



Krystal Biotech Reports First Quarter 2018 Financial Results and Provides Corporate Update

May 7, 2018

IND for KB103 to treat dystrophic epidermolysis bullosa (DEB) cleared by FDA

Patient enrollment in Phase 1/2 study of KB103 to begin in May 2018 at Stanford University

Krystal Biotech's KB103 receives Orphan Medicinal Product Designation in Europe for DEB

PITTSBURGH, May 07, 2018 (GLOBE NEWSWIRE) -- [Krystal Biotech](#) (NASDAQ:KRY5), a gene therapy company advancing "off-the-shelf" topical and intra-dermal treatments for dermatological diseases, today reports financial results for the first quarter ended March 31, 2018, and provides an update on the company's recent corporate progress.

"To date, 2018 has been marked by significant progress for Krystal Biotech, including clearance of the KB103 IND by the FDA for the treatment of dystrophic epidermolysis bullosa (DEB), granting of the U.S. composition of matter patent covering HSV vectors and methods of using the same to treat skin diseases, and the Company receiving Orphan Medicinal Product Designation in Europe for KB103," said Krish S. Krishnan, chief executive officer of Krystal Biotech. "As we continue this momentum in 2018, we look forward to announcing clinical results on KB103, filing the Investigational New Drug (IND) application on KB105 for the treatment of lamellar ichthyosis, and getting our Good Manufacturing Practice (GMP) facility ready for manufacturing our pipeline products."

Recent Corporate Highlights include:

- Granting of the composition of matter patent covering HSV vectors and methods of using the same to treat skin diseases;
- Receipt of Orphan Medicinal Product Designation (OMPD) in Europe for DEB;
- Clearance of KB103's IND by FDA for the treatment of DEB;
- Begin enrollment of patients in Phase 1/2 clinical trial at Stanford University.

Financial results for the year ended March 31, 2018:

- Cash and cash equivalents totaled \$47.2 million at March 31, 2018, compared with \$49.6 million at December 31, 2017;
- Research and development expenses for the quarters ended March 31, 2018 and 2017 were \$1.5 million and \$319 thousand, respectively;
- General and administrative expenses for the quarters ended March 31, 2018 and 2017 were \$0.8 million and \$146 thousand, respectively; and
- Net losses for the quarters ended March 31, 2018 and 2017 were \$2.2 million and \$0.5 million or (\$0.21) and (\$0.14) per common share, basic and diluted, respectively.

For further details on the company's financials, please refer to Form 10Q filed with the SEC.

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 patient's 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 Dystrophic Epidermolysis Bullosa

Dystrophic epidermolysis bullosa (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 that is apparent at birth and is present throughout life. 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 quite disfiguring and causes considerable psychological stress to affected patients. There are no approved treatments for LI and the current standard of care remains 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 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>.

Forward-Looking Statements

This press release includes certain disclosures that contain "forward-looking statements," including, without limitation, statements regarding the development of our product candidates, including KB103 and KB105, including statements regarding our plans to initiate a clinical trial of KB103 in 2018 and to file an IND for KB105 during this calendar year and our plans to develop a GMP manufacturing facility over the next 12 months. 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 periodic filings with the Securities and Exchange Commission, including its registration statement on Form S-1 and its Form 10-K, under the caption "Risk Factors."

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