FDA Accepts Krystal Biotech's Biologics License Application for Dystrophic Epidermolysis Bullosa

August 18, 2022

- FDA granted Priority Review designation
- PDUFA target action date is February 17, 2023
- FDA stated that it is not currently planning to hold an advisory committee meeting

PITTSBURGH, Aug. 18, 2022 (GLOBE NEWSWIRE) -- Krystal Biotech, Inc. (the "Company") (NASDAQ: KRYS), the leader in redosable gene therapy, today announced that the US Food and Drug Administration (FDA) has accepted for filing the Company's Biologics License Application (BLA) for B-VEC for the treatment of patients with dystrophic epidermolysis bullosa (DEB). The application has been granted Priority Review designation, and the Prescription Drug User Fee Act (PDUFA) action date is February 17, 2023. The FDA stated that it is not currently planning to hold an advisory committee meeting to discuss the application.

"We are delighted to receive the FDA's acceptance of our BLA submission and move one step closer to potentially bringing a medicine to fundamentally treat DEB patients," said Suma Krishnan, Co-Founder and President, Research & Development of Krystal Biotech. "We are committed to working closely with the FDA to bring this potential, first-ever treatment to patients living with DEB as quickly as possible."

The BLA submission for B-VEC is supported by data from two placebo controlled clinical trials - the GEM-3 trial (NCT04491604) and the GEM-1/2 trial (NCT03536143).

Priority Review designation is granted to applications for medicines that treat a serious condition, and if approved, would provide a significant improvement in the safety or effectiveness of the treatment, prevention, or diagnosis of a serious condition. B-VEC was previously granted Fast Track designation and Regenerative Medicine Advanced Therapy (RMAT) by the FDA for the treatment of DEB.

The Company plans to submit a marketing authorization application to the European Medicines Agency (EMA) in the second half of 2022.

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 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 pivotal-stage gene therapy company leveraging its proprietary, redosable gene therapy platform and in-house manufacturing capabilities to develop life-changing medicines for patients with serious diseases, including rare diseases in skin, lung, and other areas. 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 the timing of the submission of the Company's EMA marketing authorization application, 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 press release.

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