Krystal Biotech Receives FDA Platform Technology Designation for HSV-1 Viral Vector Used in KB801 for the Treatment of Neurotrophic Keratitis

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PITTSBURGH, Oct. 14, 2025 (GLOBE NEWSWIRE) -- Krystal Biotech, Inc. (the "Company") (NASDAQ: KRYS) announced today that the United States Food and Drug Administration (FDA) granted platform technology designation to the genetically modified, non-replicating herpes simplex virus type 1 (HSV-1) viral vector used in the Company's redosable eye drop gene therapy KB801, currently under evaluation in a randomized placebo controlled trial for the treatment of neurotrophic keratitis (NK).

"Receiving a platform technology designation from the FDA is a tremendous milestone for our development team and Krystal, both as recognition of the reproducibility and scalability of our HSV-1 gene delivery platform and for the potential product development benefits it may provide," said Suma Krishnan, President of Research and Development at Krystal Biotech. "We are excited to work with the FDA under this program to identify potential efficiencies, including opportunities to leverage our prior experience with FDA-approved VYJUVEK® (beremagene geperpavec-svdt), to accelerate the development of our genetic medicines pipeline, starting with KB801 for the treatment of NK."

The FDA's platform technology designation program is intended to provide efficiencies in drug development, manufacturing, and review processes for drug product applications that incorporate designated platform technologies. Potential benefits of the designation may include early and more frequent engagement with the FDA during clinical development as well as the opportunity to leverage manufacturing and nonclinical safety data from a prior product using the designated platform technology, such as VYJUVEK, in submissions to the FDA. The FDA may also consider previous inspectional findings related to the manufacture of a drug that incorporates the designated platform technology.

To be eligible for a platform technology designation, a technology must be well-understood and reproducible, used in an FDA-approved drug or biologic product, such as VYJUVEK, have the potential to support the development of multiple drugs or biologic products without compromising quality, manufacturing, or safety, and have a reasonable likelihood to bring significant efficiencies to the development or manufacturing process as well as to the FDA review process. Drug product applications that are then recognized by the FDA to incorporate this technology may leverage the potential benefits of the designation.

About KB801
KB801 is a redosable eye drop gene therapy designed to enable sustained, localized expression and secretion of nerve growth factor (NGF) by epithelial cells in the front of the eye for the treatment of NK, a rare, degenerative corneal disease that leads to corneal epithelial defects, ulcers, and perforation. Recombinant NGF eye drops have been shown to significantly improve corneal healing and are approved for the treatment of NK in multiple jurisdictions worldwide, but rapid clearance from the eye requires intensive administration six times a day, limiting therapeutic utility. By enabling the cells of the front of the eye to produce NGF locally, KB801 has the potential to significantly reduce the treatment burden for patients while also maintaining more consistent NGF levels in the front of the eye. The safety and efficacy of KB801 for the treatment of NK are currently under evaluation in EMERALD-1, the Company's 2:1 randomized, double-masked, multicenter, placebo-controlled Phase 1/2 study in patients with NK.

About Krystal Biotech, Inc.
Krystal Biotech, Inc. (NASDAQ: KRYS) is a fully integrated, commercial-stage, global biotechnology company focused on the discovery, development and commercialization of genetic medicines to treat diseases with high unmet medical needs. VYJUVEK®, the Company's first commercial product, is the first-ever redosable gene therapy, and the first genetic medicine approved in the United States, Europe, and Japan for the treatment of dystrophic epidermolysis bullosa. The Company is rapidly advancing a robust preclinical and clinical pipeline of investigational genetic medicines in respiratory, oncology, dermatology, ophthalmology, and aesthetics. Krystal Biotech is headquartered in Pittsburgh, Pennsylvania. For more information, please visit http://www.krystalbio.com, and follow @KrystalBiotech on LinkedIn and X (formerly Twitter).

Forward-Looking Statements

This press release contains "forward looking" statements within the meaning of the Private Securities Litigation Reform Act of 1995 based on the Company's current expectations and beliefs regarding the FDA's recent grant of platform technology designation to the Company's genetically modified and non-replicating HSV-1 viral vector used in the Company's investigational product KB801. These forward-looking statements include, without limitation, statements relating to potential development efficiencies the platform technology designation may provide to accelerate the development of the Company's genetic medicines pipeline, potential benefits of the designation platform technology designation, and potential benefits of KB801 in treating NK. All statements other than historical facts are or may be deemed to be forward-looking statements and involve known and unknown risks, uncertainties, and assumptions that could cause actual results to differ materially from those indicated by such forward-looking statements as a result of various important factors 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. The Company provides this information as of the date of this release and assumes no obligation to update any forward-looking statements.

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