

# **Nusinersen for Spinal Muscular Atrophy in the United States: Findings from a Retrospective Claims Database Analysis**

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| Supplemental Table 1. Health care resource utilization among patients adherent and non-adherent to nusinersen dose schedule |                             |                  |                                 |                             |                  |                             |                             |                  |                             |                             |                  |                             |                             |                  |                             |                              |                  |                             |
|-----------------------------------------------------------------------------------------------------------------------------|-----------------------------|------------------|---------------------------------|-----------------------------|------------------|-----------------------------|-----------------------------|------------------|-----------------------------|-----------------------------|------------------|-----------------------------|-----------------------------|------------------|-----------------------------|------------------------------|------------------|-----------------------------|
| Health care resource utilization <sup>a</sup>                                                                               | SMA type 1                  |                  |                                 |                             |                  |                             | SMA type 2                  |                  |                             |                             |                  |                             | SMA type 3                  |                  |                             |                              |                  |                             |
|                                                                                                                             | Adherent cohort<br>n=11     |                  |                                 | Non-adherent cohort<br>n=12 |                  |                             | Adherent cohort<br>n=12     |                  |                             | Non-adherent cohort<br>n=29 |                  |                             | Adherent cohort<br>n=122    |                  |                             | Non-adherent cohort<br>n=138 |                  |                             |
|                                                                                                                             | Patient-years=13            |                  |                                 | Patient-years=21            |                  |                             | Patient-years=11            |                  |                             | Patient-years=50            |                  |                             | Patient-years=135           |                  |                             | Patient-years=221            |                  |                             |
|                                                                                                                             | Patients with events, n (%) | Number of events | Incidence rate PPY <sup>b</sup> | Patients with events, n (%) | Number of events | Incidence rate <sup>b</sup> | Patients with events, n (%) | Number of events | Incidence rate <sup>b</sup> | Patients with events, n(%)  | Number of events | Incidence rate <sup>b</sup> | Patients with events, n (%) | Number of events | Incidence rate <sup>b</sup> | Patients with events, n (%)  | Number of events | Incidence rate <sup>b</sup> |
| <b>Days with medical services</b>                                                                                           | <b>11 (100.0)</b>           | <b>543</b>       | <b>42.21</b>                    | <b>12 (100.0)</b>           | <b>1,003</b>     | <b>48.41</b>                | <b>12 (100.0)</b>           | <b>493</b>       | <b>45.17</b>                | <b>29 (100.0)</b>           | <b>5,505</b>     | <b>109.57</b>               | <b>122 (100.0)</b>          | <b>7,417</b>     | <b>54.78</b>                | <b>138 (100.0)</b>           | <b>15,853</b>    | <b>71.70</b>                |
| <b>Hospitalizations</b>                                                                                                     |                             |                  |                                 |                             |                  |                             |                             |                  |                             |                             |                  |                             |                             |                  |                             |                              |                  |                             |
| Days                                                                                                                        | 5 (45.5)                    | 50               | 3.89                            | 7 (58.3)                    | 45               | 2.17                        | 5 (41.7)                    | 8                | 0.73                        | 16 (55.2)                   | 321              | 6.39                        | 24 (19.7)                   | 172              | 1.27                        | 42 (30.4)                    | 581              | 2.63                        |
| Admission                                                                                                                   | 5 (45.5)                    | 9                | 0.70                            | 7 (58.3)                    | 22               | 1.06                        | 5 (41.7)                    | 6                | 0.55                        | 16 (55.2)                   | 66               | 1.31                        | 24 (19.7)                   | 40               | 0.30                        | 42 (30.4)                    | 113              | 0.51                        |
| Procedures <sup>c</sup>                                                                                                     | 4 (36.4)                    | 26               | 2.02                            | 7 (58.3)                    | 114              | 5.50                        | 4 (33.3)                    | 25               | 2.29                        | 15 (51.7)                   | 295              | 5.87                        | 24 (19.7)                   | 294              | 2.17                        | 41 (29.7)                    | 694              | 3.14                        |
| <b>Days with outpatient visits (including office and clinic visits)</b>                                                     | <b>11 (100.0)</b>           | <b>287</b>       | <b>22.31</b>                    | <b>12 (100.0)</b>           | <b>469</b>       | <b>22.63</b>                | <b>12 (100.0)</b>           | <b>436</b>       | <b>39.94</b>                | <b>29 (100.0)</b>           | <b>1,662</b>     | <b>33.08</b>                | <b>118 (96.7)</b>           | <b>3,058</b>     | <b>22.59</b>                | <b>137 (99.3)</b>            | <b>4,366</b>     | <b>19.75</b>                |
| <b>Days with other medical services</b>                                                                                     | <b>10 (90.9)</b>            | <b>206</b>       | <b>16.01</b>                    | <b>9 (75.0)</b>             | <b>489</b>       | <b>23.60</b>                | <b>9 (75.0)</b>             | <b>49</b>        | <b>4.49</b>                 | <b>26 (89.7)</b>            | <b>3,522</b>     | <b>70.10</b>                | <b>90 (73.8)</b>            | <b>4,187</b>     | <b>30.93</b>                | <b>104 (75.4)</b>            | <b>10,906</b>    | <b>49.33</b>                |
| <b>Specialist visit, n (%)</b>                                                                                              |                             |                  |                                 |                             |                  |                             |                             |                  |                             |                             |                  |                             |                             |                  |                             |                              |                  |                             |
| Pulmonologist                                                                                                               | 7 (63.6)                    | 29               | 2.25                            | 9 (75.0)                    | 146              | 7.05                        | 6 (50.0)                    | 16               | 1.47                        | 23 (79.3)                   | 605              | 12.04                       | 58 (47.5)                   | 747              | 5.52                        | 76 (55.1)                    | 1,943            | 8.79                        |
| Neurologist                                                                                                                 | 9 (81.8)                    | 155              | 12.05                           | 8 (66.7)                    | 196              | 9.46                        | 9 (75.0)                    | 92               | 8.43                        | 25 (86.2)                   | 338              | 6.73                        | 92 (75.4)                   | 767              | 5.67                        | 95 (68.8)                    | 885              | 4.00                        |
| Pediatric critical care medicine specialist                                                                                 | 8 (72.7)                    | 45               | 3.50                            | 9 (75.0)                    | 71               | 3.43                        | 4 (33.3)                    | 7                | 0.64                        | 16 (55.2)                   | 172              | 3.42                        | 21 (17.2)                   | 120              | 0.89                        | 31 (22.5)                    | 414              | 1.87                        |
| Surgical specialist (e.g., neurological surgery specialist, orthopedic surgeon)                                             | 3 (27.3)                    | 15               | 1.17                            | 4 (33.3)                    | 21               | 1.01                        | 6 (50.0)                    | 18               | 1.65                        | 12 (41.4)                   | 42               | 0.84                        | 33 (27.0)                   | 171              | 1.26                        | 57 (41.3)                    | 234              | 1.06                        |
| Physical medicine and rehabilitation specialist                                                                             | 2 (18.2)                    | 18               | 1.40                            | 2 (16.7)                    | 17               | 0.82                        | 3 (25.0)                    | 8                | 0.73                        | 12 (41.4)                   | 149              | 2.97                        | 23 (18.9)                   | 157              | 1.16                        | 35 (25.4)                    | 172              | 0.78                        |
| <b>SMA-related procedures, n (%)</b>                                                                                        |                             |                  |                                 |                             |                  |                             |                             |                  |                             |                             |                  |                             |                             |                  |                             |                              |                  |                             |
| Mechanical ventilation support                                                                                              | 4 (36.4)                    | 11               | 0.86                            | 6 (50.0)                    | 30               | 1.45                        | 1 (8.3)                     | 2                | 0.18                        | 14 (48.3)                   | 517              | 10.29                       | 43 (35.2)                   | 585              | 4.32                        | 48 (34.8)                    | 1,275            | 5.77                        |
| Non-invasive                                                                                                                | 4 (36.4)                    | 11               | 0.86                            | 5 (41.7)                    | 26               | 1.25                        | 1 (8.3)                     | 2                | 0.18                        | 13 (44.8)                   | 152              | 3.03                        | 31 (25.4)                   | 228              | 1.68                        | 41 (29.7)                    | 447              | 2.02                        |
| Invasive                                                                                                                    | 0 (0.0)                     | 0                | -                               | 2 (16.7)                    | 4                | 0.19                        | 0 (0.0)                     | 0                | -                           | 6 (20.7)                    | 379              | 7.54                        | 23 (18.9)                   | 393              | 2.90                        | 21 (15.2)                    | 902              | 4.08                        |
| Tracheostomy                                                                                                                | 0 (0.0)                     | 0                | -                               | 2 (16.7)                    | 3                | 0.14                        | 0 (0.0)                     | 0                | -                           | 3 (10.3)                    | 373              | 7.42                        | 20 (16.4)                   | 380              | 2.81                        | 20 (14.5)                    | 895              | 4.05                        |
| Nutritional support                                                                                                         | 6 (54.5)                    | 46               | 3.58                            | 7 (58.3)                    | 118              | 5.69                        | 0 (0.0)                     | 0                | -                           | 13 (44.8)                   | 606              | 12.06                       | 38 (31.1)                   | 669              | 4.94                        | 49 (35.5)                    | 1,990            | 9.00                        |
| Gastrostomy                                                                                                                 | 4 (36.4)                    | 32               | 2.49                            | 7 (58.3)                    | 103              | 4.97                        | 0 (0.0)                     | 0                | -                           | 13 (44.8)                   | 514              | 10.23                       | 32 (26.2)                   | 533              | 3.94                        | 43 (31.2)                    | 1,753            | 7.93                        |
| Nasogastric tube or other (non-oral feeding support)                                                                        | 3 (27.3)                    | 17               | 1.32                            | 4 (33.3)                    | 23               | 1.11                        | 0 (0.0)                     | 0                | -                           | 11 (37.9)                   | 176              | 3.50                        | 22 (18.0)                   | 254              | 1.88                        | 30 (21.7)                    | 461              | 2.09                        |
| Nutritional counseling                                                                                                      | 0 (0.0)                     | 0                | -                               | 1 (8.3)                     | 1                | 0.05                        | 0 (0.0)                     | 0                | -                           | 1 (3.4)                     | 3                | 0.06                        | 8 (6.6)                     | 14               | 0.10                        | 6 (4.3)                      | 14               | 0.06                        |
| Physical and/or occupational therapy/rehabilitation                                                                         | 5 (45.5)                    | 190              | 14.77                           | 7 (58.3)                    | 261              | 12.60                       | 8 (66.7)                    | 293              | 26.84                       | 20 (69.0)                   | 1,397            | 27.81                       | 62 (50.8)                   | 1,135            | 8.38                        | 63 (45.7)                    | 2,802            | 12.67                       |
| Speech therapy                                                                                                              | 1 (9.1)                     | 2                | 0.16                            | 6 (50.0)                    | 40               | 1.93                        | 0 (0.0)                     | 0                | -                           | 10 (34.5)                   | 384              | 7.64                        | 11 (9.0)                    | 187              | 1.38                        | 11 (8.0)                     | 529              | 2.39                        |
| Sleep studies                                                                                                               | 5 (45.5)                    | 8                | 0.62                            | 1 (8.3)                     | 2                | 0.10                        | 3 (25.0)                    | 3                | 0.27                        | 8 (27.6)                    | 9                | 0.18                        | 11 (9.0)                    | 15               | 0.11                        | 20 (14.5)                    | 22               | 0.10                        |
| Orthopedic surgery                                                                                                          | 0 (0.0)                     | 0                | -                               | 1 (8.3)                     | 1                | 0.05                        | 1 (8.3)                     | 2                | 0.18                        | 6 (20.7)                    | 8                | 0.16                        | 6 (4.9)                     | 9                | 0.07                        | 18 (13.0)                    | 38               | 0.17                        |
| Chiropractic services                                                                                                       | 0 (0.0)                     | 0                | -                               | 0 (0.0)                     | 0                | -                           | 0 (0.0)                     | 0                | -                           | 0 (0.0)                     | 0                | -                           | 0 (0.0)                     | 0                | -                           | 1 (0.7)                      | 2                | 0.01                        |
| Other services <sup>d</sup>                                                                                                 | 0 (0.0)                     | 0                | -                               | 0 (0.0)                     | 0                | -                           | 0 (0.0)                     | 0                | -                           | 0 (0.0)                     | 0                | -                           | 0 (0.0)                     | 0                | -                           | 0 (0.0)                      | 0                | -                           |

|                                      |         |   |   |         |   |   |          |   |      |           |    |      |           |    |      |           |    |      |
|--------------------------------------|---------|---|---|---------|---|---|----------|---|------|-----------|----|------|-----------|----|------|-----------|----|------|
| SMA-related DME, n (%)               |         |   |   |         |   |   |          |   |      |           |    |      |           |    |      |           |    |      |
| Braces                               | 0 (0.0) | 0 | - | 0 (0.0) | 0 | - | 3 (25.0) | 5 | 0.46 | 17 (58.6) | 24 | 0.48 | 18 (14.8) | 22 | 0.16 | 27 (19.6) | 39 | 0.18 |
| Pediatric wheelchair (tilt-in-space) | 0 (0.0) | 0 | - | 0 (0.0) | 0 | - | 0 (0.0)  | 0 | -    | 5 (17.2)  | 5  | 0.10 | 0 (0.0)   | 0  | -    | 0 (0.0)   | 0  | -    |
| Pediatric wheelchair                 | 0 (0.0) | 0 | - | 0 (0.0) | 0 | - | 2 (16.7) | 2 | 0.18 | 2 (6.9)   | 2  | 0.04 | 3 (2.5)   | 3  | 0.02 | 0 (0.0)   | 0  | -    |
| Manual adult wheelchair              | 0 (0.0) | 0 | - | 0 (0.0) | 0 | - | 0 (0.0)  | 0 | -    | 0 (0.0)   | 0  | -    | 0 (0.0)   | 0  | -    | 2 (1.4)   | 2  | 0.01 |
| Walker or cane                       | 0 (0.0) | 0 | - | 0 (0.0) | 0 | - | 0 (0.0)  | 0 | -    | 0 (0.0)   | 0  | -    | 0 (0.0)   | 0  | -    | 0 (0.0)   | 0  | -    |

*DME* durable medical equipment, *SMA* spinal muscular atrophy

<sup>a</sup>Assessed while on treatment, that is from the first observed nusinersen dose (i.e., index date) until (i) the last dose date + expected inter-dose interval based on product label (i.e., date of next expected dose), (ii) an indicator of treatment with onasemnogene abeparvovec, or (iii) the end of data availability (i.e., Nov. 30, 2019), whichever occurred first

<sup>b</sup>The incidence rate was calculated as number of events (e.g., outpatient visits) divided by patient-years of observation to account for the different lengths of study period among patients

<sup>c</sup>Evaluation and management related procedures were excluded (CPT codes: 99201 - 99499)

<sup>d</sup>Other services were osteopathic services and acupuncture

Supplemental Table 2. Health care resource utilization among patients who discontinued and did not discontinue nusinersen

| Health care resource utilization <sup>a</sup>                                   | SMA type 1                    |                  |                             |                                  |                  |                             | SMA type 2                    |                  |                             |                                  |                  |                             | SMA type 3                     |                  |                             |                                   |                  |                             |
|---------------------------------------------------------------------------------|-------------------------------|------------------|-----------------------------|----------------------------------|------------------|-----------------------------|-------------------------------|------------------|-----------------------------|----------------------------------|------------------|-----------------------------|--------------------------------|------------------|-----------------------------|-----------------------------------|------------------|-----------------------------|
|                                                                                 | Discontinuation cohort (n=11) |                  |                             | No discontinuation cohort (n=12) |                  |                             | Discontinuation cohort (n=24) |                  |                             | No discontinuation cohort (n=17) |                  |                             | Discontinuation cohort (n=146) |                  |                             | No discontinuation cohort (n=114) |                  |                             |
|                                                                                 | Patient-years=9               |                  |                             | Patient-years=15                 |                  |                             | Patient-years=19              |                  |                             | Patient-years=25                 |                  |                             | Patient-years=167              |                  |                             | Patient-years=157                 |                  |                             |
|                                                                                 | Patients with events, N (%)   | Number of events | Incidence rate <sup>b</sup> | Patients with events, N (%)      | Number of events | Incidence rate <sup>b</sup> | Patients with events, N (%)   | Number of events | Incidence rate <sup>b</sup> | Patients with events, N (%)      | Number of events | Incidence rate <sup>b</sup> | Patients with events, N (%)    | Number of events | Incidence rate <sup>b</sup> | Patients with events, N (%)       | Number of events | Incidence rate <sup>b</sup> |
| Days with medical services                                                      | 11 (100.0)                    | 326              | 36.10                       | 12 (100.0)                       | 805              | 52.62                       | 24 (100.0)                    | 1,971            | 101.51                      | 17 (100.0)                       | 1,888            | 75.57                       | 145 (99.3)                     | 9,883            | 59.26                       | 114 (100.0)                       | 9,078            | 57.79                       |
| Hospitalizations                                                                |                               |                  |                             |                                  |                  |                             |                               |                  |                             |                                  |                  |                             |                                |                  |                             |                                   |                  |                             |
| Days                                                                            | 6 (54.5)                      | 14               | 1.55                        | 6 (50.0)                         | 69               | 4.51                        | 7 (29.2)                      | 87               | 4.48                        | 10 (58.8)                        | 181              | 7.24                        | 31 (21.2)                      | 348              | 2.09                        | 32 (28.1)                         | 267              | 1.70                        |
| Admission                                                                       | 6 (54.5)                      | 12               | 1.33                        | 6 (50.0)                         | 15               | 0.98                        | 7 (29.2)                      | 14               | 0.72                        | 10 (58.8)                        | 45               | 1.80                        | 31 (21.2)                      | 69               | 0.41                        | 32 (28.1)                         | 65               | 0.41                        |
| Procedures <sup>c</sup>                                                         | 6 (54.5)                      | 59               | 6.53                        | 5 (41.7)                         | 66               | 4.31                        | 6 (25.0)                      | 63               | 3.24                        | 9 (52.9)                         | 186              | 7.44                        | 30 (20.5)                      | 448              | 2.69                        | 31 (27.2)                         | 439              | 2.79                        |
| Days with outpatient visits (including office and clinic visits)                | 11 (100.0)                    | 85               | 9.41                        | 12 (100.0)                       | 512              | 33.47                       | 24 (100.0)                    | 798              | 41.10                       | 17 (100.0)                       | 636              | 25.46                       | 144 (98.6)                     | 2,780            | 16.67                       | 110 (96.5)                        | 3,317            | 21.12                       |
| Days with other medical services                                                | 6 (54.5)                      | 227              | 25.14                       | 11 (91.7)                        | 224              | 14.64                       | 20 (83.3)                     | 1,086            | 55.93                       | 13 (76.5)                        | 1,071            | 42.87                       | 98 (67.1)                      | 6,755            | 40.51                       | 88 (77.2)                         | 5,494            | 34.98                       |
| Specialist visit, N (%)                                                         |                               |                  |                             |                                  |                  |                             |                               |                  |                             |                                  |                  |                             |                                |                  |                             |                                   |                  |                             |
| Pulmonologist                                                                   | 4 (36.4)                      | 31               | 3.43                        | 9 (75.0)                         | 95               | 6.21                        | 16 (66.7)                     | 201              | 10.35                       | 9 (52.9)                         | 138              | 5.52                        | 60 (41.1)                      | 1,483            | 8.89                        | 62 (54.4)                         | 1,327            | 8.45                        |
| Neurologist                                                                     | 6 (54.5)                      | 22               | 2.44                        | 10 (83.3)                        | 287              | 18.76                       | 19 (79.2)                     | 139              | 7.16                        | 12 (70.6)                        | 167              | 6.68                        | 99 (67.8)                      | 689              | 4.13                        | 81 (71.1)                         | 878              | 5.59                        |
| Pediatric critical care medicine specialist                                     | 6 (54.5)                      | 46               | 5.09                        | 8 (66.7)                         | 45               | 2.94                        | 8 (33.3)                      | 75               | 3.86                        | 8 (47.1)                         | 77               | 3.08                        | 27 (18.5)                      | 247              | 1.48                        | 22 (19.3)                         | 180              | 1.15                        |
| Surgical specialist (e.g., neurological surgery specialist, orthopedic surgeon) | 2 (18.2)                      | 3                | 0.33                        | 4 (33.3)                         | 24               | 1.57                        | 6 (25.0)                      | 12               | 0.62                        | 7 (41.2)                         | 28               | 1.12                        | 37 (25.3)                      | 140              | 0.84                        | 39 (34.2)                         | 221              | 1.41                        |
| Physical medicine and rehabilitation specialist                                 | 0 (0.0)                       | 0                | -                           | 2 (16.7)                         | 18               | 1.18                        | 8 (33.3)                      | 13               | 0.67                        | 5 (29.4)                         | 45               | 1.80                        | 27 (18.5)                      | 67               | 0.40                        | 27 (23.7)                         | 224              | 1.43                        |
| SMA-related procedures, N (%)                                                   |                               |                  |                             |                                  |                  |                             |                               |                  |                             |                                  |                  |                             |                                |                  |                             |                                   |                  |                             |
| Mechanical ventilation support                                                  | 4 (36.4)                      | 14               | 1.55                        | 4 (33.3)                         | 12               | 0.78                        | 7 (29.2)                      | 56               | 2.88                        | 6 (35.3)                         | 316              | 12.65                       | 50 (34.2)                      | 1,070            | 6.42                        | 38 (33.3)                         | 749              | 4.77                        |
| Non-invasive                                                                    | 3 (27.3)                      | 12               | 1.33                        | 4 (33.3)                         | 12               | 0.78                        | 7 (29.2)                      | 49               | 2.52                        | 5 (29.4)                         | 48               | 1.92                        | 41 (28.1)                      | 472              | 2.83                        | 28 (24.6)                         | 257              | 1.64                        |
| Invasive                                                                        | 2 (18.2)                      | 2                | 0.22                        | 0 (0.0)                          | 0                | -                           | 2 (8.3)                       | 8                | 0.41                        | 3 (17.6)                         | 281              | 11.25                       | 21 (14.4)                      | 656              | 3.93                        | 21 (18.4)                         | 519              | 3.30                        |
| Tracheostomy                                                                    | 1 (9.1)                       | 1                | 0.11                        | 0 (0.0)                          | 0                | -                           | 1 (4.2)                       | 6                | 0.31                        | 2 (11.8)                         | 279              | 11.17                       | 20 (13.7)                      | 652              | 3.91                        | 18 (15.8)                         | 504              | 3.21                        |
| Nutritional support                                                             | 5 (45.5)                      | 78               | 8.64                        | 5 (41.7)                         | 46               | 3.01                        | 9 (37.5)                      | 183              | 9.43                        | 3 (17.6)                         | 161              | 6.44                        | 48 (32.9)                      | 1,306            | 7.83                        | 32 (28.1)                         | 990              | 6.30                        |
| Gastrostomy                                                                     | 4 (36.4)                      | 43               | 4.76                        | 4 (33.3)                         | 38               | 2.48                        | 8 (33.3)                      | 135              | 6.95                        | 3 (17.6)                         | 160              | 6.40                        | 40 (27.4)                      | 1,098            | 6.58                        | 28 (24.6)                         | 904              | 5.75                        |
| Nasogastric tube or other (non-oral feeding support)                            | 2 (18.2)                      | 42               | 4.65                        | 2 (16.7)                         | 11               | 0.72                        | 9 (37.5)                      | 83               | 4.27                        | 1 (5.9)                          | 1                | 0.04                        | 35 (24.0)                      | 396              | 2.37                        | 16 (14.0)                         | 226              | 1.44                        |
| Nutritional counseling                                                          | 1 (9.1)                       | 1                | 0.11                        | 0 (0.0)                          | 0                | -                           | 0 (0.0)                       | 0                | -                           | 1 (5.9)                          | 3                | 0.12                        | 6 (4.1)                        | 13               | 0.08                        | 5 (4.4)                           | 9                | 0.06                        |
| Physical and/or occupational therapy / rehabilitation                           | 2 (18.2)                      | 22               | 2.44                        | 7 (58.3)                         | 344              | 22.49                       | 13 (54.2)                     | 762              | 39.25                       | 12 (70.6)                        | 341              | 13.65                       | 65 (44.5)                      | 1,830            | 10.97                       | 51 (44.7)                         | 1,408            | 8.96                        |

|                                      |          |    |      |          |   |      |          |     |       |          |    |      |          |     |      |           |     |      |
|--------------------------------------|----------|----|------|----------|---|------|----------|-----|-------|----------|----|------|----------|-----|------|-----------|-----|------|
| Speech therapy                       | 3 (27.3) | 11 | 1.22 | 2 (16.7) | 3 | 0.20 | 8 (33.3) | 276 | 14.21 | 1 (5.9)  | 58 | 2.32 | 8 (5.5)  | 286 | 1.71 | 10 (8.8)  | 343 | 2.18 |
| Sleep studies                        | 1 (9.1)  | 2  | 0.22 | 5 (41.7) | 8 | 0.52 | 3 (12.5) | 3   | 0.15  | 4 (23.5) | 4  | 0.16 | 10 (6.8) | 12  | 0.07 | 14 (12.3) | 18  | 0.11 |
| Orthopedic surgery                   | 0 (0.0)  | 0  | -    | 0 (0.0)  | 0 | -    | 3 (12.5) | 4   | 0.21  | 1 (5.9)  | 2  | 0.08 | 11 (7.5) | 14  | 0.08 | 9 (7.9)   | 15  | 0.10 |
| Chiropractic services                | 0 (0.0)  | 0  | -    | 0 (0.0)  | 0 | -    | 0 (0.0)  | 0   | -     | 0 (0.0)  | 0  | -    | 0 (0.0)  | 0   | -    | 1 (0.9)   | 2   | 0.01 |
| Osteopathic services                 | 0 (0.0)  | 0  | -    | 0 (0.0)  | 0 | -    | 0 (0.0)  | 0   | -     | 0 (0.0)  | 0  | -    | 0 (0.0)  | 0   | -    | 0 (0.0)   | 0   | -    |
| Acupuncture                          | 0 (0.0)  | 0  | -    | 0 (0.0)  | 0 | -    | 0 (0.0)  | 0   | -     | 0 (0.0)  | 0  | -    | 0 (0.0)  | 0   | -    | 0 (0.0)   | 0   | -    |
| <b>SMA-related DME, N (%)</b>        |          |    |      |          |   |      |          |     |       |          |    |      |          |     |      |           |     |      |
| Braces                               | 0 (0.0)  | 0  | -    | 0 (0.0)  | 0 | -    | 6 (25.0) | 8   | 0.41  | 5 (29.4) | 9  | 0.36 | 14 (9.6) | 15  | 0.09 | 19 (16.7) | 25  | 0.16 |
| Pediatric wheelchair (tilt-in-space) | 0 (0.0)  | 0  | -    | 0 (0.0)  | 0 | -    | 0 (0.0)  | 0   | -     | 2 (11.8) | 2  | 0.08 | 0 (0.0)  | 0   | -    | 0 (0.0)   | 0   | -    |
| Pediatric wheelchair                 | 0 (0.0)  | 0  | -    | 0 (0.0)  | 0 | -    | 2 (8.3)  | 2   | 0.10  | 1 (5.9)  | 1  | 0.04 | 0 (0.0)  | 0   | -    | 3 (2.6)   | 3   | 0.02 |
| Manual adult wheelchair              | 0 (0.0)  | 0  | -    | 0 (0.0)  | 0 | -    | 0 (0.0)  | 0   | -     | 0 (0.0)  | 0  | -    | 0 (0.0)  | 0   | -    | 0 (0.0)   | 0   | -    |
| Walker                               | 0 (0.0)  | 0  | -    | 0 (0.0)  | 0 | -    | 0 (0.0)  | 0   | -     | 0 (0.0)  | 0  | -    | 0 (0.0)  | 0   | -    | 0 (0.0)   | 0   | -    |
| Cane                                 | 0 (0.0)  | 0  | -    | 0 (0.0)  | 0 | -    | 0 (0.0)  | 0   | -     | 0 (0.0)  | 0  | -    | 0 (0.0)  | 0   | -    | 0 (0.0)   | 0   | -    |

DME durable medical equipment, SMA spinal muscular atrophy

<sup>a</sup>Measured at any time during the study period. The study period spanned from index date until (i) the last observed month of clinical activity, (ii) date of nusinersen reinitiation after discontinuation, (iii) an indicator of treatment with onasemnogene abeparvovec, or (iv) the end of data availability (i.e., November 30, 2019), whichever occurred first

<sup>b</sup>The incidence rate was calculated as number of events (e.g., outpatient visits) divided by patient-years of observation to account for the different lengths of study period among patients

<sup>c</sup>Evaluation and management related procedures were excluded