Krystal Biotech's KB103 Granted Orphan Drug Designation by the FDA to Treat Patients With Dystrophic Epidermolysis Bullosa

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PITTSBURGH, Pa., Nov. 07, 2017 (GLOBE NEWSWIRE) -- Krystal Biotech, Inc. (Nasdaq:KRYS), a gene therapy company dedicated to developing and commercializing novel treatments for patients suffering from dermatological diseases, today announced that the U.S. Food & Drug Administration (FDA) has granted Orphan Drug Designation to the Company' lead product candidate, KB103, currently in preclinical development for dystrophic epidermolysis bullosa, or DEB.

"Receiving orphan drug designation is an important step forward in our efforts to bring hope to DEB patients and their families," said Suma M. Krishnan, Founder and Chief Operating Officer of Krystal Biotech. "We are excited by the results of the pharmacology data in the diseased hypomorphic animal model for DEB that we submitted to the Office of Orphan Product Development (OOPD) and are on track to file an IND for KB103 in the first quarter of 2018. In December 2016, we received the designation of 'rare pediatric disease' for KB103 that will qualify us to receive a Rare Pediatric Priority Review Voucher upon approval of KB103."

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 KB103, 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 Dystrophic Epidermolysis Bullosa

Dystrophic Epidermolysis Bullosa, or DEB, is an incurable, often fatal skin blistering condition caused by a lack of collagen in the skin. It is caused by mutations in the gene coding for type VII collagen, or COL7, a major component of the anchoring fibrils which anchor the epidermis to the underlying dermis, and provide structural adhesion in a normal individual. The lack of COL7 in DEB patients causes blisters to occur in the dermis as a result of separation from the epidermis. This makes the skin incredibly fragile, leading to blistering or skin loss at the slightest friction or knock. It is progressive and incredibly painful.

The most severe form of DEB is recessive DEB, or RDEB, which is caused by null mutations in the COL7A1 gene. DEB also occurs in the form of dominant DEB, or DDEB, which is considered to be a milder form of DEB. There are no known treatments which affect the outcome of either form of the disease and the current standard of care for DEB patients is limited to palliative treatments. Krystal is developing KB-103 for the treatment of the broad DEB population, including both recessive and dominant forms of the disease.

About KB103

KB103 is Krystal's lead product candidate, currently in preclinical development and seeks to use gene therapy to treat DEB. KB103 is a replication-defective, non-integrating viral vector that has been engineered employing Krystal's STAR-D platform to deliver functional human COL7A1 genes directly to the patients' dividing and non-dividing skin cells. HSV-1 is Krystal's replication-deficient, non-integrating viral vector that can penetrate skin cells more efficiently than other viral vectors. Its high payload capacity allows it to accommodate large or multiple genes and its low immunogenicity makes it a suitable choice for direct and repeat delivery to the skin.

About Krystal Biotech

Krystal Biotech, Inc. (NASDAQ:KRYS) is a gene therapy company dedicated to developing and commercializing novel treatments for patients suffering from dermatological diseases. For more information, please visit http://www.krystalbio.com.

Forward-Looking Statements

This press release includes certain disclosures which contain "forward-looking statements," including, without limitation, statements regarding the anticipated timing for Krystal's filing of an IND, prospects for the development and potential efficacy of KB103, and the benefits of receiving the "rare pediatric disease" indication. You can identify forward-looking statements because they contain words such as "believes" and "expects." Forward-looking statements are based on Krystal's current expectations and assumptions. Because forward-looking statements relate to the future, they are subject to inherent uncertainties, risks and changes in circumstances that may differ materially from those contemplated by the forward-looking statements, which are neither statements of historical fact nor guarantees or assurances of future performance. Important factors that could cause actual results to differ materially from those in the forward-looking statements are set forth in Krystal's filings with the Securities and Exchange Commission, including its registration statement on Form S-1, as amended from time to time, under the caption "Risk Factors."

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