Krystal Biotech Provides Update on Pivotal GEM-3 Study of B-VEC for DEB

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PITTSBURGH, April 26, 2021 (GLOBE NEWSWIRE) -- Krystal Biotech Inc., (“Krystal”) (NASDAQ: KRYS), the leader in redosable gene therapies for rare diseases, today announced modifications to the Statistical Analysis Plan (SAP) in the ongoing Phase 3 study of B-VEC for the treatment of dystrophic epidermolysis bullosa (DEB) based on feedback from the U.S. Food and Drug Administration (FDA).

The GEM-3 trial is a randomized, double-blind, intra patient placebo-controlled multicenter study designed to evaluate the efficacy and safety of B-VEC for patients living with both recessive and dominant forms of dystrophic epidermolysis bullosa. The trial enrolled 31 subjects with DEB, aged 6 months or older at time of consent. Each subject will serve as their own control.

To account for paired binary data in the study design and the within-pair correlation in the GEM-3 study, Krystal will use the McNemar test for primary efficacy analysis. The primary outcome measure is complete wound healing determined by the Investigator in B-VEC treated wounds versus placebo. In the updated SAP, a positive response is defined as:

- Complete wound healing at Week 22 and Week 24; *or*
- Complete wound healing at Week 24 and Week 26.

Only wounds that achieve complete wound healing for at least 2 weeks are counted as a positive response.

“As the GEM-3 trial remains double blinded until database lock occurs in the fourth quarter of 2021, we had the flexibility to adjust the planned SAP with no impact on trial conduct or progress,” said Suma Krishnan, chief operating officer of Krystal Biotech. She added, “We believe that the updated SAP accounts for the within-wound-pair correlation and will better assess the potential benefit of B-VEC against placebo.”

With 90% power and a two-sided Type 1 error rate of 5%, and an assumed 20% drop out rate, 30 subjects (i.e., 30 wound pairs) were required for the GEM-3 study assuming a 50% difference in response rate between B-VEC and placebo treated wounds. Because the calculation assumes no correlation within subjects, the estimate is conservative. Any positive correlation will cause an increase in power.

Retrospective analysis of Phase 2 study looking at complete wound healing at Week 8 and Week 10 or Week 10 and Week 12 resulted in 78.6% response rate for B-VEC treated wounds versus 0% response rate for placebo wound, with a $p<0.003$ using the McNemar test.

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.

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 pre-clinical and clinical trials of beremagene geperpavec (“B-VEC”), KB105, KB104, KB301, KB407, and KB408; the clinical utility of B-VEC, KB105, KB104, KB301, KB407 and KB408, and Krystal’s plans for filing of regulatory approvals and efforts to bring B-VEC, KB105, KB104, KB301, KB407 and KB408 to market; the market opportunity for and the potential market acceptance of B-VEC, KB105, KB104, KB301, KB407 and KB408; plans to pursue research and development of other product candidates; the sufficiency of Krystal’s existing cash resources; the unanticipated impact of COVID-19 on Krystal’s business operations, pre-clinical activities and clinical trials; 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, KB105, KB104, KB301, KB407 and KB408, 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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