Krystal Biotech Completes Dosing in the GEM-Phase 2 Study in Pediatric Patients for the Treatment of Dystrophic Epidermolysis Bullosa

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Top line results from GEM-Phase 2 study expected in H1 2019

PITTSBURGH, Dec. 19, 2018 (GLOBE NEWSWIRE) -- Krystal Biotech Inc., (“Krystal”) (NASDAQ: KRYS), a gene therapy company developing topical and intradermal "off-the-shelf" treatments for rare dermatological diseases, today announces that four patients have been dosed in the GEM-Phase 2 study of KB103, a first-in-class topical gene therapy for the treatment of dystrophic epidermolysis bullosa (DEB).

The study is being conducted in adult and pediatric populations to determine whether KB103 administered topically can safely and effectively promote healing of DEB patient wounds. In addition, the study will assess change from baseline in Investigator Global Assessments and Patient Reported Outcomes (PRO). This study, being conducted at Stanford University, is a single-center, randomized, placebo-controlled, intra-subject comparison of treatment and placebo wounds.

Four patients (age 5 and older) are being treated in this study. Three chronic wounds (wounds up to 20 cm^2 in surface area are eligible) are selected per patient; two wounds are randomized to KB103 and one to placebo. Patients will be on trial for approximately six months. The primary objective of this trial is to measure wound healing by imaging. Outcome measures include duration of wound closure relative to placebo, time to wound closure relative to placebo and change of wound surface area relative to baseline and placebo. Secondary outcome measures include change from baseline in the Investigator’s Global Assessment score, change from baseline in PRO scales of severity and pain, level of collagen VII in KB103-administrated skin as measured by immunofluorescence and presence of anchoring fibrils as measured by immunoelectron microscopy.

“Results from the GEM-Phase 2 study in 1H 2019 will enable us to select our endpoints going into the pivotal study in the second half of 2019,” said Suma Krishnan, founder and chief operating officer of Krystal. "We believe that 2019 has the potential to be a year of significant clinical progress toward the ultimate goal of bringing KB103 to patients and families devastated by DEB.”

About Dystrophic Epidermolysis Bullosa (“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 KB103

KB103 is Krystal’s lead product candidate 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.

About the STAR-D Gene Therapy Platform

Krystal’s **S**kin **TAR**geted **D**elivery 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 topical and intradermal “off-the-shelf” novel treatments for patients suffering from rare 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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