



January 14, 2015

BioCryst Receives Positive Opinion on European Orphan Drug Designation for BCX4161 for the Treatment of Hereditary Angioedema

RESEARCH TRIANGLE PARK, N.C., Jan. 14, 2015 (GLOBE NEWSWIRE) -- [BioCryst Pharmaceuticals, Inc.](#), (Nasdaq:BCRX) today announced that the [Committee for Orphan Medicinal Products](#) (COMP) of the [European Medicines Agency](#) (EMA) issued a positive opinion on the application for orphan drug designation for BCX4161 for the treatment of patients with hereditary angioedema (HAE). The European Commission will make a final decision on European Orphan Drug Designation based upon the COMP positive opinion. On December 23, 2014, the [U.S. Food and Drug Administration](#) (FDA) granted orphan drug designation for [BCX4161](#).

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 by the EMA provides regulatory and financial incentives for companies to develop and market therapies that treat a life-threatening or chronically debilitating condition affecting no more than five in 10,000 persons in the European Union (EU), and where no satisfactory treatment is available, or where a treatment exists, as with HAE, a new treatment such as BCX4161 may provide a significant benefit to patients affected by the condition. In addition to a 10-year period of marketing exclusivity in the EU after product approval, orphan drug designation provides incentives for companies seeking protocol assistance from the EMA during the product development phase, and direct access to the centralized authorization procedure.

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 conducted in the U.S. and certain EU countries, which will evaluate the efficacy and safety of BCX4161 treatment for 12 weeks in patients with HAE. BioCryst expects to report results from OPuS-2 by the end of 2015.

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, and [BCX4430](#), a broad spectrum viral RNA polymerase inhibitor. In December 2014, [RAPIVAB](#)TM (peramivir injection), a viral neuraminidase inhibitor for the treatment of influenza, was approved by the FDA and is available to treat flu patients in the U.S. 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, EMA 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 regulatory agencies may withhold market approval for BCX4161; 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.

BCRXW

CONTACT: Robert Bennett, BioCryst Pharmaceuticals, +1-781-308-1387 (investors)



Source: BioCryst Pharmaceuticals

News Provided by Acquire Media