**SUPPLEMENTARY MATERIALS**

**Table S1**

Eteplirsen-treated and CINRG DNHS cohorts: demographic and selected variables at baseline and last visit

| **Parameter** | | **Patients Aged 10 to <18 Years** | | | | **Studies 201/202 (N=12)** | | **Study 204 (N=20)** | **Study 301 (N=42)** | **Exon 51 CINRG** **DNHS  (n=20)** | **Genotyped**  **CINRG** **DNHS**  **(n=148)** | | **All**  **CINRG DNHS**  **(N=172)** | |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
| Race, n (%) | White | | | | | 11 (92) | | 19 (95) | 35 (83) | 16 (80) | 116 (78) | | 134 (78) | |
|  | Asian | | | | | 1 (8) | | 1 (5) | 2 (5) | 3 (15) | 19 (13) | | 24 (14) | |
|  | Black | | | | | 0 | | 0 | 1 (2) | 0 | 2 (1) | | 2 (1) | |
|  | Native Hawaiian or other Pacific Islander | | | | | 0 | | 0 | 2 (5) | 0 | 2 (1) | | 2 (1) | |
|  | Other | | | | | 0 | | 0 | 2 (5) | 1 (5) | 9 (6) | | 10 ( 6) | |
| Ethnicity, n (%) | Hispanic or Latino | | | | | 0 | | 1 (5) | 4 (10) | 1 (5) | 20 (14) | | 23 (13) | |
|  | Not Hispanic or Latino | | | | | 12 (100) | | 18 (90) | 37 (88) | 19 (95) | 128 (86) | | 149 (87) | |
|  | Not reported | | | | | 0 | | 1 (5) | 0 | 0 | 0 | | 0 | |
|  | Unknown | | | | | 0 | | 0 | 1 (2) | 0 | 0 | | 0 | |
| Age at baseline, years | Mean (SD) | | | | | 10.32 (0.301) | | 13.04 (2.276) | 11.07 (1.440) | 11.78 (2.235) | 11.73 (1.971) | | 11.65 (1.909) | |
|  | Median | | | | | 10.24 | | 12.97 | 10.37 | 10.40 | 10.86 | | 10.83 | |
|  | Min, max | | | | | 10.0, 11.0 | | 10.0, 17.3 | 10.0, 16.3 | 10.0, 17.9 | 10.0, 17.9 | | 10.0, 17.9 | |
| Age at last visit, years | Mean (SD) | | | | | 13.89 (1.177) | | 14.51 (2.426) | 12.15 (1.822) | 14.82 (2.497) | 14.89 (2.471) | | 14.64 (2.49) | |
|  | Median | | | | | 14.38 | | 14.80 | 11.71 | 15.01 | 15.18 | | 14.90 | |
|  | Min, max | | | | | 11.9, 15.2 | | 10.3, 18.0 | 10.2, 17.7 | 10.4, 17.9 | 10.0, 18.0 | | 10.0, 18.0 | |
| Standing height at baseline, cm | n | | | | | 11 | | 8 | 39 | 13 | 87 | | 106 | |
|  | | | | | Mean (SD) | | | 126.12 (7.596) | 127.30 (11.286) | 130.46 (7.244) | 131.45 (7.604) | 130.54  (8.381) | | 129.88  (8.195) |
|  | Median | | | | | 124.90 | | 126.50 | 130.70 | 131.60 | 130.00 | | 129.75 | |
|  | Min, max | | | | | 116.0, 140.5 | | 113.6, 150.0 | 116.7, 148.5 | 119.0, 145.0 | 114.0, 157.3 | | 114.0, 157.3 | |
| Standing height at last visit, cm | n | | | | | 12 | | 7 | 35 | 8 | 47 | | 59 | |
| Mean (SD) | | | | | 137.36 (13.757) | | 138.57 (18.959) | 133.27 (9.581) | 130.93 (8.364) | 135.12  (11.194) | | 134.05  (10.695) | |
|  | Median | | | | | 132.95 | | 133.00 | 132.00 | 128.65 | 130.60 | | 130.80 | |
|  | Min, max | | | | | 122.4, 163.6 | | 117.0, 164.6 | 117.8, 156.5 | 122.0, 149.5 | 116.1, 164.5 | | 116.1, 164.5 | |
| Calculated height at baseline, cm | n | | | | | 0 | | 20 | 41 | 20 | 148 | | 172 | |
| Mean (SD) | | | | |  | | 141.67 (10.742) | 137.74 (7.891) | 140.76 (12.063) | 139.76  (10.595) | | 139.21  (10.411) | |
|  | Median | | | | |  | | 138.94 | 138.97 | 140.65 | 138.25 | | 138.20 | |
|  | Min, max | | | | |  | | 126.7, 158.5 | 122.4, 155.2 | 124.0, 178.1 | 120.4, 178.1 | | 120.4, 178.1 | |
| Calculated height at last visit, cm | n | | | | | 0 | | 20 | 42 | 20 | 148 | | 172 | |
| Mean (SD) | | | | |  | | 147.28 (11.293) | 142.07 (9.415) | 150.91 (13.396) | 149.83  (12.176) | | 148.87  (11.984) | |
|  | Median | | | | |  | | 143.36 | 141.81 | 150.70 | 148.85 | | 147.70 | |
|  | Min, max | | | | |  | | 131.2, 165.2 | 124.9, 162.1 | 131.0, 178.1 | 124.1, 184.4 | | 124.1, 184.4 | |
| Weight at baseline, kg | | | Mean (SD) | | | 35.78 (6.510) | | 48.71 (12.620) | 39.83 (11.159) | 38.87 (14.783) | 40.03  (12.602) | | 39.96  (12.348) | |
|  | Median | | | | | 34.75 | | 48.50 | 39.30 | 32.25 | 37.35 | | 37.50 | |
|  | Min, max | | | | | 27.9, 46.0 | | 25.6, 69.6 | 22.2, 67.2 | 23.5, 79.1 | 18.0, 79.5 | | 18.0, 79.5 | |
| Weight at last visit, kg | Mean (SD) | | | | | 48.43 (16.747) | | 54.65 (13.838) | 46.40 (13.860) | 46.81 (15.202) | 49.07  (15.331) | | 48.59  (14.970) | |
|  | Median | | | | | 44.15 | | 53.15 | 48.75 | 43.30 | 46.60 | | 46.00 | |
|  | Min, max | | | | | 29.3, 80.2 | | 30.5, 88.7 | 22.2, 77.5 | 26.5, 79.1 | 26.0, 111.8 | | 23.9, 111.8 | |
| Ambulatory status at baseline, n (%) | Not ambulatory | | | | | 0 | | 15 (75) | 0 | 3 (15) | 49 (33) | | 52 (30%) | |
| Ambulatory | | | | | 12 (100) | | 5 (25) | 42 (100) | 17 (85) | 99 (67) | | 120 (70) | |
| Ambulatory status at last visit, n (%) | Not ambulatory | | | | | 2 (17) | | 19 (95) | 5 (12) | 9 (45) | 86 (58) | | 98 (57) | |
| Ambulatory | | | | | 10 (83) | | 1 (5) | 37 (88) | 11 (55) | 62 (42) | | 74 (43) | |
| FVC%p at baseline | | | | Mean (SD) | | | 96.92 (14.042) | 65.94 (16.601) | 78.48 (15.691) | 79.60 (13.300) | 78.81  (19.497) | | 78.45  (19.245) | |
|  | Median | | | | | 92.00 | | 72.25 | 80.31 | 81.00 | 79.00 | | 78.00 | |
|  | Min, max | | | | | 84.0, 121.0 | | 31.4, 89.7 | 52.6, 127.0 | 50.0, 106.0 | 14.0, 140.0 | | 14.0, 140.0 | |
| FVC%p at last visit | Mean (SD) | | | | | 86.83 (17.513) | | 61.62 (18.079) | 74.60 (12.636) | 61.90 (21.829) | 62.26  (22.184) | | 62.28  (21.143) | |
|  | Median | | | | | 81.50 | | 67.68 | 70.76 | 64.50 | 63.00 | | 62.50 | |
|  | Min, max | | | | | 61.0, 112.0 | | 31.2, 84.2 | 55.9, 102.9 | 16.0, 104 | 12.0, 122.0 | | 12.0, 122.0 | |

CINRG DNHS, Cooperative International Neuromuscular Research Group Duchenne Natural History Study; DMD, Duchenne muscular dystrophy; FVC%p, percent predicted forced vital capacity; max, maximum; min, minimum; SD, standard deviation.

**Table S2**

FVC%p slope model sensitivity analysis: studies 201/202 versus the Exon 51 CINRG DNHS cohort, with baseline height as a covariate

| **Number of Patients Aged 10 to <18 Years** | **Studies 201/202 (N=11)\*** | **Exon 51 CINRG DNHS (N=20)** |
| --- | --- | --- |
| Number of observations | 118 | 88 |
| FVC%p annual change (SE) | −1.54 (0.757) | −6.05 (0.409) |
| 95% CI | −3.04, −0.05 | −6.86, −5.25 |
| Difference in FVC%p annual change (SE) |  | 4.51 (0.860) |
| 95% CI |  | 2.81, 6.21 |
| *P* value |  | < 0.001 |

CI, confidence interval; CINRG DNHS, Cooperative International Neuromuscular Research Group Duchenne Natural History Study; FVC%p, percent predicted forced vital capacity; SE, standard error.

**\***One of the 12 patients in the age range of 10 to <18 years had a missing height value at the baseline visit. Therefore, this patient was excluded from the sensitivity analysis that included baseline height as a covariate.

**Table S3**

FVC%p slope model sensitivity analysis: studies 201/202 versus the Exon 51 CINRG DNHS cohort, with random slope for patients

| **Number of Patients Aged 10 to <18 Years** | **Studies 201/202 (N=12)** | **Exon 51 CINRG DNHS (N=20)** |
| --- | --- | --- |
| Number of observations | 132 | 88 |
| FVC%p annual change (SE) | −2.17 (0.758) | −5.62 (0.460) |
| 95% CI | −3.72, -0.61 | −6.56, −4.67 |
| Difference in FVC%p annual change (SE) |  | 3.45 (0.886) |
| 95% CI |  | 1.63, 5.27 |
| *P* value |  | < 0.001 |

CI, confidence interval; CINRG DNHS, Cooperative International Neuromuscular Research Group Duchenne Natural History Study; FVC%p, percent predicted forced vital capacity; SE, standard error.

**Table S4**

FVC%p slope model sensitivity analysis: study 204 versus the Exon 51 CINRG DNHS cohort, with baseline height as a covariate

| **Number of Patients Aged 10 to <18 Years** | **Study 204 (N=20)** | **Exon 51 CINRG DNHS  (N=20)** |
| --- | --- | --- |
| Number of observations | 117 | 88 |
| FVC%p annual change (SE) | −3.99 (0.687) | −6.09 (0.408) |
| 95% CI | −5.34, −2.63 | −6.90, −5.29 |
| Difference in FVC%p annual change (SE) |  | 2.11 (0.791) |
| 95% CI |  | 0.54, 3.67 |
| *P* value |  | 0.009 |

CI, confidence interval; CINRG DNHS, Cooperative International Neuromuscular Research Group Duchenne Natural History Study; FVC%p, percent predicted forced vital capacity; SE, standard error.

**Table S5**

FVC%p slope model sensitivity analysis: study 204 versus the Exon 51 CINRG DNHS cohort, with random slope for patients

| **Number of Patients Aged 10 to <18 Years** | **Study 204 (N=20)** | **Exon 51 CINRG DNHS (N=20)** |
| --- | --- | --- |
| Number of observations | 117 | 88 |
| FVC%p annual change (SE) | −3.65 (0.716) | −5.62 (0.460) |
| 95% CI | −5.10, −2.19 | −6.55, −4.68 |
| Difference in FVC%p annual change (SE) |  | 1.97 (0.851) |
| 95% CI |  | 0.24, 3.70 |
| *P* value |  | 0.027 |

CI, confidence interval; CINRG DNHS, Cooperative International Neuromuscular Research Group Duchenne Natural History Study; FVC%p, percent predicted forced vital capacity; SE, standard error.

**Table S6**

FVC%p slope model sensitivity analysis: study 301 versus the Exon 51 CINRG DNHS cohort, with baseline height as a covariate

| **Number of Patients Aged 10 to <18 Years** | **Study 301 (N=41)\*** | **Exon 51 CINRG DNHS (N=20)** |
| --- | --- | --- |
| Number of observations | 175 | 88 |
| FVC%p annual change (SE) | −3.71 (0.873) | −6.00 (0.410) |
| 95% CI | −5.43, −1.99 | −6.81, −5.19 |
| Difference in FVC%p annual change (SE) |  | 2.29 (0.956) |
| 95% CI |  | 0.40, 4.17 |
| Nominal p-value |  | 0.018 |

CI, confidence interval; CINRG DNHS, Cooperative International Neuromuscular Research Group Duchenne Natural History Study; FVC%p, percent predicted forced vital capacity; SE, standard error.

\*One of the 42 patients in the age range of 10 to <18 years had a missing height value at the baseline visit. Therefore, this patient was excluded from the sensitivity analysis that included baseline height as a covariate.

**Table S7**

FVC%p slope model sensitivity analysis: study 301 versus the Exon 51 CINRG DNHS cohort, with random slope for patients

| **Number of Patients Aged 10 to <18 Years** | **Study 301 (N=42)** | **Exon 51 CINRG (N=20)** |
| --- | --- | --- |
| Number of observations | 184 | 88 |
| FVC%p annual change (SE) | −3.79 (0.824) | −5.62 (0.460) |
| 95% CI | −5.44, −2.13 | −6.54, −4.69 |
| Difference in FVC%p annual change (SE) |  | 1.83 (0.994) |
| 95% CI |  | −0.06, 3.72 |
| *P* value |  | 0.058 |

CI, confidence interval; CINRG DNHS, Cooperative International Neuromuscular Research Group Duchenne Natural History Study; FVC%p, percent predicted forced vital capacity; SE, standard error.