Krystal Biotech Announces FDA's 3-Month Extension of BLA PDUFA Date and Regulatory Update for B-VEC to Treat Patients with Dystrophic Epidermolysis Bullosa

January 9, 2023

NEW PDUFA DATE OF MAY 19, 2023

PITTSBURGH, Jan. 09, 2023 (GLOBE NEWSWIRE) -- Krystal Biotech, Inc. (the "Company") (NASDAQ: KRYS) today announced that on January 5, 2023, the U.S. Food and Drug Administration (FDA) notified the Company that based on manufacturing information submitted to the Agency on December 20, 2022, in response to an information request, the PDUFA date has been revised to May 19, 2023, and proposed labeling discussions to no later than April 20, 2023.

The manufacturing information submitted by the Company included additional information about a replaced hardware unit in the concentration step of the manufacturing process and comparability data supporting the use of the unit. The unit did not affect processing parameters or product contact materials. The FDA considered this new information as a major amendment to the application that will require additional time for review.

The BLA late-cycle review meeting was completed on December 15, 2022. During this meeting, the FDA indicated that there will be no Advisory Committee meeting for B-VEC and a Risk Evaluation and Mitigation Strategies (REMS) program is not needed for the B-VEC application. All pre-approval inspections of clinical sites and internal manufacturing and testing facilities have been successfully completed.

"While we are disappointed that this change was viewed as a major amendment, we are committed to working with the FDA as it completes its review of the B-VEC application," said Krish S. Krishnan, Chairman & CEO at Krystal Biotech. "We will continue our commercial readiness efforts and upon approval bring this important treatment to DEB patients as soon as possible."

The Company submitted the B-VEC BLA to the FDA in June 2022. The FDA accepted the BLA in August 2022 and granted Priority Review.

About Dystrophic Epidermolysis Bullosa (DEB)

DEB is a rare and severe disease that affects the skin and mucosal tissues. It is caused by one or more mutations in a gene called *COL7A1*, which is responsible for the production of the protein type VII collagen (COL7) that forms anchoring fibrils that bind the dermis (inner layer of the skin) to the epidermis (outer layer of the skin). The lack of functional anchoring fibrils in DEB patients leads to extremely fragile skin that blisters and tears from minor friction or trauma. DEB patients suffer from open wounds, which leads to skin infections, fibrosis which can cause fusion of fingers and toes, and ultimately an increased risk of developing an aggressive form of squamous cell carcinoma which, in severe cases, can be fatal.

About B-VEC

B-VEC is an investigational non-invasive, topical, redosable gene therapy designed to deliver two copies of the *COL7A1* gene when applied directly to DEB wounds. B-VEC was designed to treat DEB at the molecular level by providing the patient's skin cells the template to make normal COL7 protein, thereby addressing the fundamental disease-causing mechanism.

The FDA and European Medical Agency (EMA) have each granted B-VEC orphan drug designation for the treatment of DEB, and the FDA has granted B-VEC fast track designation and rare pediatric designation for the treatment of DEB. In addition, the FDA granted Regenerative Medicine Advanced Therapy (RMAT) to B-VEC for the treatment of DEB and the EMA granted PRIority MEdicines (PRIME) eligibility for B-VEC to treat DEB.

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.

Forward-Looking Statements

Any statements in this press release about future expectations, plans and prospects for the Company, including statements about commercial and launch readiness for B-VEC 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 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 press release.

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