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BioCryst Announces Initiation of ZENITH-1, a Clinical Trial to Evaluate BCX7353 as an Acute Treatment of Hereditary Angioedema Attacks

RESEARCH TRIANGLE PARK, N.C., Aug. 02, 2017 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (NASDAQ:BCRX) announced today the dosing of the first subject into ZENITH-1, a clinical trial studying up to three dosage strengths of a liquid formulation of BCX7353 given as a single oral dose for the acute treatment of angioedema attacks in patients with hereditary angioedema (HAE).

"We are excited to launch the ZENITH-1 exploratory Phase 2 trial. Based upon pharmacokinetic and pharmacodynamic properties of BCX7353, we believe it could be an efficacious and convenient oral alternative to parenteral treatments of acute angioedema attacks in patients with HAE. A liquid formulation of BCX7353 would be a strong complement to our prophylactic treatment program," said Jon Stonehouse, Chief Executive Officer. "We believe this new formulation can fill an unmet need for patients who are looking for better and easier ways to manage their illness."

ZENITH-1 is a randomized, double-blind, placebo-controlled, adaptive dose-ranging trial of the efficacy, safety and tolerability of BCX7353 for treatment of acute angioedema attacks, and will enroll up to 60 subjects with HAE. Blinded study drug will be dosed as an oral liquid after onset of symptoms, for up to 3 attacks in each subject, with each subject receiving both BCX7353 (for 2 attacks) and placebo (for one attack) in a randomized sequence. The trial is structured with up to 3 consecutive cohorts testing single doses of 750 mg (from 12 to 36 subjects), 500 mg (up to 12 subjects) and 250 mg (up to 12 subjects), starting with 750 mg. Efficacy assessments include patient-reported composite visual analogue scale (VAS) scores, patient global assessment, change in symptoms, and use of rescue medication.

Treatment effect will be assessed on accumulating results, beginning after 12 subjects have completed study in the first cohort (750 mg), by comparing the proportion of BCX7353-treated and placebo-treated attacks which have a stable or improved composite VAS at 4 hours post dose. Once a treatment effect is demonstrated, enrollment at the 500 mg dose level will commence. If treatment effect at the 500 mg dose level is similar to 750 mg dose level, the 250 mg dose cohort will be enrolled.

Additional clinical trial details will be available on www.clinicaltrials.gov.

About BCX7353

Discovered by BioCryst, BCX7353 is a novel, once-daily, selective inhibitor of plasma kallikrein currently in development for the prevention of angioedema attacks in patients diagnosed with HAE. With the initiation of the ZENITH-1 clinical trial, an oral liquid formulation of BCX7353 will be evaluated for treating acute angioedema attacks. BCX7353 has been generally safe and well tolerated in the ongoing Phase 2 APeX-1 clinical trial and in clinical pharmacology studies in healthy volunteers.

About BioCryst Pharmaceuticals

BioCryst Pharmaceuticals designs, optimizes and develops novel small molecule drugs that block key enzymes involved in rare diseases. BioCryst has several ongoing development programs: BCX7353 and other second generation oral inhibitors of plasma kallikrein for hereditary angioedema, and galidesivir, a broad spectrum viral RNA polymerase inhibitor that is a potential treatment for filoviruses. 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, Japan, Taiwan and Korea. Post-marketing commitment development activities for RAPIVAB are ongoing, as well as activities to support regulatory approvals in other territories. 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 drug 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 APeX-1 and ZENITH-1) 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 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; that the Company may not be able to continue development of ongoing and future development programs; that such development programs may never result in future products; that actual financial results may not be consistent with expectations, including that 2017 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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