



## Krystal Biotech Reports First Quarter 2020 Financial Results and Provides Update on Operational Progress

May 4, 2020

*KB105 interim Phase 1/2 clinical data for treatment of autosomal recessive congenital ichthyosis (ARCI) to be announced at the Society for Investigative Dermatology (SID) Annual Meeting in May 2020*

*B-VEC Phase 3 pivotal study anticipated to start in 1H 2020*

*B-VEC Phase 1/2 clinical data and statistical analysis to be presented at the Society for Investigative Dermatology (SID) Annual Meeting in May 2020*

*KB407 in vitro pharmacology data to treat cystic fibrosis to be announced at the American Society of Gene & Cell Therapy (ASGCT) Annual Meeting in May 2020*

*Ended the quarter with cash, cash equivalents and short-term investments of \$186.7 million*

PITTSBURGH, May 04, 2020 (GLOBE NEWSWIRE) -- [Krystal Biotech Inc.](#), ("Krystal") (NASDAQ: KRYS), a gene therapy company developing a new class of transformative medicines to treat diseases caused by gene or protein dysfunction, reported financial results and key operational progress updates for the first quarter ending March 31, 2020.

"In 2020, we anticipate treating patients across three different clinical trials for skin diseases and conditions and remain committed to developing our pipeline to ensure that that we can translate its potential into treatments for patients affected by severe diseases," said Krish S. Krishnan, chairman and chief executive officer. "We are pleased to report that our manufacturing batch has been cleared for the upcoming B-VEC Phase 3 pivotal study. We are presently on track to start the pivotal study in the first half of 2020, barring any COVID-19 delays. We are also encouraged by the pre-clinical data to date on KB407, with potential to treat cystic fibrosis, that demonstrates the breadth of our STAR-D platform and are excited that we have an opportunity to share this at ASGCT."

### Upcoming events:

- Presentation of "First-in-Human use of a Novel In Vivo Gene Therapy for the Treatment of Autosomal Recessive Congenital Ichthyosis: Results of Phase 1/2 placebo controlled trial," by Dr. Amy S. Paller, M.D. (Chair, Department of Dermatology, Northwestern University), at the Society for Investigative Dermatology (SID) annual meeting, May 13-16 2020.
- Presentation of "In vivo correction of dystrophic epidermolysis bullosa by direct cutaneous COL7A1 gene replacement: results of Phase 1/2 trial," by Dr. Peter Marinkovich, M.D. (Associate Professor of Dermatology, Stanford University), at the SID annual meeting.
- Poster presentation of "In vitro Pharmacology of KB407, an HSV-1 based gene therapy vector, for the treatment of cystic fibrosis" at the American Society of Gene & Cell Therapy (ASGCT) 23<sup>rd</sup> Annual Meeting, May 12-15, 2020.

### Upcoming milestones:

- Commence pivotal Phase 3 study on B-VEC for the treatment of dystrophic epidermolysis bullosa and organize commercial plans for potential future global launch of B-VEC.
- Advance Phase 1/2 study to include pediatric patients on KB105 for the treatment of ARCI, following safety review by the FDA.
- Initiate Phase 1 clinical safety and efficacy study on KB301 for an aesthetic indication.
- Issuance of a new U.S. patent covering compositions of matter and methods of cosmetic use related to our product candidate KB301 after positive decision rendered by the US Patent & Trademark Office for U.S. Pat. App. No. 16/395,896, entitled "Recombinant Nucleic Acids Encoding Cosmetic Protein(s) for Aesthetic Applications."
- Work to file an IND for KB104 for the treatment of Netherton Syndrome.
- Continue pre-clinical efforts on KB407 for an anticipated IND filing in 2021.

### Financial results for the quarter ended March 31, 2020

- Cash, cash equivalents and short-term investments totaled \$186.7 million on March 31, 2020.
- Research and development expenses for the first quarter ended March 31, 2020 were \$3.5 million, compared to \$3.2 million for first quarter 2019.
- General and administrative expenses for the first quarter ended March 31, 2020 were \$2.4 million, compared to \$1.5 million for first quarter 2019.
- Net losses for the quarters ended March 31, 2020 and 2019 were \$5.3 million and \$4.1 million or (\$0.31) and (\$0.29) per common share (basic and diluted), respectively.

For additional information on the Company's financial results for the year ended December 31, 2019, refer to form 10-K filed with the SEC.

#### **About Krystal Biotech**

[Krystal Biotech Inc.](#), ("Krystal") (NASDAQ: KRYS), a gene therapy company developing a new class of transformative medicines to treat diseases caused by gene or protein dysfunction. For more information, please visit <http://www.krystalbio.com>.

#### **About B-VEC**

*B-VEC (Beremagene Geperpavec, previously "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. B-VEC 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 proprietary 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, 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 Autosomal Recessive Congenital Ichthyosis, or ARCI**

Transglutaminase 1 (TGM-1) is an essential epidermal enzyme that facilitates the formation of the epidermal barrier, which prevents dehydration, and protects the skin from unwanted toxins and surface microorganisms. The loss of TGM-1-activity results in the severe genetic skin disease autosomal recessive congenital ichthyosis (ARCI). Most patients with a TGM-1-deficiency exhibit life-long pronounced scaling with increased transepidermal water loss (TEWL). The scales are plate-like, often of a dark color, and cover the whole body surface area. Erythroderma is either absent or minimal. Such patients usually have ectropion and, at times, eclabium, hypoplasia of joint and nasal cartilage, scarring alopecia, especially at the edge of the scalp, and palmoplantar keratoderma. Additional complications include episodes of sepsis, fluid and electrolyte imbalances due to impaired skin barrier function, and failure to thrive, especially during neonatal period and infancy. Severe heat intolerance, and nail dystrophy are also frequently observed. TGM-1-deficient ARCI is associated with increased mortality in the neonatal period and has a dramatic impact on quality of life. No efficient treatment is available; current therapy only relieves some symptoms.

#### **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.

#### **Forward-Looking Statements**

Any statements in this press release about future expectations, plans and prospects for Krystal Biotech, Inc., including but not limited to statements about the development of Krystal's product candidates, such as plans for the design, conduct and timelines of ongoing clinical trials of bercolagene telserpavec ("B-VEC") and KB105; the clinical utility of B-VEC and KB105 and Krystal's plans for filing of regulatory approvals and efforts to bring B-VEC and KB105 to market; the market opportunity for and the potential market acceptance of B-VEC and KB105; plans to pursue research and development of other product candidates; the sufficiency of Krystal's existing cash resources; the unanticipated impact of COVID-19 on Krystal's business operations, pre-clinical activities and clinical trials; and other statements containing the words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan," "predict," "project," "target," "potential," "likely," "will," "would," "could," "should," "continue," and similar expressions, constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including: the uncertainties inherent in the initiation and conduct of clinical trials, availability and timing of data from clinical trials, whether results of early clinical trials or trials will be indicative of the results of ongoing or future trials, uncertainties associated with regulatory review of clinical trials and applications for marketing approvals, the availability or commercial potential of product candidates including B-VEC and KB105, the sufficiency of cash resources and need for additional financing and such other important factors as are set forth under the caption "Risk Factors" in Krystal's annual and quarterly reports on file with the U.S. Securities and Exchange Commission. In addition, the forward-looking statements included in this press release represent Krystal's views as of the date of this release. Krystal anticipates that subsequent events and developments will cause its views to change. However, while Krystal may elect to update these forward-looking statements at some point in the future, it specifically disclaims any obligation to do so. These forward-looking statements should not be relied upon as representing Krystal's views as of any date subsequent to the date of this release.

#### **CONTACTS:**

##### **Investors:**

Ashley R. Robinson  
LifeSci Advisors  
[arr@lifesciadvisors.com](mailto:arr@lifesciadvisors.com)

##### **Media:**

Darren Opland, PhD  
LifeSci Communications  
[darren@lifescicomms.com](mailto:darren@lifescicomms.com)

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