FDA Grants Orphan Drug Designation for BioCryst's ALK-2 Inhibitor, BCX9250, for the Treatment of Fibrodysplasia Ossificans Progressiva

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RESEARCH TRIANGLE PARK, N.C., Aug. 31, 2022 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq: BCRX) today announced the U.S. Food and Drug Administration (FDA) has granted orphan drug designation for BCX9250 for the treatment of fibrodysplasia ossificans progressiva (FOP).

The FDA has the option to grant orphan drug designation to applicants for the purpose of supporting the development of a drug or biologic intended to treat a disease or condition that affects fewer than 200,000 individuals in the U.S. Companies that receive this designation are eligible for incentives, including tax credits for qualified clinical trials, exemption from user fees and up to seven years of market exclusivity after approval.

"We appreciate the FDA's decision to grant orphan drug designation to BCX9250 as we work toward our goal of bringing this important oral investigational therapy to FOP patients. The benefits available to us through this designation – and the designations we previously received from the FDA and the EMA – have the potential to advance our ALK-2 inhibitor program as efficiently as possible as we move towards beginning trials in FOP patients next year," said Dr. Helen Thackray, chief research and development officer of BioCryst.

FOP is an ultra-rare, severely disabling genetic disorder characterized by heterotopic ossification (HO), or the irregular formation of bone outside the normal skeleton. HO can occur in muscles, tendons, ligaments and other connective tissues. Patients with FOP become bound by this irregular ossification over time, with restricted movement and fused joints, resulting in deformities, restricted mobility and premature mortality.

Earlier this year, BioCryst announced that BCX9250 has received Fast Track designation from the FDA, in addition to orphan drug designation and PRIME designation from the European Medicines Agency (EMA).

BioCryst will present three posters featuring data from non-clinical studies and first-in-human Phase 1 safety, tolerability and pharmacokinetics study in healthy subjects from the BCX9250 program at the American Society for Bone and Mineral Research Annual Meeting, which is being held in Austin, Texas, from Sept. 9-12, 2022. As the company previously reported in the Phase 1 study, BCX9250 was safe and well tolerated at all doses studied, with linear and dose-proportional exposure supporting the potential for once-daily dosing.

About BCX9250
BCX9250 is a novel, oral activin receptor-like kinase-2 (ALK-2) inhibitor designed to inhibit the ALK-2 enzyme, which 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 fibrodysplasia ossificans progressiva (FOP). An activating mutation in ALK-2 is necessary for the disease to occur, making the ALK-2 enzyme an ideal drug target for treatment of FOP. In a Phase 1 clinical trial in healthy subjects, BCX9250 was safe and well tolerated at all doses studied, with linear and dose-proportional exposure supporting the potential for once-daily dosing.

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. Oral, once-daily ORLADEYO® (berotralstat) is approved in the United States and multiple global markets. BioCryst has several ongoing development programs including BCX9930, an oral Factor D inhibitor for the treatment of complement-mediated diseases, BCX9250, an ALK-2 inhibitor for the treatment of fibrodysplasia ossificans progressiva, and galidesivir, a potential treatment for Marburg virus disease and yellow fever. RAPIVAB® (peramivir injection) is approved in the U.S. and multiple global markets, with post-marketing commitments ongoing. 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 BioCryst's plans and expectations for BCX9250 and the potential benefits associated with an FDA orphan drug designation. These statements involve known and unknown risks, uncertainties and other factors which may cause any actual results or benefits to be materially different from those 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: orphan drug, Fast Track, and other designations by the FDA or other applicable regulatory agency may not lead to a faster development, regulatory review, or approval process with such agency and does not increase the likelihood that BCX9250 will receive marketing approval; ongoing and future preclinical and clinical development of BCX9250 may not have positive results; BioCryst may not be able to enroll the required number of subjects in planned clinical trials of product candidates; BioCryst may not advance human clinical trials with product candidates as expected; the FDA or other applicable regulatory agency may require additional studies beyond the studies planned for products and product candidates, may not provide regulatory clearances which may result in delay of planned clinical trials, may impose certain restrictions, warnings, or other requirements on products and product candidates, may impose a clinical hold with respect to product candidates, or may withhold, delay, or withdraw market approval for products and product candidates; product candidates, if approved, may not achieve market acceptance; and the ongoing COVID-19 pandemic, which could create challenges in all aspects of BioCryst's business, including without limitation delays, stoppages, difficulties and increased expenses with respect to BioCryst's and its partners' development, regulatory processes and supply chains, negatively impact BioCryst's ability to access the capital or credit markets to finance its operations, or have the effect of heightening many of the risks described above or in the documents BioCryst periodically files with the Securities and Exchange Commission. 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, which identify important factors that could cause actual results to differ materially from those contained in BioCryst's forward-looking statements.

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