



BioCryst Begins Enrollment in Phase 1 Trial Evaluating BCX17725, a KLK5 Inhibitor, for the Treatment of Netherton Syndrome

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—Potential best-in-class targeted treatment for Netherton syndrome is company's first protein therapeutic to advance to the clinic—

—Initial data from trial expected by end of 2025—

RESEARCH TRIANGLE PARK, N.C., Oct. 02, 2024 (GLOBE NEWSWIRE) -- [BioCryst Pharmaceuticals, Inc.](#) (Nasdaq: BCRX) today announced the enrollment of the first participant in a Phase 1 trial evaluating BCX17725, a potent and selective investigational kallikrein 5 (KLK5) inhibitor designed to provide best-in-class disease-modifying treatment for people with Netherton syndrome.

"People living with Netherton syndrome face many challenges in their daily lives and currently only have topical treatments and other supportive medications available. There is a significant unmet need for a targeted therapy that can address the underlying cause of the disease. Based on the data from our nonclinical studies, we believe BCX17725 has the potential to achieve the potency, specificity and convenient dosing needed to become a best-in-class therapy for this ultra-rare disease, and have a significant impact on the lives of patients," said Dr. Helen Thackray, chief research and development officer of BioCryst.

BCX17725 is a protein therapeutic that is designed to treat the underlying protein deficiency that causes Netherton syndrome by inhibiting KLK5, a serine protease in the skin that is unregulated in people with Netherton syndrome. The trial will evaluate the safety, tolerability, pharmacokinetics and immunogenicity of BCX17725 when administered via single and multiple doses in healthy adult participants (Parts 1 and 2), and multiple doses in participants with Netherton syndrome in an open-label design (Part 3). The company expects to report initial results from the trial by the end of 2025.

Netherton syndrome is a serious, rare, lifelong genetic disorder affecting the skin, hair, and immune system, caused by lack of normal function of a natural inhibitor of KLK5. People with Netherton syndrome often have red, scaly, inflamed skin, fragile hair, and are more likely to develop skin infections, allergies, asthma and eczema. Netherton syndrome can be life threatening, especially during infancy when patients are vulnerable to dehydration and recurrent infections. Currently, there are no approved treatments for Netherton syndrome.

For more information about the trial, visit [ClinicalTrials.gov](#) and search NCT number NCT06539507.

About BioCryst Pharmaceuticals

BioCryst Pharmaceuticals is a global biotechnology company with a deep commitment to improving the lives of people living with complement-mediated and other rare diseases. BioCryst leverages its expertise in structure-guided drug design to develop first-in-class or best-in-class oral small-molecule and protein therapeutics to target difficult-to-treat diseases. BioCryst has commercialized ORLADEYO[®] (berotralstat), the first oral, once-daily plasma kallikrein inhibitor, and is advancing a pipeline of small-molecule and protein therapies. For more information, please visit [www.biocryst.com](#) or follow us on [LinkedIn](#).

Forward-Looking Statements

This press release contains forward-looking statements, including statements regarding our plans and expectations for our BCX17725 program and other 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, performance 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: ongoing and future preclinical and clinical development of BCX17725 may take longer than expected and may not have positive or best-in-class results; BioCryst may not be able to enroll the required number of subjects in planned clinical trials of product candidates; BioCryst may not advance human clinical trials with product candidates as expected; the FDA or other applicable regulatory agency may require additional studies beyond the studies planned for product candidates, may not provide regulatory clearances which may result in delay of planned clinical trials, may impose certain restrictions, warnings, or other requirements on product candidates, may impose a clinical hold with respect to product candidates, or may withhold or delay market approval for product candidates; and product candidates, if approved, may not achieve market acceptance. 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 actual results to differ materially from those contained in BioCryst's forward-looking statements.

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