Krystal Biotech's KB103 Receives Orphan Medicinal Product Designation in Europe for Dystrophic Epidermolysis Bullosa

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KB103 represents the first investigational HSV-1 based gene therapy for the severe skin disorder dystrophic epidermolysis bullosa to receive this designation

KB103 previously received Orphan Drug Designation for DEB from U.S. FDA

PITTSBURGH, April 19, 2018 (GLOBE NEWSWIRE) -- Krystal Biotech, Inc. ("Krystal") (NASDAQ:KRYS), a gene therapy company dedicated to developing and commercializing novel treatments for patients suffering from dermatological diseases, today announces that KB103, a gene therapy candidate for dystrophic epidermolysis bullosa ("DEB"), was granted Orphan Medicinal Product Designation (OMPD) from the European Medicines Agency (EMA). KB103 represents the first investigational HSV-1 based gene therapy in DEB to receive this designation. In November 2017, the Company announced that the U.S. Food and Drug Administration (FDA) granted KB103 Orphan Drug Designation (ODD) for the same indication. There are currently no approved medical treatments aimed at addressing the underlying cause of DEB, and KB103 has the potential to play a disruptive role in this area of high unmet medical need.

In Europe, OMPD is available to companies developing products intended to treat a life-threatening or chronically debilitating condition that have a patient prevalence in the European Union ("EU") of no more than five in 10,000. This designation is only granted when there is no approved satisfactory treatment for the specific condition. OMPD offers a company a) product market exclusivity for ten years in the EU following regulatory approval, b) protocol assistance from the EMA at reduced fees during product development phase, c) access to centralized marketing authorization and d) tax and financial incentives for companies developing medicines for such orphan indications.

"Being granted Orphan Medicinal Product Designation in Europe represents another important global milestone for our KB103 program," said Suma Krishnan, founder and chief operating officer of Krystal. "Last month we filed an Investigational New Drug application for KB103 in the U.S. and intend to advance the clinical development of KB103 in U.S. in 2018 and in EU in 2019."

About KB103
KB103 is Krystal's lead product candidate, currently in preclinical development, that seeks to use gene therapy to treat dystrophic epidermolysis bullosa, or DEB, an incurable skin blistering condition caused by a lack of collagen in the skin. 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. KB103 received Orphan Drug Designation (ODD) for DEB from FDA in November 2017.

About Dystrophic Epidermolysis Bullosa, or DEB
Dystrophic epidermolysis bullosa, or DEB, is an incurable, often fatal skin blistering condition caused by a lack of collagen protein in the skin. It is caused by mutations in the gene coding for type VII collagen, or COL7, a major component of anchoring fibrils, which connect the epidermis to the underlying dermis, and provide structural adhesion between these skin layers 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 affecting 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 KB103 for the treatment of the broad DEB population, including both recessive and dominant forms of the disease.

About the STAR-D Gene Therapy Platform
Krystal's Skin TARgeted Delivery platform, or STAR-D platform, is a proprietary gene therapy platform consisting of an engineered viral vector and skin-optimized gene transfer technology that Krystal is employing to develop off-the-shelf treatments for dermatological diseases for which there are no known effective treatments. The company believes that the STAR-D platform provides an optimal approach for treating dermatological conditions due to the nature of the HSV-1 viral vector it has created. Certain inherent features of the HSV-1 virus, combined with the ability to strategically modify the virus in the form employed as a gene delivery backbone, provide the STAR-D platform with several advantages over other viral vector platforms for use in dermatological applications.

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 that contain "forward-looking statements," including, without limitation, statements regarding the potential of KB103 to treat the underlying causes of DEB, the timetable for bringing GMP manufacturing in-house and the potential for rapid development of the company's clinical programs. 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 and Form 10-K, as amended from time to time, under the caption "Risk

Factors.”

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