# Journal of Allergy and Clinical Immunology Publishes Results from BioCryst's APeX-2 Pivotal Trial of Oral, Once-Daily Berotralstat for the Prevention of HAE Attacks

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***—Berotralstat significantly reduced HAE attack rates versus placebo over 24 weeks —***

RESEARCH TRIANGLE PARK, N.C., Oct. 22, 2020 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq: BCRX) today announced that data from the first 24 weeks of the APeX-2 trial of oral, once-daily berotralstat in patients with hereditary angioedema (HAE) have been published online by the *Journal of Allergy and Clinical Immunology* (JACI).

APeX-2 was a Phase 3, double-blind, placebo-controlled, parallel-group trial that evaluated the efficacy and safety of oral, once-daily berotralstat versus placebo over 24-weeks in 121 HAE patients ages 12 years or older. In the trial, after 24 weeks, both the 110-mg and 150-mg doses of berotralstat significantly reduced HAE attack rates compared with placebo and were safe and well tolerated, with greater attack rate reductions observed for the 150-mg dose.

As the authors concluded, the combination of efficacy, safety, and tolerability with convenient oral, once-daily dosing will make berotralstat an important addition to the HAE-C1-INH therapeutic armamentarium, if approved.

"The data from APeX-2 show that berotralstat may provide an important oral option for patients with HAE that could allow them to prevent HAE attacks and reduce their overall burden of therapy," said Bruce Zuraw, M.D., professor of medicine and chief of the Division of Rheumatology, Allergy and Immunology at the University of California School of Medicine, and principal investigator of the APeX-2 trial.

"We are excited to have our results published in JACI, building on the compelling pivotal and longer-term data presented at scientific congresses in the past year. HAE patients are waiting for an oral, once-daily option to control their disease. As the authors have recognized, providing patients with an effective, oral therapy is a major step towards the goal of enabling them to live a normal life," said Jon Stonehouse, president and chief executive officer of BioCryst.

Additional details can be found in the manuscript, which is available online at www.jacionline.org/inpress. JACI is an official journal of the American Academy of Allergy, Asthma, and Immunology.

The U.S. Food and Drug Administration (FDA) is reviewing a new drug application (NDA) for ORLADEYO ™ (berotralstat) and has set an action date of December 3, 2020, under the Prescription Drug User Fee Act (PDUFA).

In Japan, ORLADEYO is being reviewed under Sakigake designation. The Pharmaceutical and Medical Devices Agency (PMDA) has confirmed their regulatory review schedule and the company expects an approval decision in December 2020.

On March 30, 2020, the company announced that the European Medicines Agency (EMA) had validated its marketing authorization application (MAA) submission for ORLADEYO and begun its formal review of the MAA under the centralized procedure. The company expects an opinion from the Committee for Medicinal Products for Human Use (CHMP) within approximately 12 months from MAA validation.

**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 ORLADEYO ™ (berotralstat), an oral treatment for hereditary angioedema, BCX9930, an oral Factor D inhibitor for the treatment of complement-mediated diseases, galidesivir, a potential treatment for COVID-19, 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 are identified by use of terms such as "will," "expect," "may," "could," and similar words, although some forward-looking statements may be expressed differently. These statements involve known and unknown risks, uncertainties and other factors which may cause 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: the ongoing COVID-19 pandemic, which could create challenges in all aspects of BioCryst's business, including without limitation delays, stoppages, difficulties and increased expenses with respect to BioCryst's and its partners' development, regulatory processes and supply chains, negatively impact BioCryst's ability to access the capital or credit markets to finance its operations, or have the effect of heightening many of the risks described below or in the documents BioCryst files periodically with the Securities and Exchange Commission; developing and commercializing ORLADEYO or any HAE product candidate may take longer or may be more expensive than planned; 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, EMA, PMDA 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 such

product candidates, or may withhold market approval for product candidates; product candidates, if approved, may not achieve market acceptance; BioCryst's ability to successfully commercialize its product candidates, manage its growth, and compete effectively; and risks related to the international expansion of BioCryst's business. 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 forward-looking statements.

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