Krystal Biotech Announces Completion of the GEM-3 Pivotal Phase 3 Study Evaluating B-VEC for the Treatment of Dystrophic Epidermolysis Bullosa

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- *Topline results anticipated in the fourth quarter of 2021*
- *Of the 31 patients enrolled in the trial, 29 patients completed the study with no missing visits, including the three primary endpoint assessment visits*

PITTSBURGH, Oct. 26, 2021 (GLOBE NEWSWIRE) -- Krystal Biotech, Inc., ("Krystal") (NASDAQ: KRYS), the leader in redosable gene therapies for rare diseases, today announced that the last participant has completed the 26-week dosing period and 30-day safety follow up visit in the GEM-3 study, Krystal's pivotal Phase 3 clinical trial evaluating investigational beremagene geperpavec (B-VEC) as a topical gene therapy for the treatment of dystrophic epidermolysis bullosa (DEB). DEB is a rare, genetic skin disease that causes skin to tear or blister from minor contact and is caused by a lack of functional type VII collagen (COL7) protein in the skin.

"We want to express our gratitude to the trial participants, their families, the clinical investigators and their coordinators for their participation and commitment that enabled us to complete this study in a timely manner during such a challenging year," said Suma Krishnan, Chief Operating Officer of Krystal Biotech. "We remain on track to announce topline data from the trial this quarter, and if positive, we will work expeditiously to complete regulatory filings with the FDA and EMA with the goal of bringing a topical, genetically corrective therapy to patients living with DEB."

About the GEM-3 Pivotal Study
The GEM-3 trial is a randomized, double-blind, intra-patient placebo-controlled study designed to evaluate the efficacy and safety of B-VEC for the treatment of DEB. The trial enrolled 31 patients ranging in ages from one (1) year to forty-four (44) years old. Sixty-one percent (61%) of the patients enrolled were pediatric patients (18 years old or younger). Less than ten percent (10%) of enrolled patients have the dominant form of dystrophic epidermolysis bullosa (DDEB). Of the 31 patients enrolled in the trial, 29 patients completed the study with no missing visits, including the three primary endpoint assessment visits.

In each patient, a primary wound pair was identified by the investigator; one wound was randomized to receive a weekly topical application of B-VEC and the other a placebo. Primary wound pairs selected in the study included all three wound area segments of <20 cm^2, 20-40 cm^2 and 40-60 cm^2 and were assigned the corresponding doses of 4×10^8 PFU/wound, 8×10^8 PFU/wound or 1.2×10^9 PFU/wound, respectively. Weekly application was continued until the investigator determined the wound was completely closed. Re-application occurred at any point throughout the study if the investigator determined the wound was not completely closed. The primary outcome measure is complete wound healing determined by the investigator in B-VEC treated wounds versus placebo treated wounds at Week 22 and Week 24 or Week 24 and Week 26.

In addition to the primary target wound pair(s), additional wounds (secondary wounds) were selected and treated with B-VEC giving the treating physicians and patients flexibility to treat multiple wounds during the weekly application. For more information about the pivotal GEM-3 study, visit www.clinicaltrials.gov (NCT04491604).

Subjects returned to the clinical site 30 days following the last dosing visit (Week 26) for safety evaluation by the investigator and subsequently had the option to roll into the Open Label Extension (OLE) Study (NCT04917874). In addition, new participants who were unable to participate in the Phase 3 study but met all enrollment criteria are eligible to enroll in the OLE.

About Dystrophic Epidermolysis Bullosa
Dystrophic epidermolysis bullosa, or DEB, is an incurable, often fatal skin blistering condition. It is caused by mutations in the COL7A1 gene encoding type VII collagen, or COL7, a major component of anchoring fibrils which connect the epidermis to the underlying dermis and provide structural adhesion between these skin layers in a healthy individual. The lack of COL7 in DEB patients causes blisters to occur in the dermis as a result of its separation from the epidermis. This makes the skin incredibly fragile, leading to blistering or skin loss at the slightest friction or perturbation. DEB is both progressive and incredibly painful.

The most severe form of DEB is recessive DEB, or RDEB, which is caused by homozygous null mutations in the COL7A1 gene. DEB also occurs in a dominant form, DDEB, which is considered to be a milder form of the disease. There are no known treatments affecting the outcome of either form of the disease, and the current standard of care for DEB patients is limited to palliative treatments.

About Krystal Biotech
Krystal Biotech, Inc. (NASDAQ:KRYS) is a pivotal-stage gene therapy company leveraging its novel, redosable gene therapy platform and in-house manufacturing capabilities to develop therapies to treat serious rare diseases. 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 Krystal Biotech, Inc., including but not limited to statements about the development of Krystal's product candidates, such as plans for the design, conduct and timelines of ongoing clinical trials of beremagene geperpavec ("B-VEC"); the clinical utility of B-VEC, and Krystal's plans for filing of regulatory approvals and efforts to bring B-VEC to market; 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: the uncertainties inherent in the initiation and conduct of clinical trials, availability and timing of data from clinical trials, whether results of early clinical trials or trials will be indicative of the results of ongoing or future trials, 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 Krystal’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 Krystal’s views as of the date of this release. Krystal anticipates that subsequent events and developments will cause its views to change. However, while Krystal 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 Krystal’s views as of any date subsequent to the date of this release.

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