



BioCryst Submits Japanese New Drug Application for Oral, Once Daily Berotralstat

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RESEARCH TRIANGLE PARK, N.C., Feb. 03, 2020 (GLOBE NEWSWIRE) -- [BioCryst Pharmaceuticals, Inc.](#) (Nasdaq: BCRX) today announced that it has submitted a new drug application (JNDA) to the Japanese Pharmaceuticals and Medical Devices Agency (PMDA) for approval of oral, once daily berotralstat for the prophylactic treatment of hereditary angioedema (HAE).

"The data from APeX-J and the berotralstat clinical program are very exciting for HAE patients in Japan," said Dr. Isao Ohsawa, president of Saiyu Soka hospital and principal investigator of the APeX-J trial.

"Based on the clinical benefit observed in patients in APeX-J, I expect many patients in Japan will want to use this new treatment, once it becomes available," he added.

BioCryst received Orphan Drug and Sakigake designations for berotralstat. Under the Sakigake timeline, the company expects potential approval in the second half of 2020.

"Berotralstat would be the first approved chronic therapy for HAE patients in Japan and would address a significant unmet medical need," said Jon Stonehouse, president and chief executive officer of BioCryst.

Torii Pharmaceutical, Co. is BioCryst's commercial partner in Japan for berotralstat. Under the terms of its commercial agreement with Torii, BioCryst received a \$22 million upfront payment and is eligible to receive an additional milestone payment of up to \$20 million based on the timing of PMDA approval and upon receipt of a reimbursement price approval from Japan's National Health Insurance system in excess of the threshold specified in the agreement. In addition, BioCryst will receive tiered royalties of up to 40 percent of Japanese net sales of berotralstat.

The JNDA contains data from both the APeX-J and APeX-2 clinical trials. The company recently announced that the APeX-J trial in Japan met its primary endpoint ($p=0.003$) of a reduction in HAE attacks from baseline for berotralstat 150 mg compared to placebo, and berotralstat was safe and generally well-tolerated in the trial. In APeX-2, berotralstat also met its primary endpoint ($p<0.001$) for berotralstat 150 mg compared to placebo, and was safe and generally well-tolerated.

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 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 Marburg virus disease and Yellow Fever, and BCX9250, an oral 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 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 subject to risks and uncertainties. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Factors that could cause actual results to differ materially from the forward-looking statements contained herein include, without limitation, the following: the results of our partnership with Torii may not meet our current expectations (including with respect to the receipt or amounts of potential milestone or royalty payments); competitor products may limit the commercial potential of berotralstat in Japan and the amount of any related royalties we would be entitled to receive; there are risks related to our relying on the performance of our partner, particularly with respect to the conduct of commercialization activities in line with our current expectations; there are risks related to government actions, including that decisions and other actions relating to approval, pricing, and exclusivity of berotralstat in Japan may not be taken when expected or at all, or that the outcomes of such decisions and other actions may not be in line with our current expectations; we rely on third-party contract manufacturing organizations to manufacture berotralstat and any failure of such parties to meet their obligations may impair our ability to supply the required amounts of berotralstat to our partner; there are inherent risks related to commercializing drugs, including regulatory, manufacturing and supply risks; development activities for any indication may take longer or may be more expensive than planned; ongoing and future preclinical and clinical development of our HAE drug candidates (including for APeX-S) may not have positive results; we may not be able to enroll the required number of subjects in planned clinical trials; we may not advance human clinical trials as expected (including those for berotralstat); the FDA, EMA, PMDA or other applicable regulatory agency may refuse to review our applications, require additional studies beyond the studies planned for product candidates (including berotralstat), or may not provide regulatory clearances for studies, which could result in delays of planned clinical trials; and applicable regulatory bodies may impose a clinical hold with respect to, or withhold market approval for, product candidates (including berotralstat). Please refer to the documents the Company files periodically with the Securities and Exchange Commission, specifically our 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 our projections and forward-looking statements.

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Contact:

John Bluth

+1 919 859 7910

jbluth@biocryst.com