**Supplemental Material:**

**Supplemental Text**:

Data pre-processing: For timed function outcomes also in the Northstar Ambulatory Assessment, two levels of grading were available: based on the NSAA test (corresponding to levels 0, 1, and 2) as well as the timed tunction test (corresponding to levels 1 through 6). For time to climb and time to stand from supine tests, only grade 2 or higher attempts had times recorded while for run/walk 10 m test, only grade 3 or higher attempts had times recorded. NSAA data was only collected when a subject was deemed ambulatory; in all other cases, EK responses were collected.

**Supplemental Figures:**

**Supplemental Figure 1: Distribution of mutations**

**A graph with numbers and a bar graph

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**Supplemental Figure 2. Spearman correlation analysis of medians of each subject for each functional outcome test and age.**

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**Supplementary Figure 3. Overall trajectory plots of change in outcomes for participants in the Becker Natural History**

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**Supplemental Figure 4. Outcome individual trajectories stratified by enrollment age (< vs. ≥ 18 years of age for motor outcomes). The expected model fit for an individual with fixed baseline values based on model estimates are overlaid.**

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**Supplemental Table 1: Summary baseline characteristics by genotype (at first visit)\***

|  |  |  |  |
| --- | --- | --- | --- |
| **Characteristic mean (SD); available n** | **45-47 (n=25)** | **45-48 (n=17)** | **Other (n=41)** |
| *Age in years; n = 83* | 35.7 (17.9) | 30.2 (18.7) | 24.7 (16.5) |
| *TTSTAND (seconds) n=63* | 12.7 (22.6) | 5.7 (5.1) | 3.9 (3.6) |
| *STAND velocity (rises/s); n=63* | 0.2 (0.1) | 0.3 (0.2) | 0.4 (0.2) |
| *TTCLIMB (seconds); n=64* | 5.9 (5.5) | 6.3 (7.4) | 2.8 (2.1) |
| *CLIMB velocity (stairs/s); n=64* | 1.1 (0.7) | 1.4 (1.0) | 2.0 (0.9) |
| *TTRW (seconds); n=66* | 7.9 (3.2) | 7.7 (5.4) | 4.4 (2.3) |
| *RW velocity (m/s); n=66* | 1.5 (0.8) | 1.9 (1.1) | 2.7 (0.9) |
| *6MWD (m); n=64* | 362.3 (87.2) | 394.1 (145.0) | 489.5 (107.5) |
| *NSAA (units); n=67* | 21.8 (8.4) | 24.5 (10.4) | 29.8 (6.8) |
| *Standing height (cms); n=66* | 171.2 (16.4) | 159.8 (25.6) | 158.7 (19.5) |
| *Weight (kgs); n=82* | 72.3 (17.9) | 60.9 (27.0) | 66.6 (30.5) |
| *% predicted FVC; n=75* | 86.9 (15.1) | 89.8 (14.5) | 83.0 (20.7) |
| *PUL 1.2 score; n=63* | 78.1 (3.0) | 79.0 (1.8) | 79.1 (1.8) |
| *Ambulatory* | 88% | 94% | 78% |
| *% Minor; n = 83* | 20% | 35.3% | 51.2% |

*Note that the large differences in baseline age between different genotype groups. Abbreviations: TTSTAND: time to stand from supine; TTCLIMB: time to climb 4 steps; TTRW: time to run/walk 10m; 6MWD: six-minute walk distance; NSAA: NorthStar Ambulatory Assessment; FVC: Forced vital capacity; PUL 1.2: Performance of Upper Limb version 1.2*