Krystal Biotech Announces Dosing of First Patient in the Phase 1/2 trial of KB103, a First-in-Class Topical Gene Therapy for the Treatment of Dystrophic Epidermolysis Bullosa

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PITTSBURGH, May 10, 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 first patient has been dosed in the Phase 1/2, first in-human trial of KB103, a first-in-class topical gene therapy for the treatment of dystrophic epidermolysis bullosa (DEB).

The Phase 1/2 trial at Stanford University is a single-center, open-label, placebo-controlled Phase 1/2 study conducting an intra-subject comparison of randomized treatment and control wounds. It is designed to evaluate the safety and tolerability of KB103 in subjects with the recessive form of dystrophic epidermolysis bullosa. Efficacy is also evaluated through wound imaging and analysis of collagen VII expression and anchoring fibril formation in the basement membrane zone.

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.

“This is an important milestone for us, as it brings us a step closer to offering a meaningful therapeutic option for patients with dystrophic epidermolysis bullosa who do not have a treatment option presently,” said Suma Krishnan, founder and chief operating officer of Krystal. "We are grateful to our clinical investigators and expert collaborators, who have supported Krystal Biotech and helped guide our efforts. Most of all, we are thankful to the patients and their families affected by DEB for their participation in our trial.

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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