

# BEATCF enrolment tables

Edward Pan

03 April, 2024

Table 1: Demographics and Clinical Baseline Characteristics

Characteristic	Overall, N = 872	ppFEV1≥70 No Pseudomonas N=531	ppFEV1≥70 Pseudomonas N=228	ppFEV1<70 No Pseudomonas N=32	ppFEV1<70 Pseudomonas N=72
<b>Median age in years (IQR)</b>					
Median (IQR)	11 (6, 17)	10 (5, 14)	13 (7, 17)	29 (17, 42)	32 (23, 43)
<b>Age Groups</b>					
<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%)
<b>Mean height in cm (SD)</b>	141 (31)	135 (31)	144 (29)	162 (21)	164 (14)
(Missing)	1	1	0	0	0
<b>Mean weight in kilograms (SD)</b>	43 (23)	38 (22)	44 (22)	62 (22)	63 (17)
<b>BMI kg/m<sup>2</sup> (SD)</b>	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
<b>Sex</b>					
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%)
<b>Genotype</b>					
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
<b>Sweat Chlorine Level in mmol/L</b>					
<40	27 (3.2%)	21 (4.1%)	5 (2.2%)	1 (3.2%)	0 (0.0%)
40-<60	20 (2.4%)	19 (3.7%)	1 (0.4%)	0 (0.0%)	0 (0.0%)
≥60	172 (20.3%)	113 (21.9%)	48 (21.5%)	5 (16.1%)	3 (4.3%)
Concurrent CFTR modulators	263 (31.0%)	134 (25.9%)	68 (30.5%)	18 (58.1%)	41 (59.4%)
Enrolment before Aug2022	367 (43.2%)	230 (44.5%)	101 (45.3%)	7 (22.6%)	25 (36.2%)
(Missing)	23	14	5	1	3
<b>Pancreatic Insufficiency</b>	638 (73.2%)	374 (70.4%)	188 (82.5%)	16 (50.0%)	53 (73.6%)
<b>CF diabetes mellitus</b>	110 (12.6%)	38 (7.2%)	38 (16.7%)	10 (31.3%)	24 (33.3%)
<b>Highest ppFEV in previous 12 months</b>	90 (20)	97 (12)	95 (13)	55 (11)	52 (11)
(Missing)	158	114	35	0	0
<b>Highest ppFEV in the previous 6 months</b>	88 (20)	95 (13)	93 (13)	54 (11)	50 (12)
(Missing)	176	127	39	0	1

<sup>1</sup> n (%); Mean (SD)

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

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

<sup>1</sup> Mean (SD)Table 3: **CFQR Adolescents and Adults at Enrolment (60 days)**

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

<sup>1</sup> Mean (SD)

Table 4: CFQR Parents and Caregivers at Enrolment (60 days)

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

<sup>1</sup> 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
<b>Antibiotics</b>					
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%)
<b>Antifungal</b>	30 (3.4%)	12 (2.3%)	11 (4.8%)	2 (6.3%)	4 (5.6%)
<b>Corticosteroids</b>					
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%)
<b>Muco-active agents</b>					
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%)
<b>CFTR modulators</b>					
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%)
<b>Antidepressants/Antipsychotic</b>					
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%)
<b>Vitamin/mineral supplements</b>	684 (78.4%)	402 (75.7%)	191 (83.8%)	23 (71.9%)	61 (84.7%)
<b>Pancreatic enzyme replacement</b>	683 (78.3%)	392 (73.8%)	199 (87.3%)	21 (65.6%)	64 (88.9%)
<b>Sodium chloride</b>	393 (45.1%)	265 (49.9%)	109 (47.8%)	4 (12.5%)	12 (16.7%)
<b>Bronchodilator (Salbutamol)</b>	373 (42.8%)	179 (33.7%)	125 (54.8%)	15 (46.9%)	49 (68.1%)
<b>Proton pump inhibitor</b>	182 (20.9%)	89 (16.8%)	54 (23.7%)	6 (18.8%)	31 (43.1%)
<b>Insulin medication</b>	84 (9.6%)	30 (5.6%)	26 (11.4%)	8 (25.0%)	20 (27.8%)
<b>Ursodeoxycholic acid</b>	73 (8.4%)	32 (6.0%)	25 (11.0%)	4 (12.5%)	11 (15.3%)
<b>Iron supplement</b>	66 (7.6%)	34 (6.4%)	15 (6.6%)	3 (9.4%)	13 (18.1%)

<sup>1</sup> n (%)<sup>2</sup> Out of the antibiotics listed above there were 5 Tobramycin, 1 Flucloxacillin intravenous cases.

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
<b>Bacterial pathogen</b>					
Pseudomonas species	305 (35.0%)	0 (0.0%)	228 (100.0%)	0 (0.0%)	72 (100.0%)
Staphylococcus species (MRSA)	39 (4.5%)	17 (3.2%)	12 (5.3%)	1 (3.1%)	7 (9.7%)
Staphylococcus species (MSSA)	303 (34.7%)	186 (35.0%)	95 (41.7%)	6 (18.8%)	13 (18.1%)
Staphylococcus species (Unknown)	196 (22.5%)	120 (22.6%)	49 (21.5%)	7 (21.9%)	19 (26.4%)
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	39 (4.5%)	29 (5.5%)	7 (3.1%)	0 (0.0%)	3 (4.2%)
Streptococcus pyogenes	18 (2.1%)	10 (1.9%)	7 (3.1%)	0 (0.0%)	1 (1.4%)
Streptococcus species (other)	14 (1.6%)	10 (1.9%)	3 (1.3%)	0 (0.0%)	1 (1.4%)
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%)
<b>Viral pathogen</b>					
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%)
<b>Fungi</b>					
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%)

<sup>1</sup> n (%)