Krystal Biotech Receives Positive Opinion from EMA Pediatric Committee on the Pediatric Investigation Plan for B-VEC for the treatment of Dystrophic Epidermolysis Bullosa

September 21, 2022

• Company on track to submit European marketing application in Q4 2022

• No additional studies required

PITTSBURGH, Sept. 21, 2022 (GLOBE NEWSWIRE) -- Krystal Biotech, Inc. (the "Company") (NASDAQ: KRYS), the leader in redosable gene therapy, announced today that the European Medicines Agency (EMA) Pediatric Committee (PDCO) has adopted a positive opinion on the Pediatric Investigation Plan (PIP) for beremagene geperpavec (B-VEC) for the treatment of dystrophic epidermolysis bullosa.

As part of the regulatory process for the registration of new medicines in Europe, the EMA requires companies to provide a PIP outlining their strategy for investigation of the new medicinal product in the pediatric population. An approved PIP is a prerequisite for filing a Marketing Authorization Application (MAA). The positive opinion is based on the B-VEC non-clinical safety program as well as data from the clinical studies conducted in the US that will be included in the upcoming MAA.

"The approval of the PIP provides a clear path forward, and we look forward to working with the EMA and PDCO to bring this important treatment to the European market as soon as possible," said Suma Krishnan, President, Research & Development.

Based on this positive opinion, the Company would be eligible for up to an additional two years of marketing exclusivity in the EU, on top of the ten-year EU market exclusivity after market approval in the EU.

The U.S. Food and Drug Administration (FDA) and EMA have each granted B-VEC orphan drug designation for the treatment of DEB. The FDA has granted B-VEC fast track designation, Regenerative Medicine Advanced Therapy (RMAT) and rare pediatric disease designation for the treatment of DEB. B-VEC is eligible to receive a Priority Review Voucher (PRV) following approval of B-VEC in the US. The EMA granted PRIority MEdicines (PRIME) eligibility for B-VEC to treat DEB.

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.

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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