



Krystal Biotech Announces Virtual Presentation of Pre-clinical Data on KB407 for the Treatment of Cystic Fibrosis

October 19, 2021

PITTSBURGH, Oct. 19, 2021 (GLOBE NEWSWIRE) -- [Krystal Biotech Inc.](#), ("Krystal") (NASDAQ: KRYS), the leader in redosable gene therapies for rare diseases, today announced the presentation of pre-clinical data describing the nonclinical pharmacology of nebulized KB407, a redosable gene therapy candidate for the treatment of cystic fibrosis, in a poster at the North American Cystic Fibrosis Conference (NACFC) taking place virtually November 2 – 5, 2021. The data include more detailed results from the IND-enabling good laboratory practice (GLP) toxicology study of KB407 from which top line results were previously [announced](#). The company was recently [granted](#) approval to conduct a Phase 1 clinical study of inhaled KB407 in patients with cystic fibrosis (CF) in Australia by the Bellberry Human Research Ethics Committee (HREC) and plans to initiate a clinical trial in the United States in 2022.

Abstract Information:

e-Poster Title: Nonclinical Pharmacology of Nebulized KB407 for the Treatment of Cystic Fibrosis

Poster Number: 541

Full text of accepted abstracts is now available on the conference website at <https://www.nacfcconference.org/WorkArea/DownloadAsset.aspx?id=1073>.

About KB407

KB407 is a redosable gene therapy designed to correct the underlying cause of CF by delivering two copies of the CFTR gene directly to the airway epithelial cells when delivered via a nebulizer. By inducing expression of full length, normal CFTR protein in the lung, treatment with KB407 has potential to restore ion and water flow into and out of lung cells to correct the lung manifestations of the disease in patients regardless of their underlying genetic mutation.

About Cystic Fibrosis

Cystic fibrosis is the most common fatal inherited disease in the United States, affecting more than 80,000 patients worldwide. CF is caused by genetic mutations that result in dysfunctional or absent CFTR protein. Lack of functional CFTR in secretory airway epithelial cells causes dehydrated mucus buildup in the lungs, pancreas and other organs. This mucus buildup in the lungs leads to recurrent chest infections, increased airway secretions, and eventually, respiratory failure. According to the US Cystic Fibrosis Foundation ("CFF"), the median age at death for patients with CF in the United States was 30.8 years in 2018.

CFTR modulators are effective in a subset of patients with certain CFTR mutations, though because these therapies work to improve the function of mutated protein, patients still experience pulmonary exacerbations and a progressive decline in lung function, which represents a significant unmet need. In addition, approximately 10% of CF patients harbor genetic mutations that are not expected to be responsive to current therapies, and currently have no available disease-modifying treatment options.

About Krystal Biotech

Krystal Biotech, Inc. (NASDAQ: KRYS) is a pivotal-stage gene therapy company leveraging its novel, redosable gene therapy platform and in-house manufacturing capabilities to develop therapies to treat serious rare diseases. For more information, please visit <http://www.krystalbio.com>.

Forward-Looking Statements

Any statements in this press release about future expectations, plans and prospects for Krystal Biotech, Inc., including but not limited to statements about the development of Krystal's product candidates, such as plans for the design, conduct and timelines of ongoing pre-clinical and clinical trials of KB407, and Krystal's plans for filing of regulatory approvals and efforts to bring KB407 to market; the market opportunity for and the potential market acceptance of KB407; plans to pursue research and development of other product candidates; the sufficiency of Krystal's existing cash resources; the unanticipated impact of COVID-19 on Krystal's business operations, pre-clinical activities and clinical trials; and other statements containing the words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "likely," "will," "would," "could," "should," "continue," and similar expressions, constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including: the uncertainties inherent in the initiation and conduct of clinical trials, availability and timing of data from clinical trials, whether results of early clinical trials or trials will be indicative of the results of ongoing or future trials, uncertainties associated with regulatory review of clinical trials and applications for marketing approvals, the availability or commercial potential of product candidates including KB408 the sufficiency of cash resources and need for additional financing and such other important factors as are set forth under the caption "Risk Factors" in Krystal's annual and quarterly reports on file with the U.S. Securities and Exchange Commission. In addition, the forward-looking statements included in this press release represent Krystal's views as of the date of this release. Krystal anticipates that subsequent events and developments will cause its views to change. However, while Krystal may elect to update these forward-looking statements at some point in the future, it specifically disclaims any obligation to do so. These forward-looking statements should not be relied upon as representing Krystal's views as of any date subsequent to the date of this release.

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Source: Krystal Biotech, Inc.