U.S. FDA Grants Fast Track Designation for Krystal Biotech's KB103 for the Treatment of Dystrophic Epidermolysis Bullosa

May 24, 2018

PITTSBURGH, May 24, 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 the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to KB103 for the treatment of dystrophic epidermolysis bullosa (DEB). KB103 is the first-ever topically-applied herpes simplex virus (HSV-1) based gene therapy engineered to deliver a human collagen protein to patients suffering from DEB.

DEB is a chronic, progressive and incredibly painful skin disease caused by mutations in the gene coding for type VII collagen, or COL7. As a result of mutated COL7, DEB patients' skin is incredibly fragile, resulting in blistering or skin loss at the slightest friction. There are currently no approved treatments for DEB.

Under the FDA Modernization Act of 1997, designation as a Fast Track product means the FDA will take action to expedite both the development and the review of the application for approval. The FDA may also evaluate for filing, and commence review of, portions of an application for approval of a Fast Track product under certain conditions.

"This Fast Track designation represents another positive step for the development of KB103 and is a clear recognition of the serious unmet need that exists for patients suffering from this debilitating disease," said Suma Krishnan, founder and chief operating officer of Krystal.

A single-site, open-label, placebo-controlled Phase 1/2 clinical study of KB103 is underway at Stanford University. The study is designed to evaluate the safety and tolerability of KB103 using wound imaging, analysis of COL7 expression, and anchoring fibril formation.

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: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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