



## Krystal Biotech Announces FDA Clearance of IND on KB103 to Begin Enrolling Patients for the Treatment of Dystrophic Epidermolysis Bullosa

April 26, 2018

*KB103 is the first-ever herpes simplex virus (HSV-1) based topical gene therapy candidate engineered to produce human collagen protein to treat dystrophic epidermolysis bullosa (DEB)*

*The planned Phase 1/2 KB103 single site clinical study is expected to start in May 2018 at Stanford University*

PITTSBURGH, April 26, 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 the clearance of an Investigational New Drug (IND) application by the U.S. Food and Drug Administration (FDA) for lead product candidate KB103. KB103 is the first-ever topical herpes simplex virus (HSV-1) based gene therapy engineered to deliver a human collagen protein to patients suffering from dystrophic epidermolysis bullosa (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.

"We are excited about KB103 entering the clinic. Our goal is to demonstrate that KB103 could truly be a first-in-class potent treatment for DEB that has the potential to change the lives of people affected by this condition," said Suma Krishnan, founder and chief operating officer of Krystal. "We plan on enrolling patients in this Phase 1/2 study as soon as we can and hope to have a meaningful data readout shortly."

"Clearance of the IND by the FDA is the first of several important milestones for the KB103 program," said Krish Krishnan, chairman and chief executive officer of Krystal. "I am proud of the efforts of the Krystal team that has worked diligently to bring KB103 into the clinic in a timely manner. We are excited about the possibility of providing a simple topically-applied treatment to fundamentally treat this debilitating disease."

The planned Phase 1/2 clinical study of KB103 is a single site study at Stanford University expected to start in May 2018. Initial data from the study are expected to be released by the end of 2018.

### **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 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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