FDA Grants Fast Track Designation for BCX9930 in PNH

August 3, 2020

RESEARCH TRIANGLE PARK, N.C., Aug. 03, 2020 (GLOBE NEWSWIRE) -- BioCryst Pharmaceuticals, Inc. (Nasdaq:BCRX) today announced that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation for its oral Factor D inhibitor, BCX9930, for the treatment of paroxysmal nocturnal hemoglobinuria (PNH).

According to the FDA, the purpose of the Fast Track designation is to get important new drugs to the patient earlier by facilitating the development, and expediting the review, of drugs to treat serious conditions and fill an unmet medical need.

A drug that receives Fast Track designation is eligible for some or all of the following:

- More frequent meetings with FDA to discuss the drug's development plan and ensure collection of appropriate data needed to support drug approval.
- More frequent written communication from FDA about such things as the design of the proposed clinical trials and use of biomarkers.
- Eligibility for accelerated approval and priority review, if relevant criteria are met.
- Rolling review, which means that a drug company can submit completed sections of its new drug application (NDA) for review by FDA, rather than waiting until every section of the NDA is completed before the entire application can be reviewed.

"PNH patients have a tremendous need for therapy improvements and it is exciting for patients that the FDA has reviewed our proof of concept PNH data and granted Fast Track status. This designation can significantly accelerate the development timeline for BCX9930," said Dr. William Sheridan, chief medical officer of BioCryst.

"We look forward to working closely with the FDA to fully utilize the opportunities presented by our Fast Track designation to advance this important medicine to patients as quickly as possible in PNH. In addition, we look forward to regulatory discussions later this year on clinical trials for BCX9930 in nephrology indications," Sheridan added.

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 potential benefits associated with an FDA Fast Track designation. These statements involve known and unknown risks, uncertainties and other factors which may cause the opportunities associated with the Fast Track designation for BCX9930 to be materially different from those expressed or implied by the forward-looking statements. These statements reflect our current views 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: Fast Track designation by the FDA may not lead to a faster development, regulatory review, or approval process with the FDA and does not increase the likelihood that BCX9930 will receive marketing approval; the ongoing COVID-19 pandemic 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; 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 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 a clinical hold with respect to such product candidates, or may 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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