



## **BioCryst Begins Phase 1 Trial With BCX9250, an Oral ALK-2 Inhibitor, for Treatment of Fibrodysplasia Ossificans Progressiva**

November 1, 2019

*—BCX9250 significantly suppressed heterotrophic ossification in preclinical studies—*

*—Urgent medical need with no approved treatments for FOP —*

*—Phase 1 data expected in 2H 2020—*

RESEARCH TRIANGLE PARK, N.C., Nov. 01, 2019 (GLOBE NEWSWIRE) -- [BioCryst Pharmaceuticals, Inc.](http://www.biocryst.com) (Nasdaq: BCRX) today announced that the company has begun a Phase 1 clinical trial with BCX9250, an oral activin receptor-like kinase-2 (ALK-2) inhibitor discovered and developed by BioCryst for the treatment of fibrodysplasia ossificans progressiva (FOP).

The Phase 1 trial will evaluate single and multiple ascending doses of oral BCX9250 in healthy volunteers. The company expects to report the results from the trial in the second half of 2020.

"BCX9250 for FOP represents the third program for potent and selective oral compounds for rare diseases invented and developed by BioCryst to enter the clinic, joining our plasma kallikrein inhibitors for hereditary angioedema and Factor D inhibitor for complement-mediated diseases," said Dr. William Sheridan, chief medical officer of BioCryst.

"FOP is a devastating disease with no approved therapies, and we look forward to seeing clinical data with BCX9250 from this initial study in healthy volunteers to inform how we proceed strategically with the program," Sheridan added.

In preclinical studies, BCX9250 demonstrated potency for the target kinase, selectivity, safety and strong suppression of heterotopic ossification (HO) in animal models.

### **About Fibrodysplasia Ossificans Progressiva**

Fibrodysplasia Ossificans Progressiva 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. Patients with FOP become bound by this irregular ossification over time, with restricted movement and fused joints, resulting in deformities and premature mortality. There are currently no approved treatments for FOP.

### **About BCX9250**

Discovered by BioCryst, BCX9250 is a novel, oral, inhibitor of the ALK-2 enzyme. The ALK-2 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 ALK-2 gene are seen in all cases of FOP. An activating mutation in ALK-2 is necessary for the disease to occur, making the ALK-2 kinase an ideal drug target for treatment of FOP. The goal of the ALK-2 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 HO.

### **About BioCryst Pharmaceuticals**

BioCryst Pharmaceuticals discovers novel, oral small-molecule medicines that treat rare diseases in which significant unmet medical needs exist and an enzyme plays a key role in the biological pathway of the disease. BioCryst has several ongoing development programs including BCX7353, an oral treatment for hereditary angioedema; BCX9930, an oral Factor D inhibitor for the treatment of complement-mediated diseases; galidesivir, a potential treatment for Marburg virus disease and Yellow Fever; and BCX9250, an oral ALK-2 inhibitor for the treatment of fibrodysplasia ossificans progressiva. RAPIVAB<sup>®</sup> (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, Australia, Japan, Taiwan, Korea and the European Union. Post-marketing commitments for RAPIVAB are ongoing. For more information, please visit the Company's website at [www.BioCryst.com](http://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 BCX9250 may take longer or may be more expensive than planned; that ongoing and future preclinical and clinical development of BCX9250 may not advance as expected, enroll the required number of subjects or have positive results; that the FDA, EMA or other applicable regulatory agency may require additional studies beyond the studies planned, may not provide regulatory clearances, may impose a clinical hold or may withhold market approval with respect to BCX9250. 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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**Contact:**

John Bluth

+1 919 859 7910

[jbluth@biocryst.com](mailto:jbluth@biocryst.com)