



## BioCryst Announces Publication of APeX-1 Clinical Trial Results for BCX7353 in The New England Journal of Medicine

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73.8% reduction in attacks with significant benefits in quality of life from once-daily oral dosing of BCX7353 125 mg

RESEARCH TRIANGLE PARK, N.C., July 25, 2018 (GLOBE NEWSWIRE) -- [BioCryst Pharmaceuticals, Inc.](#) (NASDAQ:BCRX), a pharmaceutical company focused on the development and commercialization of treatments for rare diseases, today announced that results from the Phase 2, APeX-1 clinical trial of BCX7353 for the prevention of attacks in patients with hereditary angioedema ("HAE") are published online in the July 26<sup>th</sup> issue of *The New England Journal of Medicine* (DOI: 10.1056/NEJMoa1716995).

The APeX-1 trial was a double blind, randomized, parallel group, placebo-controlled Phase 2 dose ranging trial comparing the safety and efficacy of 28 days of once-daily BCX7353 treatment, at doses of 62.5 mg, 125 mg, 250 mg, and 350 mg, with placebo. The trial demonstrated that oral administration of BCX7353 at a dose of 125 mg or more resulted in a significantly lower rate of attacks compared with placebo. Significant benefits with respect to quality of life were observed in the 125 mg and 250 mg dose groups. Mild gastrointestinal symptoms were the principal side effect, particularly in the 250 mg and 350 mg BCX7353 dose groups.

"For patients with hereditary angioedema, the results of this trial suggest potential for the future to manage their disease with a prophylactic therapy that would combine efficacy and safety with the advantage of oral administration," said Emel Aygören-Pürsün, M.D., principal investigator for the APeX-1 trial and Head of Interdisciplinary Competence Center for Hereditary Angioedema, and Specialist in Internal Medicine and Hemostaseology Department for Children and Adolescents, Goethe University Hospital Frankfurt.

"We are extremely pleased to have these important results published in *The New England Journal of Medicine*," said Jon P Stonehouse, President and Chief Executive Officer of BioCryst. "We look forward to confirming the results from APeX-1 in our pivotal Phase 3 trial, APeX-2, which we expect to report out in the first half of 2019."

### About BCX7353

Discovered by BioCryst, BCX7353 is a novel, oral, once-daily, selective inhibitor of plasma kallikrein currently in development for the prevention and treatment of angioedema attacks in patients diagnosed 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, both 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 is also conducting the ZENITH-1 clinical trial. ZENITH-1 is a proof-of-concept Phase 2 clinical trial testing an oral formulation of BCX7353 for the treatment of acute angioedema attacks.

### About BioCryst Pharmaceuticals

BioCryst Pharmaceuticals designs, optimizes and develops novel small-molecule medicines that address both common and rare conditions. BioCryst has several ongoing development programs including BCX7353, an oral treatment for hereditary angioedema, galidesivir, a potential treatment for filoviruses, and a preclinical program to develop oral ALK-2 inhibitors for the treatment of fibrodysplasia ossificans progressiva ("FOP"). RAPIVAB<sup>®</sup> (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, 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](http://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 HAE second generation drug candidates (including ZENITH-1, APeX-2, and APeX-S) 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 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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