



Krystal Biotech to Present Pre-clinical Data Highlighting KB301 for Aesthetic Indications at the ASDS 2020 Virtual Meeting

October 8, 2020

- **KB301 is designed to deliver a full-length human type 3 collagen transgene via injection**

- **The Phase 1 PEARL-1 clinical study to evaluate KB301 in acne scars and fine wrinkles is currently enrolling**

PITTSBURGH--(BUSINESS WIRE)--Oct. 8, 2020-- [Krystal Biotech](#), Inc. (Nasdaq:KRYS) today announced the presentation of positive preclinical data supporting the ongoing development of KB301, an innovative, investigational therapy designed to reverse the decline of collagen in aging skin. The data will be presented at the American Society for Dermatologic Surgery (ASDS) 2020 Virtual Meeting to be held October 9-11, 2020.

The preclinical program evaluated KB301's ability to transduce clinically relevant skin cells, to express and secrete mature human COL3 in vitro, as well as to confirm proper tissue localization of the transgene without toxicity or systemic vector distribution in vivo.

Key findings from the poster presentation titled *In Vitro and In Vivo Pharmacology of KB301, an HSV-1-Based Gene Therapy, for the Treatment of Superficial Skin Depressions* include:

- In vitro, KB301 readily transduced primary aged male and female human dermal fibroblasts (HDFs) and induced full-length COL3 expression, proper maturation, and subsequent secretion.
- In vivo, successful vector transduction and subsequent COL3 expression was observed in a dose-dependent manner at both the transcript and protein levels in young and aged immunocompetent mice. COL3 was found to be properly localized to the mouse dermis.
- High concentrations of KB301 were non-cytotoxic to the cells in vitro, supporting safety of the vector. In vivo, no vector was detected in blood or other tested tissues, showing that KB301 was retained in the treated area of the skin.

The above data presentation will be available on the Scientific Publications page of the Company's website at <http://ir.krystalbio.com/select-scientific-publications>.

About collagen type III and KB301

The skin is composed of collagen-rich connective tissue composed primarily of types I and III collagen fibrils. Age-related changes in skin are largely due to aberrant collagen homeostasis, caused both by intrinsic (e.g., passage of time, genetics) and extrinsic (e.g., chronic light exposure, pollution) factors, which leads to progressive loss of dermal collagen. KB301 is an investigational therapy designed to restore collagen homeostasis locally via directed expression of full-length human type III collagen gene (COL3A1), thereby reconstructing an optimal physiologic environment in the skin to treat wrinkles and other superficial skin defects. KB301 is manufactured in-house at Krystal's fully functional GMP ANCORIS facility, located near corporate headquarters in Pittsburgh. If ultimately successful, KB301 and any additional aesthetic programs will be developed through our wholly-owned subsidiary Jeune, Inc.

About the PEARL-1 Trial

The Phase 1 trial will evaluate the safety, tolerability, and initial efficacy of KB301 injections. Approximately 22 patients will be enrolled across 3 cohorts. The initial, open-label cohort will evaluate the safety and tolerability of 2 different dose levels of KB301 in healthy buttock skin. Following dose selection enrollment will begin in Cohorts 2 and 3, which are double-blind, placebo-controlled, intra-subject evaluations of the safety and efficacy of KB301 in either shallow-to-moderately deep facial wrinkles or moderate-to-severe atrophic acne scars. More information about the PEARL-1 study is available at <https://clinicaltrials.gov/ct2/show/NCT04540900>.

About Krystal Biotech

Krystal Biotech, Inc. (NASDAQ:KRYS) is a gene therapy company dedicated to developing transformative medicines to treat diseases caused by protein or gene dysfunction. For more information, please visit <http://www.krystalbio.com>.

About Jeune, Inc.

Jeune, Inc. is the company's wholly-owned subsidiary, which was incorporated in 2019 for the purpose of undertaking preclinical studies for aesthetic skin conditions.

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"), KB105, KB104, KB301 and KB407; the clinical utility of B-VEC, KB105, KB104, KB301 and KB407, and Krystal's plans for filing of regulatory approvals and efforts to bring B-VEC, KB105, KB104, KB301 and KB407 to market; the market opportunity for and the potential market acceptance of B-VEC, KB105, KB104, KB301 and KB407; 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 and KB407, 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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