



New England Journal of Medicine Publishes Phase 3 Data on B-VEC in Patients with Dystrophic Epidermolysis Bullosa

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PITTSBURGH, Dec. 14, 2022 (GLOBE NEWSWIRE) -- [Krystal Biotech, Inc.](#) (the "Company") (NASDAQ: KRY5), a biotechnology company focused on developing and commercializing genetic medicines for patients with rare diseases, announced today that data from the pivotal Phase 3 (GEM-3) trial of beremagene geperpavec (B-VEC) for dystrophic epidermolysis bullosa (DEB) have been published [here](#) in the New England Journal of Medicine (NEJM). The full manuscript, titled "Trial of Beremagene Geperpavec (B-VEC) for Dystrophic Epidermolysis Bullosa," will appear in the December 15, 2022 issue of the *NEJM*.

In this GEM-3 trial of 31 patients, complete wound healing at 6 months occurred in 67.4% of B-VEC wounds compared to 21.6% for placebo (difference, 45.8 percentage points; 95% confidence interval [CI], 23.6 to 68.0; $p=0.002$). Complete wound healing at 3 months occurred in 70.6% of the wounds exposed to B-VEC as compared with 19.7% of those exposed to placebo (difference, 51.0 percentage points; 95% CI, 29.3 to 72.6; $p=0.0005$).

"The impressive phase 3 results with B-VEC are the best we have seen to date in patients with DEB and, if approved, B-VEC provides hope for these patients suffering through debilitating and potentially life-threatening symptoms associated with the disease," said Peter Marinkovich, M.D., Director of the Blistering Disease Clinic at Stanford Health Care, Associate Professor of Dermatology at the Stanford University School of Medicine, primary investigator of the GEM-3 trial and primary author of the manuscript.

The GEM-3 trial was a randomized, double-blind, intra-patient placebo-controlled multi-center trial designed to evaluate the efficacy and safety of B-VEC for the treatment of DEB. In the trial, matched wounds receiving topical B-VEC or placebo were evaluated in 31 DEB patients over 26 weeks. The pivotal GEM-3 trial met its primary endpoint of complete wound healing at six-months and its secondary endpoint of complete wound healing at three-months. B-VEC was well tolerated, with no drug-related serious adverse events or discontinuations due to treatment.

"B-VEC was developed by Krystal scientists as a potential first-in-class therapy for DEB," said Suma Krishnan, President, Research & Development, Krystal Biotech. "We are working closely with the FDA to get B-VEC approved and deliver a meaningful benefit to patients with this debilitating disease."

The Company received US Food and Drug Administration (FDA) filing acceptance of its Biologics License Application (BLA) for B-VEC. The BLA was granted Priority Review designation and the Prescription Drug User Fee Act action date is February 17, 2023. The Company has filed the Marketing Authorization (MA) application with the European Medical Agency (EMA) and is currently working closely with the EMA through the MA validation process.

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: KRY5) 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 the clinical utility of 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.

CONTACT:

Investors and Media

Meg Dodge

Krystal Biotech

mdodge@krystalbio.com

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