EMA Grants PRIME Eligibility for KB103 to Treat Dystrophic Epidermolysis Bullosa

March 29, 2019

First EMA PRIME eligibility for Krystal Biotech

Eligibility based on positive primary analysis of clinical and non clinical data to date

PRIME Designation allows for frequent and early interactions with the EMA aimed at supporting accelerated evaluation and approval

PITTSBURGH, March 29, 2019 (GLOBE NEWSWIRE) -- Krystal Biotech Inc., ("Krystal") (NASDAQ: KRYS), a gene therapy company developing medicines to treat dermatological diseases, announced today that the European Medicines Agency (EMA) has granted access to its PRIME (PRIority MEdicines) scheme for KB103 (bercolagene telserpavec), a first-in-class topical gene therapy for the treatment of dystrophic epidermolysis bullosa (DEB). Krystal's application was supported by clinical data to date from the ongoing GEM-1 Phase 1/2 study coupled with non-clinical data.

The PRIME designation is awarded by the EMA to promising medicines that target an unmet medical need. These medicines are considered priority medicines by the EMA. To be eligible and accepted for PRIME, a medicine has to show its potential to benefit patients with unmet medical needs based on early clinical data coupled with non-clinical data. Through PRIME, the EMA offers enhanced support to medicine developers including early interaction and dialogue, and a pathway for accelerated evaluation by the agency. The program is intended to optimize development plans and expedite the review and approval process so that these medicines may reach patients as early as possible. To be eligible for PRIME, medicines must target an unmet medical need and show potential benefit for patients based on early clinical data coupled with non clinical data

Suma Krishnan, founder and chief operating officer of Krystal Biotech, said: "We are excited to receive PRIME eligibility for KB103. Not only does this validate the importance of a truly transformative treatment for DEB but also allows us to work closely with the EMA to optimize our development plan and help us bring KB103 to patients as quickly as possible."

This is the first EMA PRIME eligibility that Krystal Biotech has received since initiating the KB103 program. To be accepted for PRIME, a medicine must demonstrate the potential to benefit patients with unmet medical needs through early clinical data.

About the Priority Medicines (PRIME) Initiative
PRIME is a program launched by the European Medicines Agency (EMA) to enhance support for the development of medicines that target an unmet medical need. This voluntary program is based on enhanced interaction and early dialogue with developers of promising medicines, to optimize development plans and speed up evaluation so these medicines can reach patients earlier. Through PRIME, the EMA offers early and proactive support to medicine developers to optimize the generation of robust data on a medicine's benefits and risks and enable accelerated assessment of medicines applications. The goal of the initiative is to help patients benefit as early as possible from therapies that may significantly improve their quality of life.

About Krystal Biotech
Krystal Biotech, Inc. (NASDAQ:KRYS) 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.

About KB103
KB103 (bercolagene telserpavec) 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.

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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