**SUPPLEMENTARY MATERIALS**

Table S-1. EQ-5D-5L among SMA Patients: overall and by self-reported SMA type

|  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- |
| Characteristics | Patient Respondents  (N = 965) | Type I  (n = 241) | Type II  (n = 399) | Type III  (n = 283) | Other  (n = 42) |
| Mobility (Walking about), n (%) | | | | | |
| No problems | 77 (8.1%) | 9 (3.8%) | 17 (4.3%) | 39 (13.8%) | 12 (29.3%) |
| Slight problems | 196 (20.5%) | 28 (11.8%) | 86 (21.9%) | 69 (24.4%) | 13 (31.7%) |
| Moderate problems | 206 (21.6%) | 35 (14.7%) | 83 (21.1%) | 79 (27.9%) | 9 (22.0%) |
| Severe problems | 127 (13.3%) | 10 (4.2%) | 44 (11.2%) | 68 (24.0%) | 5 (12.2%) |
| Unable to walk about | 349 (36.5%) | 156 (65.5%) | 163 (41.5%) | 28 (9.9%) | <5 |
| Non-respondents | 10 | <5 | <5 | 0 | <5 |
| Self-Care (Washing or Dressing), n (%) | | | | | |
| No problems | 106 (11.1%) | <5 | 21 (5.3%) | 63 (22.3%) | 18 (43.9%) |
| Slight problems | 248 (26.0%) | 46 (19.4%) | 77 (19.6%) | 114 (40.3%) | 11 (26.8%) |
| Moderate problems | 155 (16.2%) | 16 (6.8%) | 66 (16.8%) | 66 (23.3%) | 7 (17.1%) |
| Severe problems | 81 (8.5%) | 8 (3.4%) | 44 (11.2%) | 27 (9.5%) | <5 |
| Unable to wash or dress | 364 (38.2%) | 163 (68.8%) | 185 (47.1%) | 13 (4.6%) | <5 |
| Non-respondents | 11 | <5 | 6 | 0 | <5 |
| Usual Activities, n (%) | | | | | |
| No problems | 77 (8.1%) | 4 (1.7%) | 16 (4.1%) | 38 (13.4%) | 19 (46.3%) |
| Slight problems | 297 (31.1%) | 36 (15.1%) | 109 (27.7%) | 141 (49.8%) | 11 (26.8%) |
| Moderate problems | 201 (21.0%) | 31 (13.0%) | 90 (22.9%) | 73 (25.8%) | 7 (17.1%) |
| Severe problems | 102 (10.7%) | 7 (2.9%) | 72 (18.3%) | 20 (7.1%) | <5 |
| Unable to do usual activities | 278 (29.1%) | 160 (67.2%) | 106 (27.0%) | 11 (3.9%) | <5 |
| Non-respondents | 10 | <5 | 6 | 0 | <5 |
| Pain / Discomfort, n (%) | | | | | |
| No pain | 106 (11.1%) | 5 (2.1%) | 45 (11.5%) | 49 (17.3%) | 7 (17.1%) |
| Slight | 466 (48.8%) | 74 (31.2%) | 202 (51.4%) | 165 (58.3%) | 25 (61.0%) |
| Moderate | 294 (30.8%) | 126 (53.2%) | 114 (29.0%) | 47 (16.6%) | 7 (17.1%) |
| Severe | 72 (7.5%) | 31 (13.1%) | 26 (6.6%) | 14 (4.9%) | <5 |
| Extreme | 16 (1.7%) | <5 | 6 (1.5%) | 8 (2.8%) | <5 |
| Non-respondents | 11 | <5 | 6 | 0 | <5 |
| Anxiety / Depression, n (%) | | | | | |
| None | 185 (19.4%) | 19 (8.0%) | 72 (18.3%) | 77 (27.2%) | 17 (41.5%) |
| Slight | 415 (43.5%) | 72 (30.4%) | 187 (47.6%) | 143 (50.5%) | 13 (31.7%) |
| Moderate | 276 (28.9%) | 112 (47.3%) | 112 (28.5%) | 46 (16.3%) | 6 (14.6%) |
| Severe | 59 (6.2%) | 31 (13.1%) | 16 (4.1%) | 9 (3.2%) | <5 |
| Extreme | 19 (2.0%) | <5 | 6 (1.5%) | 8 (2.8%) | <5 |
| Non-respondents | 11 | <5 | 6 | 0 | <5 |
| EQ-5D VAS | | | | | |
| Mean (SD) | 62.9 (19.3) | 46.6 (19.3) | 66 (16.2) | 71.5 (14.7) | 68.6 (18.3) |
| Median (Q1 – Q3) | 65 (50-79) | 40 (30-61) | 68 (57-78) | 75 (62-82) | 70 (58-82) |
| Health Utility Index | | | | | |
| Mean (SD) | 0.49 (0.26) | 0.32 (0.25) | 0.46 (0.23) | 0.65 (0.21) | 0.71 (0.24) |

Note: Cells with fewer than 5 responses are masked using ‘<5’ for privacy standards.

SD=standard deviation; SMA=spinal muscular atrophy; Q1=first quartile; Q3=third quartile

Table S-2. Patient Demographics: overall and by self-reported SMA type among patients with no treatment or nusinersen use only

|  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- |
| Characteristics | Patient or adult proxy Respondents  (N = 442\*) | Type I  (n = 125) | Type II  (n = 155) | Type III  (n = 150) | Other  (n = 12) |
| Age in years at time of survey response, n = 432 | | | | | |
| Mean (SD) | 11.5 (11.3) | 3.5 (8.3) | 9.9 (10.5) | 18.6 (8.3) | 30.9 (8.1) |
| Median (Q1 – Q3) | 7 (2-22) | 1 (1-2) | 4 (2-20) | 17 (12-26) | 31 (25-34) |
| Age in years at time of survey response, n (%) | | | | | |
| 0 – 6 months | 0 | 0 | 0 | 0 | 0 |
| 6 – 18 months | 83 (19.2%) | 82 (66.1%) | <5 | 0 | 0 |
| 18 month – 18 years | 222 (51.4%) | 35 (28.2%) | 111 (73.5%) | 76 (51.7%) | 0 |
| ≥18 years | 127 (29.4%) | 7 (5.6%) | 39 (25.8%) | 71 (48.3%) | 10 (100%) |
| Non-respondents | 10 | <5 | <5 | <5 | <5 |
| Sex, n (%) | | | | | |
| Female | 172 (38.9%) | 60 (48%) | 57 (36.8%) | 53 (35.3%) | <5 |
| Male | 270 (61.1%) | 65 (52%) | 98 (63.2%) | 97 (64.7%) | 10 (83.3%) |
| Non-respondents | 0 | 0 | 0 | 0 | 0 |
| Age at first onset of symptoms, in years, n=423 | | | | | |
| Mean (SD) | 5.6 (7.3) | 1.5 (5.6) | 3 (4.6) | 10.2 (5.1) | 27.4 (8.9) |
| Median (Q1 – Q3) | 1 (1-9) | 0 (0-1) | 1 (1-2) | 10 (6-13) | 29 (23-32) |
| Age at diagnosis, in years, n=422 | | | | | |
| Mean (SD) | 6.5 (7.7) | 1.6 (5.6) | 3.9 (5.7) | 11.6 (5.1) | 28 (8.6) |
| Median (Q1 – Q3) | 2 (1-12) | 1 (0-1) | 1 (1-3) | 12 (8-15) | 29 (23-32) |

\*N=442 represents a subgroup of individuals from the N=965 included in the final report that 1) indicated nusinersen but not risdiplam or onasemnogene abeparvovec; or 2) did not indicate any of those three medications.

Note: Cells with fewer than 5 responses are masked using <5 for privacy standards.

SD=standard deviation; SMA=spinal muscular atrophy; Q1=first quartile; Q3=third quartile

Table S-3. Self-Reported SMA Costs: overall and by self-reported SMA type among patients with no treatment or nusinersen use only

|  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- |
| Characteristics | Patient or adult proxy Respondents  (N = 442\*) | Type I  (n = 125) | Type II  (n = 155) | Type III  (n = 150) | Other  (n = 12) |
| Estimated total spent on assistive devices for SMA (any kind) in the last year, CAD$, n=433 | | | | | |
| Mean (SD) | 8,474 (19,115) | 5,381 (8,761) | 8,063 (20,601) | 11,090 (23,416) | 12,596 (12,646) |
| Median (Q1 – Q3) | 2,200  (1,400-5,800) | 4,200  (1,700-5,800) | 2,750  (1,534-5,700) | 1,535  (1,200-5,000) | 6,500  (2,150-24,000) |
| Estimated total cost of home modifications, CAD$, n=172 | | | | | |
| Mean (SD) | 20,945 (30,928) | 8,265 (5,256) | 34,620 (48,013) | 17,940 (18,999) | 12750 (3817.7) |
| Median (Q1 – Q3) | 13,750  (8,000-18,000) | 6,900  (5,700-7,700) | 15,000  (10,000-30,000) | 15,000  (12,000-17,500) | 12,750  (10,000-15,000) |
| Estimated cost spent personally on travel and accommodation relating to SMA appointments in the last year (12 months), CAD$, n=433 | | | | | |
| Mean (SD) | 2,547 (8,154) | 2,500 (3,510) | 2,529 (12,384) | 2,309 (4,427) | 6,208 (10,927) |
| Median (Q1 – Q3) | 1,100 (600-2,150) | 1,900 (1,200-2,800) | 900 (500-1,500) | 800 (469-1,800) | 3,725 (700-6,000) |
| Estimated personal, out-of-pocket expenses for SMA-related, allied health professional services in the past 12 months, CAD$, n=434 | | | | | |
| Mean (SD) | 8,861 (11,658) | 7,480 (3,593) | 8,040 (8,317) | 10,374 (17,042) | 14,432 (16,177) |
| Median (Q1 – Q3) | 6,187  (4,000-8,300) | 6,950  (5,900-8,700) | 6,077  (4,100-8,200) | 5,186  (3,200-7,557) | 7,000  (2,000-25,000) |

\*N=442 represents a subgroup of individuals from the N=965 included in the final report that 1) indicated nusinersen but not risdiplam or onasemnogene abeparvovec; or 2) did not indicate any of those three medications.

Note: Cells with fewer than 5 responses are masked using <5 for privacy standards.

SD=standard deviation; SMA=spinal muscular atrophy; Q1=first quartile; Q3=third quartile

Table S-4. EQ-5D-5L among SMA Patients: overall and by self-reported SMA type among patients with no treatment or nusinersen use only

|  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- |
| Characteristics | Patient or adult proxy Respondents  (N = 442\*) | Type I  (n = 125) | Type II  (n = 155) | Type III  (n = 150) | Other  (n = 12) |
| Mobility (Walking about), n (%) | | | | | |
| No problems | 29 (6.6%) | <5 | 8 (5.2%) | 16 (10.7%) | <5 |
| Slight problems | 68 (15.4%) | <5 | 22 (14.2%) | 36 (24%) | 7 (58.3%) |
| Moderate problems | 73 (16.5%) | 6 (4.8%) | 19 (12.3%) | 47 (31.3%) | <5 |
| Severe problems | 58 (13.1%) | <5 | 12 (7.7%) | 45 (30%) | 0 |
| Unable to walk about | 214 (48.4%) | 113 (90.4%) | 94 (60.6%) | 6 (4%) | <5 |
| Non-respondents | 0 | 0 | 0 | 0 | 0 |
| Self-Care (Washing or Dressing), n (%) | | | | | |
| No problems | 43 (9.8%) | <5 | 7 (4.5%) | 27 (18%) | 8 (66.7%) |
| Slight problems | 112 (25.4%) | 9 (7.3%) | 23 (14.8%) | 78 (52%) | <5 |
| Moderate problems | 52 (11.8%) | 0 | 21 (13.5%) | 31 (20.7%) | 0 |
| Severe problems | 24 (5.4%) | <5 | 11 (7.1%) | 12 (8%) | 0 |
| Unable to wash or dress | 210 (47.6%) | 113 (91.1%) | 93 (60%) | <5 | <5 |
| Non-respondents | <5 | <5 | 0 | 0 | 0 |
| Usual Activities, n (%) | | | | | |
| No problems | 26 (5.9%) | <5 | <5 | 14 (9.3%) | 7 (58.3%) |
| Slight problems | 130 (29.4%) | <5 | 33 (21.3%) | 90 (60%) | <5 |
| Moderate problems | 71 (16.1%) | 8 (6.4%) | 25 (16.1%) | 38 (25.3%) | 0 |
| Severe problems | 32 (7.2%) | <5 | 23 (14.8%) | 7 (4.7%) | <5 |
| Unable to do usual activities | 183 (41.4%) | 112 (89.6%) | 70 (45.2%) | <5 | 0 |
| Non-respondents | 0 | 0 | 0 | 0 | 0 |
| Pain / Discomfort, n (%) | | | | | |
| None | 41 (9.3%) | 0 | 18 (11.6%) | 20 (13.3%) | <5 |
| Slight | 221 (50.1%) | 27 (21.8%) | 84 (54.2%) | 102 (68%) | 8 (66.7%) |
| Moderate | 152 (34.5%) | 86 (69.4%) | 44 (28.4%) | 21 (14%) | <5 |
| Severe | 24 (5.4%) | 11 (8.9%) | 8 (5.2%) | 5 (3.3%) | 0 |
| Extreme | <5 | 0 | <5 | <5 | 0 |
| Non-respondents | <5 | <5 | 0 | 0 | 0 |
| Anxiety / Depression, n (%) | | | | | |
| None | 74 (16.8%) | 5 (4%) | 24 (15.5%) | 36 (24%) | 9 (75%) |
| Slight | 185 (42%) | 27 (21.8%) | 71 (45.8%) | 84 (56%) | <5 |
| Moderate | 157 (35.6%) | 79 (63.7%) | 55 (35.5%) | 23 (15.3%) | 0 |
| Severe | 23 (5.2%) | 13 (10.5%) | 5 (3.2%) | 5 (3.3%) | 0 |
| Extreme | <5 | 0 | 0 | <5 | 0 |
| Non-respondents | <5 | <5 | 0 | 0 | 0 |
| EQ-5D VAS | | | | | |
| Mean (SD) | 59.5 (19.7) | 39.6 (14.4) | 61.4 (14.7) | 72.3 (14.1) | 80.9 (11.9) |
| Median (Q1 – Q3) | 60 (45-76) | 35 (30-45) | 61 (55-70) | 76 (62-82) | 82 (81-90) |
| Health Utility Index | | | | | |
| Mean (SD) | 0.45 (0.27) | 0.22 (0.16) | 0.4 (0.23) | 0.67 (0.17) | 0.8 (0.18) |

\*N=442 represents a subgroup of individuals from the N=965 included in the final report that 1) indicated nusinersen but not risdiplam or onasemnogene abeparvovec; or 2) did not indicate any of those three medications.

Note: Cells with fewer than 5 responses are masked using <5 for privacy standards.

SD=standard deviation; SMA=spinal muscular atrophy; Q1=first quartile; Q3=third quartile