# Krystal Biotech Announces Virtual Presentation of Pre-clinical Data on KB408 for the Treatment of Alpha-1 Antitrypsin Deficiency at ESGCT

October 14, 2021

PITTSBURGH, Oct. 14, 2021 (GLOBE NEWSWIRE) -- Krystal Biotech Inc., (“Krystal”) (NASDAQ: KRYS), the leader in redosable gene therapies for rare diseases, today announced that it will present pre-clinical pharmacology data on its second genetic pulmonary disease candidate KB408, for the treatment of alpha-1 antitrypsin deficiency (AATD), at the upcoming European Society of Gene & Cell Therapy (ESGCT) Virtual Congress held October 19 – 22, 2021.

AATD is a rare genetic disorder that predisposes affected individuals to develop several complications, including severe and progressive lung disease in adults, and in a minority of patients (~10%) severe liver disease. AATD lung disease is caused by low levels of functional AAT protein in the blood and ultimately the lungs, its primary site of action, where it is needed to prevent tissue from enzymatic destruction. KB408 is an inhaled, redosable gene therapy designed to increase production of normal, fully functional AAT protein directly in the lung tissue to correct the deficiency.

**Abstract Information:**
*e-Poster Title:* Preclinical Pharmacology of KB408, an HSV-1-based Gene Therapy Vector, for the Treatment of Alpha-1 Antitrypsin Deficiency
*Poster Number:* 40

Full text of accepted abstracts will be made available to registrants on October 19, 2021, at 8:00am CEST. To register for the congress, please visit https://esgctcongress.ada.wats-on.co.uk/Registration.aspx.

**About AATD and KB408**
AATD is caused by mutations in the *SERPINA1* gene that lead to decreased levels and/or decreased functionality of AAT. The protein is primarily produced in the liver and secreted into the bloodstream, where it acts as a circulating serine protease inhibitor whose principal substrate is neutrophil elastase in the lungs. Over time, the deficiency can lead to progressive enzymatic destruction of the lung tissue, ultimately causing life-threatening pulmonary impairment and severe respiratory insufficiency.

Current standard of care is augmentation therapy with purified AAT from pooled human plasma to normalize serum AAT levels with the goal of maintaining protein levels in the lungs. While augmentation therapy has been shown to be effective in reducing the rate of lung destruction, it requires weekly IV infusions and is associated with the risks and limitations of being a human plasma-derived product. As such, there exists a need for novel lung-targeted therapeutics capable of delivering functional AAT to the lungs. There are an estimated 90,000 to 100,000 people in the US with severe AAT deficiency.

KB408 is an inhaled (nebulized) formulation of our novel vector designed to deliver two copies of the *SERPINA1* gene, that encodes for normal human AAT protein, for the treatment of AATD.

***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 KB408, and Krystal’s plans for filing of regulatory approvals and efforts to bring KB408 to market; the market opportunity for and the potential market acceptance of 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 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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