Krystal®

KB407 for Cystic Fibrosis
CORAL-1 Clinical Data Update

January 2026

Forward Looking Statements and Disclosures

This presentation and our discussion contain forward-looking statements that involve substantial risks and uncertainties. Any statements about future expectations, plans, and prospects for Krystal Biotech, Inc. (the "Company"), including but not limited to statements about the Company's investigational product candidate, KB407, and the CORAL-1 clinical trial evaluating KB407 for the treatment of cystic fibrosis (CF); the potential transformational implications from the positive interim clinical update from the highest dose cohort (Cohort 3) of CORAL-1, including a leadership opportunity in the treatment of CF, further validation of the Company's lung platform, derisking of KB408 and KB707, support for pipeline expansion, and potential blockbuster opportunities; the Company's planned CORAL-3 study designed to evaluate the safety and efficacy of repeat KB407 administration, including the timing of expected alignment on the CORAL-3 study design with the FDA and initiation of the study, and the potential for CORAL-3 to support registration of KB407; and other statements, 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 reviews and the content and timing of regulatory authorities' decisions; uncertainties in the initiation and conduct of clinical trials and availability and timing of data from clinical trials; whether results of early clinical trials will be indicative of the results of later-stage studies; the availability or commercial potential of product candidates; and such other important factors as are set forth in the Company's filings with the SEC. The forward-looking statements represent the Company's views as of the date of this presentation and should not be relied upon as representing the Company's views as of any subsequent date. The Company specifically disclaims any obligation to update forward-looking statements.

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Today's discussions and presentation are intended for the investor community only; they are not intended to promote the products or product candidates referenced herein or otherwise influence healthcare prescribing decisions.

The Company is using the Aerogen Solo® Nebulizer System and Aerogen® Ultra in its clinical trials evaluating KB407, KB408, and inhaled KB707

Agenda

Opening Remarks
Krish Krishnan; Chairman and CEO

Treatment Gap for Modulator-Ineligible Patients with Cystic Fibrosis
Jorge Lascano, MD; Associate Director of Adult Cystic Fibrosis Program, University of Florida

KB407 Program Overview
Suma Krishnan, MS, MBA; President, Research & Development

KB407 CORAL-1 Phase 1 Highest Dose Cohort Results
David Sweet, MD, PhD; Director, Clinical Development

KB407 Next Steps and Clinical Development Outlook
Suma Krishnan, MS, MBA; President, Research & Development

Closing Remarks
Krish Krishnan; Chairman and CEO

Q&A
All Speakers and Trevor Parry, PhD; Vice President, Product Development

Today's Readout Has Transformational Implications for Krystal

Leadership Opportunity in Cystic Fibrosis

- KB407 is now the **first** gene therapy with molecular confirmation of wild-type CFTR protein expression in the lungs of patients with cystic fibrosis
- Functionality of wild-type payload confirmed in multiple models
- Strong engagement with CFF TDN to progress to repeat dosing study with longitudinal ppFEV$_1$ assessment, start expected **in 1H 2026**

10K Modulator Ineligible CF Patients

20K CF Patients with Suboptimal Modulator Responses

$2B+

Market Opportunity in Modulator Ineligible or Refractory CF

+ Lung Platform Validation

- Lung gene delivery in dozens of patients across multiple disease states including heavily obstructed airways
- Today's data further derisks KB408 and KB707 and supports pipeline expansion
- Multiple blockbuster opportunities under evaluation, rare and larger indications

CF, cystic fibrosis; CFF, Cystic Fibrosis Foundation; CFTR, cystic fibrosis transmembrane conductance regulator; ppFEV$_1$, percent predicted forced expiratory volume in 1 second; TDN, Therapeutics Development Network

Cystic Fibrosis Beyond Modulators: Persistent Unmet Need

Jorge Lascano, MD

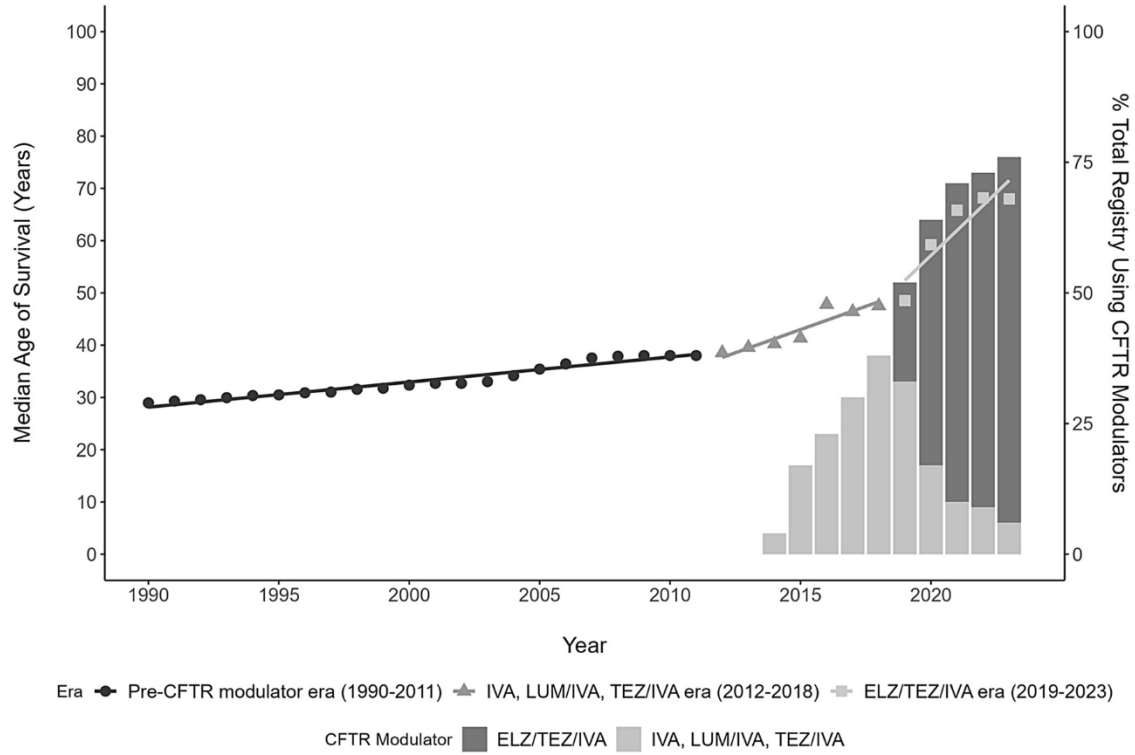
Professor of Medicine
Associate Director of the Adult Cystic Fibrosis Program
Director of the Cystic Fibrosis Therapeutics Development Center
University of Florida
CORAL-1 Investigator

UFHealth
UNIVERSITY OF FLORIDA HEALTH

The Modulator Era: A Major Advance

Modulator therapy has revolutionized care for people with CF (pwCF) who have mutations that are eligible for treatment:

- Improved pulmonary function (ppFEV1)[1]
- Decreased pulmonary exacerbations[2]
- Improved quality-of-life indices[1]
- Increased life expectancy[3]



A similar treatment revolution is needed for the remaining CF population who are not eligible for, do not tolerate, or do not benefit from modulatory therapy

[1]Wang et al. (2022); [2]Sutharsan et al. (2023); [3]Rubin et al. (2025); ppFEV1, percent predicted forced expiratory volume in 1 second

Who Is Left Behind?

Unresponsive genotype: Mutations which result in no mRNA or functional protein produced (*e.g.,* class 1 mutations)

Poor response: 'Modulator-refractory' individuals exist due to extensive heterogeneity in CF populations (*e.g.* non-CFTR genetic variants, variable immune response, comorbid conditions)

Poor tolerability: Side effects and drug-drug interactions can prove intolerable for some patients

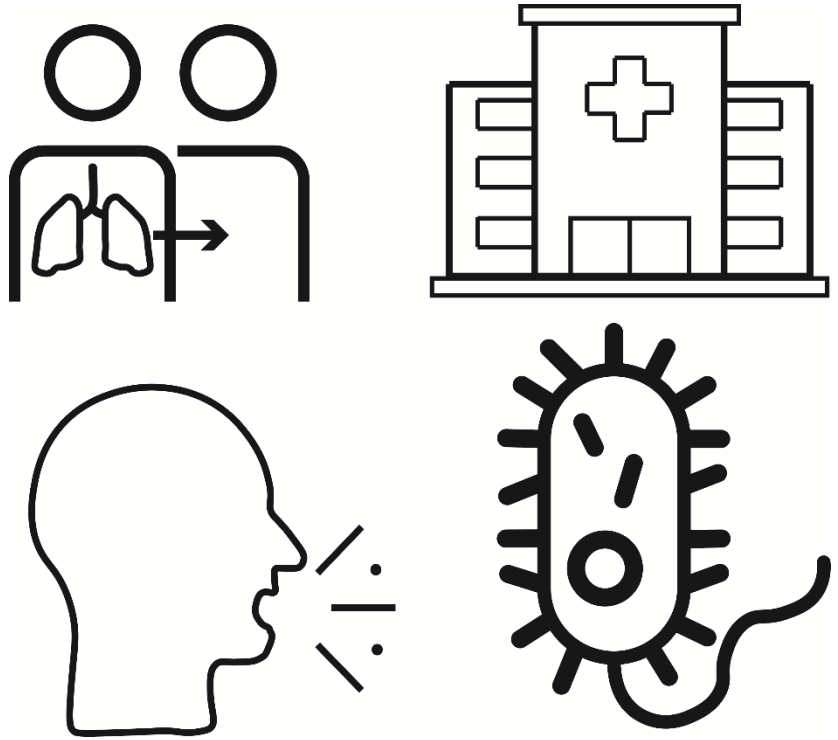
Access issues: Even those with mutations that are potentially responsive to modulator therapy are often unable to access therapy due to challenges with testing and diagnosis on nontraditional populations, understanding the functionality of uncommon mutations as well as drug availability.

10+% are modulator ineligible, with thousands more with suboptimal responses to modulator therapy

Somerville et al. (2024)

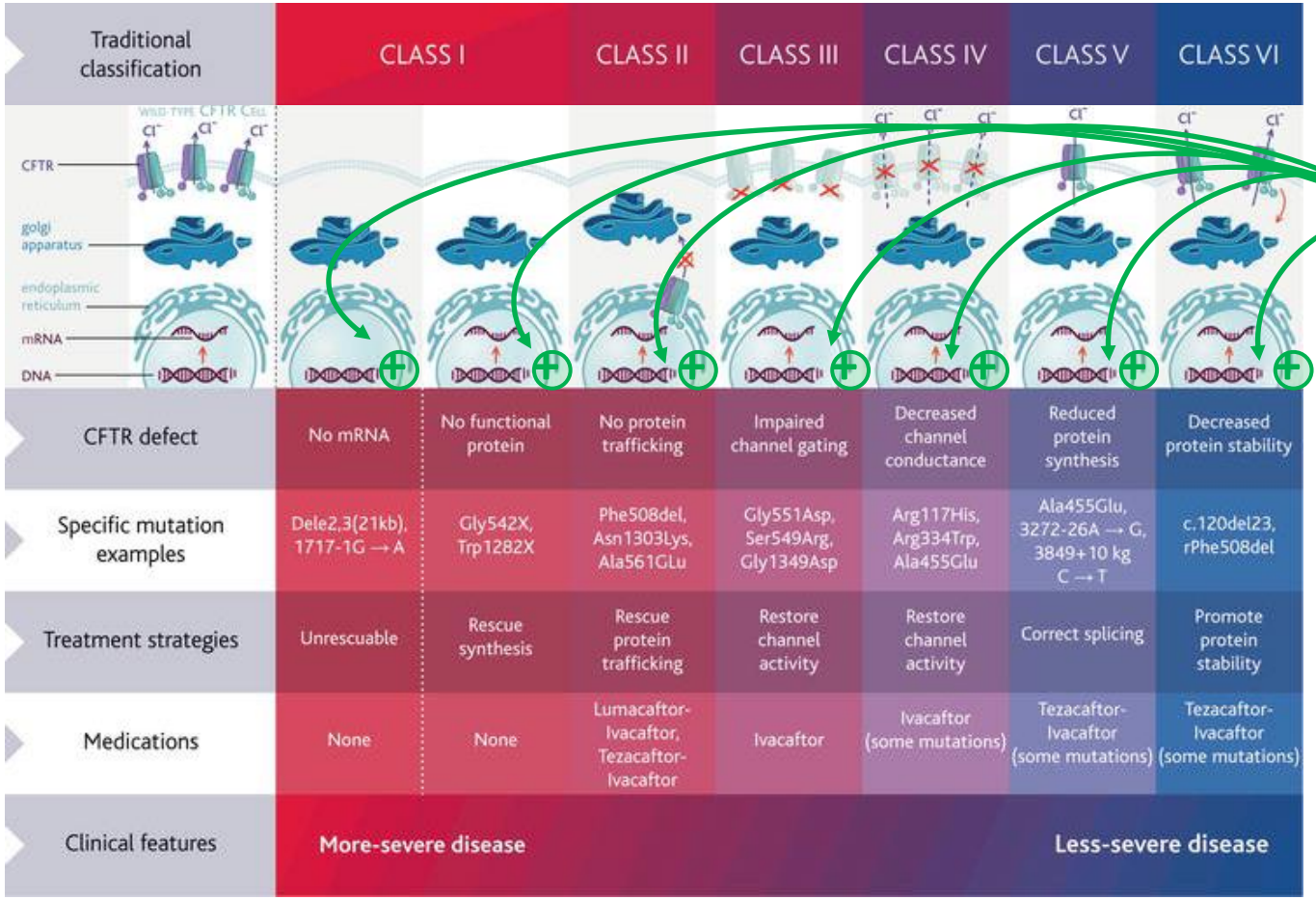
Clinical Burden in the Non-Modulator Population

- Ongoing **pulmonary function decline**
- High exacerbation burden
- Continued need for **intensive daily therapy**
- Substantial negative influence on quality-of-life; **patients can feel left behind** compared to those who benefit from modulator therapy
- More likely to **require lung transplantation**[1]
- Even when compared to those who are otherwise eligible but not on modulator therapy, patients that are not eligible have worse clinical outcomes[2]
  - Underscoring critical need for novel therapeutics for this population in particular



[1]*Clancy and Hamblett (2023);* [2]*Buyuksahin et al. (2024)*

Why Nucleic Acid-Based Therapies Are Urgently Needed



Gene replacement therapies offer a *mutation-agnostic strategy* to target CF, regardless of modulator status

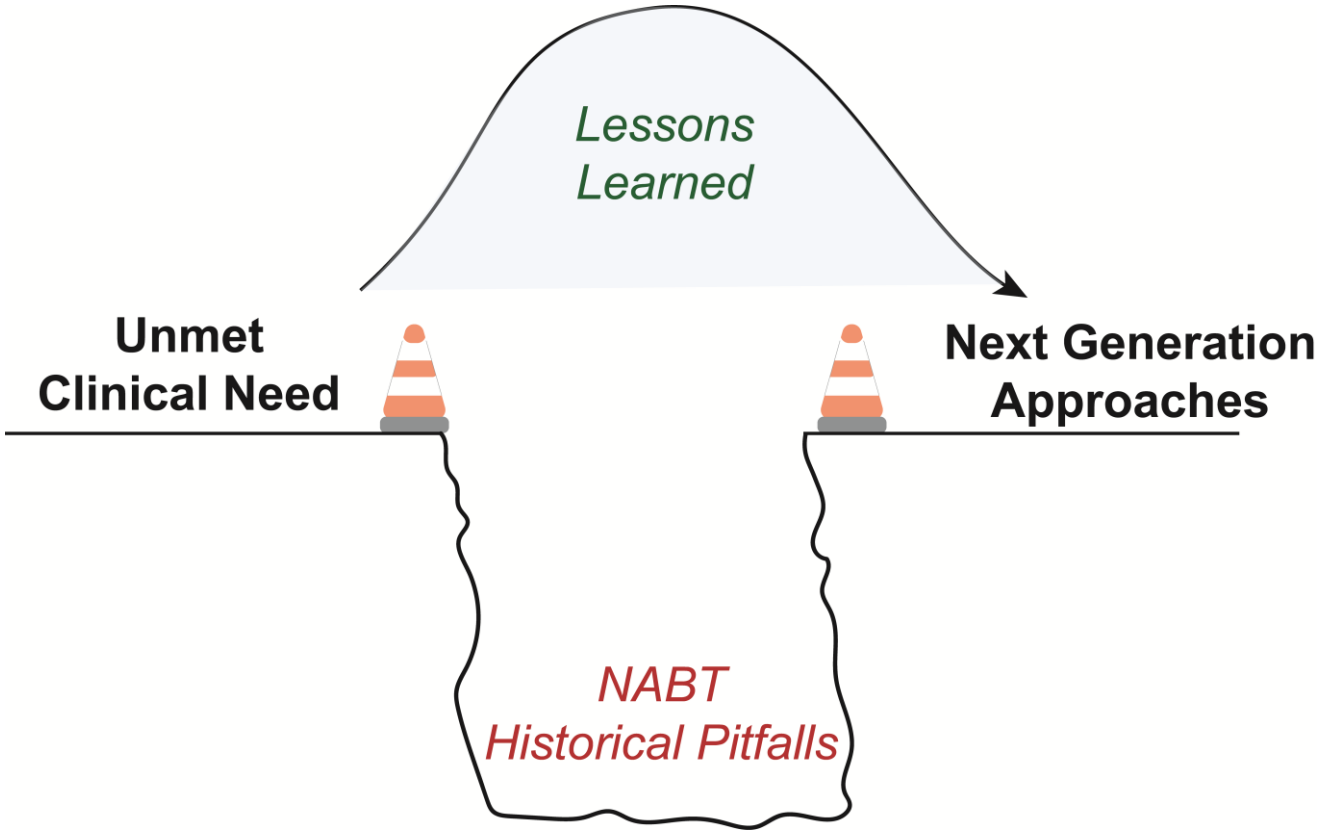
Adapted from Rang, Kotsimbos, and Wilson (2020)

Lessons from Prior Nucleic Acid-Based Efforts

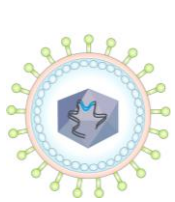
Concept	Pitfalls faced in previous efforts	Idealized quality of future NABT
Delivery & Stability	- Nebulization through thick and infected airway leads to poor delivery - Degradation of less-stable NABTs within the airway - Some delivery vectors require subtype optimization to reach target cells (*i.e.,* cell-type tropism) - Low transduction/transfection efficiency seen with many viral and non-viral approaches	**Stable** vector (viral or non-viral) capable of expressing CFTR in **5-15% of target respiratory epithelial cells**[1] in CF lungs (*i.e.,* **lungs experiencing infection**, **inflammation**, and significant **mucus** burden)
Payload	- Large size of *CFTR* gene requires gene truncation with some approaches, potentially impacting function	Able to deliver **full-length CFTR** DNA or stable mRNA
Immunogenicity	- Neutralizing immunity can lead to reduced efficacy with repeat-dosing, ultimately impacting durability of effect	Able to **repeatedly dose** without significant neutralizing immunity

[1]Thought to be enough to restore Cl- channel function to non-CF levels; NABT, nucleic acid-based therapy

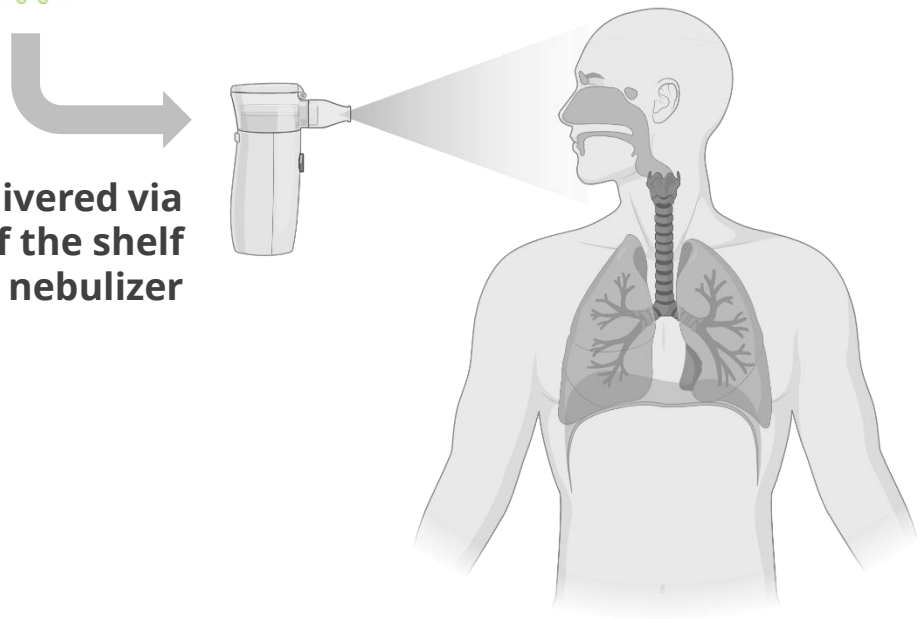
Looking Forward



Krystal's Redosable HSV-1 Platform for Lung Gene Delivery



Krystal's Proprietary HSV-1 Vectors



Delivered via off the shelf nebulizer

Historical Challenges with Inhaled Gene Therapy[1]

- Inhaled gene therapy has been explored for decades, with little success
- Focus to date has been on adenovirus, AAV, and non-viral approaches
- Multiple challenges including cargo limitations, low efficiency of gene transfer, toxicity, product instability, and burdensome delivery

HSV-1 Platform Has Potential to Overcome Historical Challenges

- Clinically validated vector; tolerated and redosable in Phase 3 for DEB
- Large cargo capacity to load in full genes, including *CFTR* for cystic fibrosis
- Ability to redose and/or adjust dose over time as lung cells turnover
- Broad cellular tropism and efficient transduction of airway epithelium
- Short expected nebulization time using off-the-shelf nebulizer

Lung delivery with HSV-1 already demonstrated with two inhaled programs: KB408 for alpha-1 antitrypsin deficiency and KB707 for lung cancer

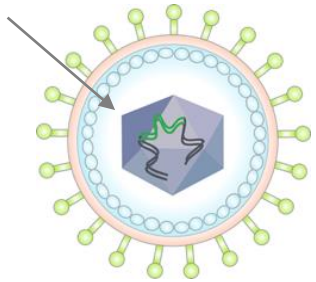
1. Vu A, et al. *Human Gene Therapy* 2020;31(17-18):921-939

AAV, adeno-associated virus; CFTR, cystic fibrosis transmembrane conductance regulator; DEB, dystrophic epidermolysis bullosa; HSV-1, herpes simplex virus 1

Inhaled Candidate KB407 for Cystic Fibrosis

KB407

2 x *CFTR* genes



Replication-incompetent HSV-1 vector containing functional human *CFTR*

Preclinical Summary

- ✓ **Cellular Tropism:** KB407 efficiently transduces human primary airway epithelial cells leading to dose dependent CFTR expression
- ✓ **Full-Length Payload:** CFTR protein expressed in KB407 transduced cells is full-length, properly localized, and glycosylated
- ✓ **Functionality:** Encoded CFTR has shown functionality in both *in vitro* CF patient model and *in vivo* rodent model
- ✓ **Tolerability:** KB407 well tolerated in multiple preclinical studies including in GLP IND-enabling repeat dose toxicology study in NHPs
- ✓ **Broad and Sustained *In Vivo* Expression:** KB407 well disseminated throughout NHP lungs via inhalation and human *CFTR* detected at least 28 days after last dose

Strong preclinical support for clinical evaluation;
KB407 Phase 1 CORAL-1 study sanctioned by CFF TDN last year

CF, cystic fibrosis; CFF, Cystic Fibrosis Foundation; CFTR, cystic fibrosis transmembrane conductance regulator; GLP, good laboratory practices; HSV-1, herpes simplex virus 1; IND, investigational new drug; NHP, non-human primate; TDN, Therapeutic Development Network

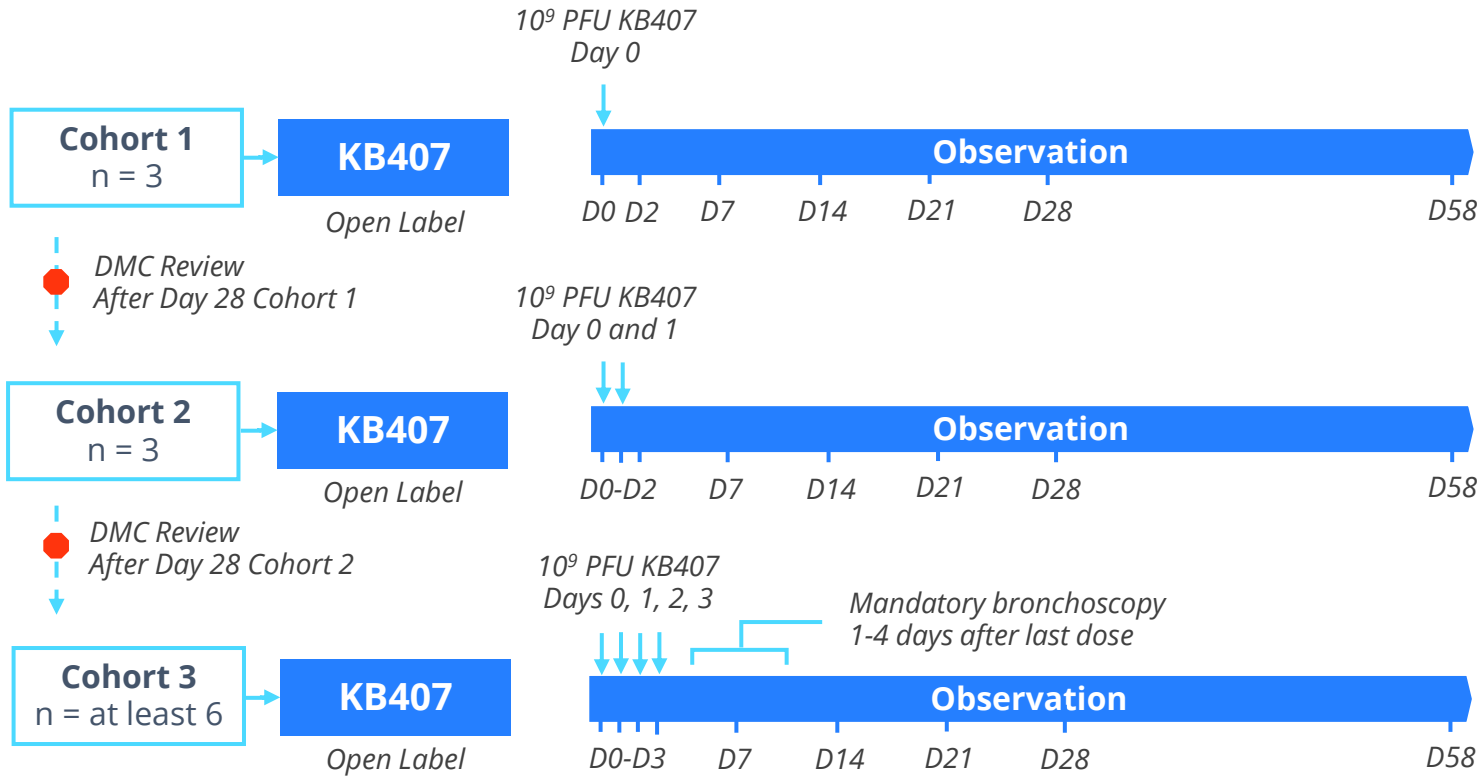
KB407 Phase 1 Study CORAL-1

Study Objectives

- Evaluate safety and tolerability of **ascending doses** of nebulized KB407, as well as preliminary efficacy evaluation
- Assessment of KB407 transduction and CFTR transgene expression in lung (bronchoscopy sub-study only)
- Vector shedding and biodistribution will also be assessed in blood, urine, buccal, and sputum samples

Key Enrollment Criteria

- Age ≥ 18 years with confirmed diagnosis of CF
- $ppFEV_1$ ≥40% and ≤100%
- Resting O_2 saturation ≥92% on room air
- **Cohort 1 and 2:** Participants may receive concurrent modulator therapy, bronchoscopy optional
- **Cohort 3:** No more than 3 participants may be on concurrent modulator therapy, bronchoscopy mandatory



Interim safety update for Cohorts 1 and 2 provided in 4Q 2024

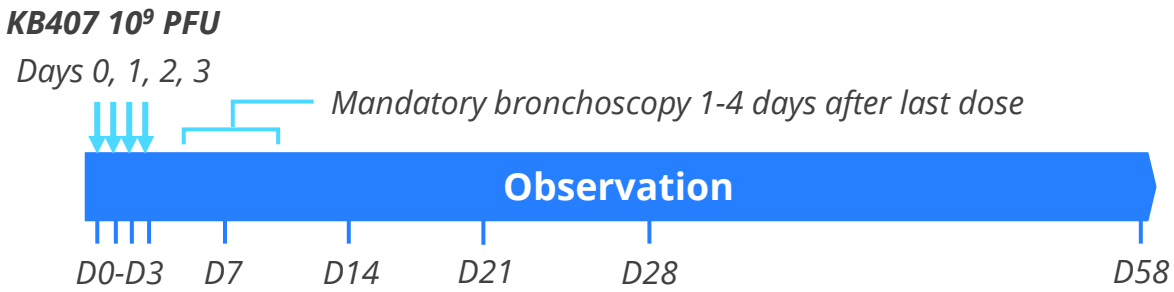
Today's update focuses on safety and molecular data for Cohort 3

CF, cystic fibrosis, CFTR, cystic fibrosis transmembrane conductance regulator; DMC, data monitoring committee; PFU, plaque forming unit; $ppFEV_1$, percent predicted forced expiratory volume in 1 second

CORAL-1 Cohort 3 Patient Demographics

Safety and Molecular Assessments

Cohort 3
Four 10^9 PFU Doses
n = at least 6



Modulator Eligibility	Patient ID	*CFTR* Genotype	Age	Sex	Baseline $ppFEV_1$	Modulator Therapy	Successful Bronchoscopy
Ineligible	03-01	2184delA/W1282X	30	Male	64	No	**Yes**
	03-02	R553X/M1V	34	Male	45	No	**Yes**
	03-03	C1210-12T/1408A>G	67	Female	45	No	**Yes**
	03-04	R334W/R1162X	34	Male	69	No	**Yes**
Eligible	03-05	F508del/F508del	33	Male	54	**Yes**	**Yes**
	03-06	F508del/F508del	39	Female	59	**Yes**	**Yes**
	03-07	G542X/R1066H	24	Female	82	**Yes**	No

Data cutoff date of January 6, 2026

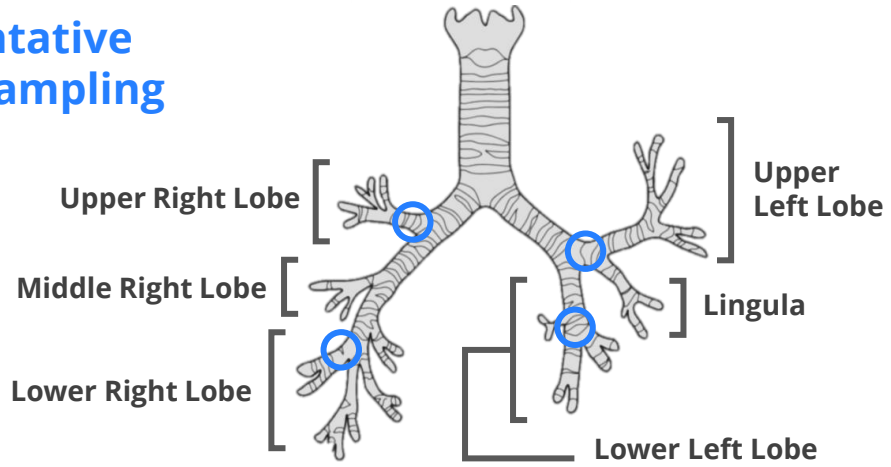
CFTR, cystic fibrosis transmembrane conductance regulator; PFU, plaque forming unit; $ppFEV_1$, percent predicted forced expiratory volume in 1 second

Scope of Today's Molecular Data Readout

Bronchoscopy Overview

- Endoscopic medical procedure to collect biopsies from conducting airways of the lungs of CF patients
- Biopsies typically collected at major junctions of the bronchi with goal of sampling multiple lung lobes
- Target of at least four samples if tolerated by patient
- Conducted 24-96 hours after last KB407 dose

Representative Airway Sampling



Immunofluorescence Analyses Conducted on Biopsies

- Biopsies were assessed for expression of CFTR or a viral marker of KB407 transduction
- When available a limited number of tissue samples were also co-stained for airway cell type markers

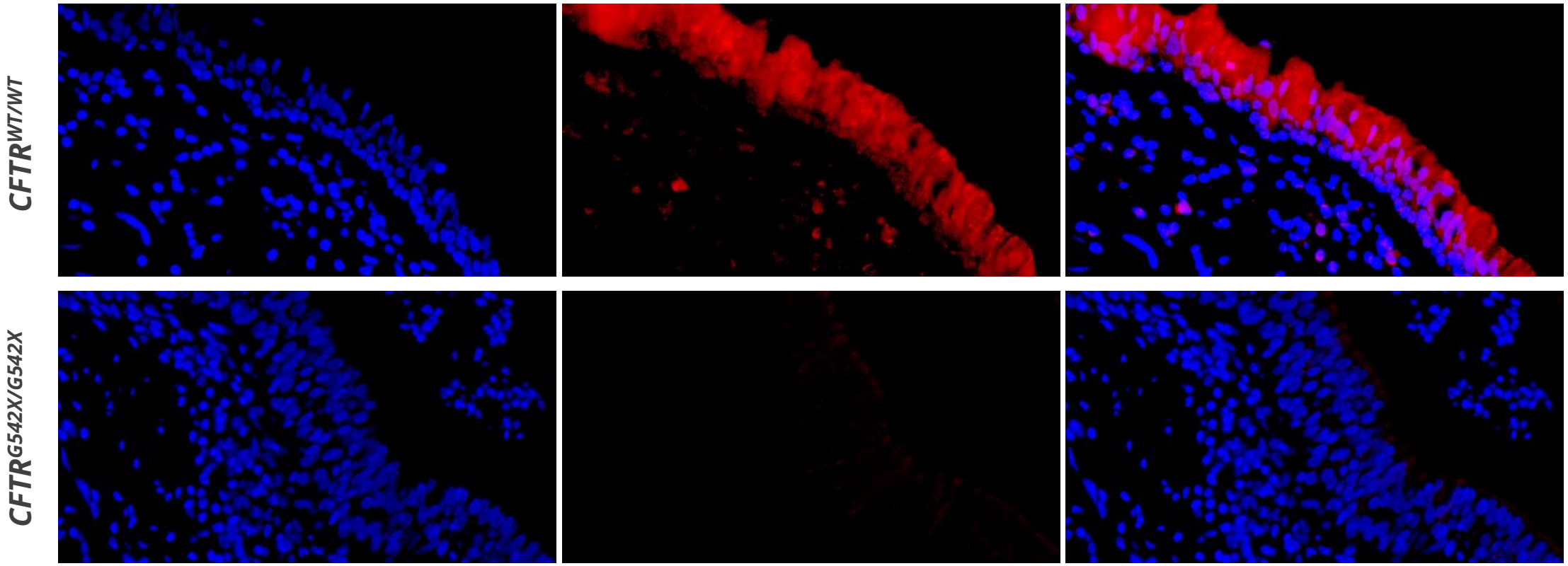
Key Molecular Data Outputs

1. **Percentage and distribution of conducting airway cells expressing CFTR** – ***patients with class I mutations***
2. **Percentage and distribution of conducting airway cells expressing viral marker** – ***all remaining patients***

CF, cystic fibrosis; CFTR, cystic fibrosis transmembrane conductance regulator

CFF Validated Protocol Used for CFTR Assessments by Immunofluorescence

Representative Control Data



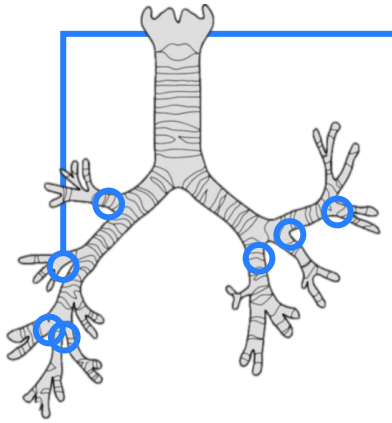
All imaging conducted at 40× magnification; CFTR staining using ab596
Null patient negative control biopsy provided by CFF TDN, positive control biopsy procured commercially
Control samples run with each immunofluorescence assessment of patient biopsies

CFF, Cystic Fibrosis Foundation; CFTR, cystic fibrosis transmembrane conductance regulator; TDN, Therapeutics Development Network

Clear Evidence of CFTR Delivery and Expression in Lungs of Class I CF Patient

CFTR expression results for patient 03-01 with *CFTR* genotype 2184delA/W1282X

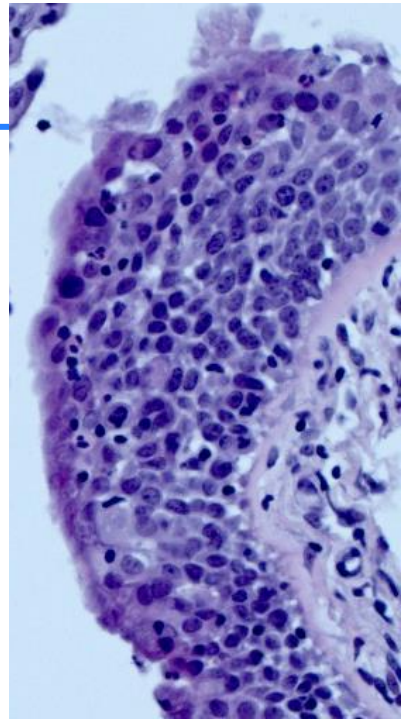
Patient 03-01
Airway Sampling



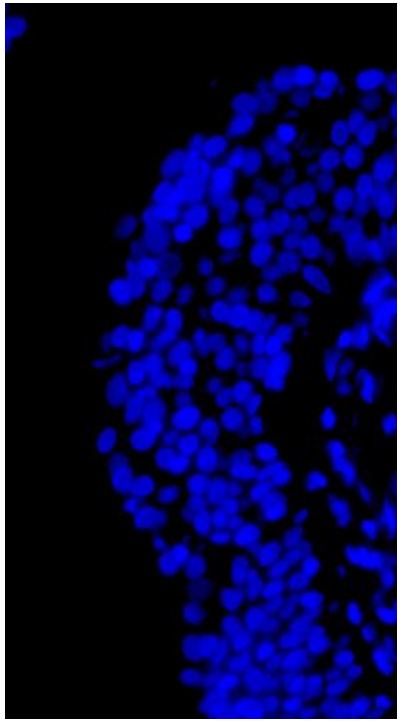
42.1%

CFTR Positive Cells*

+ all seven biopsies positive for CFTR

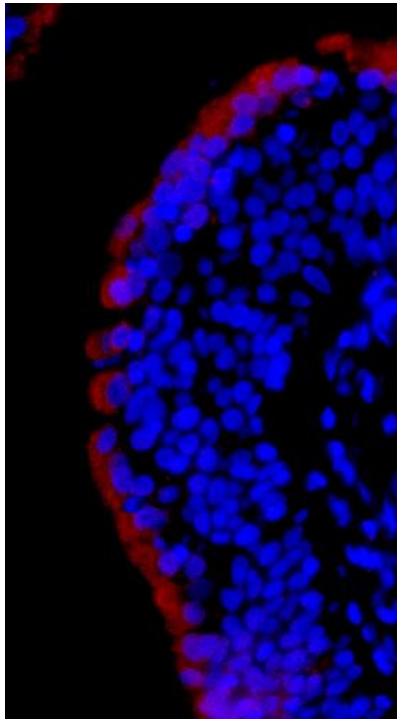


H&E



DAPI

CFTR



Merge

* Based on quantification of DAPI positive and DAPI + CFTR co-positive cells lining the conducting airways of the lung by immunofluorescence, total cell counts > 650

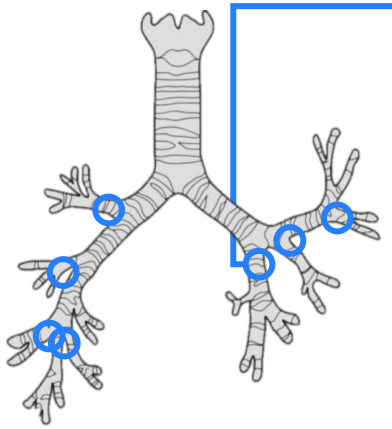
CFTR, cystic fibrosis transmembrane conductance regulator; DAPI, 4',6-diamidino-2-phenylindole; H&E, hematoxylin and eosin

All imaging conducted at 40× magnification
Post-dose biopsies harvested 96 hours after nebulization
CFTR staining using ab596

Clear Evidence of CFTR Delivery and Expression in Lungs of Class I CF Patient

CFTR expression results for patient 03-01 with *CFTR* genotype 2184delA/W1282X

Patient 03-01
Airway Sampling



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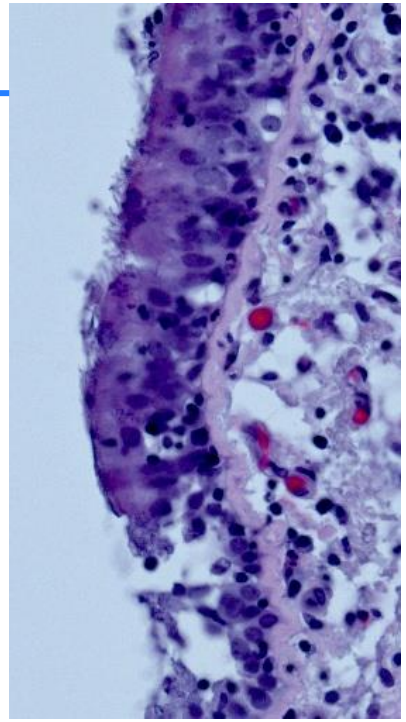
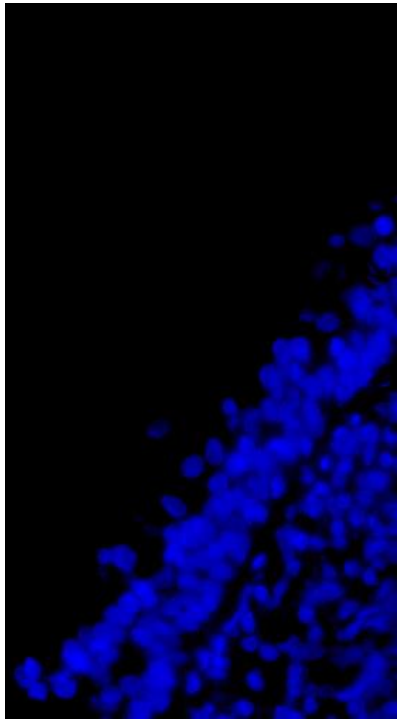


H&E



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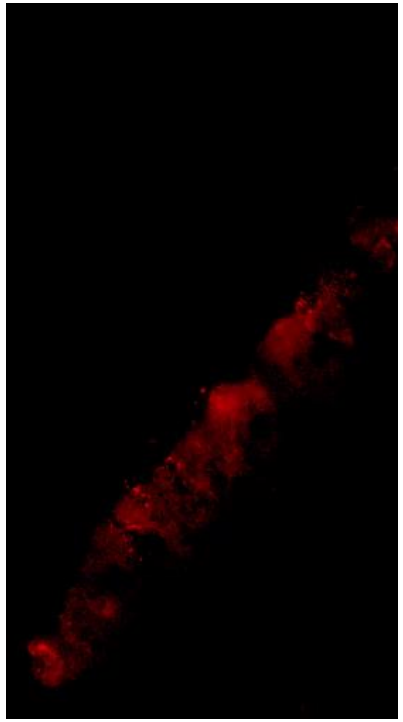
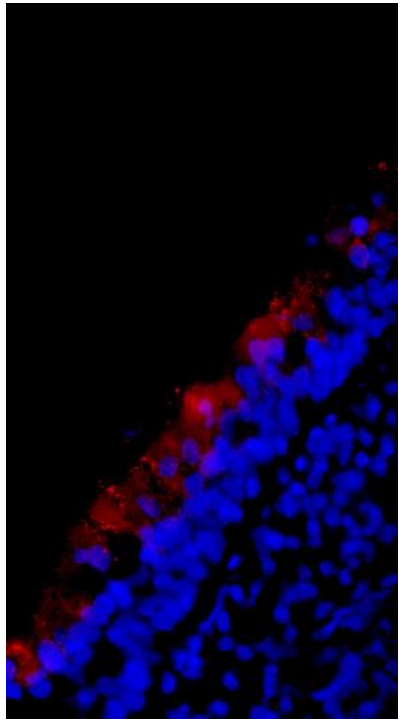


CFTR



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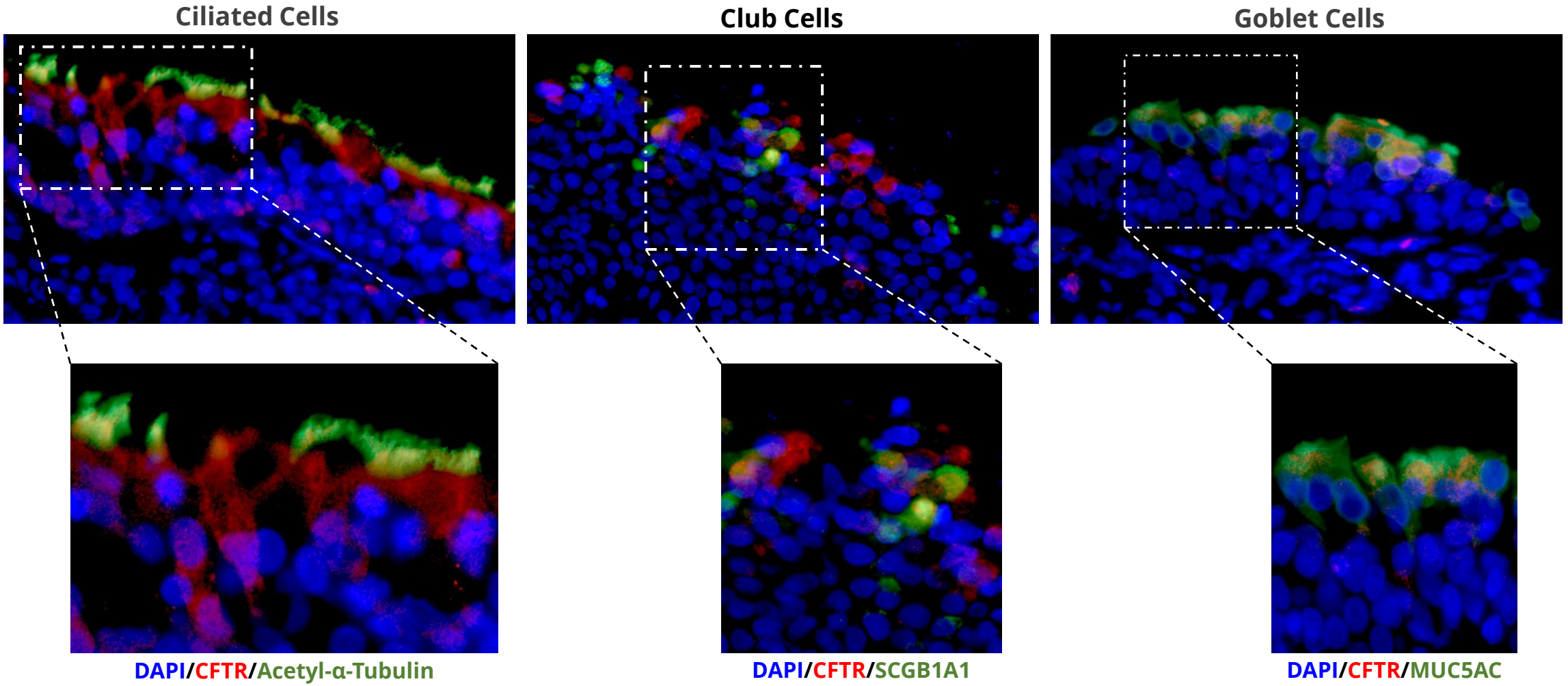
All imaging conducted at 40× magnification
Post-dose biopsies harvested 96 hours after nebulization
CFTR staining using ab596

CFTR Expression in All Relevant Conducting Airway Cell Types

CFTR expression results for patient 03-01 with *CFTR* genotype 2184delA/W1282X



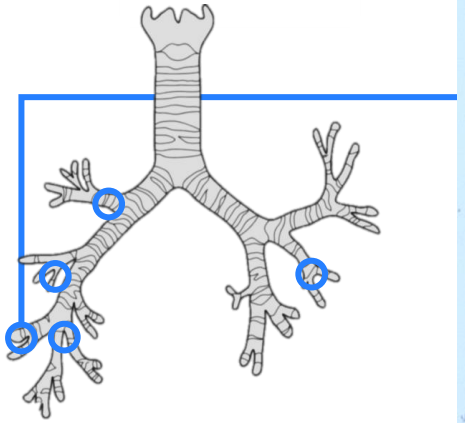
All imaging conducted at 40× magnification
Post-dose biopsies harvested 96 hours after nebulization
CFTR staining using ab596; acetyl-α-tubulin staining using PA5-105102 (Invitrogen)
SCGB1A1 staining using PAS-95864; MUC5AC staining using ab78660 (abcam)

CFTR, cystic fibrosis transmembrane conductance regulator; DAPI, 4',6-diamidino-2-phenylindole; MUC5AC, mucin 5AC; SCGB1A1, uteroglobin

Wild-Type CFTR Expression in Lungs of Second Patient with Class I CF

CFTR expression results for patient 03-02 with *CFTR* genotype R553X/M1V

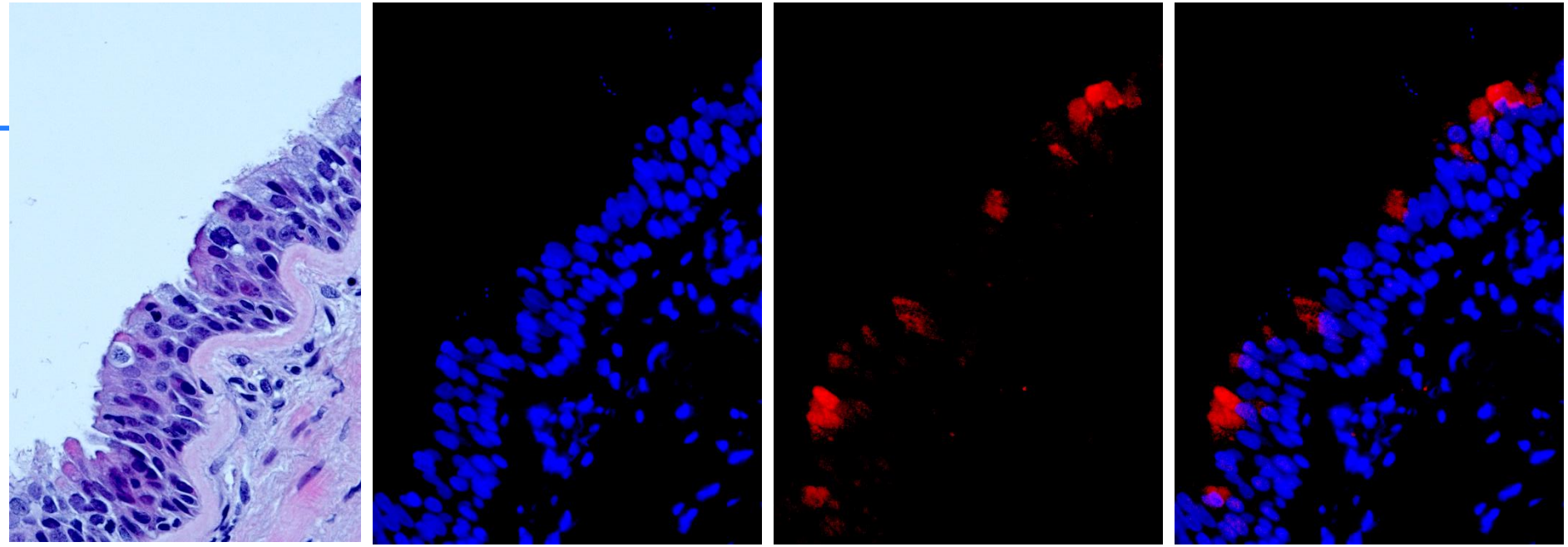
Patient 03-02
Airway Sampling



29.4%

CFTR Positive Cells*

+ all five biopsies positive for CFTR



H&E | **DAPI** | **CFTR** | **Merge**

* Based on quantification of DAPI positive and DAPI + CFTR co-positive cells lining the conducting airways of the lung by immunofluorescence, total cell counts > 400

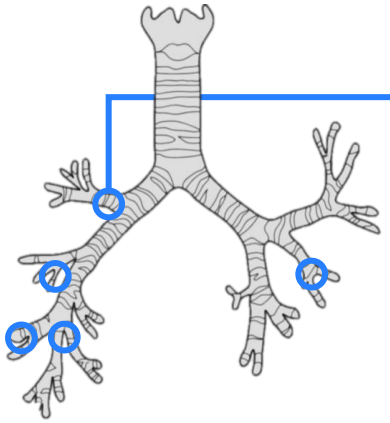
CFTR, cystic fibrosis transmembrane conductance regulator; DAPI, 4',6-diamidino-2-phenylindole; H&E, hematoxylin and eosin

All imaging conducted at 40× magnification
Post-dose biopsies harvested 96 hours after nebulization
CFTR staining using ab596

Wild-Type CFTR Expression in Lungs of Second Patient with Class I CF

CFTR expression results for patient 03-02 with *CFTR* genotype R553X/M1V

Patient 03-02
Airway Sampling



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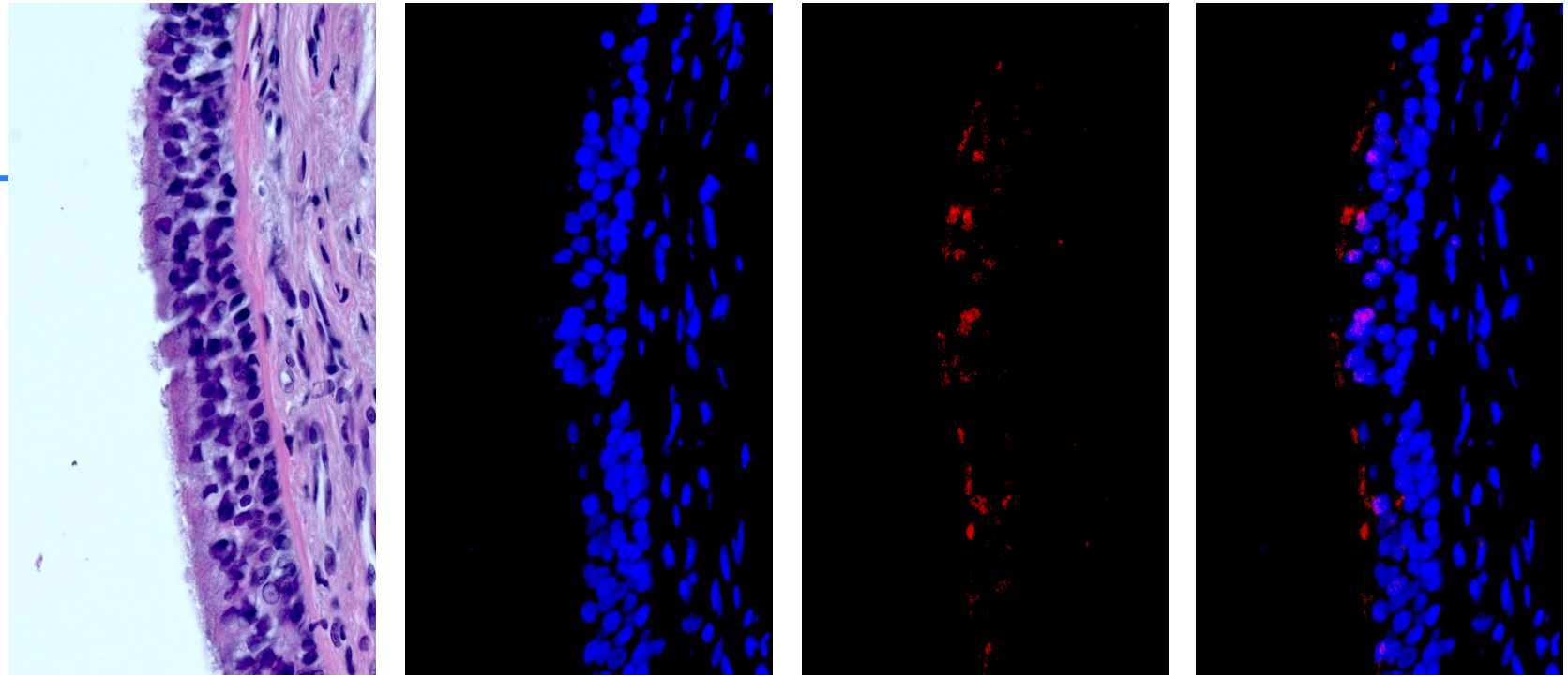


H&E | DAPI | CFTR | Merge

* Based on quantification of DAPI positive and DAPI + CFTR co-positive cells lining the conducting airways of the lung by immunofluorescence, total cell counts > 400

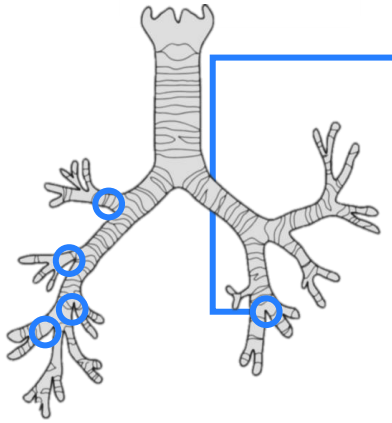
CFTR, cystic fibrosis transmembrane conductance regulator; DAPI, 4',6-diamidino-2-phenylindole; H&E, hematoxylin and eosin

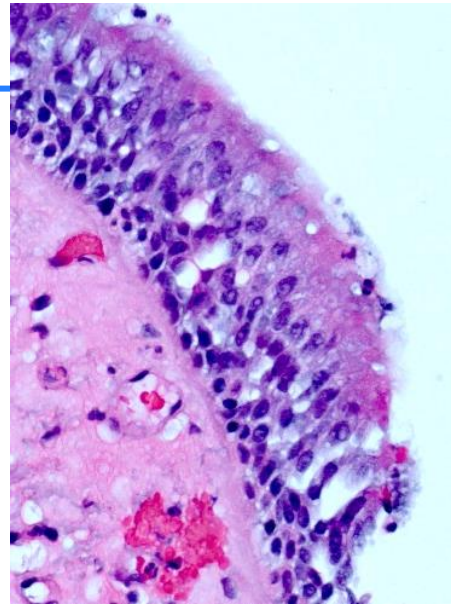
All imaging conducted at 40× magnification
Post-dose biopsies harvested 96 hours after nebulization
CFTR staining using ab596

Confirmed KB407 Transduction by Viral Marker in All Other Patients

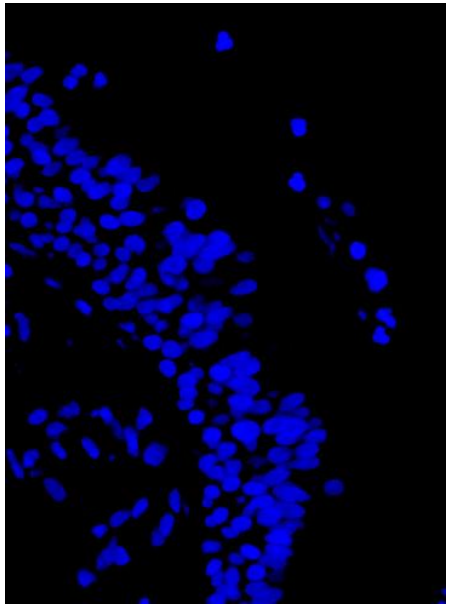
CFTR expression results for patient 03-03 with *CFTR* genotype C1210-12T/1408A>G

Patient 03-03
Airway Sampling

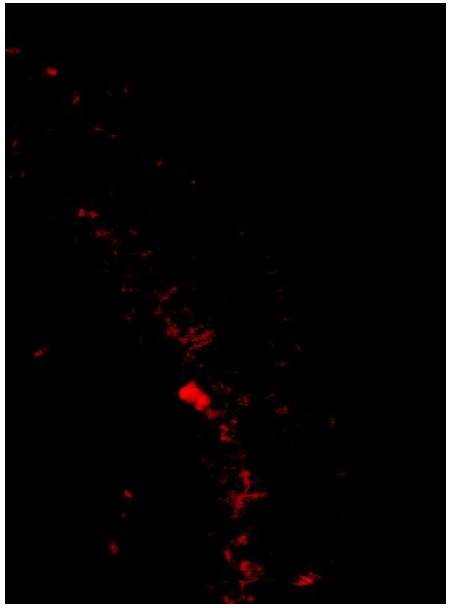




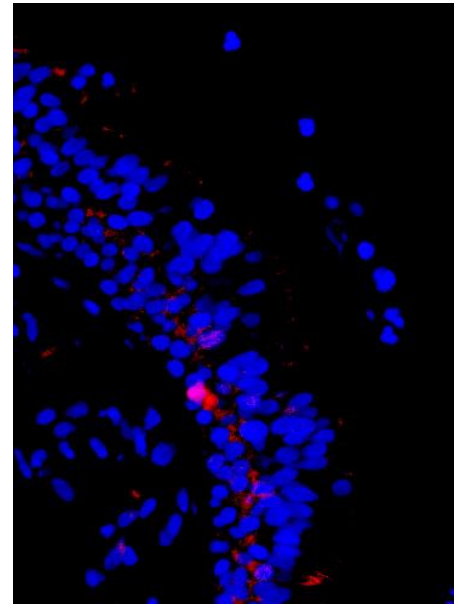
H&E



DAPI



Viral Marker



Merge

36.5%

Viral Marker Positive Cells*

+ all five biopsies positive for marker

* Based on quantification of DAPI positive and DAPI + viral marker co-positive cells lining the conducting airways of the lung by immunofluorescence, total cell counts > 500

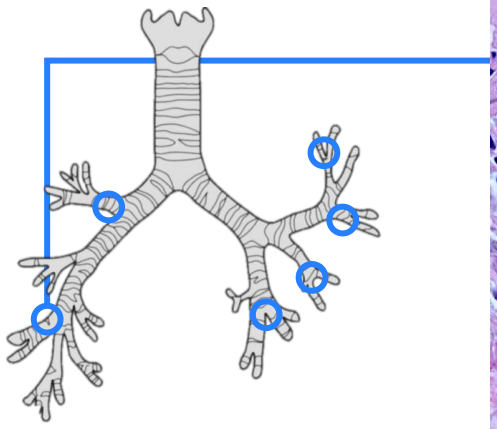
CFTR, cystic fibrosis transmembrane conductance regulator; DAPI, 4',6-diamidino-2-phenylindole; H&E, hematoxylin and eosin

All imaging conducted at 40× magnification
Post-dose biopsies harvested 24 hours after nebulization
Viral marker staining using 10-H44J (Biosynth)

Confirmed KB407 Transduction by Viral Marker in All Other Patients

CFTR expression results for patient 03-04 with *CFTR* genotype R334W/R1162X

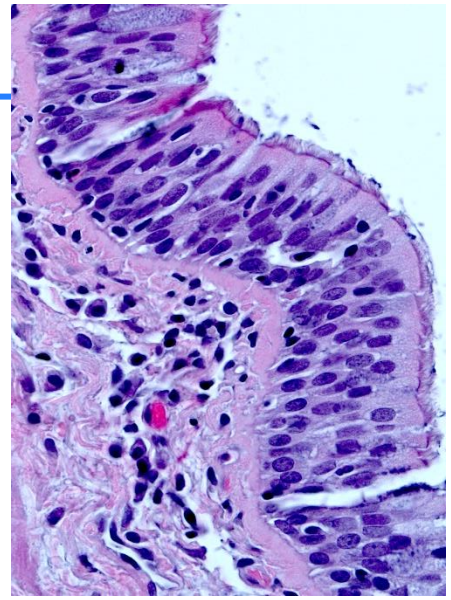
Patient 03-04
Airway Sampling



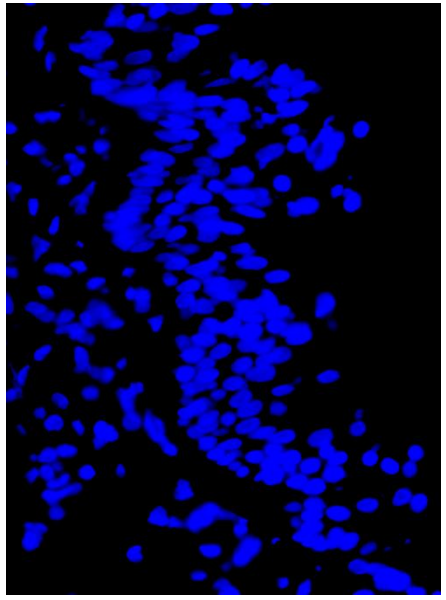
33.8%

Viral Marker Positive Cells*

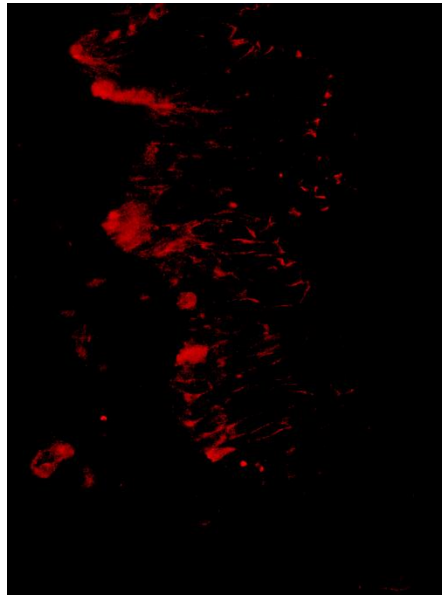
+ all six biopsies positive for marker



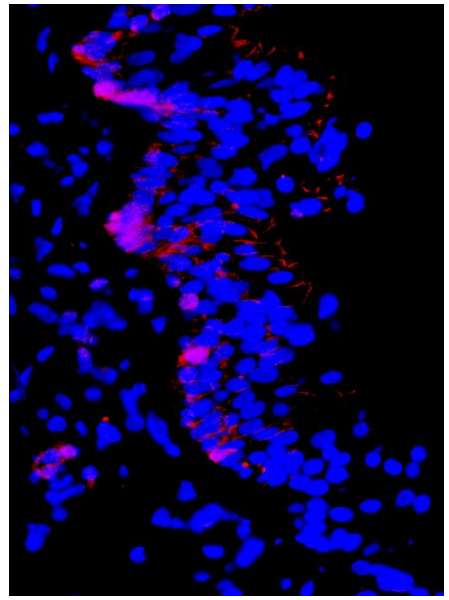
H&E



DAPI



Viral Marker



Merge

* Based on quantification of DAPI positive and DAPI + viral marker co-positive cells lining the conducting airways of the lung by immunofluorescence, total cell counts > 450

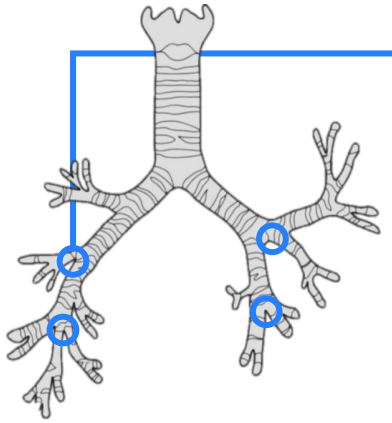
CFTR, cystic fibrosis transmembrane conductance regulator; DAPI, 4',6-diamidino-2-phenylindole; H&E, hematoxylin and eosin

All imaging conducted at 40× magnification
Post-dose biopsies harvested 24 hours after nebulization
Viral marker staining using 10-H44J (Biosynth)

Confirmed KB407 Transduction by Viral Marker in All Other Patients

CFTR expression results for patient 03-05 with *CFTR* genotype F508del/F508del

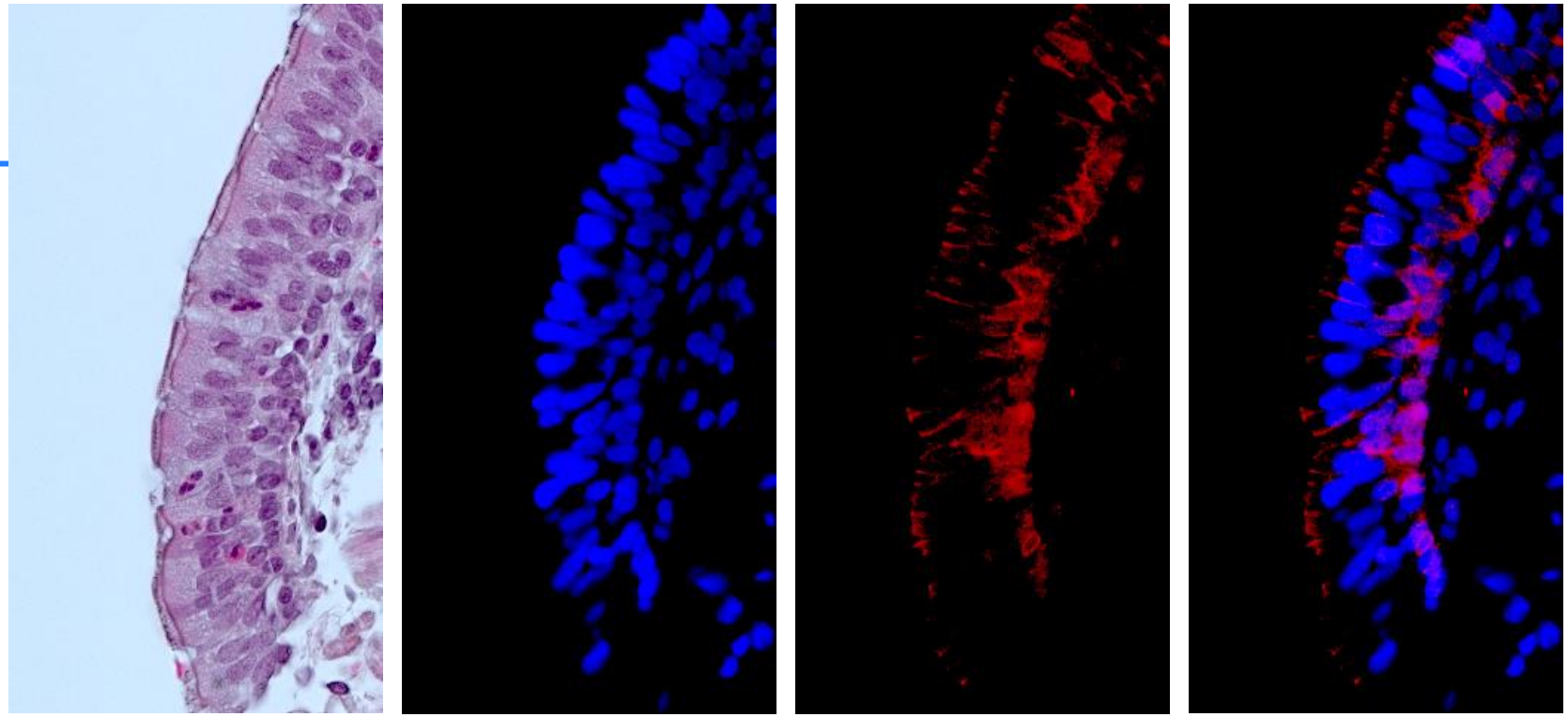
Patient 03-05
Airway Sampling



36.8%

Viral Marker Positive Cells*

+ all four biopsies positive for marker



H&E | **DAPI** | **Viral Marker** | **Merge**

* Based on quantification of DAPI positive and DAPI + viral marker co-positive cells lining the conducting airways of the lung by immunofluorescence, total cell counts > 400

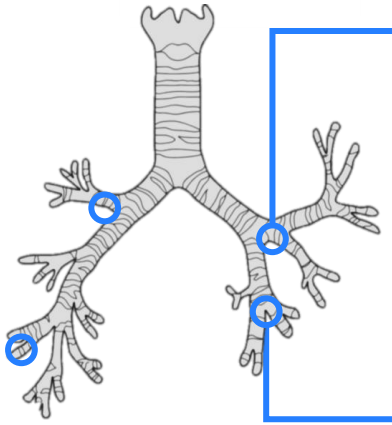
CFTR, cystic fibrosis transmembrane conductance regulator; DAPI, 4',6-diamidino-2-phenylindole; H&E, hematoxylin and eosin

All imaging conducted at 40× magnification
Post-dose biopsies harvested 72 hours after nebulization
Viral marker staining using 10-H44J (Biosynth)

Confirmed KB407 Transduction by Viral Marker in All Other Patients

CFTR expression results for patient 03-06 with *CFTR* genotype F508del/F508del

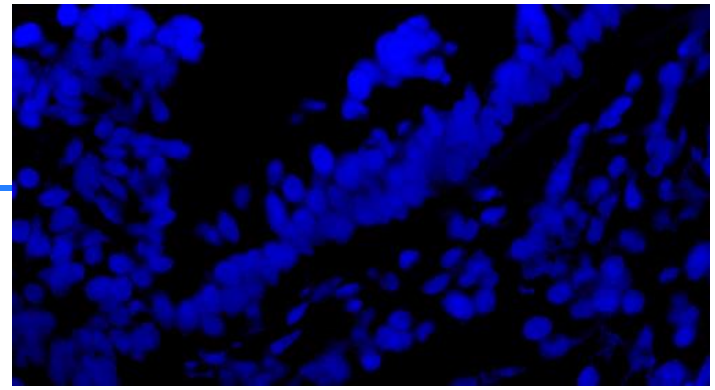
Patient 03-06
Airway Sampling



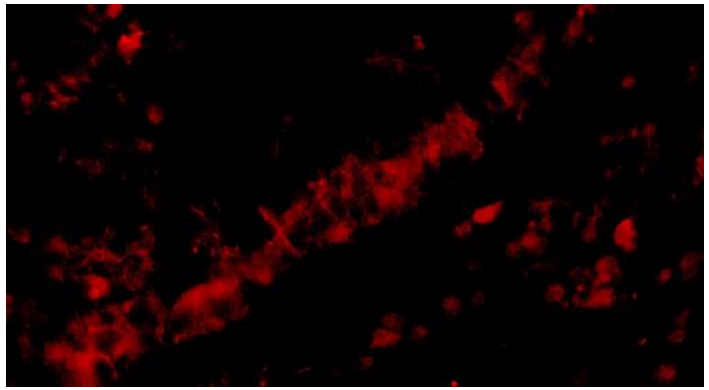
31.4%

Viral Marker Positive Cells*

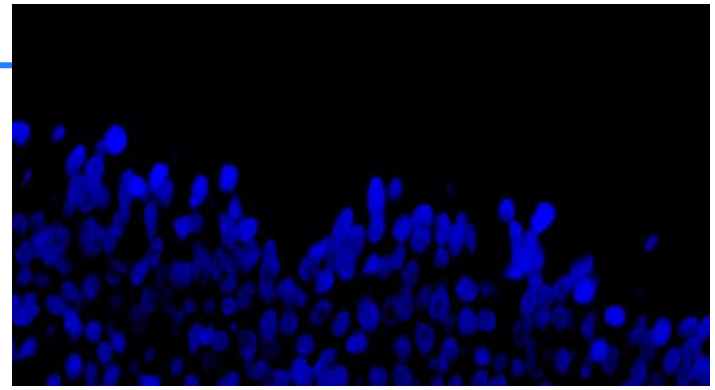
+ all four biopsies positive for marker



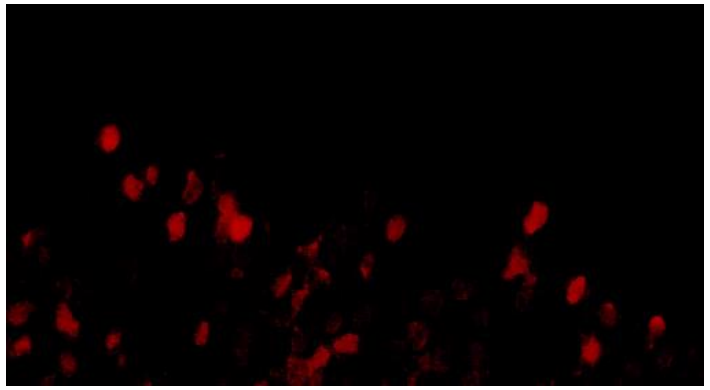
DAPI



Viral Marker



DAPI



Viral Marker

All imaging conducted at 40× magnification
Post-dose biopsies harvested 72 hours after nebulization
Viral marker staining using 10-H44J (Biosynth)

* Based on quantification of DAPI positive and DAPI + viral marker co-positive cells lining the conducting airways of the lung by immunofluorescence, total cell counts > 350

CFTR, cystic fibrosis transmembrane conductance regulator; DAPI, 4',6-diamidino-2-phenylindole

KB407 Transduction Confirmed in All Cohort 3 Patients with Bronchoscopies

Molecular Data Summary

Patient Number	03-01	03-02	03-03	03-04	03-05	03-06
CFTR Variants	2184delA / W1282X	R553X / M1V	C1210-12T / 1408A>G	R334W / R1162X	F508del / F508del	F508del / F508del
Baseline $ppFEV_1$	64	45	45	69	54	59
Total Number of Biopsies Suitable for Analysis	7	5	5	6	4	4
Percentage of Conducting Airway Cells Transduced with KB407*	42.1%	29.4%	36.5%	33.8%	36.8%	31.4%

- ✓ **Broad airway distribution:** All usable biopsies were positive for CFTR and/or viral marker of KB407 transduction
- ✓ **Exceeded transduction target:** Over 29% of conducting airway cells transduced in all six patients
- ✓ **Apical CFTR expression pattern:** Suggestive of appropriate post-translational modification and CFTR localization
- ✓ **CFTR protein expression for at least 96 hours:** Positive indicator for potential weekly or better dosing

Data cutoff date of January 6, 2026

* Based on CFTR protein expression for Patients 1 and 2, based on viral marker expression for Patients 3, 4, 5, and 6; conducting airway cells defined as airway-exposed epithelial cells lining the bronchi of the lung

CFTR, cystic fibrosis transmembrane conductance regulator; PFU, plaque forming unit; $ppFEV_1$, percent predicted forced expiratory volume in 1 second

KB407 Continues To Be Well Tolerated in Highest Dose Cohort

- **All but one KB407-related adverse events were mild-to-moderate; all were transient**
- **One serious adverse event of asthma exacerbation reported 24 hours after bronchoscopy**
  - Data monitoring committee considered the adverse event procedure related and not related to KB407
  - Event resolved in five days
- **No evidence of significant neutralizing antibody response following KB407 administration**
- **No systemic vector distribution after inhalation, based on blood and urine analysis**

Advancing into repeat dosing study

Next Steps and Accelerating The Path to Potential Registration of KB407

Advancing Into Repeat Dosing

- Today's data clearly demonstrate efficient vector delivery and expression of CFTR in patients treated with KB407
- Already working with CFF TDN on repeat dosing CORAL-3 study design which has been submitted to the FDA
- Goal will be to assess safety of repeat KB407 treatment and potential impact on lung function by spirometry

Upside: Making CORAL-3 Registrational

- Clear and urgent unmet need for patients that are either ineligible for or respond poorly to modulators
- Clinically-validated HSV-1 platform with demonstrated full-length gene delivery to lung across multiple programs
- Potential strategies to support registration with CORAL-3 under discussion with CFF TDN and the FDA

Expecting to align with FDA and initiate potentially registrational CORAL-3 study in the first half of 2026

CF, cystic fibrosis; CFF, Cystic Fibrosis Foundation; CFTR, cystic fibrosis transmembrane conductance regulator; FDA, United States Food and Drug Administration; HSV-1, herpes simplex virus 1; TDN, Therapeutics Development Network

Building Momentum in Krystal's Rare Disease Pipeline

- Confirmation of CFTR delivery sets KB407 on accelerated path towards $2B+ market
- Adds to potential registrational readouts for KB803 in ocular DEB and KB801 in NK
- Building a portfolio of rare disease medicines that Krystal can launch directly
- Gene delivery in CF also reinforces that potential of HSV-1 platform in the lung

CF, cystic fibrosis; CFTR, cystic fibrosis transmembrane conductance regulator; DEB, dystrophic epidermolysis bullosa; HSV-1, herpes simplex virus type 1; NK, neurotrophic keratitis

Krystal®

Developing Genetic Medicines to Treat Diseases with High Unmet Medical Needs