



Krystal Biotech Presents In Vivo KB103 Data at the International Investigative Dermatology Conference that Show Human COL7 Incorporating into Anchoring Fibrils with Proper Structural Orientation

May 9, 2018

Lead product candidate, KB103, shown to successfully transfer COL7A1 gene and to correct recessive dystrophic epidermolysis bullosa (RDEB)

PITTSBURGH, May 09, 2018 (GLOBE NEWSWIRE) -- [Krystal Biotech, Inc.](#), ("Krystal") (NASDAQ:KRY5), a gene therapy company developing topical and intradermal "off-the-shelf" treatments for rare dermatological diseases, today announces that Peter Marinkovich, M.D., associate professor of dermatology at Stanford University and his team will present *in vivo* data on Krystal's lead gene therapy candidate KB103 at [the International Investigative Dermatology \(IID\) conference](#) on May 19, 2018 in Orlando, Fla. Study results have demonstrated that:

- Linear deposition of human functional COL7 in the basement membrane zone of hypomorph mice after KB103 injection;
- Human COL7 incorporates into anchoring fibrils with proper structural orientation; and
- KB103 exhibits expression and minimal *in vivo* toxicity after repeated administration.

The details of the presentation are as follows:

Title: *Successful in vivo COL7A1 gene delivery and correction of recessive dystrophic epidermolysis bullosa (RDEB) skin using an off the shelf HSV-1 vector (KB103)*

Poster Presenters: Dr. Peter Marinkovich and fellow team members

Poster Session: Genetic Disease, Gene Regulation and Gene Therapy

Time: 11:45 a.m. – 1:45 p.m. ET

Date: May 19, 2018

Location: Gatlin Ballroom, Rosen Shingle Creek Resort, Orlando, FL

The International Investigative Dermatology (IID) conference is organized by the Society for Investigative Dermatology and has been held annually since 1998 when the European Society for Dermatological Research (ESDR), the Japanese Society for Investigative Dermatology (JSID), and the Society for Investigative Dermatology (SID) joined together to hold "tri-continental" meetings. Over the ensuing years, IID has grown and attracted top scientists from around the world in what is now one of the largest international dermatology conferences. For more information, including the conference schedule, refer to <https://iid2018.org/>.

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 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 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 Krystal Biotech

Krystal Biotech, Inc. (NASDAQ:KRY5) 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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