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

November 9, 2020

- Pivotal GEM-3 study of B-VEC in DEB expected to complete enrollment in early 2021

- Initiated the Phase 1 Study of KB301 in Facial Wrinkles and Acne Scars

- Today announced additional in vivo preclinical data supporting the development of KB407 in cystic fibrosis

- Strong balance sheet with cash, cash equivalents and short-term investments of $286.4 million as of September 30, 2020

PITTSBURGH--(BUSINESS WIRE)--Nov. 9, 2020-- Krystal Biotech, Inc. (Nasdaq:KRYS), a fully integrated gene therapy company driven by its proprietary, engineered herpes simplex virus type 1 vector (HSV-1) platform, today reported financial results and key operational progress updates for the third quarter ended September 30, 2020.

"Despite the challenges presented by COVID in this quarter, we initiated a pivotal trial for DEB, a phase 2 trial in ARCI and a phase 1 trial in aesthetic skin conditions, and I thank my entire team for their efforts in these difficult times," said Krish Krishnan, Chairman and CEO of Krystal Biotech, Inc. "I am particularly encouraged by progress with KB407 for the treatment of cystic fibrosis. The i*n vivo* animal data reported today demonstrates our ability to deliver functional CFTR throughout the lung, which is encouraging in this indication and gives us confidence to explore additional diseases of the lung where delivery of a therapeutic transgene may be beneficial," he added.

He further noted, "With the granting of Rare Pediatric Designation for this program by the FDA this quarter, we are now eligible to receive a Priority Review Voucher for KB407 as well as for B-VEC, KB105, KB104."

Program Highlights & Upcoming Events

Beremagene Geperpavec (B-VEC) for DEB

- Enrollment in the ongoing pivotal GEM-3 study is proceeding well and enrollment completion is anticipated in early 2021. The trial is a randomized, double-blind, intra patient placebo-controlled multicenter trial designed to evaluate the efficacy and safety of B-VEC for patients suffering from both recessive and dominant dystrophic forms of Epidermolysis Bullosa.
- Details of the pivotal study can be found at www.clinicaltrials.gov under NCT identifier NCT04491604.
- Top line data and BLA filing are anticipated in 2021, in line with prior guidance. Data from this trial will also form the basis of an MAA filing in the EU which is anticipated to occur shortly after the BLA filing.

KB105 for TGM1-ARCI

- In September 2020, preclinical data supporting the development of KB105 in TGM1-related Autosomal Recessive Congenital Ichthyosis (ARCI) were published online ahead of print in the peer-reviewed *Journal of Investigative Dermatology*.
- Dosing of the 4th patient in the Phase 1/2 study of KB105 in patients with TGM1 deficient autosomal recessive congenital ichthyosis (ARCI) has completed. Treatment of a larger area and therefore higher dose is being evaluated. Data from this patient together with the data from the 3 initial patients, will help determine next steps.
- Details of the Phase 2 study can be found at www.clinicaltrials.gov under NCT identifier NCT04047732.
- The Company plans to present an update on this program in the first half of 2021.

KB407 for Cystic Fibrosis

- New *in vivo* data from the ongoing preclinical development of KB407 shows successful nebulization, distribution of vector and functional protein expression throughout the lung in mice and a nonhuman primate. More detailed results are available in a corporate presentation available under the Investors section of our website, here.
- In September 2020, KB407 was granted Rare Pediatric Designation by the FDA.
- During the third quarter of 2020, we received a Notice of Allowance for our patent application covering methods of using KB407 for the treatment of Cystic fibrosis and other diseases causing progressive lung destruction, which is expected to issue as US Pat. No. 10,829,529 on November 10th, 2020. As previously announced, in August 2020, the FDA granted Orphan Drug Designation for KB407.
- Pre-clinical validation work is ongoing, and the Company is on track to initiate a clinical study in the first half of 2021.

KB301 for Aesthetic Indications

- In October 2020, positive preclinical data supporting the ongoing development of KB301 in aesthetic indications was presented at the American Society for Dermatologic Surgery (ASDS) 2020 Virtual Meeting.

- During the third quarter of 2020, the United States Patent Office (USPTO) granted U.S. Patent No. 10,786,438 which covers pharmaceutical compositions comprising HSV vectors encoding one or more cosmetic proteins, as well as methods of their use for improving skin condition, quality, and/or appearance.
- The Phase 1 study of KB301 for the treatment of shallow to moderately deep facial wrinkles and severe atrophic acne scars initiated in August 2020.
- Initial safety data from this study is anticipated in early 2021, followed by initial efficacy data in 2H21.

KB104 for Netherton Syndrome

- The Company continues to work towards an IND filing, which is anticipated in 2021.

Financial results for the quarter ended September 30, 2020

- Cash, cash equivalents and short-term investments totaled $286.4 million on September 30, 2020.
- Research and development expenses for the third quarter ended September 30, 2020 were $5.1 million, compared to $3.9 million for third quarter 2019.
- General and administrative expenses for the third quarter ended September 30, 2020 were $4.6 million, compared to $1.5 million for third quarter 2019.
- Net losses for the quarters ended September 30, 2020 and 2019 were $9.6 million and $4.3 million, or ($0.49) and ($0.25) respectively, per common share (basic and diluted).
- For additional information on the Company's financial results for the third quarter ended September 30, 2020, refer to form 10-Q filed with the SEC.

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

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 beremagene geperpavec ("B-VEC"), KB105, KB104, KB301 and KB407; the clinical utility of B-VEC, KB105, KB104, KB301 and KB407, and Krystal's plans for filing of regulatory approvals and efforts to bring B-VEC, KB105, KB104, KB301 and KB407 to market; the market opportunity for and the potential market acceptance of B-VEC, KB105, KB104, KB301 and KB407; 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, KB105, KB104, KB301 and KB407, 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.

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