



Krystal Biotech Announces FDA Acceptance of KB407 IND Application for Cystic Fibrosis Clinical Trial

August 1, 2022

- KB407 is an investigational, redosable gene therapy candidate designed to treat all patients with cystic fibrosis regardless of the underlying genetic mutation

PITTSBURGH, Aug. 01, 2022 (GLOBE NEWSWIRE) -- [Krystal Biotech, Inc.](#) (the "Company") (NASDAQ: KRYS), the leader in redosable gene therapy, today announced that the United States Food and Drug Administration (FDA) has accepted its Investigational New Drug (IND) application to evaluate KB407 in a clinical trial for cystic fibrosis (CF).

KB407 is a modified HSV-1 vector carrying two copies of the cystic fibrosis transmembrane conductance regulator (*CFTR*) gene to the respiratory cells in the lungs. 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.

CF is a genetic disease, affecting people of every racial and ethnic group, by causing a buildup of mucus in the lungs leading to persistent lung infections and progressive pulmonary disease. Sources including the Cystic Fibrosis Foundation indicate that there are close to 40,000 children and adults living with cystic fibrosis in the US, and an estimated 105,000 people diagnosed with CF across 94 countries.

"We are excited to advance KB407, our investigational gene therapy for patients with CF, into the clinic. It is designed to treat the root cause of the disease regardless of an individual patient's mutation by giving the body instructions to produce its own functional protein," said Hubert Chen, M.D., Senior Vice President of Clinical Development at Krystal Biotech. Dr. Chen continued, "In addition, this IND acceptance represents an important milestone for us as it allows us to demonstrate the power of our platform to deliver genes, beyond skin cells, to respiratory cells."

On July 1, 2022, the Company submitted an IND application to initiate the Phase 1 clinical trial of KB407. At the end of the 30-day review period, the Company received notification that the FDA accepted the IND allowing the Phase 1 clinical trial to begin. The Company anticipates initiating the clinical trial in 2H 2022.

Phase 1 Trial Overview

The Phase 1 clinical trial will utilize nebulized administration to deliver KB407 in up to 20 adults with CF. The study will enroll 3 cohorts sequentially to evaluate ascending doses of KB407. 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.

About KB407

KB407 is an investigational, 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 caused by genetic mutations that result in dysfunctional or absent CFTR protein and is the most common fatal inherited disease in the United States. 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 loss of lung function, and eventually, respiratory failure. According to the US Cystic Fibrosis Foundation, the median age at death for patients with CF in the United States was 34.1 years in 2020.

Although CFTR modulators are effective in patients with certain CFTR mutations, patients may still experience pulmonary symptoms requiring treatment. Importantly, approximately 10-15% 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, representing a significant unmet need.

About Krystal Biotech, Inc.

Krystal Biotech, Inc. (NASDAQ: KRYS) is a pivotal-stage gene therapy company leveraging its proprietary, redosable gene therapy platform and in-house manufacturing capabilities to develop life-changing medicines for patients with serious diseases, including rare diseases in skin, lung, and other areas. For more information please visit <http://www.krystalbio.com>, and follow @KrystalBiotech on [LinkedIn](#) and [Twitter](#).

Forward Looking Statements

Any statements in this press release about future expectations, plans and prospects for Krystal Biotech, Inc., including statements about the clinical utility of KB407 and the expected timing of the KB407 Phase 1 clinical trial program in the U.S., 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 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 the Company'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 the Company's views as of the date of this release. The Company anticipates that subsequent events and developments will cause its views to change. However, while the Company 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 the Company's views as of any date subsequent to the date of this release.

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