



Krystal Biotech Receives European Commission Orphan Designation for KB407 for the Treatment of Cystic Fibrosis

January 18, 2023

PITTSBURGH, Jan. 18, 2023 (GLOBE NEWSWIRE) -- [Krystal Biotech, Inc.](#) (the "Company") (NASDAQ: KRYS), a biotechnology company focused on developing and commercializing genetic medicines for patients with rare diseases, announced today that the European Commission has granted orphan designation for KB407 for the treatment of Cystic Fibrosis (CF).

KB407 is an investigational, redosable gene therapy designed to correct the underlying cause of cystic fibrosis 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.

"We are pleased that the European Commission has granted orphan drug designation to KB407, which we believe is an important step toward advancing this option to treat patients with CF," said Suma Krishnan, President of Research and Development at Krystal Biotech.

The U.S. Food and Drug Administration has also granted Orphan Drug designation for KB407 to treat patients with CF.

About European Commission Orphan Designation

Orphan drug designation in the European Union (EU) is granted by the European Commission based on a positive opinion issued by the European Medical Association (EMA) Committee for Orphan Medicinal Products. The EMA's orphan designation is available to companies developing treatments for life-threatening or chronically debilitating conditions that affect fewer than five in 10,000 persons in the EU. Medicines that meet the EMA's orphan designation criteria qualify for financial and regulatory incentives that include a 10-year period of marketing exclusivity in the EU after product approval, protocol assistance from the EMA at reduced fees during the product development phase and access to centralized marketing authorization.

About Krystal Biotech, Inc.

Krystal Biotech, Inc. (NASDAQ: KRYS) is a biotechnology company focused on developing and commercializing genetic medicines for patients with rare diseases. The Company's wide-ranging pipeline is based on its proprietary redosable HSV vector. Headquartered in Pittsburgh, Pennsylvania, the Company is led by an experienced management team, is fully-integrated and has core capabilities in viral vector design, vector optimization, gene therapy manufacturing and commercialization. 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 the Company, including statements about orphan drug designation for KB407 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: uncertainties associated with regulatory review of clinical trials and applications for marketing approvals, the availability or commercial potential of 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 press release.

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Source: Krystal Biotech, Inc.