European Medicines Agency Grants PRIME Designation to BioCryst's ALK-2 Inhibitor, BCX9250, for Treatment of Fibrodysplasia Ossificans Progressiva

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BCX9250 is first investigational drug for FOP to be eligible for program

RESEARCH TRIANGLE PARK, N.C., April 27, 2022 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq: BCRX) today announced the European Medicines Agency (EMA) has granted access to the Priority Medicines (PRIME) scheme for BCX9250, a novel, oral activin receptor-like kinase-2 (ALK-2) inhibitor discovered and developed by BioCryst for the treatment of fibrodysplasia ossificans progressiva (FOP).

PRIME is a program launched by the EMA to enhance support for the development of medicines that target an unmet medical need. This voluntary program is based on enhanced interaction and early dialogue with developers of promising medicines and is designed to optimize development plans and speed up evaluation so these medicines can potentially reach patients earlier. According to the EMA, developers of medicines that are eligible for PRIME can expect additional opportunities for scientific advice and be eligible for accelerated assessment at the time of application for a marketing authorization.

"Promising results from non-clinical data and the first-in-human Phase 1 safety, tolerability and pharmacokinetics study in healthy subjects formed the basis of the application for PRIME eligibility. We are pleased with the EMA's decision to grant PRIME eligibility to BCX9250 – the first investigational drug for this indication to receive this designation – based on the early evidence of the potential of BCX9250 to address the unmet need for patients living with FOP. We look forward to applying the benefits available to us through PRIME as we continue to advance our ALK-2 inhibitor program," said Dr. Helen Thackray, chief research and development officer of BioCryst.

FOP is an ultra-rare, severely disabling genetic disorder characterized by the irregular formation of bone outside the normal skeleton, also known as heterotopic ossification (HO). 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.

BCX9250 is 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 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, the European Union, Japan, the United Kingdom and the United Arab Emirates. 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) has received regulatory approval in the U.S., Canada, Australia, Japan, Taiwan and Korea. Post-marketing commitments for RAPIVAB are 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 potential benefits and opportunities associated with BCX9250 and access to PRIME. These statements involve known and unknown risks, uncertainties and other factors which may cause any actual benefits and opportunities 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: access to PRIME may not lead to a faster development, regulatory review, or approval process with the EMA 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, EMA, 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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