



BioCryst to Provide Berotralstat and BCX9930 Program Updates at 38th Annual J.P. Morgan Healthcare Conference

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RESEARCH TRIANGLE PARK, N.C., Jan. 12, 2020 (GLOBE NEWSWIRE) -- [BioCryst Pharmaceuticals, Inc.](#) (Nasdaq: BCRX) today announced that the company will provide updates on berotralstat, an oral kallikrein inhibitor for hereditary angioedema (HAE), and BCX9930, an oral Factor D inhibitor for complement-mediated diseases, this week at the 38th Annual J.P. Morgan Healthcare Conference in San Francisco.

"BioCryst is positioned for transformation in 2020 with multiple global approvals and launches of berotralstat, and PNH proof of concept data with BCX9930. The \$100 million in additional capital we brought into the company in Q4 2019 provides a foundation for progress and value creation in 2020," said Jon Stonehouse, president and chief executive officer of BioCryst.

Berotralstat Program Updates:

- New drug application (NDA) submitted to U.S Food and Drug Administration in December 2019
- APeX-J trial in Japan met its primary endpoint ($p=0.003$) for prevention of HAE attacks, and berotralstat was safe and generally well-tolerated
- JNDA submission to Japanese Pharmaceuticals and Medical Devices Agency (PMDA) on-track for Q1 2020
- Marketing authorization application to European Medicines Agency (EMA) on-track for Q1 2020

BCX9930 Program Updates:

As previously announced, results from an ongoing three part Phase 1 trial of BCX9930 showed rapid, sustained and >95% suppression of the alternative pathway (AP) of the complement system at 100 mg every 12 hours, as measured by the AP Wieslab[®] assay.

In two initial multiple ascending dose (MAD) assessment cohorts, healthy volunteers received 50 mg or 100 mg of oral BCX9930 or placebo (each MAD cohort randomized 10:2) administered every 12 hours for seven days. Healthy volunteers in the MAD cohorts were prophylactically dosed with the broad-spectrum antibiotic, amoxicillin/clavulanate. BCX9930 was safe and generally well tolerated at all doses studied in single ascending dose and MAD cohorts. There were no serious adverse events. A clinically benign rash was observed in some healthy volunteers in the MAD (two in the 50 mg cohort, seven in the 100 mg cohort), which was self-limited and resolved in 4-8 days after onset.

The company has now completed an additional MAD cohort with 50 mg of oral BCX9930 or placebo administered every 12 hours for 14 days, with vaccination instead of an antibiotic. Key observations from the additional MAD cohort include:

- Benign rash (similar to prior MAD cohorts) that was self-limited and resolved in 4 to 8 days post-onset seen in seven healthy volunteers
- Successfully dosed-through benign rash, with rash resolving on-drug, in both patients who continued dosing, per protocol
- Biopsies of rashes from multiple subjects confirm benign assessment

The company is on-track to report proof of concept data in paroxysmal nocturnal hemoglobinuria (PNH) patients in 1H 2020.

Additional details can be found on [slides](#), which can be accessed at the Investors section of BioCryst's website at <http://www.biocryst.com>.

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 berotralstat (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 oral ALK-2 inhibitor for the treatment of fibrodysplasia ossificans progressiva (FOP). 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 or BCX9250 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. 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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