BioCryst Discontinues Development of BCX9930 and Shifts Focus to Potential Once-daily, Oral Factor D Inhibitor, BCX10013

December 15, 2022

RESEARCH TRIANGLE PARK, N.C., Dec. 15, 2022 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq: BCRX) today announced that, based on new competitive data recently presented at the American Society of Hematology (ASH) annual meeting, the company no longer believes that BCX9930 would be commercially competitive, and is discontinuing the development of BCX9930. This decision allows the company to fully focus its complement inhibitor development efforts on BCX10013, a potential once-daily, oral Factor D (alternative pathway) inhibitor currently in clinical development, and pursue additional oral compounds for multiple targets across other complement pathways.

"With the new competitor efficacy data presented at ASH, and the limitations preventing us from optimizing the dosing of BCX9930 for increased efficacy, it is unlikely that BCX9930 could meet the new standard of care. We have made this decision prior to fully investing in the pivotal development program and commercialization activities, and will focus on our potential best-in-class asset, BCX10013, and our other programs," said Jon Stonehouse, BioCryst president and chief executive officer.

Patients benefitting from BCX9930 in the clinical program may remain on therapy. As the program advances, the company plans to offer these patients an opportunity for access to BCX10013.

BioCryst expects to report preliminary data from healthy volunteers receiving single ascending doses and multiple ascending doses of BCX10013 in the first quarter of 2023. The preclinical and early clinical profile from approximately 90 healthy volunteers suggests BCX10013 could have the properties of a once-daily, oral therapy. Key goals of the ongoing clinical program include confirming this once-daily profile with healthy volunteer and patient data and establishing optimal dosing for pivotal studies.

In addition to BCX10013, which targets Factor D in the alternative pathway of complement, BioCryst is pursuing oral medicines directed at other targets across the classical, lectin and terminal pathways of the complement system. The goal of the company's overall complement program is to advance several oral compounds across multiple pathways in the complement system to treat many complement-mediated diseases.

The decision to discontinue the BCX9930 program will have a positive near-term financial impact for the company. The pause in the program earlier this year allowed the company to reduce 2022 operating expense guidance by approximately $100 million, primarily from reduced research and development (R&D) expenses. The company now expects that 2023 R&D expenses will be similar to 2022 R&D expenses as the company focuses its investment on BCX10013 and its other complement programs. The company plans to provide full year 2023 operating expense guidance in the first quarter of 2023.

Conference Call and Webcast

BioCryst management will host a conference call and webcast at 8:30 a.m. ET today. The live call may be accessed by dialing 1-866-777-2509 for domestic callers and 1-412-317-5413 for international callers. A live webcast of the call will be available online at the investors section of the company website at www.biocryst.com. A replay of the call will be available on the company website.

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 many global markets. BioCryst has active programs to develop oral medicines for multiple targets across the complement system, including BCX10013, an oral Factor D inhibitor in clinical development. 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 our plans and expectations for our complement program and other future results. 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, performance 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: 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 below or in the documents BioCryst files periodically with the Securities and Exchange Commission; ongoing and future preclinical and clinical development of BCX10013 and other product candidates 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 actual financial results may not be consistent with expectations, including that revenue, operating expenses and cash

usage may not be within management's expected ranges. 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 actual results to differ materially from those contained in BioCryst's forward-looking statements.

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