Long-term (48-week) Data Show Treatment with Berotralstat Provides Robust and Durable Reductions in HAE Attacks and Improvements in Quality of Life Scores

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Data presented at European Academy of Allergy and Clinical Immunology (EAACI) Digital Congress

RESEARCH TRIANGLE PARK, N.C., June 06, 2020 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq:BCRX) today announced new data from the APeX-2 and APeX-S clinical trials, which showed that hereditary angioedema (HAE) patients taking oral, once-daily berotralstat experienced sustained decreases in their attack frequency and improvements in quality of life (QoL) scores over 48 weeks. Berotralstat was also safe and generally well-tolerated over 48 weeks in both APeX-2 and APeX-S.

The data were presented at the European Academy of Allergy and Clinical Immunology (EAACI) Digital Congress.

“As HAE patients continue in our long-term clinical trials, we are seeing reductions in attack rate and increases in QoL scores which highlight the impact oral, once-daily berotralstat could have in reducing the burden of disease for patients currently dependent on injectable or infused prophylaxis options,” said Dr. William Sheridan, chief medical officer of BioCryst.

- In APeX-2, 31 patients who were randomized to 150 mg of oral, once-daily berotralstat at the beginning of the study and completed 48 weeks of therapy had a mean baseline attack rate of 2.9 attacks per month, which declined to 1.5 attacks per month after one month and to 1.0 attack per month at 12 months.
- In APeX-S, patients completing 48 weeks of treatment on 150 mg of berotralstat (n=73) had a median attack rate of zero attacks per month in six of the 12 months, including month 12 (week 48).
- The low attack rate experienced by HAE patients on 150 mg of oral, once-daily berotralstat reduced the burden of disease and translated into clinically meaningful improvements in mean angioedema quality of life (AE-QoL) total score, as measured by the disease-specific AE-QoL questionnaire. This persisted through month 12 (week 48) in the APeX-S trial.
    - Improvements in mean change from baseline AE-QoL total score exceeding the minimum clinically important difference (MCID) of six points were observed by week four and at week 48 the mean AE-QoL total score for the berotralstat 150-mg treatment group had decreased by 14.7 points compared with baseline.

An integrated 48-week analysis across both APeX-2 and APeX-S showed no new safety findings. Berotralstat was safe and generally well tolerated in a total of 342 patients with a total of 232 patient-years of daily oral dosing. The most common adverse event was the common cold, which occurred with similar frequency in berotralstat and placebo patients. Gastrointestinal events led to discontinuation of berotralstat in 3.2 percent of patients. Drug-related serious adverse events occurred in three of 342 subjects (0.9 percent) and resolved after stopping or interrupting berotralstat 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. BioCryst has several ongoing development programs including berotralstat (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 COVID-19, 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, 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.

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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 the ongoing COVID-19 pandemic could create challenges in all aspects of our business, including without limitation delays, stoppages, difficulties and increased expenses with respect to our and our partners’ development, regulatory processes and supply chains, could negatively impact our ability to access the capital or credit markets to finance our operations, or could have the effect of heightening many of the risks described below or in the documents we file periodically with the Securities and Exchange Commission; that developing any HAE product candidate may take longer or may be more expensive than planned; that ongoing and future preclinical and clinical development of BCX9930, BCX9250 and galidesivir may not have positive results; that BioCryst may not be able to enroll the required number of subjects in planned clinical trials of product candidates; that BioCryst may not advance human clinical trials with product candidates as expected; that the FDA, EMA, PMDA or other applicable regulatory agency may require additional studies beyond the studies planned for product candidates, or may not provide regulatory clearances which may result in delay of planned clinical trials, or may impose a clinical hold with respect to

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