BioCryst Reports ZENITH-1 Results With Oral BCX7353 Which Confirm Rapid Onset of Action, Sustained Activity and Robust Dose Response for Treatment of Acute HAE Attacks

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—Based on ZENITH-1 data, company plans to commence Phase 3 trial of single-dose 750 mg oral BCX7353 for acute treatment of hereditary angioedema (HAE) attacks in summer 2019—

—Data show excellent clinical dose response in ZENITH-1, as predicted by pK data—

—BCX7353 well-tolerated at all dose levels (250 mg, 500 mg, 750 mg) in ZENITH-1—

—Data presented at American Academy of Allergy, Asthma & Immunology (AAAAI) annual meeting—

RESEARCH TRIANGLE PARK, N.C., Feb. 23, 2019 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq: BCRX) today reported additional topline data from the Phase 2 ZENITH-1 trial, including new data from the 250 mg and 500 mg dose cohorts. Data from the now complete trial confirm previously reported results showing a single dose of oral 750 mg BCX7353 was well-tolerated and superior to placebo ($p<0.05$) against the majority of efficacy endpoints evaluated in HAE patients suffering an acute attack, and demonstrate a clear dose response across the three dose levels.

Based on the results of ZENITH-1, the company plans to meet with the U.S. Food and Drug Administration (FDA) in the second quarter, and to commence a Phase 3 trial with the 750 mg dose of oral BCX7353 in the summer of 2019.

“The results of ZENITH-1, with onset of action within one hour, duration of effect of a single dose over 24 hours, and a robust efficacy dose response across all dose levels are very exciting for patients who have an urgent need for an oral treatment option for acute attacks,” said Dr. William Sheridan, chief medical officer of BioCryst.

“Based on these excellent results, we plan to quickly advance 750 mg oral BCX7353 into a Phase 3 trial that will be designed to support approval in the U.S. and European Union,” Sheridan added.

Efficacy and tolerability data for the 750 mg dose cohort were previously reported by the company in a September 4, 2018 press release. With the 750 mg dose, compared to placebo, improvement in symptoms and Visual Analog Scale (VAS) scores was seen as early as one hour after oral BCX7353 dosing, and was sustained through 24 hours. Through 24 hours, standard of care (SOC) medication use was reduced by 31.6 percent after BCX7353 compared with placebo ($p=0.0029$), and no or mild symptoms were reported in 64.1 percent of attacks treated with BCX7353 compared with 32.3 percent of attacks treated with placebo ($p=0.0038$).

In the additional data reported today at the AAAAI annual meeting, a clear dose response was observed across the 250 mg to 750 mg range. Across dose levels, BCX7353 was generally safe and well-tolerated with no notable differentiation from the adverse event profile of placebo.

The prior press release from September 4, 2018 containing safety and efficacy data from the 750 mg dose cohort, and the poster containing the additional results presented today at the AAAAI annual meeting, including figures highlighting dose response and tolerability across all three dose levels, can be found on the investor relations section of the company’s website at: http://ir.biocryst.com/.

ZENITH-1 Trial Design

ZENITH-1 was a double-blind, placebo-controlled, randomized, cross-over, dose-ranging trial of oral BCX7353 for acute treatment of angioedema attacks in patients with HAE. A total of 63 patients were randomized and 58 received at least one dose of blinded study drug: 11 in the 250 mg cohort, 14 in the 500 mg cohort and 33 in the 750 mg cohort.

ZENITH-1 was designed for compatibility with modern treatment guidelines for at-home self-administered drug administration as early as possible after onset of symptoms. The goals of the trial were to identify activity against clinically meaningful endpoints that the company could use to construct a Phase 3 registration trial, to identify the dose or doses the company could advance, and to assess safety and tolerability.

Adults with HAE Type I or II self-administered a dose of blinded study drug for three attacks; two treated with active drug and one with placebo, in a randomized sequence. Subjects were asked to administer blinded study medicine within one hour of onset of symptoms. Subjects were free to use approved prescribed acute medications but were asked to wait at least four hours post-study drug if possible. Patient completed trial diaries to collect information on symptoms, VAS scores and use of SOC medicines prior to dosing and at 1, 2, 3, 4, 8 and 24 hours after study drug administration. In the 250 mg, 500 mg and 750 mg cohorts respectively, a total of 21, 25 and 64 attacks were treated with BCX7353 and 11, 11 and 31 attacks were treated with placebo.

About BCX7353

Discovered by BioCryst, BCX7353 is a novel, oral, once-daily, selective inhibitor of plasma kallikrein currently in advanced clinical development for the prevention and treatment of angioedema attacks in patients with HAE. BCX7353 was 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, each 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 has also completed the ZENITH-1 clinical trial. ZENITH-1 was a proof-of-concept Phase 2 clinical trial testing oral BCX7353 for the treatment of acute angioedema attacks.

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 of oral drugs for rare diseases including BCX7353, an oral plasma kallikrein inhibitor for treatment of hereditary angioedema; a preclinical program with an oral ALK-2 inhibitor for treatment of fibrodysplasia ossificans progressive, and intravenous galidesivir, a broad-sepctrum viral RNA polymerase inhibitor, as a potential treatment for Marburg virus disease and Yellow Fever, under contracts from NIAID and HHS/BARDA. RAPIVAB® (peramivir injection), an intravenous influenza virus 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 ongoing and future preclinical and clinical development of HAE second generation drug candidates (including APeX-2, APeX-S and APeX-J) may not have positive results, may be more expensive or may not move as quickly as planned; that the FDA, EMA or other applicable regulatory agency may not provide regulatory clearances which may result in delay of planned clinical trials or failure to achieve 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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