Berotralstat (BCX7353) Significantly Reduced Use of Acute On-Demand Medicine in HAE Patients in APeX-2

March 16, 2020

RESEARCH TRIANGLE PARK, N.C., March 16, 2020 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq:BCRX) today announced new data from the APeX-2 trial showing 150 mg of oral, once-daily berotralstat (BCX7353) for prophylaxis of hereditary angioedema (HAE) attacks reduced patients' monthly use of standard of care (SoC) on-demand medicine by 53.6 percent ($p<0.001$) compared to placebo, and reduced the number of HAE attacks requiring acute SoC treatment by 49.2 percent ($p<0.001$) compared to placebo.

Additional new data show that the percentage of HAE attacks requiring re-treatment with multiple doses of on-demand therapy was lower for patients receiving berotralstat (150 mg) than placebo.

The posters are available on the BioCryst website at https://www.biocryst.com/our-focus/scientific-publications, and are expected to be shared online by the American Academy of Allergy, Asthma & Immunology (AAAAI), which recently cancelled its planned annual meeting.

"Patients receiving berotralstat had fewer attacks, treated fewer attacks, experienced less severe attacks and used less on-demand medication compared to placebo. These data from APeX-2 provide further evidence that HAE patients are seeing significant benefits from oral, once-daily berotralstat," said Dr. William Sheridan, chief medical officer of BioCryst.

In APeX-2, patients experienced a rapid and sustained decrease in their attack frequency over 48 weeks. Thirty patients who were randomized to 150 mg of berotralstat at the beginning of the trial and completed 48 weeks of therapy had a baseline mean attack rate of 2.9 attacks per month, which declined to 1.4 attacks per month after one month and to 1.0 attacks per month at month 12.

APeX-2 patients who switched from placebo to 150 mg of berotralstat after week 24 saw dramatic and sustained reductions in their HAE attack rate. Their mean attack rate dropped to 0.5 attacks per 28 days at month seven and to 0.4 attacks per 28 days at month 12.

An integrated 48-week analysis across both the APeX-2 and APeX-S trials showed 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 frequent adverse drug reactions were mild-to-moderate gastrointestinal events that were brief in duration and self-limited.

APeX-2 is a randomized, double-blind, placebo-controlled, three-arm trial testing two dose levels of orally administered once-daily berotralstat (110 mg and 150 mg) for prevention of angioedema attacks. The trial enrolled 121 patients with Type I and II HAE in the United States, Canada and Europe. Following completion of the 24-week analysis period, patients continued on study drug in an ongoing extension phase of APeX-2 through 48 weeks. Patients randomized to placebo for 24 weeks were re-randomized to receive one of the two doses of study drug in the extension phase of the trial. Patients who complete 48 weeks may continue in the trial on open-label berotralstat.

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 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.

Forward-Looking Statements

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, 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: that the FDA, PMDA, EMA or other applicable regulatory agency may not approve berotralstat, within the timeframe expected, or at all, may ultimately determine that there are deficiencies in the development program or execution thereof, may require additional information or studies, may disagree with our analyses regarding the safety and efficacy conclusions; in the future they could determine that an advisory committee is necessary; we may learn of previously unknown issues; ongoing studies may take longer or may be more expensive than planned; in the event that berotralstat is approved in any territory, we or our partners may be unable to successfully commercialize as expected; that actual financial results may not be consistent with expectations, including that 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 the actual results to differ materially from those contained in BioCryst's projections and forward-looking statements.

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