### Supplementary table 3. Results of initial and annual evaluations of non-ambulant participants.

|  |  |  |  |  |  |  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
|  | | | pre | | 1 year | | 2 year | | 3 year | | 4 year | | 5 year | |
|  | n |  | n |  | n |  | n |  | n |  | n |
| physical evaluation | summed MMT | | 24.5 +/- 16.2 | 20 | 23.4 +/- 15.2\*\* | 18 | 23.6 +/- 13.1\*\* | 15 | 17.3 +/- 11.2\* | 13 | 19.3 +/- 11.3\* | 9 | 18.4 +/- 11.2\*\* | 13 |
|  | GMFM (%) | | 22.4 +/- 23.3 | 17 | 19.2 +/- 20.4 \* | 16 | 15.6 +/- 16.2\* | 14 | 8.8 +/- 8.4 | 11 | 7.2 +/- 5.6 | 8 | 7.0 +/- 6.8\* | 11 |
|  | HHD (N) | | 101.5 +/- 74.5 | 10 | 91.2 +/- 91.1 | 10 | 86.8 +/- 103.1 | 7 | 18.7 +/- 25.1 | 5 | 22.6 +/- 26.1 | 4 | 36.7 +/- 65.1 | 5 |
|  | Grip power (kg) | | 3.1 +/- 2.9 | 10 | 2.9 +/- 2.7 | 10 | 2.3 +/- 2.3 | 10 | 2.3 +/- 2.2 | 7 | 1.8 +/- 1.7 | 6 | 1.8 +/- 2.2 | 8 |
|  | Pinch power (N) | | 15.5 +/- 18.7 | 15 | 10.8 +/- 13.4 | 14 | 8.2 +/- 10.4\* | 14 | 7.7 +/- 11.2\* | 10 | 6.3 +/- 9.4 | 8 | 6.1 +/- 8.7\* | 11 |
| respitaroy function | FVC (%) | | 75.4 +/- 23.3 | 20 | 71.0 +/- 24.3\* | 19 | 76.1 +/- 20.5\* | 15 | 66.6 +/- 21.6\*\* | 13 | 65.2 +/- 17.1\* | 8 | 62.3 +/- 27.6\*\* | 15 |
| VC (%) | | 75.2 +/- 22.2 | 20 | 72.4 +/- 24.4 | 19 | 78.2 +/- 19.5 | 15 | 69.5 +/- 19.0\* | 13 | 67.1 +/- 15.6\*\* | 8 | 64.3 +/- 26.0\*\* | 15 |
| DEXA | Whole-body lean body mass (kg) | | 28.8 +/- 7.1 | 20 | 28.6 +/- 7.8 | 18 | 28.4 +/- 8.3 | 14 | 27.6 +/- 6.2 | 14 | 25.2 +/- 5.3 | 9 | 26.6 +/- 5.8 | 14 |
| Arm lean body mass (kg) | | 2.4 +/- 0.9 | 20 | 2.4 +/- 1.0 | 18 | 2.4 +/- 1.0 | 14 | 2.5 +/- 0.9 | 14 | 2.2 +/- 0.6 | 9 | 2.3 +/- 0.9 | 14 |
| Leg lean body mass (kg) | | 7.5 +/- 2.4 | 20 | 7.7 +/- 2.7 | 18 | 7.7 +/- 2.9 | 14 | 7.5 +/- 2.1 | 14 | 6.8 +/- 1.9 | 9 | 6.8 +/- 1.6 | 14 |
| CK (IU/L) | | | 138.4 +/- 133.3 | 20 | 155.4 +/- 167.6 | 18 | 103.7 +/- 87.8\*\* | 15 | 63.2 +/- 57.7\* | 12 | 68.6 +/- 62.7 | 9 | 70.4 +/- 80.9 | 15 |
| ADL score | Barthel index | | 28.0 +/- 25.6 | 20 | 27.9 +/- 24.6 | 19 | 32.9 +/- 28.5 | 14 | 11.0 +/- 2.2 | 5 | 21.1 +/- 11.9 | 9 | 19.6 +/- 16.0 | 14 |
| mRS | | 4.3 +/- 0.8 | 20 | 4.1 +/- 0.7 | 19 | 4.2 +/- 0.6 | 15 | 4.4 +/- 0.5 | 7 | 4.1 +/- 0.6 | 9 | 4.3 +/- 0.5 | 15 |
| QOL score | SF36 | Pyshical functioning | -12.2 +/- 5.0 | 20 | -13.2 +/- 2.8 | 18 | -12.9 +/- 3.5 | 10 | -13.8 +/- 1.5 | 6 | -14.4 +/- 0.0 | 6 | -14.4 +/- 0.0 | 12 |
| Role physichal | 33.0 +/- 22.5 | 20 | 29.5 +/- 21.2 | 18 | 25.8 +/- 22.6 | 10 | 26.4 +/- 22.7 | 6 | 12.0 +/- 13.3 | 6 | 27.8 +/- 21.5 | 12 |
| Bodily pain | 44.2 +/- 12.3 | 20 | 41.1 +/- 10.2 | 18 | 47.4 +/- 9.7 | 10 | 37.5 +/- 8.8 | 6 | 43.0 +/- 9.9 | 6 | 41.7 +/- 11.4 | 12 |
| Social functioning | 37.7 +/- 15.9 | 20 | 37.0 +/- 16.2 | 18 | 34.5 +/- 16.7 | 10 | 24.8 +/- 10.8 | 6 | 28.0 +/- 19.0 | 6 | 37.2 +/- 18.3 | 12 |
| General health perceptions | 42.0 +/- 8.6 | 20 | 40.1 +/- 10.1 | 18 | 40.7 +/- 6.3 | 10 | 38.4 +/- 4.0 | 6 | 38.5 +/- 6.7 | 6 | 38.3 +/- 7.9\* | 12 |
| Vitality | 45.5 +/- 8.9 | 20 | 40.2 +/- 9.9\* | 18 | 42.8 +/- 8.3 | 10 | 40.2 +/- 3.5 | 6 | 39.7 +/- 7.4 | 6 | 42.1 +/- 10.7 | 12 |
| Role emotinoal | 42.3 +/- 17.8 | 20 | 37.4 +/- 19.7 | 18 | 38.2 +/- 20.7 | 10 | 42.2 +/- 8.6 | 6 | 36.0 +/- 23.6 | 6 | 33.9 +/- 22.2 | 12 |
| Mental health | 47.7 +/- 8.8 | 20 | 44.7 +/- 8.6 | 18 | 46.2 +/- 10.5 | 10 | 42.0 +/- 4.4 | 6 | 43.3 +/- 4.3 | 6 | 46.5 +/- 10.1 | 12 |

### P-values are calculated relative to baseline data. \*: p<0.05, \*\*: p<0.01