BioCryst Reports Agreement with PMDA on Phase 3 Clinical Trial and Regulatory Requirements for Marketing Authorization of BCX7353 in Japan

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BCX7353 continues development under Sakigake designation

RESEARCH TRIANGLE PARK, N.C., June 25, 2018 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (NASDAQ:BCRX), a pharmaceutical company focused on the development and commercialization of treatments for rare diseases, today announced that the Company has reached agreement on the design of a Phase 3 trial and regulatory requirements for marketing authorization of BCX7353 for Hereditary Angioedema ("HAE") with the Pharmaceuticals and Medical Devices Agency ("PMDA") in Japan.

The Phase 3 trial design agreed upon for Japan, APeX-J, is a randomized, placebo-controlled double-blind trial of 24 weeks duration with a 28-week safety extension. Approximately 24 Japanese subjects with HAE will be enrolled. The APeX-J trial design closely follows the design of APeX-2, a Phase 3 clinical trial being conducted in the U.S., Canada and European countries. APeX-J tests the same dose levels of BCX7353 as in APeX-2, 110 mg daily and 150 mg daily, and the endpoints are identical to those in APeX-2. Data from the APeX-J and APeX-2 trials will be combined for regulatory submission in Japan.

"Japanese HAE patients would welcome an oral prophylactic drug as another treatment alternative, which provides HAE patients ease in daily life as well as relief from travelling outside their home town for treatment to receive on-demand therapy of HAE attacks in a hospital," said Professor Michihiro Hide, M.D., Ph.D, Dean of the School of Medicine and Professor and Chairman, Department of Dermatology, Hiroshima University, and a leading expert in HAE in Japan.

BCX7353 was one of the first products granted Sakigake designation by the Japanese PMDA in October 2015. Sakigake designation is awarded to innovative products addressing an unmet medical need in Japan or providing improved efficacy compared to available treatment. Sakigake designation is associated with accelerated review of a Japanese NDA as well as pricing and other corporate benefits for the holder.

"Now that we have agreement on the regulatory requirements for marketing authorization of BCX7353 in Japan, we can move forward with executing APeX-J and selecting a commercialization partner for this region," said Jon P. Stonehouse, President & Chief Executive Officer. "A once-a-day oral treatment for this rare disease represents a high unmet medical need in Japan and an excellent partnering opportunity."

About BCX7353

Discovered by BioCryst, BCX7353 is a novel, oral, once-daily, selective inhibitor of plasma kallikrein currently in development for the prevention and treatment of angioedema attacks in patients diagnosed with HAE. BCX7353 has been generally safe and well tolerated in the Phase 2 APeX-1 clinical trial. BioCryst is currently conducting the Phase 3 APeX-2 clinical trial and the long-term safety APeX-S clinical trial, both evaluating two dosage strengths of BCX7353 administered orally once-daily as a preventive treatment to reduce the frequency of attacks in patients with HAE. BioCryst is also conducting the ZENITH-1 clinical trial. ZENITH-1 is a proof-of-concept Phase 2 clinical trial testing an oral liquid formulation of BCX7353 for the treatment of acute angioedema attacks.

About BioCryst Pharmaceuticals

BioCryst Pharmaceuticals designs, optimizes and develops novel small-molecule medicines that address both common and rare conditions. BioCryst has several ongoing development programs including BCX7353, an oral treatment for hereditary angioedema, galidesivir, a potential treatment for filoviruses, and a preclinical program to develop oral Alk-2 inhibitors for the treatment of fibrodysplasia ossificans progressive ("FOP"). 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, 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 developing any HAE product candidate may take longer or may be more expensive than planned; that ongoing and future preclinical and clinical development of HAE second generation drug candidates (including ZENITH-1, APeX-2, APeX-S and APeX-J) 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 the Company may not advance human clinical trials with product candidates as expected; that the FDA, EMA 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 such product candidate, or 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 projections and forward-looking statements.

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