Krystal Biotech Announces Orphan Drug Designation Granted to KB408 for the Treatment of Alpha-1 Antitrypsin Deficiency

September 5, 2023

- ***KB408 IND filed on August 15, 2023***

PITTSBURGH, Sept. 05, 2023 (GLOBE NEWSWIRE) -- Krystal Biotech, Inc. (the “Company”) (NASDAQ: KRYS), a commercial-stage biotechnology company focused on the discovery, development and commercialization of genetic medicines to treat diseases with high unmet medical needs, announced today that the U.S. Food and Drug Administration (FDA) granted Orphan Drug Designation for KB408 for the treatment of alpha-1 antitrypsin deficiency (AATD).

AATD is caused by mutations in the *SERPINA1* gene that lead to decreased levels and/or decreased functionality of alpha-1 antitrypsin protein. 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. In severe cases, current disease management includes intravenous augmentation therapy which requires weekly infusions, the clinical benefit of which remains to be established.

KB408 is an inhaled (nebulized) formulation of the Company’s novel replication-defective, non-integrating HSV-1-based vector designed to deliver two copies of the *SERPINA1* transgene, that encodes for human alpha-1 antitrypsin protein, for the treatment of AATD.

"This important designation is a milestone in the advancement of KB408, and this decision by the FDA underscores the need for potential new treatment options for patients with AATD," said Suma Krishnan, President, Research & Development, Krystal Biotech, Inc. "We are encouraged by the profile of KB408 in preclinical studies to date and look forward to dosing patients once we receive clearance from the FDA."

About Orphan Drug Designation
Orphan Drug Designation is granted by the FDA to investigational therapies addressing rare medical diseases or conditions that affect fewer than 200,000 people in the U.S. Orphan drug status provides benefits to drug developers, including assistance in the drug development process, tax credits for clinical costs, exemptions from certain FDA fees and seven years of post-approval marketing exclusivity.

About Krystal Biotech, Inc.
Krystal Biotech, Inc. (NASDAQ: KRYS) is a commercial-stage biotechnology company focused on the discovery, development and commercialization of genetic medicines to treat diseases with high unmet medical needs. VYJUVEK™ is the Company’s first commercial product, the first-ever redosable gene therapy, and the only medicine approved by the FDA 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 Twitter.

Forward Looking Statements
Any statements in this press release about future expectations, plans and prospects for Krystal Biotech, Inc., including statements about the expected timing of dosing patients with KB408, 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: uncertainties associated with regulatory review of clinical trials and applications for marketing approvals, the availability or commercial potential of product candidates, 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 the Company’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 the Company’s views as of the date of this release. The Company anticipates that subsequent events and developments will cause its views to change. However, while the Company 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 the Company’s views as of any date subsequent to the date of this release.

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