**Table 2. Comparison of clinical characteristics of children with CF between trajectory groups**

| **Characteristics** | **Overall (N=779)** | **Group “low” (N=553)** | **Group “high” (N=200)** | **Group “highest” (N=26)** | **P-value** |
| --- | --- | --- | --- | --- | --- |
| **Gender** |  |  |  |  |  |
| Male | 385 (49.4%) | 280 (50.6%) | 94 (47.0%) | 11 (42.3%) | 0.517 |
| Female | 394 (50.6%) | 273 (49.4%) | 106 (53.0%) | 15 (57.7%) |  |
| **Circumstances of CF diagnosis** |  |  |  |  |  |
| Newborn screening | 601 (77.2%) | 437 (79.0%) | 144 (72.0%) | 20 (76.9%) | 0.300a |
| Meconium ileus | 101 (13.0%) | 66 (11.9%) | 33 (16.5%) | <11 |  |
| Symptoms-based | 38 (4.9%) | 26 (4.7%) | <11 | <11 |  |
| Antenatal | 39 (5.0%) | 24 (4.3%) | 13 (6.5%) | <11 |  |
| **CFTR mutations** |  |  |  |  |  |
| F508del/F508del | 308 (39.5%) | 215 (38.9%) | 80 (40.0%) | 13 (50.0%) | 0.391a |
| F508del/Other | 332 (42.6%) | 243 (43.9%) | 79 (39.5%) | <11 |  |
| F508del/Gating | <11 | <11 | 0 (0%) | 0 (0%) |  |
| Others | 132 (16,9%) | 88 (15,9%) | 41 (20,5%) | <11 |  |
| **Anthropometricsc** |  |  |  |  |  |
| Weight z-score, at 2 y | -0.6 (1.1) | -0.5 (1.0) | -0.7 (1.2) | -1.2 (1.6) | 0.014 |
| Weight z-score, at 6 y | -0.4 (1.0) | -0.4 (1.0) | -0.5 (1.0) | -0.6 (0.7) | 0.163 |
| Weight z-score, at 10 y | -0.5 (1.0) | -0.5 (1.0) | -0.6 (1.0) | -1.0 (0.7) | 0.05 |
| Height z-score at 2 y | -0.3 (1.0) | -0.3 (1.0) | -0.3 (1.2) | -0.8 (1.2) | 0.134 |
| Height z-score at 6 y | -0.2 (1.0) | -0.2 (1.0) | -0.2 (1.0) | -0.6 (0.9) | 0.182 |
| Height z-score at 10 y | -0.4 (1.0) | -0.3 (0.9) | -0.4 (1.0) | -0.9 (0.9) | 0.022 |
| **Pulmonary function testsc** |  |  |  |  |  |
| %pred FEV1 at 6 y | 102.0 (17.8) | 103.3 (17.1) | 100.4 (19.3) | 89.7 (16.9) | 0.002 |
| %pred FEV1 at 10 y | 98.5 (16.8) | 100.5 (14.8) | 95.4 (19.0) | 73.1 (20.1) | <0.0001 |
| **Comorbidities over 10 yearsb** |  |  |  |  |  |
| Pancreatic insufficiency | 693 (89.0%) | 473 (85.5%) | 195 (97.5%) | 25 (96.2%) | <0.0001 |
| Distal intestinal obstruction syndrome occurrence | 96 (12.3%) | 69 (12.5%) | 26 (13.0%) | <11 | 0.401 |
| CF-related liver disease | 107 (13.7%) | 70 (12.7%) | 34 (17.0%) | <11 | 0.294 |
| CF-related diabetes (treated or not with insulin) | 36 (4.6%) | 13 (2.4%) | 18 (9.0%) | <11 | <0.0001 |
| **Microbiology over 10 years**b |  |  |  |  |  |
| Age at first PA colonizationc | 3.4 (2.9) | 3.9 (3.1) | 2.7 (2.4) | 2.2 (1.7) | <0.0001 |
| Chronic PA infection | 103 (13.2%) | 33 (6.0%) | 57 (28.5%) | 13 (50.0%) | <0.0001 |
| Allergic bronchopulmonary aspergillosis d | 52 (6.7%) | 25 (4.5%) | 23 (11.5%) | <11 | <0.001 |
| *Haemophilus influenzae* colonization | 692 (88.8%) | 490 (88.6%) | 178 (89.0%) | 24 (92.3%) | 0.839 |
| *Staphylococcus aureus* colonization | 767 (98.5%) | 541 (97.8%) | 200 (100.0%) | 26 (100.0%) | 0.108a |
| **Nebulized treatment prescribed over 10 years**b |  |  |  |  |  |
| Colistin d | 139 (17.8%) | 50 (9.0%) | 75 (37.5%) | 14 (53.9%) | <0.0001 |
| Tobramycin d | 181 (23.2%) | 92 (16.6%) | 79 (39.5%) | <11 | <0.0001 |
| RhDNase e | 481 (61.8%) | 320 (57.9%) | 145 (72.5%) | 16 (61.5%) | 0.001 |
| **Intravenous (IV) antibiotic therapy use** |  |  |  |  |  |
| **During the sixth year of life** |  |  |  |  |  |
| Occurrence of at least one IV therapy | 76 (9.8%) | 17 (3.1%) | 40 (20.0%) | 19 (73.1%) | <0.0001 |
| Mean IV therapies administered yearly | 1.6 (0.9) | 1.1 (0.3) | 1.5 (0.7) | 2.3 (1.3) | <0.001 |
| **During the tenth year of life** |  |  |  |  |  |
| Occurrence of at least one IV therapy | 128 (16.4%) | 48 (8.7%) | 64 (32.0%) | 16 (61.5%) | <0.0001 |
| Mean IV therapies administered yearly | 1.8 (1.3) | 1.3 (0.6) | 1.8 (1.1) | 3.6 (2.1) | <0.0001 |
| Abbreviations: PA: Pseudomonas aeruginosa.  Data comes from the FCFR and are presented as mean (SD) or N(%). (Reviewer #2, Comments #2; Reviewer #2, Comment #16)  The "<11" designation is employed for statistical confidentiality, as figures below 11 typically should not be disclosed. Nevertheless, this restriction does not hinder the execution of comparison tests.  P value is for non-parametric chi-square test (or Fisher's exact test if a theoretical number less than 6) or ANOVA  Comparison tests do not include missing data  a. Fisher’s exact test  b. At least one instance within the 10-year follow-up timeframe.  c. Data available for 70-80% of patients  d. Variable available since 2017  e. Variable not available between 2013 and 2016 inclusive | | | | | |