

Supplemental Tables and Figures

Table e1. Study characteristics and eligibility for the studies

	CHEC-SC	PROMISE	PROSPECT	GOAL
Years enrolling	2018-2021	2019-2020	2015-2017	2012, 2014
N of sites participating	106	98	52	58
N of PwCF enrolled	2,998	523	209	216
Eligibility criteria				
Eligible for modulators	X	X	X	X
F508 present		X	X (homozygous)	
G551D, R117H or other non-gating				X
Age >= 4 mo	X			
Age >= 6 yrs			X	X
Age >= 12 yrs		X		
No recent modulator use		X	X	X
On modulators 90 days	X			
No solid organ txplant			X	X

\* Cohorts 1 to 3 for GOAL were included, which each had a different time period and different genotype requirement.

Table e2. Association of Black race with potential confounders for study participation: computation of the propensity scores for each study. Each column shows the results from a single model for that study, including all covariates shown with Black (vs. White) race as the outcome.

	Adjusted risk ratio (95% CI)			
	CHEC	PROMISE	PROSPECT	GOAL
Male	1.02 (0.79, 1.32)	1.03 (0.75, 1.40)	0.59 (0.34, 1.04)	1.01 (0.31, 3.24)
BMI category				
Underweight	1.22 (0.75, 1.99)	0.73 (0.40, 1.34)	2.13 (0.89, 5.06)	1.09 (0.10, 11.5)
Normal				
Overweight	0.93 (0.65, 1.31)	0.88 (0.58, 1.36)	1.78 (0.84, 3.76)	1.87 (0.52, 6.69)
Obese	0.69 (0.42, 1.15)	0.78 (0.41, 1.46)	0.89 (0.11, 7.02)	0.00 (0.00, Inf)
Genotype				
F508 Homozygous	<b>4.34 (3.18, 5.93)</b>	<b>4.08 (2.84, 5.86)</b>	1.55 (0.18, 13.4)	N/A
F508 Heterozygous	Ref	Ref	Ref	Ref
G551D (not F508)	<b>5.82 (2.69, 12.6)</b>	N/A	(with other)	2.88 (0.79, 10.5)
R117H (not F508)	<b>5.79 (1.68, 19.9)</b>	N/A	(with other)	2.02 (0.21, 19.2)
Other	<b>14.1 (8.89, 22.4)</b>	N/A	9.35 (0.84, 104)	<b>26.3 (4.93, 140)</b>
Age >= 30	0.74 (0.49, 1.13)	0.75 (0.49, 1.15)	1.34 (0.53, 3.37)	0.50 (0.06, 4.07)
Private Insurance	<b>0.22 (0.17, 0.29)</b>	<b>0.22 (0.16, 0.31)</b>	<b>0.18 (0.10, 0.34)</b>	<b>0.17 (0.04, 0.66)</b>
Patient some college	0.68 (0.41, 1.15)	<b>0.55 (0.32, 0.93)</b>	0.55 (0.16, 1.87)	1.41 (0.06, 32.9)
Patient unknown educ	1.06 (0.62, 1.80)	0.96 (0.57, 1.62)	1.48 (0.49, 4.49)	5.23 (0.33, 82.1)
Parents some college	0.74 (0.50, 1.11)	1.20 (0.70, 2.04)	0.65 (0.26, 1.61)	1.18 (0.19, 7.27)
Parents unknown educ	1.15 (0.75, 1.76)	1.49 (0.86, 2.56)	1.67 (0.70, 3.99)	1.29 (0.27, 6.13)
FEV1% predicted				
< 40	1.07 (0.62, 1.86)	0.93 (0.49, 1.75)	0.55 (0.17, 1.77)	4.04 (0.52, 31.7)
40 to 89	0.93 (0.68, 1.28)	0.84 (0.57, 1.24)	0.67 (0.35, 1.31)	1.23 (0.32, 4.69)
90%	Ref	Ref	Ref	Ref
Unknown	0.84 (0.54, 1.32)	0.56 (0.30, 1.03)	0.56 (0.11, 2.80)	0.00 (0.00, Inf)
Exacerbation hx				
None	Ref	Ref	Ref	Ref
1 to 2	1.20 (0.85, 1.70)	<b>1.72 (1.19, 2.46)</b>	1.02 (0.55, 1.90)	2.13 (0.55, 8.26)
At least 3	0.84 (0.41, 1.74)	1.17 (0.61, 2.22)	<b>0.27 (0.08, 0.98)</b>	1.78 (0.14, 22.8)

At least 3 visits	0.77 (0.58, 1.02)	0.99 (0.71, 1.38)	0.72 (0.37, 1.41)	0.87 (0.22, 3.36)
CFRD/Diabetes	<b>2.01 (1.48, 2.72)</b>	<b>1.89 (1.36, 2.64)</b>	1.18 (0.60, 2.29)	1.09 (0.22, 5.38)
Minimum distance*				
< 30 miles	Ref	Ref	Ref	Ref
30 to 59 miles	<b>0.46 (0.32, 0.66)</b>	<b>0.35 (0.22, 0.55)</b>	0.59 (0.26, 1.31)	0.43 (0.08, 2.38)
60+ miles	<b>0.29 (0.20, 0.42)</b>	<b>0.23 (0.15, 0.35)</b>	<b>0.24 (0.11, 0.53)</b>	0.60 (0.17, 2.04)

\* Minimum distance to study site as computed between location at centroid of home zipcode to that of study participating study sites for the relevant (eligible) study.

Table e3. Association of Hispanic ethnicity with potential confounders for study participation: computation of the propensity scores for each study. Each column shows the results from a single model for that study, including all covariates shown with Hispanic ethnicity (vs. non-Hispanic White) as the outcome.

	Adjusted risk ratio (95% CI)			
	CHEC	PROMISE	PROSPECT	GOAL
Male	1.00 (0.85, 1.17)	1.13 (0.91, 1.42)	1.03 (0.74, 1.44)	0.89 (0.35, 2.30)
BMI category	N/D		N/D	
Underweight	0.84 (0.58, 1.23)	0.87 (0.56, 1.35)	0.61 (0.31, 1.22)	5.33 (0.83, 34.4)
Normal	Ref	Ref	Ref	Ref
Overweight	<b>1.29 (1.05, 1.59)</b>	1.22 (0.91, 1.64)	1.12 (0.67, 1.88)	1.95 (0.59, 6.40)
Obese	<b>1.70 (1.29, 2.23)</b>	1.04 (0.66, 1.65)	1.19 (0.43, 3.28)	1.99 (0.43, 9.26)
Genotype				
F508 Homozygous	<b>2.06 (1.73, 2.45)</b>	<b>2.49 (1.96, 3.16)</b>	<b>3.38 (1.18, 9.70)</b>	N/A
F508 Heterozygous	Ref	Ref	Ref	Ref
G551D (not F508)	1.33 (0.66, 2.68)	N/A	(with other)	0.19 (0.02, 1.60)
R117H (not F508)	0.40 (0.09, 1.68)	N/A	(with other)	0.54 (0.09, 3.24)
Other	<b>5.46 (4.08, 7.30)</b>	N/A	0.00 (0.00, Inf)	<b>28.4 (6.52, 124)</b>
Age >= 30	<b>0.45 (0.33, 0.60)</b>	<b>0.47 (0.33, 0.66)</b>	0.83 (0.42, 1.66)	0.00 (0.00, Inf)
Private Insurance	<b>0.32 (0.27, 0.38)</b>	<b>0.44 (0.35, 0.56)</b>	<b>0.36 (0.25, 0.52)</b>	<b>0.12 (0.04, 0.37)</b>
Patient some college	1.00 (0.68, 1.48)	0.77 (0.49, 1.19)	1.54 (0.51, 4.62)	223M (0.00, Inf)
Patient unknown educ	1.34 (0.91, 1.97)	1.29 (0.83, 1.98)	3.57 (1.21, 10.6)	33M (0.00, Inf)
Parents some college	<b>0.45 (0.35, 0.57)</b>	<b>0.44 (0.31, 0.63)</b>	<b>0.51 (0.31, 0.84)</b>	0.63 (0.14, 2.80)
Parents unknown educ	0.85 (0.66, 1.09)	0.86 (0.61, 1.21)	0.88 (0.53, 1.46)	1.57 (0.46, 5.43)
FEV1% predicted				
< 40	1.05 (0.69, 1.59)	1.13 (0.67, 1.90)	1.09 (0.54, 2.23)	0.00 (0.00, Inf)
40 to 89	1.13 (0.93, 1.38)	1.11 (0.84, 1.45)	1.04 (0.70, 1.53)	1.06 (0.37, 3.06)
90%	Ref	Ref	Ref	Ref
Unknown	1.13 (0.87, 1.45)	1.10 (0.70, 1.71)	0.47 (0.13, 1.71)	15.4 (0.78, 304)
Exacerbation hx				
None	Ref	Ref	Ref	Ref
1 to 2	1.00 (0.80, 1.25)	0.90 (0.67, 1.21)	1.27 (0.86, 1.87)	0.68 (0.18, 2.53)
At least 3	1.26 (0.83, 1.92)	1.33 (0.85, 2.08)	1.06 (0.59, 1.89)	0.69 (0.05, 8.91)
At least 3 visits	<b>1.34 (1.11, 1.62)</b>	1.11 (0.87, 1.42)	1.17 (0.74, 1.87)	4.19 (0.97, 18.0)
CFRD/Diabetes	0.97 (0.78, 1.20)	0.97 (0.74, 1.26)	1.41 (0.94, 2.10)	0.00 (0.00, Inf)
Minimum distance*				
< 30 miles	Ref	Ref	Ref	Ref
30 to 59 miles	<b>0.68 (0.55, 0.85)</b>	<b>0.65 (0.46, 0.92)</b>	0.79 (0.47, 1.33)	0.19 (0.02, 1.67)
60+ miles	<b>0.55 (0.44, 0.68)</b>	<b>0.64 (0.48, 0.86)</b>	<b>0.53 (0.33, 0.83)</b>	0.86 (0.31, 2.37)

\* Minimum distance to study site as computed between location at centroid of home zipcode to that of study participating study sites for the relevant (eligible) study

