial which will evaluate the efficacy and safety of BCX4161 treatment for 12 weeks in patients with HAE.

About Hereditary Angioedema

HAE is a rare, severely debilitating and potentially fatal genetic condition that occurs in about 1 in approximately 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 currently has several ongoing development programs: oral inhibitors of plasma kallikrein for hereditary angioedema, including BCX4161 and several second generation compounds; BCX4430, a broad spectrum viral RNA polymerase inhibitor, and RAPIVAB™ (peramivir injection), a viral neuraminidase inhibitor for the treatment of influenza. 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 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.

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