

BEATCF enrolment tables

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Table 1: Demographics and Clinical Baseline Characteristics

Characteristic	Overall, N = 872	ppFEV1≥70 No Pseudo- monas N=531	ppFEV1≥70 Pseudomonas N=228	ppFEV1<70 No Pseudo- monas 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 (21%)	136 (26%)	41 (18%)	1 (3.1%)	2 (2.8%)
6y-<12y	262 (30%)	191 (36%)	64 (28%)	4 (13%)	1 (1.4%)
12y-<18y	219 (25%)	132 (25%)	71 (31%)	4 (13%)	9 (13%)
18y-<30y	96 (11%)	48 (9.0%)	19 (8.3%)	9 (28%)	19 (26%)
30y-<40y	59 (6.8%)	14 (2.6%)	21 (9.2%)	4 (13%)	20 (28%)
≥40y	56 (6.4%)	10 (1.9%)	12 (5.3%)	10 (31%)	21 (29%)
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 (49%)	255 (48%)	116 (51%)	15 (47%)	37 (51%)
Male	447 (51%)	276 (52%)	112 (49%)	17 (53%)	35 (49%)
Genotype					
Homozygous F508del	412 (51%)	222 (46%)	128 (59%)	11 (39%)	45 (69%)
Heterozygous F508del	391 (49%)	263 (54%)	89 (41%)	17 (61%)	20 (31%)
(Missing)	69	46	11	4	7
Sweat Chlorine Level in mmol/L					
<40	27 (6.5%)	21 (7.8%)	5 (4.7%)	1 (13%)	0 (0%)
40-<60	20 (4.8%)	19 (7.0%)	1 (0.9%)	0 (0%)	0 (0%)
≥60	0 (0%)	0 (0%)	0 (0%)	0 (0%)	0 (0%)
Concurrent CFTR modulators	0 (0%)	0 (0%)	0 (0%)	0 (0%)	0 (0%)
Enrolment before Aug2022	367 (89%)	230 (85%)	101 (94%)	7 (88%)	25 (100%)
(Missing)	458	261	121	24	47
Pancreatic Insufficiency	638 (73%)	374 (70%)	188 (82%)	16 (50%)	53 (74%)
CF diabetes mellitus	110 (13%)	38 (7.2%)	38 (17%)	10 (31%)	24 (33%)
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)

Table 2: CFQR Ages 12 and 13 at Enrolment (60 days)

Characteristic	Overall, N = 68	ppFEV1≥70 No Pseudo- monas N=47	ppFEV1≥70 Pseudomonas N=17	ppFEV1<70 No Pseudo- monas N=1	ppFEV1<70 Pseudomonas N=2
Physical	86 (19)	87 (17)	84 (21)	100 (NA)	50 (31)
Emotional	72 (14)	73 (13)	68 (15)	92 (NA)	67 (6)
Social	71 (19)	74 (17)	62 (18)	86 (NA)	64 (51)
Body	84 (19)	86 (19)	81 (17)	67 (NA)	78 (16)
Eat	84 (21)	88 (16)	76 (24)	33 (NA)	56 (47)
Treatment burden	70 (19)	71 (19)	67 (22)	67 (NA)	72 (8)
Respiration	82 (15)	84 (16)	81 (14)	83 (NA)	63 (6)
Digestion	72 (23)	72 (22)	67 (24)	100 (NA)	67 (0)

¹ Mean (SD)

Table 3: **CFQR Adolescents and Adults at Enrolment (60 days)**

Characteristic	Overall, N = 231	ppFEV1≥70 No Pseu- domonas N=91	ppFEV1≥70 Pseudomonas N=70	ppFEV1<70 No Pseu- domonas 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)Table 4: **CFQR Parents and Caregivers at Enrolment (60 days)**

Characteristic	Overall, N = 216	ppFEV1≥70 No Pseu- domonas N=159	ppFEV1≥70 Pseudomonas N=51	ppFEV1<70 No Pseu- domonas N=4	ppFEV1<70 Pseudomonas N=1
Physical	85 (15)	87 (15)	81 (17)	74 (12)	70 (NA)
Emotional	81 (17)	81 (17)	80 (18)	88 (19)	60 (NA)
School	75 (23)	76 (22)	73 (21)	64 (26)	67 (NA)
Body	77 (25)	78 (25)	75 (26)	72 (35)	67 (NA)
Eat	72 (27)	74 (27)	68 (29)	58 (42)	67 (NA)
Treatment burden	62 (24)	67 (23)	51 (21)	50 (29)	44 (NA)
Health perceptions	79 (19)	81 (18)	74 (22)	86 (21)	78 (NA)
Weight	63 (37)	63 (38)	64 (37)	58 (42)	33 (NA)
Respiration	85 (16)	88 (14)	79 (18)	83 (10)	72 (NA)
Digestion	77 (18)	79 (18)	70 (18)	83 (11)	89 (NA)

¹ Mean (SD)

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%)	83 (16%)	73 (32%)	13 (41%)	43 (60%)
Tobramycin	135 (15%)	33 (6.2%)	63 (28%)	9 (28%)	28 (39%)
Amoxicillin	103 (12%)	77 (15%)	24 (11%)	1 (3.1%)	1 (1.4%)
Flucloxacillin	44 (5.0%)	31 (5.8%)	11 (4.8%)	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%)
Other	79 (9.1%)	22 (4.1%)	33 (14%)	7 (22%)	17 (24%)
Antifungal	30 (3.4%)	12 (2.3%)	11 (4.8%)	2 (6.3%)	4 (5.6%)
Corticosteroids					
Inhaled	258 (30%)	114 (21%)	87 (38%)	12 (38%)	41 (57%)
Nasal	73 (8.4%)	40 (7.5%)	22 (9.6%)	1 (3.1%)	10 (14%)
Systemic	23 (2.6%)	8 (1.5%)	8 (3.5%)	0 (0%)	7 (9.7%)
Topical	5 (0.6%)	5 (0.9%)	0 (0%)	0 (0%)	0 (0%)
Muco-active agents					
Dornase Alfa	588 (67%)	329 (62%)	185 (81%)	22 (69%)	46 (64%)
Hypertonic saline	468 (54%)	254 (48%)	151 (66%)	17 (53%)	41 (57%)
Mannitol Bronchitol	48 (5.5%)	31 (5.8%)	12 (5.3%)	0 (0%)	5 (6.9%)
CFTR modulators					
Elexacaftor/tezacaftor/ivacaftor (Trikafta)	213 (24%)	96 (18%)	61 (27%)	17 (53%)	38 (53%)
Lumacaftor/ivacaftor (Orkambi)	152 (17%)	104 (20%)	39 (17%)	1 (3.1%)	7 (9.7%)
Tezacaftor/ivacaftor (symdeko)	85 (9.7%)	34 (6.4%)	32 (14%)	3 (9.4%)	14 (19%)
Ivacaftor (Kalydeco)	49 (5.6%)	33 (6.2%)	5 (2.2%)	2 (6.3%)	8 (11%)
Antidepressants/Antipsychotic					
Antidepressant	55 (6.3%)	22 (4.1%)	14 (6.1%)	4 (13%)	13 (18%)
Antipsychotic	11 (1.3%)	4 (0.8%)	6 (2.6%)	0 (0%)	1 (1.4%)
ADHD medication	11 (1.3%)	8 (1.5%)	3 (1.3%)	0 (0%)	0 (0%)
Vitamin/mineral supplements	684 (78%)	402 (76%)	191 (84%)	23 (72%)	61 (85%)
Pancreatic enzyme replacement	682 (78%)	392 (74%)	198 (87%)	21 (66%)	64 (89%)
Sodium chloride	392 (45%)	265 (50%)	108 (47%)	4 (13%)	12 (17%)
Bronchodilator (Salbutamol)	373 (43%)	179 (34%)	125 (55%)	15 (47%)	49 (68%)
Proton pump inhibitor	182 (21%)	89 (17%)	54 (24%)	6 (19%)	31 (43%)
Insulin medication	84 (9.6%)	30 (5.6%)	26 (11%)	8 (25%)	20 (28%)
Ursodeoxycholic acid	73 (8.4%)	32 (6.0%)	25 (11%)	4 (13%)	11 (15%)
Iron supplement	66 (7.6%)	34 (6.4%)	15 (6.6%)	3 (9.4%)	13 (18%)

¹ n (%)

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%)	228 (100%)	0 (0%)	72 (100%)
Staphylococcus species	498 (57%)	306 (58%)	145 (64%)	10 (31%)	33 (46%)
Haemophilus species	193 (22%)	135 (25%)	51 (22%)	2 (6.3%)	5 (6.9%)
Stenotrophomonas species	101 (12%)	36 (6.8%)	49 (21%)	5 (16%)	11 (15%)
Mycobacterium species	42 (4.8%)	18 (3.4%)	18 (7.9%)	2 (6.3%)	4 (5.6%)
Streptococcus pneumoniae	39 (4.5%)	29 (5.5%)	7 (3.1%)	0 (0%)	3 (4.2%)
Streptococcus pyogenes	18 (2.1%)	10 (1.9%)	7 (3.1%)	0 (0%)	1 (1.4%)
Streptococcus species (other)	14 (1.6%)	10 (1.9%)	3 (1.3%)	0 (0%)	1 (1.4%)
Escherichia species	35 (4.0%)	27 (5.1%)	7 (3.1%)	0 (0%)	1 (1.4%)
Klebsiella species	34 (3.9%)	19 (3.6%)	14 (6.1%)	1 (3.1%)	0 (0%)
Chryseobacterium species	31 (3.6%)	11 (2.1%)	15 (6.6%)	2 (6.3%)	3 (4.2%)
Enterobacter species	32 (3.7%)	20 (3.8%)	12 (5.3%)	0 (0%)	0 (0%)
Moraxella species	27 (3.1%)	21 (4.0%)	6 (2.6%)	0 (0%)	0 (0%)
Acinetobacterium species	26 (3.0%)	12 (2.3%)	13 (5.7%)	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%)
Burkholderia species	20 (2.3%)	13 (2.4%)	1 (0.4%)	1 (3.1%)	5 (6.9%)
Other	96 (11%)	46 (8.7%)	43 (19%)	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%)	4 (5.6%)
Parainfluenza (PIV)	16 (1.8%)	8 (1.5%)	7 (3.1%)	0 (0%)	1 (1.4%)
Respiratory Syncytial Virus (RSV)	16 (1.8%)	10 (1.9%)	6 (2.6%)	0 (0%)	0 (0%)
Enterovirus	8 (0.9%)	5 (0.9%)	3 (1.3%)	0 (0%)	0 (0%)
Adenovirus	6 (0.7%)	3 (0.6%)	3 (1.3%)	0 (0%)	0 (0%)
Influenza	5 (0.6%)	4 (0.8%)	1 (0.4%)	0 (0%)	0 (0%)
Other	3 (0.3%)	2 (0.4%)	0 (0%)	0 (0%)	1 (1.4%)
Fungi					
Aspergillus species	196 (22%)	93 (18%)	83 (36%)	4 (13%)	13 (18%)
Candida species	175 (20%)	84 (16%)	63 (28%)	7 (22%)	20 (28%)
Other	104 (12%)	58 (11%)	36 (16%)	2 (6.3%)	6 (8.3%)

¹ n (%)