New Clinical Trial Results and Market Research Support Significant Commercial Opportunity for Oral, Once Daily BCX7353 in HAE

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—Results of the APeX-S and APeX-2 trials show patients taking 150 mg of oral, once daily BCX7353 achieved a stable average attack rate of ≤ 1 attack per month at 48 weeks—

—75 percent of patients taking 150 mg BCX7353 completed 48 weeks of dosing in the Phase 3 APeX2 trial—

—Comprehensive market research from 100 HAE patients and 175 treating physicians shows strong demand for oral, once daily BCX7353—

RESEARCH TRIANGLE PARK, N.C., Nov. 06, 2019 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq: BCRX) today announced 48-week results from its APeX-S and APeX-2 trials and comprehensive market research which support the significant commercial opportunity for oral, once daily BCX7353 in HAE.

“The 48-week clinical trial data we now have from APeX-S and APeX-2 highlight the control patients are having over their attacks with oral, once daily BCX7353, and consequently why 75 percent of patients stayed on-study through 48 weeks when they had other choices,” said Jon Stonehouse, chief executive officer of BioCryst.

“Since receiving the 24-week data from APeX-2 in May, we have conducted detailed and comprehensive market research to update our understanding of the commercial potential and value of BCX7353 with patients, treating physicians and payors. It is clear from this work that, regardless of their current treatment, HAE patients are eager to use, physicians are expecting to prescribe and payors are willing to reimburse oral once a day BCX7353,” Stonehouse added.

Key Findings from 48-week APeX-S and APeX-2 Data:

- In APeX-2, patients experienced a rapid and sustained decrease in their attack frequency over 48 weeks. Thirty patients who were randomized to 150 mg of BCX7353 at the beginning of the study and completed 48 weeks of therapy had a baseline attack rate of 2.9 attacks per month, which declined to 1.4 attacks per month after one month and to 1.0 attacks per month at month 12.

- APeX-2 patients who switched from placebo to 150 mg of BCX7353 at the week 24 visit saw dramatic and sustained reductions in their HAE attack rate. Their mean attack rate dropped to 0.5 attacks per month at month seven and to 0.4 attacks per month at month 12.

- APeX-S patients taking 150 mg of BCX7353 had similar attack control as those in APeX-2. Patients completing 48 weeks of treatment on 150 mg of BCX7353 (n=73) had a median attack rate of zero attacks per month in six of the 12 months, including month 12 (week 48).

- 75 percent of HAE patients who were on 150 mg of oral BCX7353 in the APeX-2 trial completed 48 weeks of treatment.

- The integrated 48-week analysis across both APeX-2 and APeX-S showed no new safety findings. BCX7353 was safe and generally well tolerated in a total of 342 patients with a total of 232 patient-years of daily oral dosing. The most common adverse event was the common cold, which occurred with similar frequency in BCX7353 and placebo patients. Gastrointestinal events led to discontinuation of BCX7353 in three percent of patients. Drug-related serious adverse events occurred in three of 342 subjects (0.9%) and resolved after stopping or interrupting BCX7353 dosing.

- In APeX-S, alanine aminotransferase levels >3xULN were seen in 14 of 49 patients who discontinued androgens within 28 days prior to study entry, compared to one of 104 patients who discontinued androgens more than 28 days prior to study entry and zero of 74 patients who had never used androgens. These observations support a proposed four-week washout period for current androgen patients before beginning therapy with BCX7353.

Key Findings from Market Research

- The prevalence of HAE in the U.S. is higher than previously estimated. A comprehensive study of U.S. administrative claims data from 274 million covered lives establishes a prevalence of approximately 10,000 total HAE patients and 7,500 diagnosed and treated HAE patients in U.S.

- More than 80 percent of the 100 HAE patients in the market research self-reported being on prophylactic therapy.
- The 175 physicians in the market research, who in total treat more than 1,300 HAE patients, report they currently treat 58 percent of HAE patients with prophylactic therapy and anticipate they will treat 80 percent of HAE patients with prophylactic therapy in the future.
- Patient demand for BCX7353 is strong, regardless of their current therapy. When 100 patients were shown the APeX-2 24-week product profile, 60 percent of HAE patients said they would be very willing to use BCX7353.
- HAE-treating physicians expect to prescribe BCX7353 to 41 percent of their HAE patients.
- Payors expressed a broad willingness to reimburse oral BCX7353 in pricing research with insurance plans and pharmacy benefit managers representing more than 100 million covered lives.

Conference Call and Webcast

BioCryst management will host a conference call and webcast at 8:00 a.m. ET today. The live call may be accessed by dialing 877-303-8027 for domestic callers and 760-536-5165 for international callers and using conference ID # 4891026. A live webcast of the call and any slides will be available online at the investors section of the company website at www.biocryst.com. A telephone replay of the call will be available by dialing 855-859-2056 for domestic callers or 404-537-3406 for international callers and entering the conference ID # 4891026.

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 including BCX7353, an oral treatment for hereditary angioedema, BCX9930, an oral Factor D inhibitor for the treatment of complement-mediated diseases, galidesivir, a potential treatment for Marburg virus disease and Yellow Fever, and BCX9250, an ALK-2 inhibitor for the treatment of fibrodysplasia ossificans progressiva. 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 BCX9930, BCX9250 and 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, PMDA 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; that actual financial results may not be consistent with expectations, including that 2019 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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