



Krystal Biotech Receives Approval from the Human Research Ethics Committee in Australia for Phase 1 Trial of KB407 for Cystic Fibrosis

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- *KB407 is an investigational inhaled gene therapy derived from Krystal's clinically validated redosable gene delivery technology platform*

PITTSBURGH, Sept. 29, 2021 (GLOBE NEWSWIRE) -- [Krystal Biotech Inc.](#), ("Krystal") (NASDAQ: KRY5), the leader in redosable gene therapies for rare diseases, announced today that it has been granted approval by the Bellberry Human Research Ethics Committee (HREC) in Australia to conduct a Phase 1 clinical study of inhaled KB407 in patients with cystic fibrosis (CF). The Company previously received license to evaluate KB407 from Australia's Office of the Gene Technology Regulator (OGTR). KB407 is an engineered viral vector designed to deliver full-length human cystic fibrosis transmembrane conductance regulator (CFTR) to the airways of people with cystic fibrosis via nebulization. Study enrollment is expected to begin shortly following approval by the trial site's institutional governance and biosafety committee.

"There remains need for a genetically corrective CF therapy that can help lung cells make full-length, normal CFTR protein in any CF patient - KB407 was designed to do just that," said Suma Krishnan, Chief Operating Officer of Krystal Biotech. "While COVID related delays have presented a challenge, we look forward to initiating this proof-of-concept trial in Australia in the coming weeks and initiating a clinical trial in the US in 2022."

Phase 1 Trial Overview

This open-label Phase 1 study is designed to evaluate KB407 in up to 13 adult patients with cystic fibrosis regardless of their underlying genotype, but will exclude patients who are currently taking a CFTR modulator, or have taken a CFTR modulator within seven days of the first KB407 dose (7-day washout period required). Each KB407 dose will be nebulized over approximately 20 minutes. This study will include three cohorts and enroll four participants each into the first two cohorts and five subjects into the last cohort with dosing as follows:

- Participants in Cohort 1 will receive a single dose of KB407 on Day 0. Subjects in Cohort 1 may rollover into Cohort 2 at the Day 28 visit.
- Participants in Cohort 2 will be dosed bi-weekly on Day 0 and Day 14. A Data Safety Monitoring Board (DSMB) will meet to determine study progress from Cohort 2 into Cohort 3.
- Participants in Cohort 3 will be dosed weekly on Day 0, Day 7, Day 14 and Day 21.

The primary endpoint of the trial will be the safety and tolerability of nebulized KB407. Changes in lung function from baseline will be assessed by forced expiratory volume in one second (FEV1) and measurement of lung clearance index (LCI) from multiple-breath washout (MBW) testing. Subjects may receive an optional intradermal dose of neat drug product, enabling a minimally invasive biopsy and assessment of CFTR expression via q-PCR.

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:KRY5) 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; the clinical utility 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 KB407, 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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