



## Krystal Biotech Announces Final Update from Phase 1/2 Clinical Trial of KB103 ("bercolagene telsepavec," "B-VEC")

October 29, 2019

- *Safety data from all patients (Phase 1 and Phase 2) show that B-VEC was well tolerated with no related adverse events reported.*
- *Nine out of 10 of the wounds closed completely (100% closure) following initial administrations of B-VEC.*
- *The average time to wound closure was 17.4 days.*
- *The average duration of wound closure at the last measured timepoint was 113 days.*
- *Re-administered chronic wound closed in 7 days with a wound closure duration of 100 days as of the last measurement timepoint.*

PITTSBURGH, Oct. 29, 2019 (GLOBE NEWSWIRE) -- [Krystal Biotech](#), Inc. (Nasdaq:KRY5), a gene therapy company dedicated to developing and commercializing novel treatments for patients suffering from rare dermatological diseases, today announced the final update from the Phase 1/2 clinical trial of bercolagene telsepavec (B-VEC), formerly KB103.

### **Phase 1/2 Study of B-VEC**

In Phase 1, two (2) adult patients with severe generalized RDEB were enrolled in May 2018. In each patient, two (2) wounds with an approximate surface area of 10 cm<sup>2</sup> were randomized to receive either topical B-VEC or placebo.

In Phase 2, four (4) patients (2 adults, ages 22 and 19, and two pediatric, ages 14 and 15) with severe generalized RDEB were enrolled in December 2018. Prior to dosing, three (3) wounds up to 20cm<sup>2</sup> were selected from each patient and subsequently randomized to receive either B-VEC or placebo in a 2:1 (B-VEC:placebo) ratio. One of patients (age 19) voluntarily discontinued from the study after 30 days due to an inability to travel to the clinical site. With respect to the six (6) B-VEC-administered wounds from the patients that remained on-study, two (2) were categorized as chronic and four (4) as recurring based on patient reporting. Chronic wounds were defined as having remained open for greater than 12 weeks, while recurring wounds are less than 12 weeks old, and open and close spontaneously.

As part of the Phase 2 study, a chronic wound that had not healed completely at the Day 90 timepoint was re-administered B-VEC. This wound was subsequently observed for approximately four (4) additional months.

In addition, two new patients (ages 21 and 33) with severe generalized RDEB were enrolled in June 2019. Prior to dosing, two wounds were selected in each patient and were randomized to receive either B-VEC or placebo in a 1:1 (B-VEC:placebo) ratio. Patients were administered B-VEC every other day for two weeks, or until the wound closed completely. Patients returned to the clinic for monthly follow-ups including imaging, biopsies for molecular correction analyses, and safety assessments.

"We are encouraged by the results to date and believe that B-VEC has the potential to be a very convenient and elegant approach to treating recurring and chronic wounds for patients suffering from DEB, a debilitating disease that affects patients and their extended families," said Suma Krishnan, founder and chief operating officer of Krystal Biotech. "With the successful completion of our Phase 1/2 study, we look forward to commencing our pivotal trial following alignment with the FDA on CMC and design of our pivotal trial."

### **Clinical Data Update Summary**

- Safety data from all of the patients in the combined Phase 1/2 study demonstrate that B-VEC was well tolerated following initial and repeat dosing. No inflammation or irritation was observed in B-VEC-administered wounds.
- Of the ten (10) wounds that were administered B-VEC, seven were categorized as recurring and three were categorized as chronic.
- *Percent of Wound Closure:* 90% of the wounds (9 of 10) closed completely (100% closure) following initial administrations of B-VEC.
- *Time to Wound Closure:* The average time to 100% wound closure on the nine (9) B-VEC-administered wounds was 17.4 days (median 14 days).
- *Duration of Wound Closure:* The average duration of wound closure on the nine (9) B-VEC-administered wounds at the last measured timepoint was 113 days (median 110 days).
- One chronic wound, reported to be open for over four years, did not close completely following the initial administration of B-VEC in the Phase 2 study.
  - The wound was 42% closed on Day 90 following the initial administration of B-VEC.
  - The wound was re-administered B-VEC, and the time to complete wound closure following re-administration was seven (7) days.
  - The wound has remained closed for over 100 days following the second administration.

### **Clinical Update on Two Additional Patients Enrolled in the Phase 2 Study**

1. Both of the B-VEC-administered wounds (one recurring and one chronic) closed 100%.
2. The time to complete wound closure for both wounds was eight (8) days.
3. The average duration of wound closure at the four (4) month timepoint was 114.5 days.
4. Consistent with prior wound data, the data on these additional patients show a strong correlation between molecular correction and wound healing.
5. Immunofluorescence (IF) analyses of biopsies from B-VEC-administered wounds demonstrate functional collagen VII (COL7) expression.
6. Functional COL7 was determined by staining the tissue samples with antibodies that bind to NC1 and NC2 domains of the COL7 protein respectively. The tissues from the skin biopsies show the presence of both NC1 and NC2 domains demonstrating production of functional COL7 that is linearly deposited along the Basement Membrane Zone (BMZ).
7. NC1- and NC2-reactive anchoring fibrils were observed by immunoelectron microscopy (IEM) in the biopsy samples.

#### **Webcast**

To hear Management elaborate on the clinical data please follow the links below for a recording:

[www.netroadshow.com/nrs/home#!/?show=10cde91d](http://www.netroadshow.com/nrs/home#!/?show=10cde91d) and enter the Code: **KRYSINC2019** (not case-sensitive)

or visit [www.netroadshow.com](http://www.netroadshow.com) and enter the Code: KRYSINC2019

#### **About Krystal Biotech**

Krystal Biotech, Inc. (NASDAQ:KRY5) is a gene therapy company dedicated to developing and commercializing novel treatments for patients suffering from dermatological diseases. For more information, please visit <http://www.krystalbio.com>.

#### **About B-VEC**

B-VEC is Krystal's lead product candidate that seeks to use gene therapy to treat dystrophic epidermolysis bullosa, or DEB, an incurable skin blistering condition caused by a lack of collagen in the skin. B-VEC is a replication-defective, non-integrating viral vector employing Krystal's STAR-D platform to deliver functional human COL7A1 genes directly to the patients' dividing and non-dividing skin cells. Krystal's HSV-1 is a proprietary vector that can penetrate skin cells more efficiently than other viral vectors. Its high payload capacity allows it to accommodate large or multiple genes and its low immunogenicity makes it a suitable choice for direct and repeat delivery to the skin.

#### **About Dystrophic Epidermolysis Bullosa**

Dystrophic epidermolysis bullosa, or DEB, is an incurable, often fatal skin blistering condition caused by a lack of collagen protein in the skin. It is caused by mutations in the gene coding for 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 normal individual. The lack of COL7 in DEB patients causes blisters to occur in the dermis as a result of separation from the epidermis. This makes the skin incredibly fragile, leading to blistering or skin loss at the slightest friction or knock. It is progressive and incredibly painful.

The most severe form of DEB is recessive DEB, or RDEB, which is caused by null mutations in the COL7A1 gene. DEB also occurs in the form of dominant DEB, or DDEB, which is considered to be a milder form of DEB. There are no known treatments affecting the underlying cause of either form of the disease, and the current standard of care for DEB patients is limited to palliative treatments. Krystal is developing B-VEC for the treatment of the broad DEB population, including both recessive and dominant forms of the disease.

#### **Forward-Looking Statements**

This press release on announcing positive data from Krystal's phase 1/2 trial evaluating B-VEC in patients suffering from Dystrophic epidermolysis bullosa, or DEB, contains "forward-looking statements" regarding matters that are not historical facts, including statements relating to the Company's clinical trials, including plans to commence future trials. There can be no assurance that the data contained in these results will be replicated in additional current and future patients enrolled in this or any future trial or that these results will prove clinically meaningful in the development of B-VEC as a potential drug. Because such statements are subject to risks and uncertainties, actual results may differ materially from those expressed or implied by such forward-looking statements. Words such as "anticipates," "plans," "expects," "intends," "will," "potential," "hope" and similar expressions are intended to identify forward-looking statements. These forward-looking statements are based upon current expectations of the Company and involve assumptions that may never materialize or may prove to be incorrect. Actual results and the timing of events could differ materially from those anticipated in such forward-looking statements as a result of various risks and uncertainties. Detailed information regarding factors that may cause actual results to differ materially from the results expressed or implied by statements in this press release relating to the Company may be found in the Company's periodic filings with the Securities and Exchange Commission, including the factors described in the section entitled "Risk Factors" in its annual report on Form 10-K for the fiscal year ended December 31, 2018, and supplemented from time to time and the Company's Quarter Reports on Form 10-Q and other filings submitted by the Company to the SEC, copies of which may be obtained from the SEC's website at [www.sec.gov](http://www.sec.gov). The parties do not undertake any obligation to update forward-looking statements contained in this press release.

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