# SUPPLEMENTARY DATA

Table S1. Baseline characteristics for *post-hoc* 6MWT-matched comparator analysis

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| **Characteristic** | **PROMOVI trial**a  **(*n*** **=** **42)** | **Eteplirsen studies 201/202**b  **(*n*** **=** **12)** |
| Age, years |  |  |
| Mean ± SD | 9.0 ± 2.1 | 9.5 ± 1.2 |
| Min, max | 7.0, 13.0 | 7.4, 11.0 |
| 6MWT distance, m |  |  |
| Mean ± SD | 389.3 ± 41.9 | 363.2 ± 42.2 |
| Min, max | 301.0, 450.0 | 256.0, 416.0 |
| NSAA total score |  |  |
| Mean ± SD | 25.0 ± 4.2 | 24.9 ± 4.9 |
| Min, max | 17.0, 31.0 | 17.0, 31.0 |
| 10-m run, s |  |  |
| Mean ± SD | 5.2 ± 0.8 | 6.2 ± 1.5 |
| Min, max | 3.8, 7.2 | 3.9, 8.7 |
| Age at start of corticosteroid use, y |  |  |
| Mean ± SD | 5.8 ± 1.9 | 5.1 ± 1.1 |
| Min, max | (1.9, 10.0) | 3.4, 6.6 |
| Duration of corticosteroid use, mo |  |  |
| Mean ± SD | 43.0 ± 28.4 | 52.1 ± 24.1 |
| Min, max | 5.7, 120.4 | 15.5, 91.7 |
| Time since DMD diagnosis, mo |  |  |
| Mean ± SD | 59.4 ± 33.0 | 58.3 ± 26.0 |
| Min, max | 5.5, 131.2 | 18.0, 112.0 |

Abbreviations: DMD = Duchenne Muscular Dystrophy; 6MWT = 6-minute walk test; NSAA = North Star Ambulatory Assessment; SD = standard deviation.

a Primary efficacy subgroup for comparison to study 201/202 consisted of all treated patients with ≥1 post-baseline assessment who have a baseline 6MWT distance of 300–450 m, baseline NSAA score 17–31, and age 7–13 years.

b Eteplirsen studies 201 (NCT01396239) [24] and 202 (NCT01540409) [28].

Table S2. Baseline characteristics for *post-hoc* FVC%p-matched comparator analysis

|  |  |  |  |
| --- | --- | --- | --- |
| **Characteristic** | **PROMOVI trial**a **(*n*** **=** **52)** | **CINRG DNHS exon 51 cohort**b  **(*n*** **=** **20)** | **Eteplirsen studies 201/202**c **(*n*** **=** **12)** |
| Age at baseline, y |  |  |  |
| Mean ± SD | 11.0 ± 1.4 | 11.8 ± 2.2 | 10.3 ± 0.3 |
| Min, max | 10.0, 16.3 | 10.0, 17.9 | 10.0, 11.0 |
| Height at baseline,d cm |  |  |  |
| Mean ± SD | 138.3 ± 7.7 e | 140.8 ± 12.1 | 126.1 ± 7.6 |
| Min, max | 122.4, 155.2 | 124.0, 178.1 | 116.0, 140.5 |
| FVC%p at baseline,d % |  |  |  |
| Mean ± SD | 78.5 ± 14.5e | 79.6 ± 13.3 | 96.9 ± 14.0 |
| Min, max | 52.6, 127.0 | 50.0, 106.0 | 84.0, 121.0 |

Abbreviations: FVC%p = percent predicted forced vital capacity; SD = standard deviation.

a The analysis set included all treated patients with assessments falling into the age group of 10 to <18 years.

b Untreated patients in the CINRG DNHS exon 51 cohort (age 10–18 years) [11].

c Eteplirsen studies 201 (NCT01396239) [24] and 202 (NCT01540409) [28].

d PROMOVI trial and CINRG DNHS used ulnar length calculated height, studies 201/202 used actual standing height.

e *n* = 51.