



Krystal Biotech Reports First Quarter 2019 Financial Results and Provides Corporate Update

May 6, 2019

EMA grants PRIME eligibility for KB103 (bercolagene telserpavec) to treat dystrophic epidermolysis bullosa

Krystal Biotech announces five presentations of clinical and preclinical data at the Society for Investigative Dermatology ("SID") Annual Meeting

PITTSBURGH, May 06, 2019 (GLOBE NEWSWIRE) -- [Krystal Biotech Inc.](#), ("Krystal") (NASDAQ: KRY5), a gene therapy company developing medicines to treat dermatological diseases, announced financial results for first quarter 2019 and an update on its business progress.

"We are very pleased to have KB103 accepted into the PRIME program and believe that KB103 could potentially be a transformative treatment option for patients with dystrophic epidermolysis bullosa," said Krish S. Krishnan, chairman and chief executive officer. "Data to be presented at SID will demonstrate the pipeline potential of our platform technology as we look to file an IND on KB105 for the treatment of autosomal recessive congenital ichthyosis and initiate Phase 1/2 clinical trials in the second half of 2019. We remain steadfast in our goal to commence pivotal trials on KB103 in the second half of 2019 and make our gene therapy available as quickly and safely as possible for families suffering from DEB."

Recent Corporate Highlights

- On April 29, 2019, we announced five presentations of clinical and preclinical data at the upcoming Society for Investigative Dermatology annual meeting on May 9, 2019 in Chicago.
- On March 29, 2019, the European Medicines Agency (EMA) granted PRIME eligibility for KB103 to treat dystrophic epidermolysis bullosa. The PRIME scheme was established by the EMA to enhance support for the development of medicines that target unmet medical needs, with the goals of optimizing development plans and speeding up evaluation so these medicines can potentially reach patients earlier.
- On March 5, 2019, we had the official inauguration of Ancoris, our commercial scale cGMP-compliant manufacturing facility, following completion of a manufacturing trial run of KB103.

Financial results for the quarter ended March 31, 2019

- Cash, cash equivalents and short-term investments totaled \$106.6 million on March 31, 2019.
- Research and development expenses for the first quarter ended March 31, 2019 were \$3.2 million, compared to \$1.5 million for first quarter 2018.
- General and administrative expenses for the first quarter ended March 31, 2019 were \$1.5 million, compared to \$0.8 million for first quarter 2018.
- Net losses for the quarters ended March 31, 2019 and 2018 were \$4.1 million and \$2.2 million or (\$0.29) and (\$0.21) per common share (basic and diluted), respectively.

For additional information on the Company's financial results for the year ended December 31, 2018, refer to form 10K filed with the SEC.

About KB103

KB103 is Krystal's lead product candidate, currently in clinical development and 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 using the HSV-1 virus employing Krystal's STAR-D platform to deliver functional human COL7A1 genes directly to the patients' dividing and non-dividing skin cells. Krystal's vector 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 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 the anchoring fibrils, which anchor the epidermis to the underlying dermis, and provide structural adhesion 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 which affect 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 KB-103 for the treatment of the broad DEB population, including both recessive and dominant forms of the disease.

About Lamellar Ichthyosis

Lamellar ichthyosis (LI) is an autosomal recessive disorder. There are 22 known types of LI, a number of which are known to be caused by defects in one of several skin-related genes. LI usually appears in the first few days of life, lasts lifelong and in certain variants can be very severe. A newborn with LI is born encased in a collodion membrane that sheds within 10-14 days. The shedding of the membrane reveals generalized scaling with variable redness of the skin. The scaling may be fine or plate-like, resembling fish skin. Although the disorder is not life threatening, it is disfiguring and

can cause considerable psychological stress to affected patients. There are no approved treatments for LI and the current standard of care is palliative treatments to manage symptoms.

About the STAR-D Gene Therapy Platform

Krystal has developed a proprietary gene therapy platform, the Skin TARgeted Delivery platform, or STAR-D platform, that consists of an engineered viral vector and skin-optimized gene transfer technology, to develop off-the-shelf treatments for dermatological diseases. 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 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: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 our intention to commence a pivotal study of KB103 in the second half 2019, our plans to file an IND for and commence a Phase 1/2 clinical trial of KB105 in the second half of 2019, and the ability of KB103 to be a transformative treatment option for DEB patients. 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 nor 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-3, and in its Forms 10-K and 10-Q, as modified or supplemented from time to time, under the caption "Risk Factors."

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