BioCryst Provides Update on Galidesivir Program

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RESEARCH TRIANGLE PARK, N.C., Dec. 22, 2020 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq:BCRX) today announced that data from part 1 of a clinical trial of its broad-spectrum antiviral, galidesivir, showed that galidesivir was safe and generally well tolerated in patients infected with SARS-CoV-2, the virus that causes COVID-19. The trial was not designed or sized to demonstrate clinical efficacy and no clinical efficacy benefit with galidesivir treatment compared to placebo treatment was observed in the trial.

Based on the company's ongoing discussions with the National Institute of Allergy and Infectious Diseases (NIAID), part of the National Institutes of Health, a major funding partner for the program, BioCryst expects NIAID to continue their support for the development of galidesivir with a focus on biodefense threats, such as Marburg virus disease, and to discontinue the pursuit of a COVID-19 indication for galidesivir.

"We are encouraged by the first patient data with galidesivir and the growing body of evidence that galidesivir could be an important broad-spectrum antiviral. We appreciate the government's continued investment to investigate galidesivir as a biodefense drug," said Jon Stonehouse, chief executive officer of BioCryst.

BioCryst is engaged in ongoing discussions with NIAID to define specific further galidesivir studies that NIAID would support.

Part 1 Trial Design
Part 1 of the trial enrolled 24 hospitalized adults diagnosed with moderate to severe COVID-19 confirmed by PCR. Three cohorts of eight patients were randomized to receive intravenous galidesivir (n=6) or placebo (n=2) every 12 hours for seven days.

Each dosing regimen began with a single 10 mg/kg or 20 mg/kg loading dose, followed by a maintenance dose of 2 mg/kg or 5 mg/kg administered twice daily. The three dosing regimens evaluated were 10 mg/kg then 2 mg/kg (cohort 1), 10 mg/kg then 5 mg/kg (cohort 2) and 20 mg/kg then 5 mg/kg (cohort 3).

The trial was conducted in Brazil under a U.S. investigational new drug application. The protocol also was approved by the Agência Nacional de Vigilância Sanitária (ANVISA) and the Brazilian National Ethics Committee (CONEP).

Part 1 Results
The primary objective of part 1 of the dose-ranging study was to evaluate the safety of galidesivir. No safety signals were identified, and all three dose levels were equally safe.

Secondary objectives were to evaluate the effect of galidesivir on the clinical course of COVID-19 and on SARS-CoV-2 infection in the respiratory tract.

Galidesivir treatment was associated with a more rapid decline in viral RNA levels in the respiratory tract in an apparent dose-dependent manner.

A separate study of galidesivir in a COVID-19 animal model showed that early administration of galidesivir reduced SARS-CoV-2 viral burden in lung tissue (1.4-1.6 log lower tissue viral burden) and was associated with a significant reduction in damage to lung tissue, compared to vehicle control treated animals. These results suggest that early antiviral treatment of SARS-CoV-2 infection may protect against developing severe COVID-19 lung disease.

NIAID is a major funding partner of the galidesivir program under contracts 75N93020C00055 and HHSN272201300017C.

About Galidesivir (BCX4430)
Galidesivir, a broad-spectrum antiviral drug, is an adenosine nucleoside analog that acts to block viral RNA polymerase. It is in advanced development for the treatment of Marburg virus disease. In animal studies, galidesivir has demonstrated activity against a variety of serious pathogens, including, Ebola, Marburg, Yellow Fever and Zika viruses. Galidesivir has also demonstrated broad-spectrum activity *in vitro* against more than 20 RNA viruses in nine different families, including coronaviruses, filoviruses, togaviruses, bunyaviruses, arenaviruses, paramyxoviruses, and flaviviruses. BioCryst is developing galidesivir in collaboration with U.S. government agencies and other institutions.

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 for the prevention of HAE attacks in adults and pediatric patients 12 years and older, and under regulatory review for approval in Japan and the European Union. BioCryst has several ongoing development programs including 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 ALK-2 inhibitor for the treatment of fibrodysplasia ossificans progressiva. 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, 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 BioCryst's plans and expectations for its galidesivir development program. These statements involve known and unknown risks, uncertainties and other factors which may cause actual results and developments of such program to be materially different from any future results or developments expressed or implied by the forward-looking

statements. These statements reflect our current views and are based on assumptions and 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: NIAID's ability to determine not to exercise available options under its contracts with BioCryst or to terminate such contracts at any time, causing BioCryst not to realize the aggregate value of the contracts; funding for galidesivir under government contracts is dependent on the progress toward, and the achievement of, developmental milestones; the ongoing COVID-19 pandemic 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 herein or in the documents BioCryst files periodically with the Securities and Exchange Commission; developing and manufacturing any product candidate, including galidesivir, may take longer or may be more expensive than planned; funding for the continued development and manufacture of galidesivir may not be available; ongoing and future preclinical and clinical studies with galidesivir may not have positive results; BioCryst may not be able to enroll the required number of subjects in planned clinical trials of product candidates, including galidesivir; BioCryst may not advance human clinical trials with product candidates, including galidesivir, as expected; and the FDA, or other applicable regulatory agency may require additional studies beyond the studies planned for product candidates, may not provide regulatory clearances which may result in delay of planned clinical trials, may impose a clinical hold with respect to such product candidates, or may withhold market approval for product candidates. 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 forward-looking statements.

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