Allergy Publishes Results from BioCryst's APeX-J Trial of Oral, Once-Daily Berotralstat for the Prevention of HAE Attacks

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RESEARCH TRIANGLE PARK, N.C., Nov. 30, 2020 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq: BCRX) today announced the journal *Allergy* has published data from the APeX-J trial, a randomized, placebo-controlled trial conducted in Japan evaluating oral, once-daily berotralstat for the prophylactic treatment of hereditary angioedema (HAE).

The APeX-J trial met its primary endpoint of a reduction in the rate of HAE attacks for berotralstat 150 mg compared to placebo during the 24-week period (p=0.003). Berotralstat was safe and generally well-tolerated in the trial.

These results are consistent with the global phase 3 APeX-2 trial, where berotralstat 150 mg also reduced the rate of HAE attacks compared to placebo (p<0.001) and was safe and generally well-tolerated.

"APeX-J is the first placebo-controlled trial of an HAE medicine conducted in Japan and the berotralstat data are very exciting for patients, who currently have no approved prophylactic treatment options. Based on the safety and efficacy profile, I believe berotralstat, if approved, would be an important advancement in HAE management for Japanese patients," said Dr. Isao Ohsawa, president of Saiyu Soka hospital and principal investigator of the APeX-J trial.

Dr. Ohsawa and the study authors note that HAE is estimated to affect 2,500 patients in Japan and the recognition of HAE by physicians is low. Although two on-demand treatments are approved, no therapies are currently approved for long-term prophylaxis in Japan.

"Berotralstat would be the first approved prophylactic therapy for HAE patients in Japan and we believe there is a significant opportunity for berotralstat to accelerate the diagnosis of HAE patients and dramatically improve the quality of life for patients," said Jon Stonehouse, president and chief executive officer of BioCryst.

A new drug application (JNDA) is under review in Japan for approval of oral, once-daily berotralstat for the prophylactic treatment of HAE. Berotralstat is being reviewed under Sakigake designation and the company expects a decision on approval in December 2020.

Torii Pharmaceutical, Co., Ltd. is BioCryst's commercial partner in Japan for 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 ORLADEYO ™ (berotralstat), 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.

Forward-Looking Statements

This press release contains forward-looking statements, including statements regarding future results, performance or achievements. These statements are identified by use of terms such as "believe," "will," "would," "expect," and similar words, although some forward-looking statements may be expressed differently. These statements involve known and unknown risks, uncertainties and other factors which may cause 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: 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; developing and commercializing ORLADEYO or any HAE product candidate may take longer or may be more expensive than planned; 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, PMDA 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 certain restrictions, warnings, or other requirements on product candidates, may impose a clinical hold with respect to such product candidates, or may withhold or delay market approval for product candidates; product candidates, if approved, may not achieve market acceptance; BioCryst's ability to successfully commercialize its product candidates, manage its growth, and compete effectively; and risks related to the international expansion of BioCryst's business. 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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