Krystal Biotech Announces Fourth Quarter and Full Year 2022 Financial Results and Operational Progress

February 27, 2023

• B-VEC PDUFA date May 19, 2023

• Data from the pivotal Phase 3 (GEM-3) trial of B-VEC for dystrophic epidermolysis bullosa published in the New England Journal of Medicine

• Four pipeline clinical trials in dermatology, respiratory and aesthetics to initiate in 2023

• Strong balance sheet, closing the quarter with $383.8 million in cash, cash equivalents and investments

PITTSBURGH, Feb. 27, 2023 (GLOBE NEWSWIRE) -- Krystal Biotech, Inc., (the "Company") (NASDAQ: KRYS), a biotechnology company focused on developing and commercializing genetic medicines, today reported financial results and key operational updates for the fourth quarter and year ending December 31, 2022.

"In 2022, we were focused on advancing B-VEC toward approval and getting commercially prepared for a potential launch this year, and I thank the entire Krystal team for their dedication and perseverance in helping us achieve our objectives," said Krish S. Krishnan, Chairman and CEO of Krystal Biotech, Inc. He added, "Our focus in 2023 will be on advancing our pipeline and demonstrating the breadth of our gene delivery platform. We plan to have ASTRA, our second manufacturing facility, operational early this year which will enable us to advance our pipeline products in a timely manner."

Dermatology

B-VEC (beremagene geperpavec) for Dystrophic Epidermolysis Bullosa (DEB)

- On January 5, 2023, the Company was notified by the U.S. Food & Drug Administration (FDA) that the Prescription Drug User Fee Act (PDUFA) date for the Company's Biologics License Application (BLA) for B-VEC for the treatment of patients with DEB, will be May 19, 2023 and that proposed labeling discussions would begin no later than April 20, 2023. All clinical and manufacturing inspections conducted by the FDA as part of the BLA review process are now successfully complete.
- In December 2022, the results of the B-VEC pivotal Phase 3 GEM-3 trial were published in the *New England Journal of Medicine* (*NEJM*). The GEM-3 trial was a randomized, double-blind, intra-patient, placebo-controlled, multi-center study designed to evaluate the efficacy and safety of B-VEC for the treatment of DEB. Two editorials were also published in the same issue of *NEJM* on the GEM-3 trial.
- The Company submitted a request for Marketing Authorization Application (MAA) with the European Medicines Agency (EMA) in November 2022 for B-VEC for the treatment of DEB in patients 6 months and older. The Company was informed by the EMA in January 2023 to modify the PIP waiver request to include patients between birth and 6 months. The Company is modifying the application so that the MAA procedure can officially start in the second half of 2023 with an approval expected in early 2024.

Rare Dermatological Pipeline

- The Company expects to initiate dosing in the Phase 2 cohort of its KB105-02 (JADE-1) trial in the first half of 2023. The Phase 2 cohort will enroll both pediatric and adult patients with TGM1 deficient autosomal recessive congenital ichthyosis for assessment of KB105 safety and efficacy. Details of the Phase 1/2 study can be found at www.clinicaltrials.gov under NCT identifier NCT04047732.
- The Company plans to file an investigational new drug application (IND) for KB104 for the treatment of Netherton Syndrome in 2023.

Respiratory

KB407 for the treatment of Cystic Fibrosis (CF)

- The Company announced in August 2022, that the FDA had accepted its IND application to evaluate KB407 in a clinical trial to treat patients with CF. The Company is working closely with the Therapeutics Development Network of the Cystic Fibrosis Foundation to validate its clinical protocol and plans to initiate a Phase 1 dose ranging clinical (CORAL-1) trial in the US in the first half of 2023 to assess safety and preliminary efficacy. Details of the Phase 1 study can be found at www.clinicaltrials.gov under NCT identifier NCT05095246. The Company is presently screening patients for enrollment in a Phase 1 clinical trial in Australia to study safety and determine frequency of dosing application.
- In November 2022, the Company presented new preclinical data at the North American Cystic Fibrosis Conference, including assessments of KB407 biodistribution in the lungs of non-human primates following inhaled delivery.

KB408 for the treatment of Alpha-1 Antitrypsin Deficiency (AATD)

- The Company is completing preclinical and manufacturing activities for KB408 and expects to initiate a Phase 1 clinical trial for the treatment of AATD in the second half of 2023.

Aesthetics

KB301 for the treatment of Aesthetic Indications

- In November 2022, the Company announced up to nine months of durability results from the extension cohort of KB301 Phase 1 (PEARL-1) study in patients aged 55 to 76. Details of the PEARL-1 study can be found at www.clinicaltrials.gov under NCT identifier NCT04540900.
- The Company plans to initiate a Phase 2 (PEARL-2) study for the treatment of fine lines in 2023 following completion of a smaller proof-of-concept study in lateral canthal lines.

Fourth Quarter and Full Year 2022 Financial Results:

- Cash, cash equivalents, and investments totaled $383.8 million on December 31, 2022.
- Research and development expenses for the fourth quarter ended December 31, 2022 were $10.7 million, compared to $9.0 million for the fourth quarter 2021, and $42.5 million for the year ended December 31, 2022, compared to $27.9 million for the year ended December 31, 2021.
- General and administrative expenses for the fourth quarter ended December 31, 2022 were $24.0 million, compared to $12.9 million for the fourth quarter 2021, and $77.7 million for the year ended December 31, 2022, compared to $40.4 million for the year ended December 31, 2021.
- Net losses for the quarters ended December 31, 2022 and 2021 were $32.1 million and $21.8 million, or $(1.25) and $(0.94) respectively, per common share (basic and diluted). Net losses for the years ended December 31, 2022 and 2021 were $140.0 million and $69.6 million, or $(5.49) and $(3.13), respectively, per common share (basic and diluted).
- For additional information on the Company's financial results for the year ended December 31, 2022, please refer to the Form 10-K filed with the Securities and Exchange Commission.

About Krystal Biotech, Inc.
Krystal Biotech, Inc. (NASDAQ: KRYS) is a biotechnology company focused on developing and commercializing genetic medicines for patients with rare diseases. The Company's wide-ranging pipeline is based on its proprietary redosable HSV vector. Headquartered in Pittsburgh, Pennsylvania, the Company is led by an experienced management team, is fully-integrated and has core capabilities in viral vector design, vector optimization, gene therapy manufacturing and commercialization. For more information, please visit http://www.krystalbio.com, and follow @KrystalBiotech on LinkedIn and Twitter.

About Jeune Aesthetics, Inc.
Jeune Aesthetics, Inc., a wholly-owned subsidiary of Krystal Biotech, Inc., is a biotechnology company leveraging a clinically validated gene-delivery platform to develop products to fundamentally address – and reverse – the biology of aging and/or damaged skin. For more information, please visit http://www.jeuneinc.com.

Forward-Looking Statements

Any statements in this press release about future expectations, plans and prospects for Krystal Biotech, Inc., including statements about the B-VEC PDUFA date of May 19, 2023; our focus in 2023 on advancing our pipeline and demonstrating the breadth of our gene delivery platform; our plans to have our second manufacturing facility operational; the timing of the Company's EMA marketing authorization application for B-VEC; our expectations regarding initiating dosing in the Phase 2 cohort of our KB105-02 (JADE-1) trial; our plans to file an investigational new drug application for KB104; our plan to initiate a Phase 1 dose ranging clinical (CORAL-1) trial for KB407 in the US in the first half of 2023; our plans to initiate a Phase 1 clinical trial for KB408 for the treatment of AATD; our plans for the commencement of a Phase 2 study of KB301; 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: uncertainties associated with regulatory review of clinical trials and applications for marketing approvals, the availability or commercial potential of product candidates including B-VEC, 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 the Company'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 the Company's views as of the date of this release. The Company anticipates that subsequent events and developments will cause its views to change. However, while the Company 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 the Company's views as of any date subsequent to the date of this release.

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Condensed Balance Sheet:

(In thousands)	December 31, 2022	December 31, 2021
Balance sheet data:		
Cash and cash equivalents	$ 161,900	$ 341,246
Short-term investments	217,271	96,850
Long-term investments	4,621	64,371
Total assets	558,450	626,295
Total liabilities	36,219	32,719
Total stockholders' equity	$ 522,231	$ 593,576

Consolidated Statement of Operations:

	Three Months Ended December 31,		
(In thousands, except shares and per share data)	**2022**	**2021**	**Change**
Expenses			
Research and development	$ 10,741	$ 9,010	$ 1,731
General and administrative	24,030	12,867	11,163
Total operating expenses	34,771	21,877	12,894
Loss from operations	(34,771)	(21,877)	(12,894)
Other Income (Expense)			
Interest and other income, net	2,719	71	2,648
Net loss	$ (32,052)	$ (21,806)	$ (10,246)
Net loss per common share: Basic and diluted	$ (1.25)	$ (0.94)	
Weighted-average common shares outstanding: Basic and diluted	25,680,520	23,096,530	

	Years Ended December 31,		
(In thousands, except shares and per share data)	**2022**	**2021**	**Change**
Expenses			
Research and development	$ 42,461	$ 27,884	$ 14,577
General and administrative	77,735	40,391	37,344
Litigation settlement	25,000	—	25,000
Total operating expenses	145,196	68,275	76,921
Loss from operations	(145,196)	(68,275)	(76,921)
Other Income (Expense)			
Interest and other income, net	5,221	197	5,024
Interest expense	—	(1,492)	1,492
Total other income (expense)	5,221	(1,295)	6,516
Net loss	$ (139,975)	$ (69,570)	$ (70,405)
Net loss per common share: Basic and diluted	$ (5.49)	$ (3.13)	
Weighted-average common shares outstanding: Basic and diluted	25,491,721	22,196,846	

Source: Krystal Biotech, Inc.