BEATCF enrolment tables

Edward Pan

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Table 1: **Demographics and Clinical Baseline Characteristics**

Characteristic	**Overall**, N = 872	ppFEV1≥70 No Pseu-domonas N=531	ppFEV1≥70 Pseudomonas N=228	ppFEV1<70 No Pseu-domonas N=32	ppFEV1<70 Pseudomonas N=72
Median age in years (IQR)					
Median (IQR)	11 (6, 17)	10 (5, 14)	13 (7, 17)	29 (17, 42)	32 (23, 43)
Age Groups					
<6y	180 (20.6%)	136 (25.6%)	41 (18.0%)	1 (3.1%)	2 (2.8%)
6y-<12y	262 (30.0%)	191 (36.0%)	64 (28.1%)	4 (12.5%)	1 (1.4%)
12y-<18y	219 (25.1%)	132 (24.9%)	71 (31.1%)	4 (12.5%)	9 (12.5%)
18y-<30y	96 (11.0%)	48 (9.0%)	19 (8.3%)	9 (28.1%)	19 (26.4%)
30y-<40y	59 (6.8%)	14 (2.6%)	21 (9.2%)	4 (12.5%)	20 (27.8%)
≥40y	56 (6.4%)	10 (1.9%)	12 (5.3%)	10 (31.3%)	21 (29.2%)
Mean height in cm (SD)	141 (31)	135 (31)	144 (29)	162 (21)	164 (14)
(Missing)	1	1	0	0	0
Mean weight in kilograms (SD)	43 (23)	38 (22)	44 (22)	62 (22)	63 (17)
BMI kg/m² (SD)	19.6 (4.3)	18.9 (4.0)	19.6 (4.1)	22.4 (4.3)	23.0 (4.4)
(Missing)	1	1	0	0	0
Sex					
Female	425 (48.7%)	255 (48.0%)	116 (50.9%)	15 (46.9%)	37 (51.4%)
Male	447 (51.3%)	276 (52.0%)	112 (49.1%)	17 (53.1%)	35 (48.6%)
Genotype					
Homozygous F508del	412 (47.5%)	222 (42.0%)	128 (56.1%)	11 (34.4%)	45 (64.3%)
Heterozygous F508del	391 (45.1%)	263 (49.8%)	89 (39.0%)	17 (53.1%)	20 (28.6%)
Other	64 (7.4%)	43 (8.1%)	11 (4.8%)	4 (12.5%)	5 (7.1%)
(Missing)	5	3	0	0	2
Sweat Chloride Level					
People not on CFTR Modulators	167 (19.2%)	122 (23.0%)	34 (14.9%)	6 (18.8%)	3 (4.2%)
<40	25 (2.9%)	20 (3.8%)	4 (1.8%)	1 (3.1%)	0 (0.0%)
40-<60	20 (2.3%)	19 (3.6%)	1 (0.4%)	0 (0.0%)	0 (0.0%)
≥60	108 (12.4%)	74 (13.9%)	26 (11.4%)	4 (12.5%)	2 (2.8%)
Missing	14 (1.6%)	9 (1.7%)	3 (1.3%)	1 (3.1%)	1 (1.4%)
People on CFTR Modulators	261 (29.9%)	146 (27.5%)	69 (30.3%)	16 (50.0%)	28 (38.9%)
<40	6 (0.7%)	3 (0.6%)	0 (0.0%)	3 (9.4%)	0 (0.0%)
40-<60	12 (1.4%)	4 (0.8%)	3 (1.3%)	3 (9.4%)	2 (2.8%)
≥60	159 (18.2%)	102 (19.2%)	42 (18.4%)	4 (12.5%)	9 (12.5%)
Missing	84 (9.6%)	37 (7.0%)	24 (10.5%)	6 (18.8%)	17 (23.6%)
Enrolment before Aug2022	444 (50.9%)	263 (49.5%)	125 (54.8%)	10 (31.3%)	41 (56.9%)
Pancreatic Insufficiency	638 (73.2%)	374 (70.4%)	188 (82.5%)	16 (50.0%)	53 (73.6%)
CF diabetes mellitus	110 (12.6%)	38 (7.2%)	38 (16.7%)	10 (31.3%)	24 (33.3%)
Highest ppFEV in previous 12 months	90 (20)	97 (12)	95 (13)	55 (11)	52 (11)
(Missing)	158	114	35	0	0
Highest ppFEV in the previous 6 months	88 (20)	95 (13)	93 (13)	54 (11)	50 (12)
(Missing)	176	127	39	0	1

[1] n (%); Mean (SD)

[2] Includes 9 records with missing spirometry

[a] Sweat chloride level was recorded only for those participants enrolled from 1 Aug 2022.

[b] ppFEV1: percentage predicted FEV1; IQR: Inter-quartile range; SD: standard deviation; BMI: Body mass index; CFTR: cystic fibrosis transmembrane conductance regulator

Table 2: **Mean (SD) CFQR for participants aged 12-13 years at Enrolment (60 days)**

Characteristic	Overall, N=68[a]	ppFEV1≥70 No Pseudomonas N=47	ppFEV1≥70 Pseudomonas N=17
Physical	86 (19)	87 (17)	84 (21)
Emotional	72 (14)	73 (13)	68 (15)
Social	71 (19)	74 (17)	62 (18)
Body	84 (19)	86 (19)	81 (17)
Eat	84 (21)	88 (16)	76 (24)
Treatment burden	70 (19)	71 (19)	67 (22)
Respiration	82 (15)	84 (16)	81 (14)
Digestion	72 (23)	72 (22)	67 (24)

[1] Mean (SD)
[a] Includes 1 record with missing spirometry.
[b] Data for stratums loNEG and loPOS were not reported due to privacy concerns regarding small numbers.
[c] There were 89 participants aged 12 or 13 at the time of enrolment.
[d] This dataset includes 2 participants of age 11 at enrolment.

Table 3: **Mean (SD) CFQR for participants (aged ≥14 years) at Enrolment (60 days)**

Characteristic	Overall, N=231[a]	ppFEV1≥70 No Pseudomonas N=91	ppFEV1≥70 Pseudomonas N=70	ppFEV1<70 No Pseudomonas N=18	ppFEV1<70 Pseudomonas N=48
Physical	75 (25)	82 (20)	77 (23)	72 (25)	59 (30)
Role	78 (20)	83 (16)	75 (22)	81 (20)	74 (22)
Vitality	55 (19)	58 (19)	55 (20)	56 (19)	49 (19)
Emotional	73 (20)	75 (20)	72 (23)	83 (13)	70 (19)
Social	69 (21)	73 (19)	67 (23)	66 (17)	65 (21)
Body	72 (24)	73 (25)	72 (24)	80 (21)	68 (24)
Eat	88 (20)	88 (21)	87 (22)	98 (4)	86 (18)
Treatment burden	66 (23)	71 (20)	61 (26)	69 (19)	63 (25)
Health perceptions	69 (22)	76 (19)	65 (25)	69 (22)	61 (22)
Weight	78 (33)	83 (29)	72 (35)	91 (19)	73 (38)
Respiration	73 (21)	79 (19)	72 (20)	68 (24)	66 (24)
Digestion	78 (19)	81 (18)	78 (19)	76 (24)	74 (19)

[1] Mean (SD)
[a] Includes 4 records with missing spirometry.
[b] There were 342 participants 14 or older at the time of enrolment.

Table 4: **Mean (SD) CFQR as reported by parents and caregivers at Enrolment (60 days)**

Characteristic	Overall, N=216[a]	ppFEV1≥70 No Pseudomonas N=159	ppFEV1≥70 Pseudomonas N=51
Physical	85 (15)	87 (15)	81 (17)
Emotional	81 (17)	81 (17)	80 (18)
School	75 (23)	76 (22)	73 (21)
Body	77 (25)	78 (25)	75 (26)
Eat	72 (27)	74 (27)	68 (29)
Treatment burden	62 (24)	67 (23)	51 (21)
Health perceptions	79 (19)	81 (18)	74 (22)
Weight	63 (37)	63 (38)	64 (37)
Respiration	85 (16)	88 (14)	79 (18)
Digestion	77 (18)	79 (18)	70 (18)

[1] Mean (SD)
[a] Includes 1 record with missing spirometry.
[b] Data for stratums loNEG and loPOS were not reported due to privacy concerns regarding small numbers.
[c] There were 263 participants aged 6 to 11 at the time of enrolment.
[d] This dataset includes 4 participants of age 5 at enrolment.

Table 5: **Concomitant medications at enrolment**

Medication Name	Overall N=872	ppFEV1≥70 No Pseu-domonas N=531	ppFEV1≥70 Pseudomonas N=228	ppFEV1<70 No Pseu-domonas N=32	ppFEV1<70 Pseudomonas N=72
Antibiotics					
Azithromycin	213 (24.4%)	83 (15.6%)	73 (32.0%)	13 (40.6%)	43 (59.7%)
Tobramycin	135 (15.5%)	33 (6.2%)	63 (27.6%)	9 (28.1%)	28 (38.9%)
Amoxicillin	104 (11.9%)	78 (14.7%)	24 (10.5%)	1 (3.1%)	1 (1.4%)
Flucloxacillin	44 (5.0%)	31 (5.8%)	11 (4.8%)	0 (0.0%)	2 (2.8%)
Cotrimoxazole	33 (3.8%)	18 (3.4%)	11 (4.8%)	1 (3.1%)	3 (4.2%)
Ciprofloxacin	32 (3.7%)	11 (2.1%)	12 (5.3%)	1 (3.1%)	8 (11.1%)
Other	79 (9.1%)	22 (4.1%)	33 (14.5%)	7 (21.9%)	17 (23.6%)
Antifungal	30 (3.4%)	12 (2.3%)	11 (4.8%)	2 (6.3%)	4 (5.6%)
Corticosteroids					
Inhaled	258 (29.6%)	114 (21.5%)	87 (38.2%)	12 (37.5%)	41 (56.9%)
Nasal	73 (8.4%)	40 (7.5%)	22 (9.6%)	1 (3.1%)	10 (13.9%)
Systemic	24 (2.8%)	8 (1.5%)	9 (3.9%)	0 (0.0%)	7 (9.7%)
Topical	5 (0.6%)	5 (0.9%)	0 (0.0%)	0 (0.0%)	0 (0.0%)
Muco-active agents					
Dornase Alfa	588 (67.4%)	329 (62.0%)	185 (81.1%)	22 (68.8%)	46 (63.9%)
Hypertonic saline	468 (53.7%)	254 (47.8%)	151 (66.2%)	17 (53.1%)	41 (56.9%)
Mannitol Bronchitol	48 (5.5%)	31 (5.8%)	12 (5.3%)	0 (0.0%)	5 (6.9%)
CFTR modulators					
Elexacaftor/tezacaftor/ivacaftor (Trikafta)	213 (24.4%)	96 (18.1%)	61 (26.8%)	17 (53.1%)	38 (52.8%)
Lumacaftor/ivacaftor (Orkambi)	152 (17.4%)	104 (19.6%)	39 (17.1%)	1 (3.1%)	7 (9.7%)
Tezacaftor/ivacaftor (symdeko)	85 (9.7%)	34 (6.4%)	32 (14.0%)	3 (9.4%)	14 (19.4%)
Ivacaftor (Kalydeco)	49 (5.6%)	33 (6.2%)	5 (2.2%)	2 (6.3%)	8 (11.1%)
Antidepressants/Antipsychotic					
Antidepressant	55 (6.3%)	22 (4.1%)	14 (6.1%)	4 (12.5%)	13 (18.1%)
Antipsychotic	11 (1.3%)	4 (0.8%)	6 (2.6%)	0 (0.0%)	1 (1.4%)
ADHD medication	11 (1.3%)	8 (1.5%)	3 (1.3%)	0 (0.0%)	0 (0.0%)
Vitamin/mineral supplements	684 (78.4%)	402 (75.7%)	191 (83.8%)	23 (71.9%)	61 (84.7%)
Pancreatic enzyme replacement	683 (78.3%)	392 (73.8%)	199 (87.3%)	21 (65.6%)	64 (88.9%)
Sodium chloride	393 (45.1%)	265 (49.9%)	109 (47.8%)	4 (12.5%)	12 (16.7%)
Bronchodilator (Salbutamol)	373 (42.8%)	179 (33.7%)	125 (54.8%)	15 (46.9%)	49 (68.1%)
Proton pump inhibitor	182 (20.9%)	89 (16.8%)	54 (23.7%)	6 (18.8%)	31 (43.1%)
Insulin medication	84 (9.6%)	30 (5.6%)	26 (11.4%)	8 (25.0%)	20 (27.8%)
Ursodeoxycholic acid	73 (8.4%)	32 (6.0%)	25 (11.0%)	4 (12.5%)	11 (15.3%)
Iron supplement	66 (7.6%)	34 (6.4%)	15 (6.6%)	3 (9.4%)	13 (18.1%)

[1] n (%)

[a] Participants may have received more than one CFTR modulator in the two years prior to enrolment and are included for each modulator prescribed.

Table 6: **Microbiology at enrolment**

Microbiology	Overall N=872	ppFEV1≥70 No Pseu-domonas N=531	ppFEV1≥70 Pseudomonas N=228	ppFEV1<70 No Pseu-domonas N=32	ppFEV1<70 Pseudomonas N=72
Bacterial pathogen					
Pseudomonas species	305 (35.0%)	0 (0.0%)	228 (100.0%)	0 (0.0%)	72 (100.0%)
Staphylococcus aureus	423 (48.5%)	258 (48.6%)	125 (54.8%)	8 (25.0%)	28 (38.9%)
Staphylococcus aureus (MRSA)	35 (4.0%)	16 (3.0%)	12 (5.3%)	1 (3.1%)	5 (6.9%)
Staphylococcus aureus (MSSA)	384 (44.0%)	234 (44.1%)	114 (50.0%)	7 (21.9%)	25 (34.7%)
Staphylococcus aureus (Unknown)	26 (3.0%)	17 (3.2%)	5 (2.2%)	1 (3.1%)	3 (4.2%)
Staphylococcus species	92 (10.6%)	57 (10.7%)	24 (10.5%)	5 (15.6%)	6 (8.3%)
Haemophilus species	193 (22.1%)	135 (25.4%)	51 (22.4%)	2 (6.3%)	5 (6.9%)
Stenotrophomonas species	101 (11.6%)	36 (6.8%)	49 (21.5%)	5 (15.6%)	11 (15.3%)
Mycobacterium species	42 (4.8%)	18 (3.4%)	18 (7.9%)	2 (6.3%)	4 (5.6%)
Streptococcus pneumoniae	41 (4.7%)	32 (6.0%)	8 (3.5%)	0 (0.0%)	1 (1.4%)
Streptococcus pyogenes	18 (2.1%)	11 (2.1%)	7 (3.1%)	0 (0.0%)	0 (0.0%)
Streptococcus species (other)	11 (1.3%)	6 (1.1%)	2 (0.9%)	0 (0.0%)	3 (4.2%)
Escherichia species	35 (4.0%)	27 (5.1%)	7 (3.1%)	0 (0.0%)	1 (1.4%)
Klebsiella species	34 (3.9%)	19 (3.6%)	14 (6.1%)	1 (3.1%)	0 (0.0%)
Enterobacter species	32 (3.7%)	20 (3.8%)	12 (5.3%)	0 (0.0%)	0 (0.0%)
Chryseobacterium species	31 (3.6%)	11 (2.1%)	15 (6.6%)	2 (6.3%)	3 (4.2%)
Moraxella species	27 (3.1%)	21 (4.0%)	6 (2.6%)	0 (0.0%)	0 (0.0%)
Acinetobacterium species	26 (3.0%)	12 (2.3%)	13 (5.7%)	0 (0.0%)	1 (1.4%)
Achromobacter species	25 (2.9%)	7 (1.3%)	10 (4.4%)	3 (9.4%)	4 (5.6%)
Serratia species	23 (2.6%)	12 (2.3%)	10 (4.4%)	1 (3.1%)	0 (0.0%)
Burkholderia species	20 (2.3%)	13 (2.4%)	1 (0.4%)	1 (3.1%)	5 (6.9%)
Other	96 (11.0%)	46 (8.7%)	43 (18.9%)	3 (9.4%)	4 (5.6%)
Viral pathogen					
Rhinovirus	39 (4.5%)	17 (3.2%)	16 (7.0%)	2 (6.3%)	4 (5.6%)
SARS-CoV-2	20 (2.3%)	10 (1.9%)	6 (2.6%)	0 (0.0%)	4 (5.6%)
Parainfluenza (PIV)	16 (1.8%)	8 (1.5%)	7 (3.1%)	0 (0.0%)	1 (1.4%)
Respiratory Syncytial Virus (RSV)	16 (1.8%)	10 (1.9%)	6 (2.6%)	0 (0.0%)	0 (0.0%)
Enterovirus	8 (0.9%)	5 (0.9%)	3 (1.3%)	0 (0.0%)	0 (0.0%)
Adenovirus	6 (0.7%)	3 (0.6%)	3 (1.3%)	0 (0.0%)	0 (0.0%)
Influenza	5 (0.6%)	4 (0.8%)	1 (0.4%)	0 (0.0%)	0 (0.0%)
Other	3 (0.3%)	2 (0.4%)	0 (0.0%)	0 (0.0%)	1 (1.4%)
Fungi					
Aspergillus species	196 (22.5%)	93 (17.5%)	83 (36.4%)	4 (12.5%)	13 (18.1%)
Candida species	175 (20.1%)	84 (15.8%)	63 (27.6%)	7 (21.9%)	20 (27.8%)
Other	104 (11.9%)	58 (10.9%)	36 (15.8%)	2 (6.3%)	6 (8.3%)

[1] n (%)