



Krystal Biotech Reports 2017 Financial Results and Business Progress

March 12, 2018

IND for KB103 to treat Dystrophic Epidermolysis Bullosa (DEB) to be filed in March 2018

IND for KB105 to treat Lamellar Ichthyosis to be filed in Q4 2018

Company was granted composition of matter and method of use patent by USPTO in January 2018

GMP facility for production of KB103 and pipeline products to be completed by Q1 2019

PITTSBURGH, March 12, 2018 (GLOBE NEWSWIRE) -- Krystal Biotech (NASDAQ:KRYS), a gene therapy company for rare dermatological diseases announced financial results for 2017 and an update on its business progress.

"2018 promises to be a productive and exciting year for Krystal. Our successful IPO last year has positioned us to build a fully integrated company dedicated to discovering, developing and delivering off-the-shelf easy to administer chronic treatments for rare dermatological diseases that provide transformative outcomes to patients and families," said Krish S. Krishnan, Chief Executive Officer of Krystal Biotech. "Our immediate focus is to execute on our planned Phase 1/2 clinical study for our lead product candidate KB103 for treatment of Dystrophic Epidermolysis Bullosa, and to file an IND in the fourth quarter for our second product candidate, KB105 for the treatment of Lamellar Ichthyosis. In parallel, we are building out our infrastructure with the goal of establishing a GMP facility in Pittsburgh, PA in the next 12 months for the production of our pipeline products using our STAR-D (Skin Targeted Delivery) platform."

2017 and Recent Corporate Highlights

- Granted U.S. Patent No. 9,877,990 by the USPTO which covers compositions comprising herpes simplex viral (HSV) vectors and methods of using the same for providing prophylactic, palliative or therapeutic relief of a wound, disorder or disease of the skin in a subject. This patent is 100% owned by Krystal.
- Phase 1/2 clinical trial protocol for KB103 to treat Dystrophic Epidermolysis Bullosa (DEB) received clearance from Recombinant DNA Advisory Committee (RAC). The company plans to file an IND for KB103 in March 2018, and to begin the clinical trial in DEB shortly thereafter.
- KB103 granted Orphan Drug Designation by the Office of Orphan Product Development (OOPD).
- Presented preclinical data assessing the *in vitro* and *in vivo* delivery of HSV-1 mediated KB103 at EB2017, the 5th World Conference on EB Research in Salzburg, Austria.
- Received non-dilutive funding totaling \$770,000 from the EB Research Partnership (EBRP) and the EB Medical Research Foundation (EBMRF). This funding was provided following a highly competitive application and screening process overseen by EBRP's Scientific Advisory Board (SAB), which is composed of leading scientists and physicians.
- Completed a successful initial public offering raising approximately \$40.7 million in net proceeds.

Financial results for the year ended December 31, 2017

- Cash totaled \$49.6 million at December 31, 2017.
- Research and development expenses for the year were \$3.2 million.
- General and administrative expenses for the year were \$1.6 million.
- Net losses for the years ended December 31, 2017 and 2016 were \$7.9 million and \$1.2 million or (\$1.48) and (\$1.31) per common share (basic and diluted), respectively.

About KB103

KB103 is Krystal's lead product candidate, currently in preclinical 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 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 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 that is apparent at birth and is present throughout life. The newborn 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 platelike, resembling fish skin. Although the disorder is not life threatening, it is quite disfiguring and causes considerable psychological stress to affected patients. It usually appears in the first few days of life, lasts lifelong and can be very severe.

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 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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