September 5, 2017

BioCryst Announces Positive Results from its APeX-1 Phase 2 Trial in HAE

73% Reduction in Overall Attack Frequency at 125mg Dose (p < 0.001)

FDA and EMA Meetings Planned for Fourth Quarter 2017 to Finalize Phase 3

RESEARCH TRIANGLE PARK, N.C., Sept. 05, 2017 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (NASDAQ:BCRX) today announced final results from its Phase 2 APeX-1 clinical trial in hereditary angioedema (HAE). APeX-1 was a 3-part dose ranging trial designed to evaluate the efficacy, safety, tolerability, pharmacokinetics (PK) and pharmacodynamics of orally administered once-daily (QD) BCX7353 for 28 days, as a preventative treatment to reduce the frequency of attacks in HAE patients. This final analysis evaluated data from all patients in Parts 1, 2 and 3 of the trial.

"We are delighted to see a robust treatment effect after completing the largest ever Phase 2 trial in HAE patients. We now have the information necessary to select doses for Phase 3," said Jon Stonehouse, Chief Executive Officer & President. "The 125 mg once-daily oral dose of BCX7353 provided a high level of efficacy and excellent tolerability. This product profile will be an extremely attractive treatment option for physicians and patients."

"An effective and tolerable prophylaxis of hereditary angioedema attacks is of paramount importance for many HAE patients and the benefit of an oral administration route for these chronically ill patients cannot be overestimated. In that respect, the results of this trial are extremely encouraging for the HAE patient community," said Dr. Emel Aygören-Pürsün, MD, principal investigator for the APeX-1 trial and Head of Interdisciplinary Competence Center for Hereditary Angioedema, and Specialist in Internal Medicine and Hemostaseology Department of Child and Adolescent Medicine, Goethe University Hospital Frankfurt.

"With an active IND in the U.S. and the completion of APeX-1, we are now preparing for meetings in the fourth quarter of this year with the FDA and EMA to finalize the Phase 3 program," said William Sheridan, Chief Medical Officer. "Our goal is to start the Phase 3 efficacy and long-term safety trials in the first quarter of 2018."

Seventy-five subjects were randomized and included in the final analysis of pooled data from Parts 1, 2 and 3: 7 at 62.5 mg, 14 at 125 mg, 14 at 250 mg and 18 at 350 mg of BCX7353 QD; and 22 placebo. The qualifying attack rate was approximately 1/week. Baseline characteristics were generally well balanced across the treatment groups. Compliance with daily study drug dosing for 28 days was excellent (≥ 98% across all treatment groups).

Subjects recorded angioedema symptoms in a diary; diary records were reviewed and attacks adjudicated by an independent expert group. The primary endpoint of the trial was the number of HAE attacks. The pre-specified per-protocol (PP) final analysis included data on a total of 67 subjects with Type 1 or Type 2 HAE completing > 90% of planned study drug doses. The percentage reductions by treatment group in the mean rate of angioedema attacks for the pre-defined effective dosing period (weeks 2 through 4) in BCX7353 treated subjects are indicated in the Table below. Results from a pre-planned analysis of peripheral and abdominal attacks are also shown. Similar results to those shown were seen in the analysis of weeks 1 through 4 and the intent-to-treat population (ITT).

	Percentage change in attacks vs placebo (p-Value) Per protocol population, weeks 2-4 of treatment			
	N	All Attacks	Peripheral Attacks	Abdominal Attacks
BCX7353 350 mg	14	-58% (p < 0.001)	-90% (p < 0.001)	-5% (p=0.884)
BCX7353 250 mg	12	-46% (p=0.006)	-66% (p=0.005)	-13% (p=0.700)
BCX7353 125 mg	13	-73% (p < 0.001)	-79% (p < 0.001)	-63% (p=0.048)
BCX7353 62.5 mg	7	-7% (p=0.715)	-25% (p=0.371)	+22% (p=0.578)

The 125 mg dose level showed statistically significant and similar benefit for all attacks, and also when split into abdominal attacks and peripheral attacks. In contrast, at the 250 mg and 350 mg dose levels, there was no statistically significant effect for abdominal attacks, despite strong and statistically significant effects on peripheral attacks. Based on these findings, it is

likely that subjects in the 250 mg and 350 mg arms recorded transient drug-related abdominal adverse events (AEs) as HAE attack symptoms in their diary. As expected, the lowest dose tested (62.5 mg QD) showed no statistically significant differences in attack rates (total, or when split into abdominal and peripheral) compared with placebo. The range of doses studied and associated results complete the dose response evaluation required to inform Phase 3 dose selection.

A significant increase in the proportion of attack-free subjects was observed in the 125 mg QD dose group compared to placebo (46% versus 10%, p= 0.033). Furthermore, a clinically important and statistically significant improvement in patient quality of life total score, measured using the AE-QoL instrument, was seen in the 125 mg QD group compared to placebo ($p < 0.001$). The mean improvement in the 125 mg QD group was more than four times the minimum clinically important difference.

Oral BCX7353 once-daily for 28 days was generally safe and well tolerated in subjects with HAE. No new clinically significant safety findings were seen in Part 3 of the trial. Overall, there was one serious adverse event of moderate gastrointestinal infection that was determined by the investigator not to be drug-related. As previously reported, study drug was discontinued before day 28 in three subjects in the BCX7353 350 mg treatment arm (unrelated pre-existing liver disorder; related gastroenteritis with liver disorder; and related vomiting/abdominal cramps). The most common treatment-emergent AEs in descending order of frequency were the common cold, headache, diarrhea, nausea and abdominal pain. Gastrointestinal AEs were infrequent at the 125 mg and 62.5 mg dose levels, and there were no clinically significant laboratory abnormalities at these dose levels.

Steady state BCX7353 plasma levels and kallikrein inhibition levels were consistent with previous analyses. Steady state trough drug levels (24 hours after dosing) exceeded the proposed target threshold for efficacy of 4 times the 50% effective concentration (EC_{50}) in 0%, 64%, 100% and 100% of subjects at the 62.5 mg, 125 mg, 250 mg and 350 mg dose levels, respectively.

The observed efficacy, dose response, PK, safety and tolerability profile of BCX7353 strongly support its advancement into Phase 3 development. BioCryst intends to meet with the U.S. Food and Drug Administration (FDA) and European Medicines Agency (EMA) during the fourth quarter of 2017 to finalize the Phase 3 program required to support New Drug Application (NDA) and Marketing Authorization Application (MAA) submissions.

Conference Call and Webcast

BioCryst's leadership team will host a conference call and webcast today, September 5, 2017 at 9:00 a.m. Eastern Time, to discuss its APeX-1 final results. To participate in the conference call, please dial 1-877-303-8027 (United States) or 1-760-536-5165 (International). No passcode is needed for the call. The webcast can be accessed by logging onto www.BioCryst.com. Please connect to the website at least 15 minutes prior to the start of the conference call to ensure adequate time for any software download that may be necessary.

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 or MAA 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 receive additional government funding to further support the development of galidesivir; that galidesivir development may not be successful; that BARDA and/or NIAID may further condition, reduce or eliminate future funding; that revenue from peramivir injection is unpredictable and may never result in significant revenue for the Company; 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 that 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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