



Krystal Biotech Provides Update on Operational Progress and Reports Fourth Quarter and Full Year 2020 Financial Results

March 1, 2021

- Enrollment anticipated to complete in 1Q21 and topline pivotal data from the GEM-3 study of B-VEC in DEB expected in 4Q21.
- Today announced Positive Opinion from the European Medicines Agency on Orphan Drug Designation for KB407 for the treatment of cystic fibrosis.
- Strong balance sheet with December 31, 2020 ending cash, cash equivalents and short-term investments of \$271.3 million. In addition, our cash position was strengthened by \$151.9 million of net proceeds from 2021 subsequent offerings.

PITTSBURGH, March 01, 2021 (GLOBE NEWSWIRE) -- [Krystal Biotech Inc.](#), ("Krystal") (NASDAQ: KRYS), the leader in redosable gene therapies for rare diseases, today reported financial results and key operational progress updates for the fourth quarter ending December 31, 2020.

"As the gene therapy landscape evolves, we grow increasingly confident in the unique positioning of our proprietary technology. The ability to episomally deliver therapeutic transgenes repeatedly over time has afforded us a great opportunity to develop transformative medicines for debilitating rare diseases," said Krish Krishnan, Chairman and CEO of Krystal Biotech, Inc. "2021 is an exciting year for our company as we will have Phase 3 data for B-VEC, continue to advance our rare skin pipeline, and begin dosing patients for the first time with our lung targeted vector in cystic fibrosis."

Program Highlights & Upcoming Events:

B-VEC for DEB

- Enrollment in the ongoing pivotal GEM-3 study is expected to complete in 1Q21. Details of the pivotal study can be found at www.clinicaltrials.gov under NCT identifier NCT04491604
- Top line data is anticipated in 4Q21 followed by BLA filing shortly after
- Data from this trial will also form the basis of a Marketing Authorisation Application (MAA) filing in the EU which is anticipated following the BLA filing
- More details on the safety of repeat dose B-VEC from the completed Phase 1/2 study will be presented as an e-Poster talk at the Society of Investigative Dermatology (SID) Annual Meeting, which will be held May 3-8 as a virtual event
 - **Title:** Assessment of safety in repeat dosing of an in vivo topical gene therapy for the treatment of recessive dystrophic epidermolysis bullosa (RDEB) in a phase I/II trial
 - **Session Name:** Poster Session I - Genetic Disease, Gene Regulation, and Gene Therapy; 2:30pm – 4:00pm EST on May 5th 2021
 - *The presentation will also be available on-demand for those registered for the SID conference from May 3, 2021 – May 31, 2021*

KB105 for TGM1-ARCI

- The Company plans to present an update on this program in 1H21. The update will consist of safety, TGM1 expression level and efficacy data from the 4th patient enrolled. Data from this patient, together with the data from the 3 initial patients will help determine next steps
- Details of the Phase 1/2 study can be found at www.clinicaltrials.gov under NCT identifier NCT04047732

KB407 for Cystic Fibrosis

- The GLP toxicology study evaluating repeat dosing of inhaled KB407 in a large cohort of nonhuman primates is nearly complete, and the Company plans to announce data from the study in 1H21
- In February 2021 the European Medicines Agency (EMA) Committee for Orphan Medicinal Products issued a positive opinion on the Company's application for orphan drug designation for its investigational medicine KB407 as a potential treatment for cystic fibrosis
- Clinical trial initiation is anticipated in 1H21

KB301 for Aesthetic Indications

- Dosing in the first cohort of the Phase 1 study of KB301 is complete. Patients in cohort 1 received intradermal injections of KB301 in the buttock region, which was compared to an untreated or saline injected control. The Company plans to announce initial data from this cohort, consisting of safety and COL3 expression levels, in 1Q21 and present more details at the upcoming SID Annual meeting
 - **Title:** First-in-human safety and mechanism of action (MOA) analyses of repeatedly dosed in vivo gene delivery for directed human type III collagen (COL3) expression in aesthetics

- **Session Name:** Poster Session I - Genetic Disease, Gene Regulation, and Gene Therapy; 2:30pm – 4:00pm EST on May 5th 2021

- *The presentation will also be available on-demand for those registered for the SID conference from May 3, 2021 – May 31, 2021*

- Enrollment in the efficacy cohorts is expected to begin in 2H21

KB104 for Netherton Syndrome

- The Company continues to work towards an IND filing, which is anticipated in 2H21

Fourth Quarter and Full Year 2020 Financial Results:

- Cash, cash equivalents and short-term investments totaled \$271.3 million on December 31, 2020, compared to \$193.7 million as of December 31, 2019.
- Research and development expenses for the fourth quarter ended December 31, 2020 were \$5.7 million, compared to \$4.3 million for the fourth quarter 2019, and \$17.9 million for the year ended December 31, 2020, compared to \$15.6 million for the year ended December 31, 2019.
- General and administrative expenses for the fourth quarter ended December 31, 2020 were \$4.8 million, compared to \$1.8 million for the fourth quarter 2019, and \$15.1 million for the year ended December 31, 2020, compared to \$6.5 million for the year ended December 31, 2019.
- Net losses for the quarters ended December 31, 2020 and 2019 were \$10.5 million and \$5.4 million, or \$(0.53) and \$(0.31) respectively, per common share (basic and diluted). Net losses for the years ended December 31, 2020 and 2019 were \$32.2 million and \$19.1 million or \$(1.71) and \$(1.20) respectively, per common share (basic and diluted).
- For additional information on the Company's financial results for the year ended December 31, 2020, refer to form 10-K filed with the SEC.

Subsequent Events:

- On February 1, 2021, the Company completed a public offering of 2,211,538 shares of its common stock, including 288,461 shares purchased by the underwriters, at \$65.00 per share. Net proceeds to the Company from the offering were \$135.0 million.
- In January 2021, 262,500 shares of common stock were issued pursuant to our at-the-market equity offering program ("ATM Program") for net proceeds of \$16.9 million, resulting in a remaining \$132.5 million available for issuance under the ATM Program.

About Krystal Biotech

Krystal Biotech, Inc. (NASDAQ:KRY5) is a pivotal-stage gene therapy company leveraging its novel, redosable gene therapy platform and in-house manufacturing capabilities to develop therapies to treat serious rare 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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Consolidated Balance Sheet Data:

(In thousands)	December 31, 2020	December 31, 2019
Balance sheet data:		
Cash and cash equivalents	\$ 268,269	\$ 187,514
Working capital	259,606	192,553
Total assets	310,844	209,023
Total liabilities	18,760	6,109
Total stockholders' equity	\$ 292,084	\$ 202,914

Consolidated Statement of Operations:

(In thousands, except shares and per share data)	Years Ended December 31,		
	2020	2019	Change
Expenses			
Research and development	\$ 17,936	\$ 15,616	\$ 2,320
General and administrative	15,063	6,465	8,598
Total operating expenses	32,999	22,081	10,918
Loss from operations	(32,999)	(22,081)	(10,918)
Other Expense			
Interest and other income, net	832	2,993	(2,161)
Total interest and other income	832	2,993	(2,161)
Net loss	(32,167)	(19,088)	(13,079)
Net loss applicable to stockholders	\$ (32,167)	\$ (19,088)	\$ (13,079)
Net loss attributable to common stockholders per share: Basic and diluted	\$ (1.71)	\$ (1.20)	
Weighted-average common shares outstanding: Basic and diluted	18,787,161	15,901,083	

Source: Krystal Biotech, Inc.



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