



## Krystal Biotech Announces Positive Interim Clinical Update from KB407 Phase 1 CORAL-1 Study with Confirmation of Wild-Type CFTR Delivery to the Lungs of Patients with Cystic Fibrosis

January 8, 2026

*Confirmed wild-type CFTR delivery and expression in conducting airway cells of patients with class I mutations*

*KB407 transduction confirmed in all six patients with successful bronchoscopies irrespective of modulator-status; percentage of conducting airway cells transduced in each patient ranged from 29.4% to 42.1%*

*Registrational repeat dosing CORAL-3 study design submitted to FDA in late December; anticipating enrollment in study to start in 1H 2026 following alignment with the FDA*

*Investor call to be held January 8 at 4:30 pm ET to discuss data update and timelines for KB407 repeat dosing study start*

PITTSBURGH, Jan. 08, 2026 (GLOBE NEWSWIRE) -- [Krystal Biotech, Inc.](https://www.krystalbiotech.com) (the "Company") (NASDAQ: KRYS) today announced a positive interim clinical update from the highest dose cohort of CORAL-1, the Company's multi-center, dose escalation Phase 1 study evaluating KB407 in patients with cystic fibrosis (CF), confirming the successful lung delivery and expression of wild-type cystic fibrosis transmembrane conductance regulator (CFTR) protein following inhaled administration of KB407.

"Molecular confirmation of delivery and expression of unmodified, wild-type CFTR protein in clinically relevant ciliated and secretory cells of the lungs of patients with CF is a tremendous breakthrough and a first for our field," said Jorge Lascano, MD, Professor of Medicine, Associate Director of the Adult Cystic Fibrosis Program, and Director of the Cystic Fibrosis Therapeutics Development Center at the University of Florida. "High rates of KB407 transduction and broad distribution across patient airways irrespective of underlying lung disease, genetic background, or modulator status – combined with the redosability of KB407 and demonstrated functionality of its full-length CFTR payload – underscore the transformative potential of KB407 as a mutation-agnostic therapy for the many patients either ineligible for or underserved by currently available modulators."

The Company will host an investor conference call and webcast today, Thursday, January 8, 2026, at 4:30 pm ET, to discuss the clinical data updates. Investors and the general public can access the live webcast at: <https://www.webcaster5.com/Webcast/Page/3018/53466>. For those unable to listen to the live webcast, an archived version will also be available on the Investors section of the Company's website for at least 30 days.

### CORAL-1 Highest Dose Cohort Interim Results

KB407 is being evaluated in the Company's CORAL-1 study, an open label, multi-center Phase 1 study in patients with CF that includes three dose escalation cohorts evaluating either one, two, or four daily administrations of  $10^9$  PFU of KB407 via inhalation. Additional details of the CORAL-1 study can be found at [www.clinicaltrials.gov](https://www.clinicaltrials.gov) under NCT identifier NCT05504837.

Positive interim safety results from the first two dose escalation cohorts, referred to as Cohorts 1 and 2, were [reported](#) in 4Q 2024. Today's update is focused on safety and molecular findings from patients dosed in the highest dose cohort of CORAL-1, Cohort 3.

As of the January 6, 2026 data cut-off, a total of seven (7) patients have been dosed with KB407 in the highest dose Cohort 3 of CORAL-1. Four of the seven patients were ineligible for modulator therapy. All patients received four daily administrations of  $10^9$  PFU of KB407 via inhalation, followed by a bronchoscopy 24-96 hours after receiving their last dose of KB407. Bronchoscopies for six of the seven patients were successful and yielded biopsies suitable for molecular analysis. At least 28 days of safety follow up data was available for all seven patients dosed with KB407 as of data cut-off.

KB407 transduction was confirmed in all Cohort 3 patients with successful bronchoscopies irrespective of modulator-status and genetic background, with broad airway distribution and transduction as assessed by CFTR or viral marker immunofluorescence, ranging from 29.4% to 42.1% across these six patients. Key molecular findings are summarized below:

#### Modulator Ineligible Patients (n = 4)

Patient Number	1	2	3	4
CFTR Variants	2184delA/W1282X Class I Patient	R553X/M1V Class I Patient	C1210-12T/1408A>G	R334W/R1162X
Baseline ppFEV <sub>1</sub>	64	45	45	69
Total Number of Biopsies Suitable for Analysis	7	5	5	6
Protein Marker Assessed*	CFTR	CFTR	Viral Marker	Viral Marker
Percentage of Conducting Airway Cells Positive for CFTR or Viral Marker**	Overall: 42.1% Range: 33.1%-62.0%	Overall: 29.4% Range: 24.6%-32.3%	Overall: 36.5% Range: 29.1%-44.8%	Overall: 33.8% Range: 29.0%-36.3%

\* Positive cell counts for patients 3 and 4 based on viral marker expression given potential for background endogenous CFTR

\*\* Overall values are based on the combined cell counts across all analyzable biopsies from a given patient while range values reflect cell counts from individual biopsies; conducting airway cells defined as airway-exposed epithelial cells lining the bronchi of the lung

All biopsies suitable for analysis from the four modulator ineligible patients (combined n = 23 biopsies) were positive for CFTR or viral marker expression, indicative of widespread dissemination of KB407 throughout the conducting airways of the lung.

## Modulator Eligible Patients (n = 2)

Patient Number	5	6
CFTR Variants	F508del/F508del	F508del/F508del
Baseline ppFEV <sub>1</sub>	54	59
Total Number of Biopsies Suitable for Analysis	4	4
Percentage of Conducting Airway Cells Positive for Viral Marker*	<b>Overall: 36.8%</b> Range: 28.3%-46.4%	<b>Overall: 31.4%</b> Range: 27.3%-38.0%

\* Overall values are based on the combined cell counts across all analyzable biopsies from a given patient while range values reflect cell counts from individual biopsies; conducting airway cells defined as airway-exposed epithelial cells lining the bronchi of the lung

All biopsies suitable for analysis from the two modulator eligible patients (combined n = 8 biopsies) were positive for viral marker expression.

Consistent with the safety profile previously reported from Cohorts 1 and 2, inhaled KB407 continued to be well tolerated by patients treated with the highest dose in Cohort 3. All but one KB407-related adverse event were mild to moderate in severity and transient in nature. One serious adverse event (SAE) of asthma exacerbation was reported 24 hours after completion of the bronchoscopy. The SAE was deemed procedure related, and not related to KB407, by the independent data monitoring committee. The SAE resolved in 5 days.

"Today's update has profound implications for Krystal and for the many CF patients unable to benefit from modulator therapy," said Suma Krishnan, President, Research & Development, Krystal Biotech, Inc. "With clear evidence of CFTR protein expression in patients with class I mutations and reproducible KB407 transduction across a diverse CF population, we are moving forward with conviction into our repeat dosing study with registrational intent, CORAL-3. We are excited to be working with the Cystic Fibrosis Foundation to accelerate clinical development and potential registrational timelines."

The Company submitted the CORAL-3 study design to the United States Food and Drug Administration (FDA) in late December. CORAL-3 is designed to evaluate the safety and efficacy of repeat KB407 administration, including regular assessments of lung function by spirometry, and support potential registration. The Company expects to align on the CORAL-3 study design with the FDA in 1Q 2026 and start enrollment in CORAL-3 in 2Q 2026. Additional details on the study design will be provided by the time of study initiation.

### About CF

CF is an inherited disease caused by genetic mutations that result in dysfunctional or absent CFTR protein. Lack of functional CFTR causes dehydrated mucus buildup in the lungs, pancreas, and other organs. This mucus buildup in the lungs leads to loss of lung function, and eventually, respiratory failure. According to the US Cystic Fibrosis Foundation, close to 40,000 children and adults are living with CF in the United States, and an estimated 105,000 are diagnosed with CF across 94 countries. Although CFTR modulators are effective in patients with certain CFTR mutations, patients may still experience pulmonary symptoms requiring treatment. Further, a meaningful proportion of CF patients harbor genetic mutations that are not expected to be responsive to modulators and currently have no available disease-modifying treatment options, representing a significant unmet need.

### About KB407

KB407 is a redosable gene therapy designed to deliver two copies of the full-length *CFTR* transgene to the lung via inhalation for the treatment of CF. By enabling expression of full-length, wild-type CFTR protein in the lung, treatment with KB407 has potential to restore CFTR-mediated ion transport, mucus clearance, and lung function in patients with CF regardless of their underlying genetic mutation. KB407 has been shown to successfully transduce patient-derived epithelial cells and deliver functional CFTR in 2D and 3D *in vitro* organotypic systems and, as demonstrated in the CORAL-1 Phase 1 study, is amendable to inhaled administration via nebulization, with successful lung delivery and broad airway distribution confirmed in multiple patients with cystic fibrosis.

### About Krystal Biotech, Inc.

Krystal Biotech, Inc. (NASDAQ: KRY5) 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 its product candidate, KB407 for the treatment of patients with cystic fibrosis. These forward-looking statements include, without limitation, statements relating to the potentially transformative potential of KB407 as a mutation-agnostic therapy; and the Company's planned study, CORAL-3, that is designed to evaluate the safety and efficacy of repeat KB407 administration, including expected alignment on the CORAL-3 study design with the FDA in 1Q 2026 and initiation of enrollment in CORAL-3 in 2Q 2026. 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, including uncertainties inherent in the initiation and conduct of clinical trials, regulatory review of clinical trials, and applications for marketing approvals; whether results of early clinical trials will be indicative of the results of later-stage studies; 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. The Company provides this information as of the date of this press release and assumes no obligation to update any forward-looking statements.

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