

**Supplementary Figures and Tables**

1.1 **Supplementary Figures**

**Supplementary Figure S1.** Total number of actions at baseline, Week 9, and Week 24 in the cohort of patients excluding those who showed ceiling effects (Patients 1 and 2). Action count includes numbers of mistakes + number of correct answers. The median values at baseline, Week 9 and Week 24 are shown by the black dotted line. Analyses were performed using data from Patients 3–7. The level 3 test scores for Patients 5 and 6 are excluded.
1.2 Supplementary Tables

**Supplementary Table S1.** Individual CHOP-INTEND, PedsQL-NM and NRS scores at each assessment.

|                     | Patient 1 | Patient 2 | Patient 3 | Patient 4 | Patient 5 | Patient 6 | Patient 7 |
|---------------------|-----------|-----------|-----------|-----------|-----------|-----------|-----------|
| **CHOP-INTEND**     |           |           |           |           |           |           |           |
| Baseline            | 10        | 11        | 0         | 0         | 0         | 1         | 4         |
| Week 9              | 8         | 13        | 0         | 0         | 0         | 1         | 4         |
| Week 24             | 5         | 16        | 0         | 1         | 2         | 1         | 4         |
| **PedsQL-NM**       |           |           |           |           |           |           |           |
| Disease symptoms    |           |           |           |           |           |           |           |
| Baseline            | 34        | 47        | 0         | 19        | 49        | –         | 15        |
| Week 9              | 44        | 47        | 24        | 19        | 46        | –         | 15        |
| Week 24             | 59        | 65        | 18        | 29        | 46        | –         | 16        |
| Communication       |           |           |           |           |           |           |           |
| Baseline            | 75        | 100       | 0         | 0         | 67        | –         | 0         |
| Week 9              | 50        | 100       | 0         | 0         | 67        | –         | 0         |
| Week 24             | 67        | 100       | 0         | 8         | 67        | –         | 0         |
| Family function     |           |           |           |           |           |           |           |
| Baseline            | 65        | 65        | 83        | 35        | 80        | –         | 30        |
| Week 9              | 60        | 65        | 50        | 35        | 80        | –         | 30        |
| Week 24             | 70        | 90        | 45        | 45        | 80        | –         | 40        |
| **Total**           |           |           |           |           |           |           |           |
| Baseline            | 45        | 57        | 15        | 20        | 57        | –         | 16        |
| Week 9              | 48        | 57        | 26        | 20        | 55        | –         | 16        |
| Week 24             | 62        | 74        | 22        | 30        | 55        | –         | 19        |
| **NRS score**       |           |           |           |           |           |           |           |
| Motor function      |           |           |           |           |           |           |           |
| Baseline            | 20        | 20        | 20        | 20        | 20        | 20        | 20        |
| Week 9              | 21        | 20        | 10        | 23        | 22        | –         | 21        |
| Week 24             | 23        | 20        | 20        | 23        | 24        | –         | 21        |
| Breathing           |           |           |           |           |           |           |           |
| Baseline            | 15        | 15        | 15        | 15        | 15        | 15        | 15        |
| Week 9              | 15        | 15        | 10        | 17        | 15        | –         | 16        |
| Week 24             | 15        | 15        | 15        | 18        | 15        | –         | 16        |
| Gastrointestinal    |           |           |           |           |           |           |           |
| Baseline            | 20        | 20        | 20        | 20        | 20        | 20        | –         |
| Week 9              | 20        | 24        | 10        | 23        | 20        | –         | –         |
| Week 24             | 20        | 25        | 20        | 23        | 20        | –         | –         |
| Sleep               |           |           |           |           |           |           |           |
| Baseline            | 10        | 10        | 10        | 10        | 10        | 10        | –         |
| Week 9              | 10        | 12        | 11        | 10        | 10        | –         | –         |
|            | Week 24 | 10 | 12 | 10 | 12 | 10 | – | – |
|------------|---------|----|----|----|----|----|---|---|
| **Fatigue**|         |    |    |    |    |    |   |   |
| Baseline   | 10      | 10 | 10 | 10 | 10 | 10 | 10 | – |
| Week 9     | 11      | 14 | 12 | 11 | –  | –  |   |   |
| Week 24    | 11      | 14 | 10 | 13 | 13 | –  | – | – |
| **Total**  |         |    |    |    |    |    |   |   |
| Baseline   | 75      | 75 | 75 | 75 | 75 | 75 | 75 | 1 |
| Week 9     | 77      | 85 | 30 | 86 | 78 | –  | 6 |   |
| Week 24    | 79      | 86 | 75 | 89 | 82 | –  | 2 |   |

CHOP-INTEND, Children’s Hospital of Philadelphia Infant Test of Neuromuscular Disorders; NRS, numerical rating scale; PedsQL-NM, Pediatric Quality of Life inventory for patients with Neuromuscular Disease.