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BioCryst Reports Additional Positive Results From the Second Interim Analysis of Its APeX-1 Trial

125 mg dose of BCX7353 showed a reduction of 73% in overall attack rate (p=0.002)

RESEARCH TRIANGLE PARK, N.C., May 25, 2017 (GLOBE NEWSWIRE) -- [BioCryst Pharmaceuticals, Inc.](http://www.biocryst.com) (NASDAQ:BCRX) today announced results from a second interim analysis of its Phase 2 APeX-1 clinical trial in hereditary angioedema (HAE). APeX-1 is a 3-part dose ranging trial designed to evaluate the efficacy, safety, tolerability, pharmacokinetics 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 second interim analysis evaluated data from all patients in Parts 1 and 2 of the trial. The first interim analysis evaluated data from 28 of 36 patients in Part 1.

"These data support our hypothesis regarding the initial findings seen from the first interim analysis," said Jon Stonehouse, Chief Executive Officer & President of BioCryst. "We are delighted to see that a daily dose of 125 mg of BCX7353 results in a high level of efficacy with an improved tolerability profile compared to the 350 mg dose observed in the first interim analysis. We look forward to completing Part 3 of the trial to select appropriate doses for our pivotal program."

This second interim analysis of pooled data from Parts 1 and 2 evaluated doses of BCX7353 125 mg (n=7), 250 mg (n=6) and 350 mg (n=18) QD versus placebo (n=20) for 28 days. The baseline attack rate was approximately 1/week. Baseline characteristics were generally well balanced between the treatment groups. Compliance with study drug dosing was excellent ($\geq 98\%$).

The pre-specified per-protocol (PP) interim analysis included data on a total of 44 subjects with confirmed Type 1 or Type 2 HAE completing 28 days of treatment. The percentage reductions by treatment group in the mean rate of independently-adjudicated angioedema attacks for the pre-defined effective dosing period (weeks 2 through 4) in BCX7353 treated subjects were: 125 mg QD, 73% (p=0.002); 250 mg QD, 37% (p=0.128) and 350 mg QD, 58% (p=0.001) compared to placebo. In the intent-to-treat (ITT) population, corresponding reductions by treatment group were: 125 mg QD, 73% (p=0.004); 250 mg QD, 44% (p=0.090) and 350 mg QD, 45% (p=0.014) compared to placebo.

A pre-planned analysis of peripheral and abdominal attacks showed reductions in peripheral attacks of 74% (125 mg QD), 54% (250 mg QD) and 90% (350 mg QD) compared with placebo (PP analysis, weeks 2 through 4) and reductions in abdominal attacks of 72% (125 mg QD), 10% (250 mg QD) and 8% (350 mg QD) compared with placebo (PP analysis, weeks 2 through 4). Based on this distribution, it is likely that subjects in the 250 mg and 350 mg arms recorded transient abdominal adverse events (AEs) as HAE attack symptoms in their diary. In contrast, a consistent reduction in attacks regardless of anatomical location was observed in the 125 mg arm.

Oral BCX7353 once-daily for 28 days was generally safe and well tolerated in subjects with HAE. There were no serious AEs and no severe AEs. Three subjects in the BCX7353 350 mg treatment arm, two of which were previously reported, discontinued study drug before day 28. A third subject in this arm discontinued study drug due to vomiting and abdominal cramps concurrent with menses. The most common treatment-emergent adverse events were the common cold and diarrhea. The gastrointestinal AEs previously observed in the 350 mg arm were not seen at the 125 mg dose. Additionally, no significant laboratory abnormalities were observed in the two lower dose groups.

Steady state BCX7353 plasma levels and kallikrein inhibition levels in HAE subjects were similar to those seen in healthy subjects administered the same doses in a previously completed Phase 1 trial. Steady state trough drug levels (24 hours after dosing) greatly exceeded the target therapeutic range at the 250 mg and 350 mg dose levels. Trough levels for the 125 mg dose were generally within the target range.

The efficacy, safety and tolerability profile of BCX7353 observed in this interim analysis strongly supports its continued investigation as a prophylactic treatment for HAE. Enrollment into Part 3 of the trial is progressing well. Completion of Part 3 will enable a full evaluation of the dose response necessary to select doses for a pivotal program.

Conference Call and Webcast

BioCryst's leadership team will host a conference call and webcast today, May 25, 2017 at 9:00 a.m. Eastern Time, to

discuss its APeX-1 second interim analysis and to respond to questions on the APeX-1 interim 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 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 BioCryst 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 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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