BioCryst Advancing Potential Treatment for Rare and Severely Debilitating Fibrodysplasia Ossificans Progressiva

January 5, 2018

Novel approach using ALK2 inhibition

RESEARCH TRIANGLE PARK, N.C., Jan. 05, 2018 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (NASDAQ:BCRX) announced today that it has advanced a program exploring activin receptor-like kinase-2 (ALK2) inhibitors for treatment of Fibrodysplasia Ossificans Progressiva (FOP). Investigational New Drug Application (IND) enabling nonclinical development of optimized lead candidates BCX9250 and BCX9499 has been initiated with the goal of progressing to Phase 1 clinical trials in the first half of 2019.

FOP is a very rare disease that affects approximately 1 in 2 million people worldwide. In patients with FOP, minor trauma can result in rapid development of painful inflammatory masses. These progress over several weeks resulting in the replacement of the affected soft tissue by permanent bone masses. There is no cure, and there are no approved treatments for FOP.

“We are thrilled that our drug discovery culture has succeeded in bringing forward attractive oral ALK2 inhibitors that have the potential to treat patients with FOP,” said Dr. William P. Sheridan, Senior Vice President and Chief Medical Officer. “At BioCryst, we constantly strive to create medicines that not only treat serious rare diseases, but do so in a way that retains the best quality of life possible for patients and, ultimately, their families and caregivers as well. We expect our core integrated drug discovery capabilities, refined over years of research and applied in new ways, to impact the BioCryst pipeline in an accelerating fashion in coming periods.”

ALK2 enzyme is a part of the normal signaling pathway for bone formation and responds to binding its specific ligands (bone morphogenic proteins, BMPs), by stimulating normal bone growth and renewal in healthy children and adults. Specific activating mutations of the ALK2 gene are seen in all cases of FOP. An activating mutation in ALK2 is necessary for the disease to occur, making the ALK2 kinase an ideal drug target for treatment of FOP with an ALK2 kinase inhibitor.

The goal of the ALK2 inhibitor project at BioCryst is to discover and develop orally administered kinase inhibitor drug candidates that are able to slow or prevent the progressive formation of bone in soft tissues, also known as heterotopic ossification (HO). The two lead candidate molecules dramatically reduced HO in an experimental model of ALK2-driven HO in laboratory rats, with up to 89 percent reduction in volume of HO compared to controls.

“I am encouraged by pharmaceutical company efforts directed at ALK2, the central target of FOP,” stated Dr. Frederick Kaplan, the Isaac & Rose Nassau Professor of Orthopedic Molecular Medicine and Co-Director of The Center for Research in FOP & Related Disorders at The Perelman School of Medicine at The University of Pennsylvania.

The lead candidates for the FOP program, BCX9250 and BCX9499, were selected from a number of potential candidates based on potency for the target kinase, selectivity, and safety screening criteria that included industry-standard in vitro panels and in vivo PK and safety studies in laboratory animals. BioCryst plans to complete IND-enabling manufacturing and nonclinical safety studies to support Phase 1 trials beginning in 2019, and as early as possible thereafter, clinical trials in patients with FOP.

About Fibrodysplasia Ossificans Progressiva (FOP)

FOP is a rare, severely disabling condition characterized by the irregular formation of bone outside the normal skeleton, also known as heterotopic ossification (HO). HO can occur in muscles, tendons and soft tissue. FOP patients progressively become bound by this irregular ossification, with restricted movement and fused joints, resulting in deformities and premature mortality. There are currently no approved treatments for FOP. More information on this disease can be found at the International Fibrodysplasia Ossificans Progressiva Association and OrphaNet websites.

About BioCryst Pharmaceuticals

BioCryst Pharmaceuticals designs, optimizes and develops novel small-molecule medicines that address both common and rare conditions. BioCryst has several ongoing development programs including an oral product candidate for hereditary angioedema, and galidesivir, a potential treatment for filoviruses. RAPIVAB® (peramivir injection), a viral neuraminidase inhibitor for the treatment of influenza, is BioCryst's first approved product and has received regulatory approval in the U.S., Canada, Japan, Taiwan and Korea. Post-marketing commitments for RAPIVAB are ongoing, as well as activities to support regulatory approvals in other territories. 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 are 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 developing any FOP drug candidate may take longer or may be more expensive than planned; that ongoing and future preclinical and clinical development of FOP candidates may not have positive results; that BioCryst may not be able to enroll the required number of subjects in planned clinical trials of product candidates; that the Company may not advance human clinical trials with product candidates as expected; that the FDA and EMA may require additional studies beyond the studies planned for product candidates, or may

not provide regulatory clearances which may result in delay of planned clinical trials, or may impose a clinical hold with respect to such product candidate, or withhold market approval for product candidates; that the Company may not be able to continue development of ongoing and future development programs; that such development programs may never result in future products. 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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