BioCryst Presents Data Demonstrating >99 Percent Suppression of Alternative Pathway Complement Activity with BCX9930 in C3G Patients

August 26, 2022

Study represents first data with BCX9930 in C3G patients

RESEARCH TRIANGLE PARK, N.C., Aug. 26, 2022 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq: BCRX) today announced that its oral Factor D inhibitor, BCX9930, demonstrated >99 percent suppression of the alternative pathway (AP), and that >98 percent suppression was maintained for 24 hours post-dosing, in patients with C3 glomerulopathy (C3G), a rare renal disease that is characterized by dysregulation of the AP of the complement system.

The data, which are the first data with BCX9930 in patients with C3G, are being presented in a poster session at the 18th European Meeting on Complement in Human Disease, which is being held in Bern, Switzerland, from August 26-29, 2022.

"The analyses from this study add to the body of evidence that show Factor D inhibition can correct dysregulation of the alternative pathway and has the potential to be a meaningful treatment approach for multiple complement-mediated diseases. The data are consistent using both commercially available assays and sophisticated proprietary assays our team has developed, which we can also utilize with our next-generation pipeline programs to quickly assess complement activation *in vivo* early in development," said Dr. Yarlagadda S. Babu, chief discovery officer of BioCryst.

BioCryst EMCHD 2022 Presentation Highlights

***BCX9930, an Oral Factor D Inhibitor, Suppresses Complement Alternative Pathway C3 Convertase Activity* in vitro*, and in Patients with Complement 3 Glomerulopathy* (Poster #80); Sunday, August 28, 2022; 11:45am-1:30pm CET; Hintere Gasse, Building Fab8**

- The study assessed the effects of oral BCX9930 on AP activity in *ex vivo* activated serum from healthy volunteers (n=16) and C3G patients (n=6) who participated in a phase 1, open-label study to evaluate the pharmacokinetics and pharmacodynamics of a single dose of BCX9930.
- One assay was used to assess the ability of BCX9930 to inhibit formation of AP C3 convertase *in vitro* (Elisa-detecting properdin (P)-bound C3 convertase), two assays were used to assess the ability of BCX9930 to inhibit AP complement activity *ex vivo* (AP Wieslab kit and multiplex assays), and another assay was used to assess the ability of BCX9930 to inhibit formation of AP C3 convertase *ex vivo* (immunofixation electrophoresis).
- The analyses found that a single oral dose (600 mg) of BCX9930:
    - Demonstrated >99 percent (median) suppression of the AP, and that >98 percent (median) suppression was maintained for 24 hours post-dosing, in C3G patients.
    - Achieved rapid (within two hours) and maximal (median >99 percent relative to pre-dose levels) suppression of the generation of C3a, a product of C3 convertase activity, in both healthy volunteers and C3G patients.
- These data support further development of Factor D inhibitors for the treatment of complement-mediated diseases, including C3G and other diseases driven by dysregulation of the AP.

In the study, a single dose of BCX9930 600 mg was generally safe and well-tolerated. Based on prior work with BCX9930, the proposed clinical dose of 400 mg (bid) provides a similar level of AP suppression as the 600 mg single dose used in this study in C3G patients.

Currently, BioCryst is conducting the RENEW proof-of-concept basket study evaluating BCX9930 in C3G, immunoglobulin A nephropathy (IgAN) and primary membranous nephropathy (PMN), all rare renal diseases caused by dysregulation of the alternative pathway.

BioCryst is also currently conducting the REDEEM-1 and REDEEM-2 pivotal trials evaluating BCX9930 in paroxysmal nocturnal hemoglobinuria (PNH).

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. Oral, once-daily ORLADEYO® (berotralstat) is approved in the United States and multiple global markets. BioCryst has several ongoing development programs including BCX9930, an oral Factor D inhibitor for the treatment of complement-mediated diseases, BCX9250, an ALK-2 inhibitor for the treatment of fibrodysplasia ossificans progressiva, and galidesivir, a potential treatment for Marburg virus disease and yellow fever. RAPIVAB® (peramivir injection) is approved in the U.S. and multiple global markets, with post-marketing commitments 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 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: 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 periodically files with the Securities and Exchange Commission; ongoing and future preclinical and clinical development of BCX9930 may not have positive 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; and 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. 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, which identify important factors that could cause the actual results to differ materially from those contained in BioCryst's forward-looking statements.

BCRXW

Investor Contact:
John Bluth
+1 919 859 7910
jbluth@biocryst.com

Media Contact:
Catherine Collier Kyroulis
+1 917 886 5586
ckyroulis@biocryst.com