



## Krystal Biotech's KB407 Granted Orphan Drug Designation by the FDA to Treat Patients With Cystic Fibrosis

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### The company is on track to file an IND for KB407 in 2021

PITTSBURGH, Aug. 17, 2020 (GLOBE NEWSWIRE) -- [Krystal Biotech](#), Inc. (Nasdaq:KRY5), a fully integrated gene therapy company driven by its proprietary, engineered herpes simplex virus type 1 vector (HSV-1) platform, today announced that the U.S. Food & Drug Administration (FDA) has granted Orphan Drug Designation to KB407, currently in preclinical development for the treatment of cystic fibrosis ("CF").

"We are pleased to receive Orphan Drug Designation for KB407 to treat cystic fibrosis as this is an important step forward in our efforts to address the continued unmet need in this devastating disease," said Suma M. Krishnan, founder and chief operating officer of Krystal Biotech. "We are excited by the results of the in vitro data thus far, as presented at ASGCT earlier this year, and we look forward to sharing in vivo animal data later this year."

The FDA's Office of Orphan Drug Products grants Orphan Drug Designation to support the development of medicines for underserved patient populations, or rare disorders, that affect fewer than 200,000 people in the United States. Orphan Drug Designation may allow Krystal Biotech to be eligible for a seven-year period of U.S. marketing exclusivity upon approval of KB407, tax credits for certain clinical research costs and a waiver of the Prescription Drug User Fee Act (PDUFA) filing fees, subject to certain conditions.

#### About KB407 for Cystic Fibrosis

KB407 is an inhaled, repeat-dose gene therapy product currently in the preclinical phase with plans to file an IND in 2021. In pre-clinical studies to date, KB407 has been able to successfully transduce human CF patient-derived epithelial cells and deliver functional cystic fibrosis transmembrane conductance regulator ("CFTR") in vitro in 2D and 3D organotypic systems. Additional data has shown that the therapy is amendable to non-invasive inhaled administration in vivo, as indicated by successful delivery to the lungs through the use of a clinically relevant nebulizer in rodent healthy and diseased small animal models.

#### About Krystal Biotech

Krystal Biotech, Inc. (NASDAQ:KRY5) is a gene therapy company dedicated to developing transformative medicines to treat diseases caused by protein or gene dysfunction. 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 clinical trials of beremagene geperpavec ("B-VEC"), KB105, KB104, KB301 and KB407; the clinical utility of B-VEC, KB105, KB104, KB301 and KB407, and Krystal's plans for filing of regulatory approvals and efforts to bring B-VEC, KB105, KB104, KB301 and KB407 to market; the market opportunity for and the potential market acceptance of B-VEC, KB105, KB104, KB301 and 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 B-VEC, KB105, KB104, KB301 and 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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