| All \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 2188)** | **SMA Type 1 (N = 432)** | **SMA Type 2 (N = 914)** | **SMA Type 3 (N = 779)** | **SMA Type 4 (N = 22)** | **SMA Type Other (N = 7)** | **SMA Type Presymptomatic (N = 8)** | **SMA Type Missing (N = 26)** |
| **Calendar year of registry entry; n (%)** | | | | | | | | |
| 2008 | 27 (1.2%) | 6 (1.4%) | 14 (1.5%) | 5 (0.6%) | - | - | - | * |
| 2009 | 23 (1.1%) | 5 (1.2%) | 9 (1.0%) | 8 (1.0%) | - | - | - | * |
| 2010 | 41 (1.9%) | * | 25 (2.7%) | 10 (1.3%) | - | - | - | * |
| 2011 | 59 (2.7%) | 14 (3.2%) | 20 (2.2%) | 24 (3.1%) | - | * | - | - |
| 2012 | 147 (6.7%) | 21 (4.9%) | 64 (7.0%) | 62 (8.0%) | - | - | - | - |
| 2013 | 101 (4.6%) | 15 (3.5%) | 49 (5.4%) | 37 (4.7%) | - | - | - | - |
| 2014 | 203 (9.3%) | 32 (7.4%) | 88 (9.6%) | 83 (10.7%) | - | - | - | - |
| 2015 | 187 (8.5%) | 29 (6.7%) | 89 (9.7%) | 67 (8.6%) | * | - | - | * |
| 2016 | 136 (6.2%) | 43 (10.0%) | 55 (6.0%) | 37 (4.7%) | - | * | - | - |
| 2017 | 199 (9.1%) | 36 (8.3%) | 90 (9.8%) | 72 (9.2%) | * | - | - | - |
| 2018 | 428 (19.6%) | 75 (17.4%) | 188 (20.6%) | 157 (20.2%) | 5 (22.7%) | - | - | * |
| 2019 | 193 (8.8%) | 48 (11.1%) | 66 (7.2%) | 70 (9.0%) | 5 (22.7%) | * | - | * |
| 2020 | 150 (6.9%) | 32 (7.4%) | 69 (7.5%) | 46 (5.9%) | * | - | - | * |
| 2021 | 114 (5.2%) | 25 (5.8%) | 33 (3.6%) | 48 (6.2%) | * | * | * | * |
| 2022 | 121 (5.5%) | 33 (7.6%) | 37 (4.0%) | 36 (4.6%) | * | * | * | 7 (26.9%) |
| 2023 | 43 (2.0%) | 7 (1.6%) | 15 (1.6%) | 15 (1.9%) | * | - | * | * |
| Missing | 16 (0.7%) | 7 (1.6%) | * | * | * | - | - | * |
| **Calendar year of death; n (%)** | | | | | | | | |
| 2009 | - | - | * | - | - | - | - | - |
| 2010 | - | - | - | - | * | - | - | - |
| 2012 | - | * | - | - | - | - | - | - |
| 2013 | * | * | - | - | * | - | - | - |
| 2014 | * | * | - | - | - | - | - | - |
| 2015 | * | * | - | - | - | - | - | - |
| 2016 | * | * | * | - | - | - | - | - |
| 2017 | * | * | - | * | - | - | - | - |
| 2018 | * | - | * | * | - | - | - | - |

| All \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 2188)** | **SMA Type 1 (N = 432)** | **SMA Type 2 (N = 914)** | **SMA Type 3 (N = 779)** | **SMA Type 4 (N = 22)** | **SMA Type Other (N = 7)** | **SMA Type Presymptomatic (N = 8)** | **SMA Type Missing (N = 26)** |
| 2019 | 5 (0.2%) | * | - | * | - | - | - | - |
| 2020 | * | * | * | - | - | - | - | - |
| 2021 | 6 (0.3%) | * | * | * | - | - | - | - |
| 2022 | 9 (0.4%) | * | 6 (0.7%) | * | - | - | - | - |
| 2023 | - | * | - | - | - | - | - | - |
| Missing | 2142 (97.9%) | 408 (94.4%) | 900 (98.5%) | 773 (99.2%) | 20 (90.9%) | 7 (100.0%) | 8 (100.0%) | 26 (100.0%) |
| **Sex; n (%)** | | | | | | | | |
| Female | 1059 (48.4%) | 206 (47.7%) | 457 (50.0%) | 367 (47.1%) | 10 (45.5%) | * | * | 15 (57.7%) |
| Male | 1129 (51.6%) | 226 (52.3%) | 457 (50.0%) | 412 (52.9%) | 12 (54.5%) | * | 7 (87.5%) | 11 (42.3%) |
| Missing | - | - | - | - | - | - | - | - |
| **Class of age at symptom onset; n (%)** | | | | | | | | |
| Presymptomatic | 8 (0.4%) | - | - | - | - | - | 8 (100.0%) | - |
| Prenatal | 6 (0.3%) | * | * | - | - | - | - | - |
| < 1 month | 32 (1.5%) | 22 (5.1%) | * | 5 (0.6%) | - | * | - | - |
| [1 - 3 months) | 111 (5.1%) | 102 (23.6%) | 7 (0.8%) | * | - | - | - | * |
| [3 - 6 months) | 146 (6.7%) | 108 (25.0%) | 36 (3.9%) | * | - | - | - | - |
| [6 - 18 months) | 645 (29.5%) | 47 (10.9%) | 500 (54.7%) | 95 (12.2%) | - | - | - | * |
| [1.5 - 2 years) | 116 (5.3%) | * | 47 (5.1%) | 68 (8.7%) | - | - | - | - |
| [2 - 6 years) | 254 (11.6%) | - | 18 (2.0%) | 236 (30.3%) | - | - | - | - |
| [6 - 11 years) | 59 (2.7%) | - | * | 54 (6.9%) | * | - | - | * |
| [11 - 18 years) | 102 (4.7%) | * | * | 94 (12.1%) | 5 (22.7%) | * | - | - |
| 18 years + | 30 (1.4%) | - | - | 16 (2.1%) | 13 (59.1%) | * | - | - |
| Missing | 679 (31.0%) | 148 (34.3%) | 296 (32.4%) | 208 (26.7%) | * | * | - | 21 (80.8%) |
| **Best functional SMA status; n (%)** | | | | | | | | |
| Non-sitter | 123 (5.6%) | 68 (15.7%) | 42 (4.6%) | 11 (1.4%) | - | - | * | - |
| Sitter | 554 (25.3%) | 108 (25.0%) | 409 (44.7%) | 31 (4.0%) | - | * | * | * |
| Walker | 757 (34.6%) | 33 (7.6%) | 161 (17.6%) | 535 (68.7%) | 18 (81.8%) | * | * | 7 (26.9%) |
| Missing | 754 (34.5%) | 223 (51.6%) | 302 (33.0%) | 202 (25.9%) | * | * | * | 17 (65.4%) |

| All \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 2188)** | **SMA Type 1 (N = 432)** | **SMA Type 2 (N = 914)** | **SMA Type 3 (N = 779)** | **SMA Type 4 (N = 22)** | **SMA Type Other (N = 7)** | **SMA Type Presymptomatic (N = 8)** | **SMA Type Missing (N = 26)** |
| **Best achieved motor milestone; n (%)** | | | | | | | | |
| Climb stairs | 427 (19.5%) | * | 20 (2.2%) | 385 (49.4%) | 14 (63.6%) | * | - | 5 (19.2%) |
| Walk 10 metres without assistance | 137 (6.3%) | 5 (1.2%) | 18 (2.0%) | 110 (14.1%) | * | * | - | * |
| Walk without assistance | 65 (3.0%) | 8 (1.9%) | 18 (2.0%) | 36 (4.6%) | * | - | - | * |
| Walk with assistance | 128 (5.9%) | 18 (4.2%) | 105 (11.5%) | * | - | - | * | - |
| Stand without assistance | 14 (0.6%) | - | 10 (1.1%) | * | - | - | - | * |
| Stand with assistance | 76 (3.5%) | 21 (4.9%) | 55 (6.0%) | - | - | - | - | - |
| Crawl | 106 (4.8%) | 6 (1.4%) | 89 (9.7%) | 9 (1.2%) | - | - | * | - |
| Sit without support | 358 (16.4%) | 81 (18.8%) | 255 (27.9%) | 19 (2.4%) | - | * | - | * |
| Roll onto side | 70 (3.2%) | 52 (12.0%) | 15 (1.6%) | * | - | - | * | - |
| Hold head without support | 53 (2.4%) | 16 (3.7%) | 27 (3.0%) | 9 (1.2%) | - | - | * | - |
| Unknown | - | - | - | - | - | - | - | - |
| Missing | 754 (34.5%) | 223 (51.6%) | 302 (33.0%) | 202 (25.9%) | * | * | * | 17 (65.4%) |
| **SMN1 gene mutation type; n (%)** | | | | | | | | |
| Compound heterozygous deletion exon 7 | 55 (2.5%) | 9 (2.1%) | 20 (2.2%) | 24 (3.1%) | * | - | - | * |
| Compound heterozygous substitutions | * | - | * | * | - | - | - | - |
| Homozygous deletion exon 7 | 1914 (87.5%) | 384 (88.9%) | 801 (87.6%) | 687 (88.2%) | 19 (86.4%) | 5 (71.4%) | 7 (87.5%) | 11 (42.3%) |
| Missing | 215 (9.8%) | 39 (9.0%) | 90 (9.8%) | 67 (8.6%) | * | * | * | 14 (53.8%) |
| **Number of SMN2 copies; n (%)** | | | | | | | | |
| 0 | 15 (0.7%) | * | 7 (0.8%) | 6 (0.8%) | - | - | - | - |
| 1 | 10 (0.5%) | * | * | * | - | * | - | - |
| 2 | 321 (14.7%) | 235 (54.4%) | 52 (5.7%) | 29 (3.7%) | * | - | * | * |

| All \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 2188)** | **SMA Type 1 (N = 432)** | **SMA Type 2 (N = 914)** | **SMA Type 3 (N = 779)** | **SMA Type 4 (N = 22)** | **SMA Type Other (N = 7)** | **SMA Type Presymptomatic (N = 8)** | **SMA Type Missing (N = 26)** |
| 3 | 800 (36.6%) | 85 (19.7%) | 479 (52.4%) | 223 (28.6%) | * | * | * | * |
| 4 | 251 (11.5%) | - | 30 (3.3%) | 208 (26.7%) | 10 (45.5%) | - | * | * |
| >4 | 10 (0.5%) | * | * | 7 (0.9%) | - | - | - | - |
| Other | 52 (2.4%) | * | * | 39 (5.0%) | * | * | - | * |
| Missing | 729 (33.3%) | 105 (24.3%) | 337 (36.9%) | 263 (33.8%) | 5 (22.7%) | * | * | 16 (61.5%) |
| **Methods used for genetic testing; n (%)** | | | | | | | | |
| DNA Sequencing | - | - | * | - | - | - | - | - |
| HRM | - | - | - | * | - | - | - | - |
| MLPA | 527 (24.1%) | 88 (20.4%) | 195 (21.3%) | 215 (27.6%) | 10 (45.5%) | 5 (71.4%) | 6 (75.0%) | 8 (30.8%) |
| RFLP | 5 (0.2%) | - | 5 (0.5%) | - | - | - | - | - |
| ddPCR | * | - | * | * | - | - | - | - |
| qRT-PCR | 25 (1.1%) | * | 7 (0.8%) | 13 (1.7%) | * | - | - | - |
| Missing | 1625 (74.3%) | 340 (78.7%) | 705 (77.1%) | 547 (70.2%) | 11 (50.0%) | * | * | 18 (69.2%) |
| **Duration of follow up (months)** | | | | | | | | |
| Mean (SD) | 70.9 (38.4) | 59.2 (33.2) | 76.4 (38.4) | 73.1 (38.6) | 41.8 (18.7) | 54.7 (45.1) | 13.1 (5) | 45.2 (47) |
| Median [IQR] | - | - | - | - | - | - | - | - |
| Missing; n (%) | 16 (0.7%) | 7 (1.6%) | * | * | * | - | - | * |
| **Duration of SMA (months)** | | | | | | | | |
| Mean (SD) | 252.1 (195.8) | 90.1 (78) | 240 (166.6) | 343.1 (210.2) | 319.9 (155.8) | * | - | 71.8 (17.8) |
| Median [IQR] | - | - | - | - | - | * | - | - |
| Missing; n (%) | 699 (31.9%) | 153 (35.4%) | 299 (32.7%) | 210 (27.0%) | * | * | 8 (100.0%) | 21 (80.8%) |
| **Duration between two consecutive visits (months)** | | | | | | | | |
| Mean (SD) | 20.4 (116.4) | 15.8 (19.8) | 19.7 (25.3) | 24 (191.7) | 9.2 (5.8) | 10.1 (8.9) | 2.4 (1.3) | 27.1 (50.1) |
| Median [IQR] | - | - | - | - | - | - | - | - |
| Missing; n (%) | 182 (8.3%) | 43 (10.0%) | 60 (6.6%) | 60 (7.7%) | * | * | * | 12 (46.2%) |

| All \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 2188)** | **SMA Type 1 (N = 432)** | **SMA Type 2 (N = 914)** | **SMA Type 3 (N = 779)** | **SMA Type 4 (N = 22)** | **SMA Type Other (N = 7)** | **SMA Type Presymptomatic (N = 8)** | **SMA Type Missing (N = 26)** |
| **Duration between genetic report date and registry entry (months)** | | | | | | | | |
| Mean (SD) | 72.8 (94) | 22.6 (50.7) | 82.6 (90.5) | 90.4 (105.9) | 56.9 (88.1) | 2.4 (3.5) | 0.3 (0.5) | 10.8 (27) |
| Median [IQR] | - | - | - | - | - | - | - | - |
| Missing; n (%) | 569 (26.0%) | 124 (28.7%) | 249 (27.2%) | 173 (22.2%) | * | * | * | 17 (65.4%) |
| **Reason for genetic testing; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Family screening | 8 (1.3%) | - | * | 5 (1.9%) | * | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 303 (49.1%) | 15 (40.5%) | 154 (50.5%) | 132 (48.9%) | * | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Family screening | * | - | - | * | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 80 (44.4%) | 11 (34.4%) | 38 (50.0%) | 30 (42.3%) | * | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| Family screening | 8 (3.2%) | * | * | * | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 99 (39.9%) | 19 (27.5%) | 41 (40.6%) | 38 (51.4%) | * | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| Family screening | * | * | - | * | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 66 (50.4%) | 23 (59.0%) | 27 (55.1%) | 15 (35.7%) | * | - | - | - |

| All \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 2188)** | **SMA Type 1 (N = 432)** | **SMA Type 2 (N = 914)** | **SMA Type 3 (N = 779)** | **SMA Type 4 (N = 22)** | **SMA Type Other (N = 7)** | **SMA Type Presymptomatic (N = 8)** | **SMA Type Missing (N = 26)** |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| Family screening | * | - | * | - | - | - | - | - |
| Newborn screening | * | - | - | - | - | - | - | * |
| No screening | 74 (56.9%) | 15 (41.7%) | 28 (63.6%) | 27 (61.4%) | * | - | - | * |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| Family screening | 7 (5.4%) | * | * | * | - | - | - | * |
| Newborn screening | 5 (3.8%) | * | - | - | - | * | - | * |
| No screening | 77 (59.2%) | 21 (51.2%) | 26 (68.4%) | 30 (66.7%) | - | - | - | - |
| Prenatal screening | * | - | - | * | - | - | - | - |
| **2020** | | | | | | | | |
| Family screening | * | * | - | - | - | - | - | - |
| Newborn screening | * | * | - | - | - | - | - | - |
| No screening | 35 (56.5%) | 7 (36.8%) | 11 (52.4%) | 15 (75.0%) | * | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| Family screening | 5 (5.7%) | - | * | * | - | * | - | * |
| Newborn screening | * | - | - | - | - | * | - | - |
| No screening | 75 (85.2%) | 26 (96.3%) | 20 (87.0%) | 26 (89.7%) | * | - | - | - |
| Prenatal screening | * | - | - | - | - | - | * | - |
| **2022** | | | | | | | | |
| Family screening | * | - | - | * | - | - | - | - |
| Newborn screening | 6 (14.3%) | * | - | - | - | - | * | - |
| No screening | 34 (81.0%) | 12 (85.7%) | 9 (100.0%) | 11 (84.6%) | - | * | - | * |

| All \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 2188)** | **SMA Type 1 (N = 432)** | **SMA Type 2 (N = 914)** | **SMA Type 3 (N = 779)** | **SMA Type 4 (N = 22)** | **SMA Type Other (N = 7)** | **SMA Type Presymptomatic (N = 8)** | **SMA Type Missing (N = 26)** |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | * | - | - | - | - | - | * | - |
| No screening | - | - | - | - | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **Age at onset of SMA symptoms (years)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | 3.7 (5.8) | 0.7 (2) | 1 (0.9) | 5.5 (5.8) | 24.9 (12.3) | * | - | - |
| Median [IQR] | - | - | - | - | - | * | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | 3.2 (8) | 0.4 (0.2) | 0.8 (0.3) | 5.2 (9) | * | - | - | - |
| Median [IQR] | - | - | - | - | * | - | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | 1.3 (2.4) | 0.3 (0.2) | 0.9 (0.3) | 4 (4.2) | - | - | - | * |
| Median [IQR] | - | - | - | - | - | - | - | * |
| **2017** | | | | | | | | |
| Mean (SD) | 1.1 (2.2) | 0.2 (0.1) | 1 (0.3) | 3.3 (4.2) | - | - | - | * |
| Median [IQR] | - | - | - | - | - | - | - | * |
| **2018** | | | | | | | | |
| Mean (SD) | 0.6 (0.6) | 0.2 (0.2) | 0.8 (0.4) | 1.7 (0.8) | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| Mean (SD) | 1.5 (3.6) | 0.2 (0.1) | 0.8 (0.3) | 5.2 (5.2) | * | - | - | * |
| Median [IQR] | - | - | - | - | * | - | - | * |
| **2020** | | | | | | | | |
| Mean (SD) | 1.1 (2.1) | 0.2 (0.2) | 1.5 (2.3) | 2.9 (2.7) | - | - | - | - |

| All \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 2188)** | **SMA Type 1 (N = 432)** | **SMA Type 2 (N = 914)** | **SMA Type 3 (N = 779)** | **SMA Type 4 (N = 22)** | **SMA Type Other (N = 7)** | **SMA Type Presymptomatic (N = 8)** | **SMA Type Missing (N = 26)** |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | 1.3 (2.7) | 0.3 (0.2) | 0.7 (0.3) | 5.1 (4.5) | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2022** | | | | | | | | |
| Mean (SD) | 1.2 (4.1) | 0.2 (0.1) | * | * | - | * | - | - |
| Median [IQR] | - | - | * | * | - | * | - | - |
| **2023** | | | | | | | | |
| Mean (SD) | * | - | - | * | - | - | - | - |
| Median [IQR] | * | - | - | * | - | - | - | - |
| **Age at genetic report date (years)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | 11.5 (14.1) | 1.4 (2.2) | 6.3 (10.2) | 18 (15) | 47 (2.6) | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | 11.3 (15.8) | 0.8 (0.9) | 5.8 (9) | 21.9 (18.7) | * | - | - | - |
| Median [IQR] | - | - | - | - | * | - | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | 9.2 (14.9) | 1.5 (6.2) | 5.3 (9.7) | 19.7 (18.1) | * | - | - | - |
| Median [IQR] | - | - | - | - | * | - | - | - |
| **2017** | | | | | | | | |
| Mean (SD) | 14.4 (17.3) | 2.2 (6.1) | 13.4 (14.9) | 26.4 (18.7) | * | - | - | - |
| Median [IQR] | - | - | - | - | * | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | 15.8 (19.2) | 0.5 (0.6) | 16.8 (18.7) | 27 (18.9) | * | - | - | * |
| Median [IQR] | - | - | - | - | * | - | - | * |
| **2019** | | | | | | | | |
| Mean (SD) | 14.6 (19.4) | 0.5 (1) | 13.1 (17.8) | 27.8 (19.4) | * | * | - | * |
| Median [IQR] | - | - | - | - | * | * | - | * |

| All \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 2188)** | **SMA Type 1 (N = 432)** | **SMA Type 2 (N = 914)** | **SMA Type 3 (N = 779)** | **SMA Type 4 (N = 22)** | **SMA Type Other (N = 7)** | **SMA Type Presymptomatic (N = 8)** | **SMA Type Missing (N = 26)** |
| **2020** | | | | | | | | |
| Mean (SD) | 15.4 (20.1) | 1.5 (5.1) | 14.4 (16.8) | 26.9 (23) | * | - | - | - |
| Median [IQR] | - | - | - | - | * | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | 15.9 (20.3) | 2.1 (5.8) | 21.4 (19.6) | 22.9 (20.2) | * | * | * | * |
| Median [IQR] | - | - | - | - | * | * | * | * |
| **2022** | | | | | | | | |
| Mean (SD) | 12.1 (18.6) | 0.3 (0.2) | 15.2 (15.7) | 27.1 (22.5) | - | * | * | * |
| Median [IQR] | - | - | - | - | - | * | * | * |
| **2023** | | | | | | | | |
| Mean (SD) | * | - | - | - | - | - | * | - |
| Median [IQR] | * | - | - | - | - | - | * | - |
| **Age at registry entry (years)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | 14.6 (16.2) | 1.7 (2.1) | 13.6 (12.8) | 21.9 (18.5) | - | - | - | 28.6 (25.8) |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | 18.1 (16.6) | 3.1 (5) | 14.1 (12.6) | 28.2 (16.9) | - | * | - | - |
| Median [IQR] | - | - | - | - | - | * | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | 15.9 (17.6) | 3 (6.2) | 11.8 (12.2) | 27.9 (19.8) | * | * | - | * |
| Median [IQR] | - | - | - | - | * | * | - | * |
| **2017** | | | | | | | | |
| Mean (SD) | 20.8 (18.2) | 5.6 (6.6) | 16.5 (15) | 33.3 (17.5) | * | - | - | - |
| Median [IQR] | - | - | - | - | * | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | 17.5 (17) | 2.8 (4.5) | 14.7 (12.6) | 27.5 (18.4) | 44.7 (14.7) | - | - | * |
| Median [IQR] | - | - | - | - | - | - | - | * |
| **2019** | | | | | | | | |

| All \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 2188)** | **SMA Type 1 (N = 432)** | **SMA Type 2 (N = 914)** | **SMA Type 3 (N = 779)** | **SMA Type 4 (N = 22)** | **SMA Type Other (N = 7)** | **SMA Type Presymptomatic (N = 8)** | **SMA Type Missing (N = 26)** |
| Mean (SD) | 16.8 (18.9) | 0.8 (1) | 15.4 (16.8) | 27.8 (18.1) | 46.7 (14.3) | * | - | * |
| Median [IQR] | - | - | - | - | - | * | - | * |
| **2020** | | | | | | | | |
| Mean (SD) | 18.8 (18.3) | 3 (6.6) | 17.5 (14.5) | 30.5 (19.3) | * | - | - | * |
| Median [IQR] | - | - | - | - | * | - | - | * |
| **2021** | | | | | | | | |
| Mean (SD) | 20.1 (19.5) | 4.2 (10) | 21.6 (17.2) | 28.6 (19.2) | * | * | * | * |
| Median [IQR] | - | - | - | - | * | * | * | * |
| **2022** | | | | | | | | |
| Mean (SD) | 20.4 (20.3) | 1.8 (3.6) | 24.6 (17.8) | 33.6 (18.8) | * | * | * | 17.4 (9.1) |
| Median [IQR] | - | - | - | - | * | * | * | - |
| **2023** | | | | | | | | |
| Mean (SD) | 24.1 (19.2) | 2.5 (1.9) | 31.5 (13.9) | 26.6 (15.5) | * | - | * | * |
| Median [IQR] | - | - | - | - | * | - | * | * |
| **Age at death (years)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | * | - | * | - | * | - | - | - |
| Median [IQR] | * | - | * | - | * | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | * | * | - | - | * | - | - | - |
| Median [IQR] | * | * | - | - | * | - | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | 1.6 (1.7) | 1 (1) | * | - | - | - | - | - |
| Median [IQR] | - | - | * | - | - | - | - | - |
| **2017** | | | | | | | | |
| Mean (SD) | * | * | - | * | - | - | - | - |
| Median [IQR] | * | * | - | * | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | * | - | * | * | - | - | - | - |

| All \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 2188)** | **SMA Type 1 (N = 432)** | **SMA Type 2 (N = 914)** | **SMA Type 3 (N = 779)** | **SMA Type 4 (N = 22)** | **SMA Type Other (N = 7)** | **SMA Type Presymptomatic (N = 8)** | **SMA Type Missing (N = 26)** |
| Median [IQR] | * | - | * | * | - | - | - | - |
| **2019** | | | | | | | | |
| Mean (SD) | 19.3 (21.2) | * | - | * | - | - | - | - |
| Median [IQR] | - | * | - | * | - | - | - | - |
| **2020** | | | | | | | | |
| Mean (SD) | * | * | * | - | - | - | - | - |
| Median [IQR] | * | * | * | - | - | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | 20 (22.2) | * | * | * | - | - | - | - |
| Median [IQR] | - | * | * | * | - | - | - | - |
| **2022** | | | | | | | | |
| Mean (SD) | 32 (21.4) | * | 33.4 (13) | * | - | - | - | - |
| Median [IQR] | - | * | - | * | - | - | - | - |
| **2023** | | | | | | | | |
| Mean (SD) | * | * | - | - | - | - | - | - |
| Median [IQR] | * | * | - | - | - | - | - | - |
| **Lost to follow-up; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 85 (93.4%) | 14 (93.3%) | 44 (91.7%) | 22 (95.7%) | - | - | - | 5 (100.0%) |
| Yes | 6 (6.6%) | * | * | * | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 383 (97.7%) | 61 (95.3%) | 174 (98.3%) | 143 (98.6%) | - | * | - | * |
| Yes | 9 (2.3%) | * | * | * | - | - | - | * |
| **2014-2016** | | | | | | | | |
| No | 851 (93.9%) | 139 (85.8%) | 392 (96.6%) | 313 (94.8%) | * | * | - | * |
| Yes | 55 (6.1%) | 23 (14.2%) | 14 (3.4%) | 17 (5.2%) | - | - | - | * |
| **2017** | | | | | | | | |
| No | 1017 (97.2%) | 157 (91.8%) | 474 (98.3%) | 379 (98.4%) | * | * | - | * |
| Yes | 29 (2.8%) | 14 (8.2%) | 8 (1.7%) | 6 (1.6%) | - | - | - | * |

| All \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 2188)** | **SMA Type 1 (N = 432)** | **SMA Type 2 (N = 914)** | **SMA Type 3 (N = 779)** | **SMA Type 4 (N = 22)** | **SMA Type Other (N = 7)** | **SMA Type Presymptomatic (N = 8)** | **SMA Type Missing (N = 26)** |
| **2018** | | | | | | | | |
| No | 1428 (98.9%) | 227 (97.8%) | 657 (99.2%) | 529 (98.9%) | 7 (100.0%) | * | - | 6 (100.0%) |
| Yes | 16 (1.1%) | 5 (2.2%) | 5 (0.8%) | 6 (1.1%) | - | - | - | - |
| **2019** | | | | | | | | |
| No | 1598 (98.8%) | 271 (98.5%) | 714 (99.2%) | 591 (98.8%) | 11 (91.7%) | * | - | 8 (88.9%) |
| Yes | 19 (1.2%) | * | 6 (0.8%) | 7 (1.2%) | * | - | - | * |
| **2020** | | | | | | | | |
| No | 1725 (99.0%) | 293 (97.7%) | 777 (99.2%) | 630 (99.2%) | 13 (100.0%) | * | - | 9 (100.0%) |
| Yes | 18 (1.0%) | 7 (2.3%) | 6 (0.8%) | 5 (0.8%) | - | - | - | - |
| **2021** | | | | | | | | |
| No | 1805 (98.4%) | 307 (97.2%) | 800 (99.0%) | 667 (98.4%) | 14 (93.3%) | 6 (100.0%) | * | 10 (90.9%) |
| Yes | 30 (1.6%) | 9 (2.8%) | 8 (1.0%) | 11 (1.6%) | * | - | - | * |
| **2022** | | | | | | | | |
| No | 1611 (83.9%) | 283 (84.2%) | 698 (83.5%) | 585 (83.3%) | 17 (100.0%) | 7 (100.0%) | 5 (100.0%) | 16 (94.1%) |
| Yes | 309 (16.1%) | 53 (15.8%) | 138 (16.5%) | 117 (16.7%) | - | - | - | * |
| **2023** | | | | | | | | |
| No | 1446 (87.9%) | 264 (91.7%) | 625 (88.4%) | 517 (86.3%) | 12 (66.7%) | 6 (85.7%) | 8 (100.0%) | 14 (77.8%) |
| Yes | 199 (12.1%) | 24 (8.3%) | 82 (11.6%) | 82 (13.7%) | 6 (33.3%) | * | - | * |
| **Treated with at least one DMT; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 91 (100.0%) | 15 (100.0%) | 48 (100.0%) | 23 (100.0%) | - | - | - | 5 (100.0%) |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 389 (100.0%) | 64 (100.0%) | 175 (100.0%) | 145 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 853 (97.8%) | 135 (91.8%) | 389 (98.5%) | 321 (99.7%) | * | * | - | 5 (100.0%) |
| Yes | 19 (2.2%) | 12 (8.2%) | 6 (1.5%) | * | - | - | - | - |
| **2017** | | | | | | | | |

| All \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 2188)** | **SMA Type 1 (N = 432)** | **SMA Type 2 (N = 914)** | **SMA Type 3 (N = 779)** | **SMA Type 4 (N = 22)** | **SMA Type Other (N = 7)** | **SMA Type Presymptomatic (N = 8)** | **SMA Type Missing (N = 26)** |
| No | 877 (87.8%) | 100 (66.2%) | 421 (90.0%) | 349 (93.6%) | * | * | - | * |
| Yes | 122 (12.2%) | 51 (33.8%) | 47 (10.0%) | 24 (6.4%) | - | - | - | - |
| **2018** | | | | | | | | |
| No | 920 (65.3%) | 102 (45.9%) | 450 (69.2%) | 361 (69.0%) | * | * | - | * |
| Yes | 488 (34.7%) | 120 (54.1%) | 200 (30.8%) | 162 (31.0%) | * | - | - | * |
| **2019** | | | | | | | | |
| No | 862 (54.6%) | 102 (38.8%) | 405 (57.1%) | 342 (58.5%) | 7 (63.6%) | * | - | * |
| Yes | 717 (45.4%) | 161 (61.2%) | 304 (42.9%) | 243 (41.5%) | * | * | - | * |
| **2020** | | | | | | | | |
| No | 794 (46.8%) | 86 (30.3%) | 384 (49.9%) | 311 (50.2%) | 7 (58.3%) | * | - | * |
| Yes | 901 (53.2%) | 198 (69.7%) | 385 (50.1%) | 308 (49.8%) | 5 (41.7%) | * | - | * |
| **2021** | | | | | | | | |
| No | 473 (31.6%) | 46 (18.1%) | 215 (32.5%) | 200 (36.4%) | 7 (50.0%) | * | - | * |
| Yes | 1023 (68.4%) | 208 (81.9%) | 447 (67.5%) | 350 (63.6%) | 7 (50.0%) | * | * | 6 (66.7%) |
| **2022** | | | | | | | | |
| No | 438 (33.9%) | 50 (20.7%) | 206 (36.6%) | 167 (36.2%) | 6 (66.7%) | * | * | 5 (55.6%) |
| Yes | 854 (66.1%) | 191 (79.3%) | 357 (63.4%) | 294 (63.8%) | * | * | * | * |
| **2023** | | | | | | | | |
| No | 330 (42.1%) | 36 (26.5%) | 154 (45.6%) | 128 (44.8%) | * | * | - | 6 (75.0%) |
| Yes | 454 (57.9%) | 100 (73.5%) | 184 (54.4%) | 158 (55.2%) | * | * | 7 (100.0%) | * |
| **Treated with more than one DMT; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 91 (100.0%) | 15 (100.0%) | 48 (100.0%) | 23 (100.0%) | - | - | - | 5 (100.0%) |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 389 (100.0%) | 64 (100.0%) | 175 (100.0%) | 145 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 872 (100.0%) | 147 (100.0%) | 395 (100.0%) | 322 (100.0%) | * | * | - | 5 (100.0%) |

| All \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 2188)** | **SMA Type 1 (N = 432)** | **SMA Type 2 (N = 914)** | **SMA Type 3 (N = 779)** | **SMA Type 4 (N = 22)** | **SMA Type Other (N = 7)** | **SMA Type Presymptomatic (N = 8)** | **SMA Type Missing (N = 26)** |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 999 (100.0%) | 151 (100.0%) | 468 (100.0%) | 373 (100.0%) | * | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 1408 (100.0%) | 222 (100.0%) | 650 (100.0%) | 523 (100.0%) | 6 (100.0%) | * | - | 5 (100.0%) |
| Yes | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 1576 (99.8%) | 261 (99.2%) | 708 (99.9%) | 585 (100.0%) | 11 (100.0%) | * | - | 8 (100.0%) |
| Yes | * | * | * | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 1661 (98.0%) | 267 (94.0%) | 752 (97.8%) | 619 (100.0%) | 12 (100.0%) | * | - | 8 (100.0%) |
| Yes | 34 (2.0%) | 17 (6.0%) | 17 (2.2%) | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 1449 (96.9%) | 235 (92.5%) | 645 (97.4%) | 539 (98.0%) | 14 (100.0%) | 6 (100.0%) | * | 9 (100.0%) |
| Yes | 47 (3.1%) | 19 (7.5%) | 17 (2.6%) | 11 (2.0%) | - | - | - | - |
| **2022** | | | | | | | | |
| No | 1223 (94.7%) | 217 (90.0%) | 543 (96.4%) | 438 (95.0%) | 9 (100.0%) | * | * | 9 (100.0%) |
| Yes | 69 (5.3%) | 24 (10.0%) | 20 (3.6%) | 23 (5.0%) | - | * | * | - |
| **2023** | | | | | | | | |
| No | 778 (99.2%) | 130 (95.6%) | 338 (100.0%) | 286 (100.0%) | 6 (100.0%) | * | 7 (100.0%) | 8 (100.0%) |
| Yes | 6 (0.8%) | 6 (4.4%) | - | - | - | - | - | - |
| **Treated with nusinersen; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 91 (100.0%) | 15 (100.0%) | 48 (100.0%) | 23 (100.0%) | - | - | - | 5 (100.0%) |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 389 (100.0%) | 64 (100.0%) | 175 (100.0%) | 145 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |

| All \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 2188)** | **SMA Type 1 (N = 432)** | **SMA Type 2 (N = 914)** | **SMA Type 3 (N = 779)** | **SMA Type 4 (N = 22)** | **SMA Type Other (N = 7)** | **SMA Type Presymptomatic (N = 8)** | **SMA Type Missing (N = 26)** |
| **2014-2016** | | | | | | | | |
| No | 853 (97.8%) | 135 (91.8%) | 389 (98.5%) | 321 (99.7%) | * | * | - | 5 (100.0%) |
| Yes | 19 (2.2%) | 12 (8.2%) | 6 (1.5%) | * | - | - | - | - |
| **2017** | | | | | | | | |
| No | 882 (88.3%) | 100 (66.2%) | 425 (90.8%) | 350 (93.8%) | * | * | - | * |
| Yes | 117 (11.7%) | 51 (33.8%) | 43 (9.2%) | 23 (6.2%) | - | - | - | - |
| **2018** | | | | | | | | |
| No | 954 (67.8%) | 103 (46.4%) | 474 (72.9%) | 369 (70.6%) | * | * | - | * |
| Yes | 454 (32.2%) | 119 (53.6%) | 176 (27.1%) | 154 (29.4%) | * | - | - | * |
| **2019** | | | | | | | | |
| No | 910 (57.6%) | 105 (39.9%) | 438 (61.8%) | 351 (60.0%) | 7 (63.6%) | * | - | 6 (75.0%) |
| Yes | 669 (42.4%) | 158 (60.1%) | 271 (38.2%) | 234 (40.0%) | * | - | - | * |
| **2020** | | | | | | | | |
| No | 894 (52.7%) | 109 (38.4%) | 450 (58.5%) | 319 (51.5%) | 7 (58.3%) | * | - | 6 (75.0%) |
| Yes | 801 (47.3%) | 175 (61.6%) | 319 (41.5%) | 300 (48.5%) | 5 (41.7%) | - | - | * |
| **2021** | | | | | | | | |
| No | 731 (48.9%) | 101 (39.8%) | 382 (57.7%) | 228 (41.5%) | 7 (50.0%) | 5 (83.3%) | * | 7 (77.8%) |
| Yes | 765 (51.1%) | 153 (60.2%) | 280 (42.3%) | 322 (58.5%) | 7 (50.0%) | * | - | * |
| **2022** | | | | | | | | |
| No | 743 (57.5%) | 130 (53.9%) | 361 (64.1%) | 230 (49.9%) | 7 (77.8%) | * | * | 8 (88.9%) |
| Yes | 549 (42.5%) | 111 (46.1%) | 202 (35.9%) | 231 (50.1%) | * | * | * | * |
| **2023** | | | | | | | | |
| No | 521 (66.5%) | 81 (59.6%) | 234 (69.2%) | 185 (64.7%) | 5 (83.3%) | * | 6 (85.7%) | 7 (87.5%) |
| Yes | 263 (33.5%) | 55 (40.4%) | 104 (30.8%) | 101 (35.3%) | * | - | * | * |
| **Treated with onasemnogene abeparvovec-xioi; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 91 (100.0%) | 15 (100.0%) | 48 (100.0%) | 23 (100.0%) | - | - | - | 5 (100.0%) |
| Yes | - | - | - | - | - | - | - | - |

| All \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 2188)** | **SMA Type 1 (N = 432)** | **SMA Type 2 (N = 914)** | **SMA Type 3 (N = 779)** | **SMA Type 4 (N = 22)** | **SMA Type Other (N = 7)** | **SMA Type Presymptomatic (N = 8)** | **SMA Type Missing (N = 26)** |
| **2011-2013** | | | | | | | | |
| No | 389 (100.0%) | 64 (100.0%) | 175 (100.0%) | 145 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 872 (100.0%) | 147 (100.0%) | 395 (100.0%) | 322 (100.0%) | * | * | - | 5 (100.0%) |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 999 (100.0%) | 151 (100.0%) | 468 (100.0%) | 373 (100.0%) | * | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 1406 (99.9%) | 221 (99.5%) | 649 (99.8%) | 523 (100.0%) | 6 (100.0%) | * | - | 5 (100.0%) |
| Yes | * | * | * | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 1572 (99.6%) | 258 (98.1%) | 708 (99.9%) | 585 (100.0%) | 11 (100.0%) | * | - | 8 (100.0%) |
| Yes | 7 (0.4%) | 5 (1.9%) | * | - | - | * | - | - |
| **2020** | | | | | | | | |
| No | 1666 (98.3%) | 263 (92.6%) | 762 (99.1%) | 619 (100.0%) | 12 (100.0%) | * | - | 8 (100.0%) |
| Yes | 29 (1.7%) | 21 (7.4%) | 7 (0.9%) | - | - | * | - | - |
| **2021** | | | | | | | | |
| No | 1435 (95.9%) | 216 (85.0%) | 644 (97.3%) | 547 (99.5%) | 14 (100.0%) | 5 (83.3%) | - | 9 (100.0%) |
| Yes | 61 (4.1%) | 38 (15.0%) | 18 (2.7%) | * | - | * | * | - |
| **2022** | | | | | | | | |
| No | 1216 (94.1%) | 189 (78.4%) | 547 (97.2%) | 457 (99.1%) | 9 (100.0%) | * | * | 9 (100.0%) |
| Yes | 76 (5.9%) | 52 (21.6%) | 16 (2.8%) | * | - | - | * | - |
| **2023** | | | | | | | | |
| No | 742 (94.6%) | 110 (80.9%) | 332 (98.2%) | 282 (98.6%) | 6 (100.0%) | * | * | 8 (100.0%) |
| Yes | 42 (5.4%) | 26 (19.1%) | 6 (1.8%) | * | - | - | 6 (85.7%) | - |
| **Treated with risdiplan; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |

| All \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 2188)** | **SMA Type 1 (N = 432)** | **SMA Type 2 (N = 914)** | **SMA Type 3 (N = 779)** | **SMA Type 4 (N = 22)** | **SMA Type Other (N = 7)** | **SMA Type Presymptomatic (N = 8)** | **SMA Type Missing (N = 26)** |
| No | - | - | - | - | - | - | - | - |
| Yes | | | | | | | | |
| **2011-2013** | | | | | | | | |
| No | 91 (100.0%) | 15 (100.0%) | 48 (100.0%) | 23 (100.0%) | - | - | - | 5 (100.0%) |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 389 (100.0%) | 64 (100.0%) | 175 (100.0%) | 145 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 872 (100.0%) | 147 (100.0%) | 395 (100.0%) | 322 (100.0%) | * | * | - | 5 (100.0%) |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 994 (99.5%) | 151 (100.0%) | 464 (99.1%) | 372 (99.7%) | * | * | - | * |
| Yes | 5 (0.5%) | - | * | * | - | - | - | - |
| **2019** | | | | | | | | |
| No | 1376 (97.7%) | 222 (100.0%) | 627 (96.5%) | 515 (98.5%) | 6 (100.0%) | * | - | * |
| Yes | 32 (2.3%) | - | 23 (3.5%) | 8 (1.5%) | - | - | - | * |
| **2020** | | | | | | | | |
| No | 1535 (97.2%) | 263 (100.0%) | 676 (95.3%) | 576 (98.5%) | 11 (100.0%) | * | - | 6 (75.0%) |
| Yes | 44 (2.8%) | - | 33 (4.7%) | 9 (1.5%) | - | - | - | * |
| **2021** | | | | | | | | |
| No | 1590 (93.8%) | 265 (93.3%) | 693 (90.1%) | 611 (98.7%) | 12 (100.0%) | * | - | 6 (75.0%) |
| Yes | 105 (6.2%) | 19 (6.7%) | 76 (9.9%) | 8 (1.3%) | - | - | - | * |
| **2022** | | | | | | | | |
| No | 1252 (83.7%) | 218 (85.8%) | 496 (74.9%) | 514 (93.5%) | 14 (100.0%) | * | * | 5 (55.6%) |
| Yes | 244 (16.3%) | 36 (14.2%) | 166 (25.1%) | 36 (6.5%) | - | * | - | * |
| **2023** | | | | | | | | |
| No | 993 (76.9%) | 189 (78.4%) | 404 (71.8%) | 379 (82.2%) | 8 (88.9%) | * | * | 6 (66.7%) |
| Yes | 299 (23.1%) | 52 (21.6%) | 159 (28.2%) | 82 (17.8%) | * | * | * | * |

| All \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 2188)** | **SMA Type 1 (N = 432)** | **SMA Type 2 (N = 914)** | **SMA Type 3 (N = 779)** | **SMA Type 4 (N = 22)** | **SMA Type Other (N = 7)** | **SMA Type Presymptomatic (N = 8)** | **SMA Type Missing (N = 26)** |
| **At least one episode of invasive ventilation; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 91 (100.0%) | 15 (100.0%) | 48 (100.0%) | 23 (100.0%) | - | - | - | 5 (100.0%) |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 381 (97.9%) | 57 (89.1%) | 174 (99.4%) | 145 (100.0%) | - | * | - | * |
| Yes | 8 (2.1%) | 7 (10.9%) | * | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 855 (98.1%) | 133 (90.5%) | 393 (99.5%) | 321 (99.7%) | * | * | - | 5 (100.0%) |
| Yes | 17 (1.9%) | 14 (9.5%) | * | * | - | - | - | - |
| **2017** | | | | | | | | |
| No | 967 (96.8%) | 123 (81.5%) | 464 (99.1%) | 373 (100.0%) | * | * | - | * |
| Yes | 32 (3.2%) | 28 (18.5%) | * | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 1302 (92.5%) | 173 (77.9%) | 600 (92.3%) | 517 (98.9%) | 6 (100.0%) | * | - | * |
| Yes | 106 (7.5%) | 49 (22.1%) | 50 (7.7%) | 6 (1.1%) | - | - | - | * |
| **2019** | | | | | | | | |
| No | 1467 (92.9%) | 210 (79.8%) | 660 (93.1%) | 576 (98.5%) | 11 (100.0%) | * | - | 7 (87.5%) |
| Yes | 112 (7.1%) | 53 (20.2%) | 49 (6.9%) | 9 (1.5%) | - | - | - | * |
| **2020** | | | | | | | | |
| No | 1566 (92.4%) | 220 (77.5%) | 713 (92.7%) | 611 (98.7%) | 12 (100.0%) | * | - | 7 (87.5%) |
| Yes | 129 (7.6%) | 64 (22.5%) | 56 (7.3%) | 8 (1.3%) | - | - | - | * |
| **2021** | | | | | | | | |
| No | 1380 (92.2%) | 200 (78.7%) | 610 (92.1%) | 541 (98.4%) | 14 (100.0%) | 6 (100.0%) | * | 8 (88.9%) |
| Yes | 116 (7.8%) | 54 (21.3%) | 52 (7.9%) | 9 (1.6%) | - | - | - | * |
| **2022** | | | | | | | | |
| No | 1252 (96.9%) | 208 (86.3%) | 558 (99.1%) | 460 (99.8%) | 9 (100.0%) | * | 5 (100.0%) | 9 (100.0%) |
| Yes | 40 (3.1%) | 33 (13.7%) | 5 (0.9%) | * | - | * | - | - |
| **2023** | | | | | | | | |

| All \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 2188)** | **SMA Type 1 (N = 432)** | **SMA Type 2 (N = 914)** | **SMA Type 3 (N = 779)** | **SMA Type 4 (N = 22)** | **SMA Type Other (N = 7)** | **SMA Type Presymptomatic (N = 8)** | **SMA Type Missing (N = 26)** |
| No | 769 (98.1%) | 123 (90.4%) | 336 (99.4%) | 286 (100.0%) | 6 (100.0%) | * | 7 (100.0%) | 8 (100.0%) |
| Yes | 15 (1.9%) | 13 (9.6%) | * | - | - | - | - | - |
| **At least one episode of non-invasive ventilation; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 87 (95.6%) | 15 (100.0%) | 45 (93.8%) | 23 (100.0%) | - | - | - | * |
| Yes | * | - | * | - | - | - | - | * |
| **2011-2013** | | | | | | | | |
| No | 362 (93.1%) | 59 (92.2%) | 157 (89.7%) | 142 (97.9%) | - | * | - | * |
| Yes | 27 (6.9%) | 5 (7.8%) | 18 (10.3%) | * | - | - | - | * |
| **2014-2016** | | | | | | | | |
| No | 788 (90.4%) | 117 (79.6%) | 345 (87.3%) | 320 (99.4%) | * | * | - | * |
| Yes | 84 (9.6%) | 30 (20.4%) | 50 (12.7%) | * | - | * | - | * |
| **2017** | | | | | | | | |
| No | 869 (87.0%) | 112 (74.2%) | 384 (82.1%) | 366 (98.1%) | * | * | - | * |
| Yes | 130 (13.0%) | 39 (25.8%) | 84 (17.9%) | 7 (1.9%) | - | - | - | - |
| **2018** | | | | | | | | |
| No | 1162 (82.5%) | 152 (68.5%) | 492 (75.7%) | 507 (96.9%) | 6 (100.0%) | * | - | * |
| Yes | 246 (17.5%) | 70 (31.5%) | 158 (24.3%) | 16 (3.1%) | - | - | - | * |
| **2019** | | | | | | | | |
| No | 1296 (82.1%) | 171 (65.0%) | 542 (76.4%) | 564 (96.4%) | 11 (100.0%) | * | - | 6 (75.0%) |
| Yes | 283 (17.9%) | 92 (35.0%) | 167 (23.6%) | 21 (3.6%) | - | * | - | * |
| **2020** | | | | | | | | |
| No | 1293 (76.3%) | 160 (56.3%) | 526 (68.4%) | 586 (94.7%) | 12 (100.0%) | * | - | 6 (75.0%) |
| Yes | 402 (23.7%) | 124 (43.7%) | 243 (31.6%) | 33 (5.3%) | - | - | - | * |
| **2021** | | | | | | | | |
| No | 1164 (77.8%) | 143 (56.3%) | 469 (70.8%) | 524 (95.3%) | 14 (100.0%) | 6 (100.0%) | * | 7 (77.8%) |
| Yes | 332 (22.2%) | 111 (43.7%) | 193 (29.2%) | 26 (4.7%) | - | - | - | * |
| **2022** | | | | | | | | |

| All \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 2188)** | **SMA Type 1 (N = 432)** | **SMA Type 2 (N = 914)** | **SMA Type 3 (N = 779)** | **SMA Type 4 (N = 22)** | **SMA Type Other (N = 7)** | **SMA Type Presymptomatic (N = 8)** | **SMA Type Missing (N = 26)** |
| No | 1030 (79.7%) | 154 (63.9%) | 409 (72.6%) | 441 (95.7%) | 9 (100.0%) | * | 5 (100.0%) | 8 (88.9%) |
| Yes | 262 (20.3%) | 87 (36.1%) | 154 (27.4%) | 20 (4.3%) | - | - | - | * |
| **2023** | | | | | | | | |
| No | 641 (81.8%) | 87 (64.0%) | 254 (75.1%) | 276 (96.5%) | 6 (100.0%) | * | 7 (100.0%) | 8 (100.0%) |
| Yes | 143 (18.2%) | 49 (36.0%) | 84 (24.9%) | 10 (3.5%) | - | - | - | - |
| **At least one episode of feeding tube usage; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 91 (100.0%) | 15 (100.0%) | 48 (100.0%) | 23 (100.0%) | - | - | - | 5 (100.0%) |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 365 (93.8%) | 48 (75.0%) | 167 (95.4%) | 145 (100.0%) | - | * | - | * |
| Yes | 24 (6.2%) | 16 (25.0%) | 8 (4.6%) | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 790 (90.6%) | 93 (63.3%) | 369 (93.4%) | 320 (99.4%) | * | * | - | 5 (100.0%) |
| Yes | 82 (9.4%) | 54 (36.7%) | 26 (6.6%) | * | - | - | - | - |
| **2017** | | | | | | | | |
| No | 934 (93.5%) | 106 (70.2%) | 448 (95.7%) | 373 (100.0%) | * | * | - | * |
| Yes | 65 (6.5%) | 45 (29.8%) | 20 (4.3%) | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 1271 (90.3%) | 131 (59.0%) | 607 (93.4%) | 520 (99.4%) | 6 (100.0%) | * | - | 5 (100.0%) |
| Yes | 137 (9.7%) | 91 (41.0%) | 43 (6.6%) | * | - | - | - | - |
| **2019** | | | | | | | | |
| No | 1433 (90.8%) | 153 (58.2%) | 674 (95.1%) | 584 (99.8%) | 11 (100.0%) | * | - | 8 (100.0%) |
| Yes | 146 (9.2%) | 110 (41.8%) | 35 (4.9%) | * | - | - | - | - |
| **2020** | | | | | | | | |
| No | 1541 (90.9%) | 175 (61.6%) | 725 (94.3%) | 618 (99.8%) | 12 (100.0%) | * | - | 8 (100.0%) |
| Yes | 154 (9.1%) | 109 (38.4%) | 44 (5.7%) | * | - | - | - | - |
| **2021** | | | | | | | | |
| No | 1352 (90.4%) | 151 (59.4%) | 622 (94.0%) | 549 (99.8%) | 14 (100.0%) | 6 (100.0%) | * | 9 (100.0%) |

| All \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 2188)** | **SMA Type 1 (N = 432)** | **SMA Type 2 (N = 914)** | **SMA Type 3 (N = 779)** | **SMA Type 4 (N = 22)** | **SMA Type Other (N = 7)** | **SMA Type Presymptomatic (N = 8)** | **SMA Type Missing (N = 26)** |
| Yes | 144 (9.6%) | 103 (40.6%) | 40 (6.0%) | * | - | - | - | - |
| **2022** | | | | | | | | |
| No | 1176 (91.0%) | 150 (62.2%) | 540 (95.9%) | 460 (99.8%) | 9 (100.0%) | * | 5 (100.0%) | 9 (100.0%) |
| Yes | 116 (9.0%) | 91 (37.8%) | 23 (4.1%) | * | - | * | - | - |
| **2023** | | | | | | | | |
| No | 730 (93.1%) | 92 (67.6%) | 329 (97.3%) | 285 (99.7%) | 6 (100.0%) | * | 7 (100.0%) | 8 (100.0%) |
| Yes | 54 (6.9%) | 44 (32.4%) | 9 (2.7%) | * | - | - | - | - |
| **At least one episode of wheelchair usage; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 40 (44.0%) | 13 (86.7%) | 16 (33.3%) | 10 (43.5%) | - | - | - | * |
| Yes | 51 (56.0%) | * | 32 (66.7%) | 13 (56.5%) | - | - | - | * |
| **2011-2013** | | | | | | | | |
| No | 182 (46.8%) | 50 (78.1%) | 53 (30.3%) | 75 (51.7%) | - | * | - | * |
| Yes | 207 (53.2%) | 14 (21.9%) | 122 (69.7%) | 70 (48.3%) | - | - | - | * |
| **2014-2016** | | | | | | | | |
| No | 314 (36.0%) | 91 (61.9%) | 81 (20.5%) | 136 (42.2%) | * | * | - | * |
| Yes | 558 (64.0%) | 56 (38.1%) | 314 (79.5%) | 186 (57.8%) | - | * | - | * |
| **2017** | | | | | | | | |
| No | 369 (36.9%) | 79 (52.3%) | 117 (25.0%) | 167 (44.8%) | * | * | - | * |
| Yes | 630 (63.1%) | 72 (47.7%) | 351 (75.0%) | 206 (55.2%) | - | - | - | * |
| **2018** | | | | | | | | |
| No | 509 (36.2%) | 120 (54.1%) | 162 (24.9%) | 217 (41.5%) | * | * | - | * |
| Yes | 899 (63.8%) | 102 (45.9%) | 488 (75.1%) | 306 (58.5%) | * | - | - | * |
| **2019** | | | | | | | | |
| No | 622 (39.4%) | 135 (51.3%) | 198 (27.9%) | 270 (46.2%) | 9 (81.8%) | * | - | 7 (87.5%) |
| Yes | 957 (60.6%) | 128 (48.7%) | 511 (72.1%) | 315 (53.8%) | * | - | - | * |
| **2020** | | | | | | | | |
| No | 700 (41.3%) | 144 (50.7%) | 244 (31.7%) | 293 (47.3%) | 10 (83.3%) | * | - | 6 (75.0%) |

| All \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 2188)** | **SMA Type 1 (N = 432)** | **SMA Type 2 (N = 914)** | **SMA Type 3 (N = 779)** | **SMA Type 4 (N = 22)** | **SMA Type Other (N = 7)** | **SMA Type Presymptomatic (N = 8)** | **SMA Type Missing (N = 26)** |
| Yes | 995 (58.7%) | 140 (49.3%) | 525 (68.3%) | 326 (52.7%) | * | - | - | * |
| **2021** | | | | | | | | |
| No | 506 (33.8%) | 96 (37.8%) | 151 (22.8%) | 234 (42.5%) | 12 (85.7%) | 5 (83.3%) | * | 7 (77.8%) |
| Yes | 990 (66.2%) | 158 (62.2%) | 511 (77.2%) | 316 (57.5%) | * | * | - | * |
| **2022** | | | | | | | | |
| No | 480 (37.2%) | 88 (36.5%) | 153 (27.2%) | 216 (46.9%) | 7 (77.8%) | * | 5 (100.0%) | 8 (88.9%) |
| Yes | 812 (62.8%) | 153 (63.5%) | 410 (72.8%) | 245 (53.1%) | * | * | - | * |
| **2023** | | | | | | | | |
| No | 330 (42.1%) | 56 (41.2%) | 103 (30.5%) | 151 (52.8%) | * | * | 7 (100.0%) | 7 (87.5%) |
| Yes | 454 (57.9%) | 80 (58.8%) | 235 (69.5%) | 135 (47.2%) | * | * | - | * |
| **At least one reported measure by available motor function scale or test and by at least one PRO; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 91 (100.0%) | 15 (100.0%) | 48 (100.0%) | 23 (100.0%) | - | - | - | 5 (100.0%) |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 389 (100.0%) | 64 (100.0%) | 175 (100.0%) | 145 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 872 (100.0%) | 147 (100.0%) | 395 (100.0%) | 322 (100.0%) | * | * | - | 5 (100.0%) |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 999 (100.0%) | 151 (100.0%) | 468 (100.0%) | 373 (100.0%) | * | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 1361 (96.7%) | 222 (100.0%) | 636 (97.8%) | 492 (94.1%) | * | * | - | 5 (100.0%) |
| Yes | 47 (3.3%) | - | 14 (2.2%) | 31 (5.9%) | * | - | - | - |
| **2019** | | | | | | | | |

| All \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 2188)** | **SMA Type 1 (N = 432)** | **SMA Type 2 (N = 914)** | **SMA Type 3 (N = 779)** | **SMA Type 4 (N = 22)** | **SMA Type Other (N = 7)** | **SMA Type Presymptomatic (N = 8)** | **SMA Type Missing (N = 26)** |
| No | 1507 (95.4%) | 263 (100.0%) | 681 (96.1%) | 544 (93.0%) | 8 (72.7%) | * | - | 8 (100.0%) |
| Yes | 72 (4.6%) | - | 28 (3.9%) | 41 (7.0%) | * | - | - | - |
| **2020** | | | | | | | | |
| No | 1588 (93.7%) | 281 (98.9%) | 724 (94.1%) | 564 (91.1%) | 8 (66.7%) | * | - | 8 (100.0%) |
| Yes | 107 (6.3%) | * | 45 (5.9%) | 55 (8.9%) | * | - | - | - |
| **2021** | | | | | | | | |
| No | 1368 (91.4%) | 251 (98.8%) | 604 (91.2%) | 492 (89.5%) | 8 (57.1%) | 5 (83.3%) | * | 7 (77.8%) |
| Yes | 128 (8.6%) | * | 58 (8.8%) | 58 (10.5%) | 6 (42.9%) | * | - | * |
| **2022** | | | | | | | | |
| No | 1287 (99.6%) | 241 (100.0%) | 562 (99.8%) | 457 (99.1%) | 9 (100.0%) | * | 5 (100.0%) | 9 (100.0%) |
| Yes | 5 (0.4%) | - | * | * | - | - | - | - |
| **2023** | | | | | | | | |
| No | 779 (99.4%) | 135 (99.3%) | 337 (99.7%) | 283 (99.0%) | 6 (100.0%) | * | 7 (100.0%) | 8 (100.0%) |
| Yes | 5 (0.6%) | * | * | * | - | - | - | - |
| **At least three reported measures by available motor function scale or test and by at least one PRO; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 91 (100.0%) | 15 (100.0%) | 48 (100.0%) | 23 (100.0%) | - | - | - | 5 (100.0%) |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 389 (100.0%) | 64 (100.0%) | 175 (100.0%) | 145 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 872 (100.0%) | 147 (100.0%) | 395 (100.0%) | 322 (100.0%) | * | * | - | 5 (100.0%) |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 999 (100.0%) | 151 (100.0%) | 468 (100.0%) | 373 (100.0%) | * | * | - | * |
| Yes | - | - | - | - | - | - | - | - |

| All \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 2188)** | **SMA Type 1 (N = 432)** | **SMA Type 2 (N = 914)** | **SMA Type 3 (N = 779)** | **SMA Type 4 (N = 22)** | **SMA Type Other (N = 7)** | **SMA Type Presymptomatic (N = 8)** | **SMA Type Missing (N = 26)** |
| **2018** | | | | | | | | |
| No | 1383 (98.2%) | 222 (100.0%) | 644 (99.1%) | 506 (96.7%) | * | * | - | 5 (100.0%) |
| Yes | 25 (1.8%) | - | 6 (0.9%) | 17 (3.3%) | * | - | - | - |
| **2019** | | | | | | | | |
| No | 1532 (97.0%) | 263 (100.0%) | 686 (96.8%) | 563 (96.2%) | 9 (81.8%) | * | - | 8 (100.0%) |
| Yes | 47 (3.0%) | - | 23 (3.2%) | 22 (3.8%) | * | - | - | - |
| **2020** | | | | | | | | |
| No | 1628 (96.0%) | 281 (98.9%) | 741 (96.4%) | 584 (94.3%) | 11 (91.7%) | * | - | 8 (100.0%) |
| Yes | 67 (4.0%) | * | 28 (3.6%) | 35 (5.7%) | * | - | - | - |
| **2021** | | | | | | | | |
| No | 1422 (95.1%) | 252 (99.2%) | 622 (94.0%) | 519 (94.4%) | 13 (92.9%) | 6 (100.0%) | * | 9 (100.0%) |
| Yes | 74 (4.9%) | * | 40 (6.0%) | 31 (5.6%) | * | - | - | - |
| **2022** | | | | | | | | |
| No | 1288 (99.7%) | 241 (100.0%) | 563 (100.0%) | 457 (99.1%) | 9 (100.0%) | * | 5 (100.0%) | 9 (100.0%) |
| Yes | * | - | - | * | - | - | - | - |
| **2023** | | | | | | | | |
| No | 781 (99.6%) | 136 (100.0%) | 338 (100.0%) | 283 (99.0%) | 6 (100.0%) | * | 7 (100.0%) | 8 (100.0%) |
| Yes | * | - | - | * | - | - | - | - |
| **Available number of records of each motor function scale by patient** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | 1.8 (1.1) | - | 2.1 (1.2) | * | - | - | - | - |
| Median [IQR] | - | - | - | * | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | 1.7 (0.7) | * | 1.9 (0.8) | 1.2 (0.4) | - | - | - | * |
| Median [IQR] | - | * | - | - | - | - | - | * |
| **2014-2016** | | | | | | | | |
| Mean (SD) | 1.6 (0.7) | 1.2 (0.4) | 1.7 (0.8) | 1.5 (0.7) | - | - | - | * |
| Median [IQR] | - | - | - | - | - | - | - | * |

| All \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 2188)** | **SMA Type 1 (N = 432)** | **SMA Type 2 (N = 914)** | **SMA Type 3 (N = 779)** | **SMA Type 4 (N = 22)** | **SMA Type Other (N = 7)** | **SMA Type Presymptomatic (N = 8)** | **SMA Type Missing (N = 26)** |
| **2017** | | | | | | | | |
| Mean (SD) | 1.1 (0.3) | 1.3 (0.5) | 1.1 (0.2) | 1 (0.2) | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | 1.5 (1) | 1.9 (1.2) | 1.5 (1) | 1.3 (0.9) | 1 (-) | * | - | 2 (0.8) |
| Median [IQR] | - | - | - | - | - | * | - | - |
| **2019** | | | | | | | | |
| Mean (SD) | - | 1.8 (0.8) | 1.5 (0.7) | 1.3 (0.6) | 1.4 (0.5) | * | - | 1.2 (0.7) |
| Median [IQR] | - | - | - | - | - | * | - | - |
| **2020** | | | | | | | | |
| Mean (SD) | 1.5 (0.7) | 1.6 (0.9) | 1.5 (0.7) | 1.5 (0.7) | 1 (-) | - | - | 1.5 (0.7) |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | 1.6 (0.7) | 1.5 (0.8) | 1.6 (0.7) | 1.6 (0.7) | 1.3 (0.4) | * | * | 1.5 (0.7) |
| Median [IQR] | - | - | - | - | - | * | * | - |
| **2022** | | | | | | | | |
| Mean (SD) | 1.5 (0.7) | 1.4 (0.6) | 1.5 (0.7) | 1.5 (0.8) | * | * | 1.5 (0.8) | 1 (-) |
| Median [IQR] | - | - | - | - | * | * | - | - |
| **2023** | | | | | | | | |
| Mean (SD) | 1 (0.1) | 1 (0.2) | 1 (-) | 1 (0.2) | 1 (-) | - | * | 1 (-) |
| Median [IQR] | - | - | - | - | - | - | * | - |
| **Available number of records of PRO** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |

| All \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 2188)** | **SMA Type 1 (N = 432)** | **SMA Type 2 (N = 914)** | **SMA Type 3 (N = 779)** | **SMA Type 4 (N = 22)** | **SMA Type Other (N = 7)** | **SMA Type Presymptomatic (N = 8)** | **SMA Type Missing (N = 26)** |
| Mean (SD) | * | - | - | * | - | - | - | - |
| Median [IQR] | * | - | - | * | - | - | - | - |
| **2017** | | | | | | | | |
| Mean (SD) | 1 (-) | - | 1 (-) | 1 (-) | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | 1 (-) | * | 1 (-) | 1 (-) | * | - | - | - |
| Median [IQR] | - | * | - | - | * | - | - | - |
| **2019** | | | | | | | | |
| Mean (SD) | 1 (-) | * | 1 (-) | 1 (-) | * | - | - | - |
| Median [IQR] | - | * | - | - | * | - | - | - |
| **2020** | | | | | | | | |
| Mean (SD) | 1.2 (0.4) | 1.2 (0.4) | 1.2 (0.4) | 1.2 (0.4) | 1 (-) | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | 1 (0.2) | 1.1 (0.3) | 1.1 (0.2) | 1 (0.2) | 1 (-) | * | - | * |
| Median [IQR] | - | - | - | - | - | * | - | * |
| **2022** | | | | | | | | |
| Mean (SD) | 1.2 (0.5) | 1.2 (0.4) | 1.1 (0.3) | 1.2 (0.6) | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| Mean (SD) | 1 (0.2) | 1 (-) | 1.1 (0.3) | 1 (-) | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |

| Belgium \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 256)** | **SMA Type 1 (N = 34)** | **SMA Type 2 (N = 101)** | **SMA Type 3 (N = 103)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 7)** |
| **Calendar year of registry entry; n (%)** | | | | | | | | |
| 2008 | - | - | - | - | - | - | - | - |
| 2009 | - | - | - | - | - | - | - | - |
| 2010 | - | - | - | - | - | - | - | - |
| 2011 | - | - | - | - | - | - | - | - |
| 2012 | - | - | - | - | - | - | - | - |
| 2013 | - | - | - | - | - | - | - | - |
| 2014 | - | - | - | - | - | - | - | - |
| 2015 | - | - | - | - | - | - | - | - |
| 2016 | - | - | - | - | - | - | - | - |
| 2017 | - | - | - | - | - | - | - | - |
| 2018 | 190 (74.2%) | 20 (58.8%) | 86 (85.1%) | 76 (73.8%) | 5 (62.5%) | - | - | * |
| 2019 | 38 (14.8%) | 6 (17.6%) | 10 (9.9%) | 18 (17.5%) | * | * | - | * |
| 2020 | 15 (5.9%) | * | * | 5 (4.9%) | * | - | - | - |
| 2021 | 11 (4.3%) | * | * | * | - | * | - | * |
| 2022 | - | - | - | - | - | - | - | - |
| 2023 | - | - | - | - | - | - | - | - |
| Missing | * | * | - | - | - | - | - | - |
| **Calendar year of death; n (%)** | | | | | | | | |
| 2009 | - | - | - | - | - | - | - | - |
| 2010 | - | - | - | - | - | - | - | - |
| 2012 | - | - | - | - | - | - | - | - |
| 2013 | - | - | - | - | - | - | - | - |
| 2014 | - | - | - | - | - | - | - | - |
| 2015 | - | - | - | - | - | - | - | - |
| 2016 | - | - | - | - | - | - | - | - |
| 2017 | * | * | - | - | - | - | - | - |
| 2018 | * | - | * | * | - | - | - | - |

| Belgium \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 256)** | **SMA Type 1 (N = 34)** | **SMA Type 2 (N = 101)** | **SMA Type 3 (N = 103)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 7)** |
| 2019 | * | - | - | * | - | - | - | - |
| 2020 | * | * | - | - | - | - | - | - |
| 2021 | * | - | * | * | - | - | - | - |
| 2022 | - | - | - | - | - | - | - | - |
| 2023 | - | - | - | - | - | - | - | - |
| Missing | 247 (96.5%) | 30 (88.2%) | 99 (98.0%) | 100 (97.1%) | 8 (100.0%) | * | - | 7 (100.0%) |
| **Sex; n (%)** | | | | | | | | |
| Female | 129 (50.4%) | 18 (52.9%) | 50 (49.5%) | 50 (48.5%) | 5 (62.5%) | * | - | * |
| Male | 127 (49.6%) | 16 (47.1%) | 51 (50.5%) | 53 (51.5%) | * | * | - | * |
| Missing | - | - | - | - | - | - | - | - |
| **Class of age at symptom onset; n (%)** | | | | | | | | |
| Presymptomatic | - | - | - | - | - | - | - | - |
| Prenatal | * | * | * | - | - | - | - | - |
| < 1 month | * | * | * | - | - | - | - | - |
| [1 - 3 months) | 13 (5.1%) | 11 (32.4%) | - | * | - | - | - | * |
| [3 - 6 months) | 20 (7.8%) | 14 (41.2%) | 6 (5.9%) | - | - | - | - | - |
| [6 - 18 months) | 88 (34.4%) | * | 65 (64.4%) | 18 (17.5%) | - | - | - | * |
| [1.5 - 2 years) | 18 (7.0%) | - | 10 (9.9%) | 8 (7.8%) | - | - | - | - |
| [2 - 6 years) | 35 (13.7%) | - | * | 33 (32.0%) | - | - | - | - |
| [6 - 11 years) | 10 (3.9%) | - | * | 8 (7.8%) | * | - | - | - |
| [11 - 18 years) | 15 (5.9%) | - | - | 12 (11.7%) | * | - | - | - |
| 18 years + | * | - | - | * | * | - | - | - |
| Missing | 48 (18.8%) | * | 14 (13.9%) | 22 (21.4%) | * | * | - | 5 (71.4%) |
| **Best functional SMA status; n (%)** | | | | | | | | |
| Non-sitter | 11 (4.3%) | 8 (23.5%) | * | - | - | - | - | - |
| Sitter | 107 (41.8%) | 19 (55.9%) | 83 (82.2%) | * | - | * | - | * |
| Walker | 131 (51.2%) | * | 14 (13.9%) | 101 (98.1%) | 8 (100.0%) | * | - | 6 (85.7%) |
| Missing | 7 (2.7%) | 6 (17.6%) | * | - | - | - | - | - |

| Belgium \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 256)** | **SMA Type 1 (N = 34)** | **SMA Type 2 (N = 101)** | **SMA Type 3 (N = 103)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 7)** |
| **Best achieved motor milestone; n (%)** | | | | | | | | |
| Climb stairs | 103 (40.2%) | - | * | 88 (85.4%) | 8 (100.0%) | * | - | * |
| Walk 10 metres without assistance | 11 (4.3%) | - | - | 10 (9.7%) | - | - | - | * |
| Walk without assistance | 5 (2.0%) | - | * | * | - | - | - | * |
| Walk with assistance | 12 (4.7%) | * | 9 (8.9%) | * | - | - | - | - |
| Stand without assistance | * | - | * | * | - | - | - | - |
| Stand with assistance | 14 (5.5%) | * | 12 (11.9%) | - | - | - | - | - |
| Crawl | 10 (3.9%) | * | 9 (8.9%) | - | - | - | - | - |
| Sit without support | 79 (30.9%) | 16 (47.1%) | 59 (58.4%) | * | - | * | - | * |
| Roll onto side | * | * | * | - | - | - | - | - |
| Hold head without support | 7 (2.7%) | 6 (17.6%) | * | - | - | - | - | - |
| Unknown | - | - | - | - | - | - | - | - |
| Missing | 7 (2.7%) | 6 (17.6%) | * | - | - | - | - | - |
| **SMN1 gene mutation type; n (%)** | | | | | | | | |
| Compound heterozygous deletion exon 7 | * | * | * | * | - | - | - | * |
| Compound heterozygous substitutions | * | - | * | - | - | - | - | - |
| Homozygous deletion exon 7 | 251 (98.0%) | 33 (97.1%) | 99 (98.0%) | 102 (99.0%) | 8 (100.0%) | * | - | 6 (85.7%) |
| Missing | - | - | - | - | - | - | - | - |
| **Number of SMN2 copies; n (%)** | | | | | | | | |
| 0 | - | - | - | - | - | - | - | - |
| 1 | * | - | * | - | - | - | - | - |
| 2 | 37 (14.5%) | 24 (70.6%) | 8 (7.9%) | * | * | - | - | * |

| Belgium \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 256)** | **SMA Type 1 (N = 34)** | **SMA Type 2 (N = 101)** | **SMA Type 3 (N = 103)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 7)** |
| 3 | 126 (49.2%) | 5 (14.7%) | 69 (68.3%) | 46 (44.7%) | * | * | - | * |
| 4 | - | - | - | - | - | - | - | - |
| >4 | - | - | - | - | - | - | - | - |
| Other | 50 (19.5%) | * | * | 38 (36.9%) | * | * | - | * |
| Missing | 42 (16.4%) | * | 20 (19.8%) | 17 (16.5%) | * | - | - | - |
| **Methods used for genetic testing; n (%)** | | | | | | | | |
| DNA Sequencing | * | - | * | - | - | - | - | - |
| HRM | * | - | - | * | - | - | - | - |
| MLPA | 201 (78.5%) | 30 (88.2%) | 78 (77.2%) | 78 (75.7%) | 6 (75.0%) | * | - | 6 (85.7%) |
| RFLP | * | - | * | - | - | - | - | - |
| ddPCR | * | - | * | * | - | - | - | - |
| qRT-PCR | 15 (5.9%) | * | 5 (5.0%) | 8 (7.8%) | * | - | - | - |
| Missing | 30 (11.7%) | * | 12 (11.9%) | 13 (12.6%) | * | - | - | * |
| **Duration of follow up (months)** | | | | | | | | |
| Mean (SD) | 55.5 (13.7) | 52.2 (14.8) | 59.1 (11) | 54.2 (14.6) | 52.6 (10.6) | * | - | 48.6 (16) |
| Median [IQR] | 60 [48.2, 66] | 54 [42, 65] | 63 [57, 67] | 59 [48, 64.5] | 56.5 [48.2, 59.2] | * | - | 48 [36.5, 63] |
| Missing; n (%) | * | * | - | - | - | - | - | - |
| **Duration of SMA (months)** | | | | | | | | |
| Mean (SD) | 235.8 (163.3) | 83.3 (52.9) | 221.7 (137.6) | 301.4 (178.5) | 310.7 (71.2) | - | - | * |
| Median [IQR] | 199.5 [101.8, 342] | 75 [48, 86.2] | 188.5 [112.2, 285] | 273 [152, 430] | 313 [255.5, 364.5] | - | - | * |
| Missing; n (%) | 52 (20.3%) | 6 (17.6%) | 15 (14.9%) | 22 (21.4%) | * | * | - | 5 (71.4%) |
| **Duration between two consecutive visits (months)** | | | | | | | | |
| Mean (SD) | 27 (323.9) | 3.8 (2.8) | 6.4 (10.8) | 59 (510.3) | 7.6 (3.4) | * | - | 3.3 (0.5) |
| Median [IQR] | 4.5 [3.4, 6.4] | 3.1 [2.4, 4.3] | 4.4 [3.5, 6.5] | 5.2 [3.7, 7.8] | 6.5 [5.6, 7.9] | * | - | 3.1 [2.8, 3.8] |
| Missing; n (%) | 6 (2.3%) | - | * | * | - | - | - | * |

| Belgium \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 256)** | **SMA Type 1 (N = 34)** | **SMA Type 2 (N = 101)** | **SMA Type 3 (N = 103)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 7)** |
| **Duration between genetic report date and registry entry (months)** | | | | | | | | |
| Mean (SD) | 99.2 (96.7) | 23.5 (43.7) | 109.5 (88.3) | 124.7 (103.8) | 66.6 (81.1) | * | - | 1.4 (2) |
| Median [IQR] | 68 [10, 178.5] | 8.5 [1, 22.8] | 92 [36, 169] | 124 [17.5, 220.5] | 24 [7.5, 100.2] | * | - | - |
| Missing; n (%) | * | * | - | - | - | - | - | - |
| **Reason for genetic testing; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Family screening | * | - | - | * | * | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 106 (93.8%) | * | 48 (94.1%) | 54 (94.7%) | * | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 24 (100.0%) | - | 15 (100.0%) | 8 (100.0%) | * | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| Family screening | * | * | * | * | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 29 (85.3%) | 5 (62.5%) | 14 (93.3%) | 10 (90.9%) | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| Family screening | * | - | - | * | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 25 (96.2%) | 9 (100.0%) | 10 (100.0%) | 5 (83.3%) | * | - | - | - |

| Belgium \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 256)** | **SMA Type 1 (N = 34)** | **SMA Type 2 (N = 101)** | **SMA Type 3 (N = 103)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 7)** |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | * | - | - | - | - | - | - | * |
| No screening | 16 (88.9%) | * | 5 (100.0%) | 5 (100.0%) | * | - | - | * |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| Family screening | * | * | * | * | - | - | - | * |
| Newborn screening | 5 (22.7%) | * | - | - | - | * | - | * |
| No screening | 12 (54.5%) | * | * | 8 (80.0%) | - | - | - | - |
| Prenatal screening | * | - | - | * | - | - | - | - |
| **2020** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | * | * | - | - | - | - | - | - |
| No screening | 7 (87.5%) | * | * | * | * | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| Family screening | * | - | - | - | - | - | - | * |
| Newborn screening | * | - | - | - | - | * | - | - |
| No screening | 7 (63.6%) | * | - | * | * | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2022** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | - | - | - | - | - | - | - | - |

| Belgium \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 256)** | **SMA Type 1 (N = 34)** | **SMA Type 2 (N = 101)** | **SMA Type 3 (N = 103)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 7)** |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | - | - | - | - | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **Age at onset of SMA symptoms (years)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | 4.3 (7.3) | * | 1 (1.1) | 4.9 (4.9) | 25.5 (14.6) | - | - | - |
| Median [IQR] | 1.4 [0.8, 3.4] | * | 0.8 [0.5, 1.2] | 2.5 [1.5, 8] | 17 [14.4, 38.3] | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | 1.9 (1.5) | - | 1 (0.3) | 2.9 (1.6) | - | - | - | - |
| Median [IQR] | 1.4 [1, 2.1] | - | 1 [0.7, 1.1] | 2.2 [1.9, 3.7] | - | - | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | 1.2 (2.4) | 0.2 (0.1) | 0.9 (0.4) | 4.3 (4.6) | - | - | - | - |
| Median [IQR] | 0.5 [0.3, 1] | 0.2 [0.2, 0.3] | 0.8 [0.5, 1] | 2.4 [2, 2.7] | - | - | - | - |
| **2017** | | | | | | | | |
| Mean (SD) | 1.6 (3.3) | 0.2 (0.1) | * | * | - | - | - | * |
| Median [IQR] | 0.3 [0.1, 1.2] | 0.2 [0.1, 0.3] | * | * | - | - | - | * |
| **2018** | | | | | | | | |
| Mean (SD) | 1.1 (0.8) | * | * | * | - | - | - | - |
| Median [IQR] | 1 [0.7, 1.2] | * | * | * | - | - | - | - |
| **2019** | | | | | | | | |
| Mean (SD) | 1.7 (2.8) | * | * | * | - | - | - | * |
| Median [IQR] | 0.8 [0.3, 1.1] | * | * | * | - | - | - | * |
| **2020** | | | | | | | | |
| Mean (SD) | 1 (1.1) | * | * | * | - | - | - | - |

| Belgium \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 256)** | **SMA Type 1 (N = 34)** | **SMA Type 2 (N = 101)** | **SMA Type 3 (N = 103)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 7)** |
| Median [IQR] | 0.4 [0.2, 1.2] | * | * | * | - | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | * | * | - | - | - | - | - | - |
| Median [IQR] | * | * | - | - | - | - | - | - |
| **2022** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **Age at genetic report date (years)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | 11.4 (13.4) | * | 5.1 (7.5) | 16.3 (14) | * | - | - | - |
| Median [IQR] | 3.7 [1.6, 17.7] | * | 1.7 [1.2, 4.6] | 13 [3.3, 26.5] | * | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | 9 (11.2) | - | 8.5 (11.4) | 8.7 (10.9) | * | - | - | - |
| Median [IQR] | 3.1 [1.4, 14.1] | - | 1.6 [1, 14.3] | 4.2 [3.1, 7.1] | * | - | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | 9 (12.6) | 0.3 (0.3) | 7.9 (12.3) | 16.9 (13) | - | - | - | - |
| Median [IQR] | 1.8 [0.9, 16.6] | 0.3 [0.2, 0.3] | 1.7 [1.3, 7.6] | 17.7 [2.8, 25.2] | - | - | - | - |
| **2017** | | | | | | | | |
| Mean (SD) | 9.2 (13) | 0.8 (0.6) | 11.6 (16.5) | 13.6 (6.2) | * | - | - | - |
| Median [IQR] | 1.6 [0.9, 11.4] | 0.6 [0.3, 0.8] | 1.6 [1.4, 19.1] | 10.9 [9.4, 17.5] | * | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | 23.5 (23.1) | * | 17.2 (19.1) | 47.2 (3) | * | - | - | * |
| Median [IQR] | 19.3 [0.9, 45.2] | * | 1.8 [1.7, 36.8] | 47.6 [45.6, 49.5] | * | - | - | * |
| **2019** | | | | | | | | |
| Mean (SD) | 14.4 (21.4) | 0.3 (0.2) | * | 24.8 (22) | - | * | - | * |
| Median [IQR] | 2.8 [0.4, 19.3] | 0.2 [0.1, 0.5] | * | 18.3 [4.3, 45.4] | - | * | - | * |

| Belgium \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 256)** | **SMA Type 1 (N = 34)** | **SMA Type 2 (N = 101)** | **SMA Type 3 (N = 103)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 7)** |
| **2020** | | | | | | | | |
| Mean (SD) | 11.7 (16.3) | * | * | * | * | - | - | - |
| Median [IQR] | 1.1 [0.4, 18.2] | * | * | * | * | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | 10.1 (18.5) | * | - | * | * | * | - | * |
| Median [IQR] | 1.9 [0.5, 6] | * | - | * | * | * | - | * |
| **2022** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **Age at registry entry (years)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | 20.2 (16.2) | 3.3 (4.2) | 16.5 (12.4) | 28.1 (15.8) | 44.7 (14.7) | - | - | * |
| Median [IQR] | 15.8 [6.5, 30.8] | 1.8 [1.2, 3.4] | 12.8 [6.4, 25.3] | 25.3 [14.7, 41.5] | 37.2 [36.1, 61.2] | - | - | * |
| **2019** | | | | | | | | |

| Belgium \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 256)** | **SMA Type 1 (N = 34)** | **SMA Type 2 (N = 101)** | **SMA Type 3 (N = 103)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 7)** |
| Mean (SD) | 21.8 (21.4) | 0.2 (0.2) | 18.7 (17.7) | 31.9 (19.3) | * | * | - | * |
| Median [IQR] | 15.6 [2.2, 37] | 0.1 [0.1, 0.3] | 14.8 [5.6, 28.1] | 30.9 [16.5, 49.2] | * | * | - | * |
| **2020** | | | | | | | | |
| Mean (SD) | 22.9 (22.7) | * | * | 30.5 (19.6) | * | - | - | - |
| Median [IQR] | 12.6 [1.1, 43] | * | * | 35.1 [12.6, 43.2] | * | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | 5.8 (7.8) | * | * | * | - | * | - | * |
| Median [IQR] | 2 [0.5, 6.3] | * | * | * | - | * | - | * |
| **2022** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **Age at death (years)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| Mean (SD) | * | * | - | - | - | - | - | - |
| Median [IQR] | * | * | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | * | - | * | * | - | - | - | - |

| Belgium \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 256)** | **SMA Type 1 (N = 34)** | **SMA Type 2 (N = 101)** | **SMA Type 3 (N = 103)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 7)** |
| Median [IQR] | * | - | * | * | - | - | - | - |
| **2019** | | | | | | | | |
| Mean (SD) | * | - | - | * | - | - | - | - |
| Median [IQR] | * | - | - | * | - | - | - | - |
| **2020** | | | | | | | | |
| Mean (SD) | * | * | - | - | - | - | - | - |
| Median [IQR] | * | * | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | * | - | * | * | - | - | - | - |
| Median [IQR] | * | - | * | * | - | - | - | - |
| **2022** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **Lost to follow-up; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |

| Belgium \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 256)** | **SMA Type 1 (N = 34)** | **SMA Type 2 (N = 101)** | **SMA Type 3 (N = 103)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 7)** |
| **2018** | | | | | | | | |
| No | 190 (100.0%) | 20 (100.0%) | 86 (100.0%) | 76 (100.0%) | 5 (100.0%) | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 226 (100.0%) | 26 (100.0%) | 95 (100.0%) | 93 (100.0%) | 6 (100.0%) | * | - | 5 (100.0%) |
| Yes | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 239 (99.6%) | 30 (100.0%) | 98 (99.0%) | 97 (100.0%) | 8 (100.0%) | * | - | 5 (100.0%) |
| Yes | * | - | * | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 237 (95.6%) | 27 (90.0%) | 97 (98.0%) | 95 (94.1%) | 8 (100.0%) | * | - | 7 (100.0%) |
| Yes | 11 (4.4%) | * | * | 6 (5.9%) | - | - | - | - |
| **2022** | | | | | | | | |
| No | 223 (94.9%) | 26 (96.3%) | 92 (95.8%) | 87 (92.6%) | 8 (100.0%) | * | - | 7 (100.0%) |
| Yes | 12 (5.1%) | * | * | 7 (7.4%) | - | - | - | - |
| **2023** | | | | | | | | |
| No | 120 (53.8%) | 20 (76.9%) | 51 (55.4%) | 41 (47.1%) | * | * | - | * |
| Yes | 103 (46.2%) | 6 (23.1%) | 41 (44.6%) | 46 (52.9%) | 5 (62.5%) | * | - | * |
| **Treated with at least one DMT; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |

| Belgium \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 256)** | **SMA Type 1 (N = 34)** | **SMA Type 2 (N = 101)** | **SMA Type 3 (N = 103)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 7)** |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 96 (50.5%) | 6 (30.0%) | 53 (61.6%) | 34 (44.7%) | * | - | - | * |
| Yes | 94 (49.5%) | 14 (70.0%) | 33 (38.4%) | 42 (55.3%) | * | - | - | * |
| **2019** | | | | | | | | |
| No | 67 (29.9%) | 7 (26.9%) | 27 (28.7%) | 29 (31.5%) | * | - | - | * |
| Yes | 157 (70.1%) | 19 (73.1%) | 67 (71.3%) | 63 (68.5%) | * | * | - | * |
| **2020** | | | | | | | | |
| No | 55 (24.1%) | * | 22 (22.9%) | 23 (25.3%) | * | - | - | * |
| Yes | 173 (75.9%) | 23 (85.2%) | 74 (77.1%) | 68 (74.7%) | * | * | - | * |
| **2021** | | | | | | | | |
| No | 33 (14.7%) | * | 12 (12.9%) | 15 (17.0%) | * | - | - | * |
| Yes | 192 (85.3%) | 25 (96.2%) | 81 (87.1%) | 73 (83.0%) | 5 (62.5%) | * | - | 5 (71.4%) |
| **2022** | | | | | | | | |
| No | * | - | - | * | * | - | - | - |
| Yes | * | * | * | - | - | - | - | - |
| **2023** | | | | | | | | |
| No | * | - | - | * | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **Treated with more than one DMT; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |

| Belgium \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 256)** | **SMA Type 1 (N = 34)** | **SMA Type 2 (N = 101)** | **SMA Type 3 (N = 103)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 7)** |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 190 (100.0%) | 20 (100.0%) | 86 (100.0%) | 76 (100.0%) | 5 (100.0%) | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 222 (99.1%) | 25 (96.2%) | 93 (98.9%) | 92 (100.0%) | 6 (100.0%) | * | - | 5 (100.0%) |
| Yes | * | * | * | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 218 (95.6%) | 20 (74.1%) | 93 (96.9%) | 91 (100.0%) | 8 (100.0%) | * | - | 5 (100.0%) |
| Yes | 10 (4.4%) | 7 (25.9%) | * | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 218 (96.9%) | 23 (88.5%) | 89 (95.7%) | 88 (100.0%) | 8 (100.0%) | * | - | 7 (100.0%) |
| Yes | 7 (3.1%) | * | * | - | - | - | - | - |
| **2022** | | | | | | | | |
| No | 5 (100.0%) | * | * | * | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| No | * | - | - | * | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **Treated with nusinersen; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |

| Belgium \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 256)** | **SMA Type 1 (N = 34)** | **SMA Type 2 (N = 101)** | **SMA Type 3 (N = 103)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 7)** |
| **2014-2016** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 110 (57.9%) | 6 (30.0%) | 61 (70.9%) | 39 (51.3%) | * | - | - | * |
| Yes | 80 (42.1%) | 14 (70.0%) | 25 (29.1%) | 37 (48.7%) | * | - | - | * |
| **2019** | | | | | | | | |
| No | 92 (41.1%) | 8 (30.8%) | 42 (44.7%) | 35 (38.0%) | * | * | - | * |
| Yes | 132 (58.9%) | 18 (69.2%) | 52 (55.3%) | 57 (62.0%) | * | - | - | * |
| **2020** | | | | | | | | |
| No | 91 (39.9%) | 8 (29.6%) | 46 (47.9%) | 28 (30.8%) | * | * | - | * |
| Yes | 137 (60.1%) | 19 (70.4%) | 50 (52.1%) | 63 (69.2%) | * | - | - | * |
| **2021** | | | | | | | | |
| No | 97 (43.1%) | 12 (46.2%) | 50 (53.8%) | 23 (26.1%) | * | * | - | 6 (85.7%) |
| Yes | 128 (56.9%) | 14 (53.8%) | 43 (46.2%) | 65 (73.9%) | 5 (62.5%) | - | - | * |
| **2022** | | | | | | | | |
| No | * | - | * | * | * | - | - | - |
| Yes | * | * | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| No | * | - | - | * | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **Treated with onasemnogene abeparvovec-xioi; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |

| Belgium \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 256)** | **SMA Type 1 (N = 34)** | **SMA Type 2 (N = 101)** | **SMA Type 3 (N = 103)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 7)** |
| **2011-2013** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 190 (100.0%) | 20 (100.0%) | 86 (100.0%) | 76 (100.0%) | 5 (100.0%) | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 221 (98.7%) | 24 (92.3%) | 94 (100.0%) | 92 (100.0%) | 6 (100.0%) | - | - | 5 (100.0%) |
| Yes | * | * | - | - | - | * | - | - |
| **2020** | | | | | | | | |
| No | 223 (97.8%) | 24 (88.9%) | 95 (99.0%) | 91 (100.0%) | 8 (100.0%) | - | - | 5 (100.0%) |
| Yes | 5 (2.2%) | * | * | - | - | * | - | - |
| **2021** | | | | | | | | |
| No | 218 (96.9%) | 22 (84.6%) | 91 (97.8%) | 88 (100.0%) | 8 (100.0%) | * | - | 7 (100.0%) |
| Yes | 7 (3.1%) | * | * | - | - | * | - | - |
| **2022** | | | | | | | | |
| No | 5 (100.0%) | * | * | * | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| No | * | - | - | * | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **Treated with risdiplan; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |

| Belgium \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 256)** | **SMA Type 1 (N = 34)** | **SMA Type 2 (N = 101)** | **SMA Type 3 (N = 103)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 7)** |
| No | - | - | - | - | - | - | - | - |
| Yes | | | | | | | | |
| **2011-2013** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 176 (92.6%) | 20 (100.0%) | 78 (90.7%) | 71 (93.4%) | 5 (100.0%) | - | - | * |
| Yes | 14 (7.4%) | - | 8 (9.3%) | 5 (6.6%) | - | - | - | * |
| **2020** | | | | | | | | |
| No | 200 (89.3%) | 26 (100.0%) | 78 (83.0%) | 86 (93.5%) | 6 (100.0%) | * | - | * |
| Yes | 24 (10.7%) | - | 16 (17.0%) | 6 (6.5%) | - | - | - | * |
| **2021** | | | | | | | | |
| No | 187 (82.0%) | 19 (70.4%) | 70 (72.9%) | 86 (94.5%) | 8 (100.0%) | * | - | * |
| Yes | 41 (18.0%) | 8 (29.6%) | 26 (27.1%) | 5 (5.5%) | - | - | - | * |
| **2022** | | | | | | | | |
| No | 161 (71.6%) | 16 (61.5%) | 53 (57.0%) | 80 (90.9%) | 8 (100.0%) | * | - | * |
| Yes | 64 (28.4%) | 10 (38.5%) | 40 (43.0%) | 8 (9.1%) | - | * | - | * |
| **2023** | | | | | | | | |
| No | * | * | - | * | * | - | - | - |
| Yes | * | - | * | - | - | - | - | - |

| Belgium \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 256)** | **SMA Type 1 (N = 34)** | **SMA Type 2 (N = 101)** | **SMA Type 3 (N = 103)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 7)** |
| **At least one episode of invasive ventilation; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 126 (66.3%) | 5 (25.0%) | 43 (50.0%) | 71 (93.4%) | 5 (100.0%) | - | - | * |
| Yes | 64 (33.7%) | 15 (75.0%) | 43 (50.0%) | 5 (6.6%) | - | - | - | * |
| **2019** | | | | | | | | |
| No | 157 (70.1%) | 10 (38.5%) | 52 (55.3%) | 84 (91.3%) | 6 (100.0%) | * | - | * |
| Yes | 67 (29.9%) | 16 (61.5%) | 42 (44.7%) | 8 (8.7%) | - | - | - | * |
| **2020** | | | | | | | | |
| No | 156 (68.4%) | 10 (37.0%) | 49 (51.0%) | 84 (92.3%) | 8 (100.0%) | * | - | * |
| Yes | 72 (31.6%) | 17 (63.0%) | 47 (49.0%) | 7 (7.7%) | - | - | - | * |
| **2021** | | | | | | | | |
| No | 154 (68.4%) | 11 (42.3%) | 46 (49.5%) | 80 (90.9%) | 8 (100.0%) | * | - | 6 (85.7%) |
| Yes | 71 (31.6%) | 15 (57.7%) | 47 (50.5%) | 8 (9.1%) | - | - | - | * |
| **2022** | | | | | | | | |
| No | * | * | * | * | * | - | - | - |
| Yes | * | * | - | - | - | - | - | - |
| **2023** | | | | | | | | |

| Belgium \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 256)** | **SMA Type 1 (N = 34)** | **SMA Type 2 (N = 101)** | **SMA Type 3 (N = 103)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 7)** |
| No | * | - | - | * | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **At least one episode of non-invasive ventilation; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 125 (65.8%) | 5 (25.0%) | 42 (48.8%) | 71 (93.4%) | 5 (100.0%) | - | - | * |
| Yes | 65 (34.2%) | 15 (75.0%) | 44 (51.2%) | 5 (6.6%) | - | - | - | * |
| **2019** | | | | | | | | |
| No | 153 (68.3%) | 9 (34.6%) | 49 (52.1%) | 84 (91.3%) | 6 (100.0%) | * | - | * |
| Yes | 71 (31.7%) | 17 (65.4%) | 45 (47.9%) | 8 (8.7%) | - | - | - | * |
| **2020** | | | | | | | | |
| No | 154 (67.5%) | 10 (37.0%) | 48 (50.0%) | 83 (91.2%) | 8 (100.0%) | * | - | * |
| Yes | 74 (32.5%) | 17 (63.0%) | 48 (50.0%) | 8 (8.8%) | - | - | - | * |
| **2021** | | | | | | | | |
| No | 153 (68.0%) | 10 (38.5%) | 46 (49.5%) | 80 (90.9%) | 8 (100.0%) | * | - | 6 (85.7%) |
| Yes | 72 (32.0%) | 16 (61.5%) | 47 (50.5%) | 8 (9.1%) | - | - | - | * |
| **2022** | | | | | | | | |

| Belgium \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 256)** | **SMA Type 1 (N = 34)** | **SMA Type 2 (N = 101)** | **SMA Type 3 (N = 103)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 7)** |
| No | * | * | * | * | * | - | - | - |
| Yes | * | * | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| No | * | - | - | * | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **At least one episode of feeding tube usage; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 160 (84.2%) | * | 73 (84.9%) | 75 (98.7%) | 5 (100.0%) | - | - | * |
| Yes | 30 (15.8%) | 16 (80.0%) | 13 (15.1%) | * | - | - | - | - |
| **2019** | | | | | | | | |
| No | 194 (86.6%) | 10 (38.5%) | 81 (86.2%) | 91 (98.9%) | 6 (100.0%) | * | - | 5 (100.0%) |
| Yes | 30 (13.4%) | 16 (61.5%) | 13 (13.8%) | * | - | - | - | - |
| **2020** | | | | | | | | |
| No | 194 (85.1%) | 9 (33.3%) | 80 (83.3%) | 91 (100.0%) | 8 (100.0%) | * | - | 5 (100.0%) |
| Yes | 34 (14.9%) | 18 (66.7%) | 16 (16.7%) | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 196 (87.1%) | 11 (42.3%) | 79 (84.9%) | 88 (100.0%) | 8 (100.0%) | * | - | 7 (100.0%) |

| Belgium \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 256)** | **SMA Type 1 (N = 34)** | **SMA Type 2 (N = 101)** | **SMA Type 3 (N = 103)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 7)** |
| Yes | 29 (12.9%) | 15 (57.7%) | 14 (15.1%) | - | - | - | - | - |
| **2022** | | | | | | | | |
| No | * | * | * | * | * | - | - | - |
| Yes | * | * | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| No | * | - | - | * | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **At least one episode of wheelchair usage; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 40 (21.1%) | 12 (60.0%) | * | 19 (25.0%) | * | - | - | * |
| Yes | 150 (78.9%) | 8 (40.0%) | 83 (96.5%) | 57 (75.0%) | * | - | - | - |
| **2019** | | | | | | | | |
| No | 65 (29.0%) | 17 (65.4%) | 7 (7.4%) | 31 (33.7%) | * | * | - | 5 (100.0%) |
| Yes | 159 (71.0%) | 9 (34.6%) | 87 (92.6%) | 61 (66.3%) | * | - | - | - |
| **2020** | | | | | | | | |
| No | 64 (28.1%) | 14 (51.9%) | 6 (6.2%) | 33 (36.3%) | 6 (75.0%) | * | - | * |

| Belgium \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 256)** | **SMA Type 1 (N = 34)** | **SMA Type 2 (N = 101)** | **SMA Type 3 (N = 103)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 7)** |
| Yes | 164 (71.9%) | 13 (48.1%) | 90 (93.8%) | 58 (63.7%) | * | - | - | * |
| **2021** | | | | | | | | |
| No | 63 (28.0%) | 14 (53.8%) | * | 30 (34.1%) | 7 (87.5%) | * | - | 6 (85.7%) |
| Yes | 162 (72.0%) | 12 (46.2%) | 90 (96.8%) | 58 (65.9%) | * | - | - | * |
| **2022** | | | | | | | | |
| No | * | * | - | * | * | - | - | - |
| Yes | * | * | * | - | - | - | - | - |
| **2023** | | | | | | | | |
| No | * | - | - | * | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **At least one reported measure by available motor function scale or test and by at least one PRO; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 143 (75.3%) | 20 (100.0%) | 72 (83.7%) | 45 (59.2%) | * | - | - | * |
| Yes | 47 (24.7%) | - | 14 (16.3%) | 31 (40.8%) | * | - | - | - |
| **2019** | | | | | | | | |

| Belgium \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 256)** | **SMA Type 1 (N = 34)** | **SMA Type 2 (N = 101)** | **SMA Type 3 (N = 103)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 7)** |
| No | 152 (67.9%) | 26 (100.0%) | 66 (70.2%) | 51 (55.4%) | * | * | - | 5 (100.0%) |
| Yes | 72 (32.1%) | - | 28 (29.8%) | 41 (44.6%) | * | - | - | - |
| **2020** | | | | | | | | |
| No | 124 (54.4%) | 24 (88.9%) | 52 (54.2%) | 38 (41.8%) | * | * | - | 5 (100.0%) |
| Yes | 104 (45.6%) | * | 44 (45.8%) | 53 (58.2%) | * | - | - | - |
| **2021** | | | | | | | | |
| No | 106 (47.1%) | 23 (88.5%) | 39 (41.9%) | 33 (37.5%) | * | * | - | 5 (71.4%) |
| Yes | 119 (52.9%) | * | 54 (58.1%) | 55 (62.5%) | 5 (62.5%) | - | - | * |
| **2022** | | | | | | | | |
| No | 5 (100.0%) | * | * | * | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| No | * | - | - | * | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **At least three reported measures by available motor function scale or test and by at least one PRO; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |

| Belgium \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 256)** | **SMA Type 1 (N = 34)** | **SMA Type 2 (N = 101)** | **SMA Type 3 (N = 103)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 7)** |
| **2018** | | | | | | | | |
| No | 165 (86.8%) | 20 (100.0%) | 80 (93.0%) | 59 (77.6%) | * | - | - | * |
| Yes | 25 (13.2%) | - | 6 (7.0%) | 17 (22.4%) | * | - | - | - |
| **2019** | | | | | | | | |
| No | 177 (79.0%) | 26 (100.0%) | 71 (75.5%) | 70 (76.1%) | * | * | - | 5 (100.0%) |
| Yes | 47 (21.0%) | - | 23 (24.5%) | 22 (23.9%) | * | - | - | - |
| **2020** | | | | | | | | |
| No | 162 (71.1%) | 24 (88.9%) | 68 (70.8%) | 57 (62.6%) | 7 (87.5%) | * | - | 5 (100.0%) |
| Yes | 66 (28.9%) | * | 28 (29.2%) | 34 (37.4%) | * | - | - | - |
| **2021** | | | | | | | | |
| No | 156 (69.3%) | 24 (92.3%) | 57 (61.3%) | 58 (65.9%) | 7 (87.5%) | * | - | 7 (100.0%) |
| Yes | 69 (30.7%) | * | 36 (38.7%) | 30 (34.1%) | * | - | - | - |
| **2022** | | | | | | | | |
| No | 5 (100.0%) | * | * | * | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| No | * | - | - | * | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **Available number of records of each motor function scale by patient** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | * | - | - | * | - | - | - | - |
| Median [IQR] | * | - | - | * | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |

| Belgium \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 256)** | **SMA Type 1 (N = 34)** | **SMA Type 2 (N = 101)** | **SMA Type 3 (N = 103)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 7)** |
| **2017** | | | | | | | | |
| Mean (SD) | 1 (-) | 1 (-) | 1 (-) | 1 (-) | - | - | - | - |
| Median [IQR] | 1 [1, 1] | 1 [1, 1] | 1 [1, 1] | 1 [1, 1] | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | 1.3 (0.7) | 1.8 (1) | 1.4 (0.7) | 1.2 (0.5) | 1 (-) | - | - | * |
| Median [IQR] | 1 [1, 1] | 1 [1, 2] | 1 [1, 2] | 1 [1, 1] | 1 [1, 1] | - | - | * |
| **2019** | | | | | | | | |
| Mean (SD) | 1.5 (0.7) | 1.9 (0.7) | 1.6 (0.8) | 1.4 (0.6) | 1.4 (0.5) | * | - | 1.4 (0.8) |
| Median [IQR] | 1 [1, 2] | 2 [1, 2] | 1 [1, 2] | 1 [1, 2] | 1 [1, 2] | * | - | 1 [1, 1] |
| **2020** | | | | | | | | |
| Mean (SD) | 1.7 (0.7) | 1.7 (0.8) | 1.7 (0.7) | 1.7 (0.7) | 1 (-) | - | - | 1.6 (0.7) |
| Median [IQR] | 2 [1, 2] | 1.5 [1, 2] | 2 [1, 2] | 2 [1, 2] | 1 [1, 1] | - | - | 1.5 [1, 2] |
| **2021** | | | | | | | | |
| Mean (SD) | 1.6 (0.7) | 1.7 (0.7) | 1.7 (0.7) | 1.6 (0.6) | 1.4 (0.5) | * | - | 1.7 (0.8) |
| Median [IQR] | 2 [1, 2] | 2 [1, 2] | 2 [1, 2] | 1 [1, 2] | 1 [1, 2] | * | - | 1.5 [1, 2] |
| **2022** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **Available number of records of PRO** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |

| Belgium \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 256)** | **SMA Type 1 (N = 34)** | **SMA Type 2 (N = 101)** | **SMA Type 3 (N = 103)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 7)** |
| Mean (SD) | * | - | - | * | - | - | - | - |
| Median [IQR] | * | - | - | * | - | - | - | - |
| **2017** | | | | | | | | |
| Mean (SD) | 1 (-) | - | 1 (-) | 1 (-) | - | - | - | - |
| Median [IQR] | 1 [1, 1] | - | 1 [1, 1] | 1 [1, 1] | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | 1 (-) | * | 1 (-) | 1 (-) | * | - | - | - |
| Median [IQR] | 1 [1, 1] | * | 1 [1, 1] | 1 [1, 1] | * | - | - | - |
| **2019** | | | | | | | | |
| Mean (SD) | 1 (-) | * | 1 (-) | 1 (-) | * | - | - | - |
| Median [IQR] | 1 [1, 1] | * | 1 [1, 1] | 1 [1, 1] | * | - | - | - |
| **2020** | | | | | | | | |
| Mean (SD) | 1.1 (0.3) | * | 1 (0.2) | 1.1 (0.3) | 1 (-) | - | - | - |
| Median [IQR] | 1 [1, 1] | * | 1 [1, 1] | 1 [1, 1] | 1 [1, 1] | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | 1 (-) | * | 1 (-) | 1 (-) | 1 (-) | - | - | * |
| Median [IQR] | 1 [1, 1] | * | 1 [1, 1] | 1 [1, 1] | 1 [1, 1] | - | - | * |
| **2022** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |

| Czech Republic & Slovakia \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 348)** | **SMA Type 1 (N = 96)** | **SMA Type 2 (N = 117)** | **SMA Type 3 (N = 120)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 1)** | **SMA Type Presymptomatic (N = 6)** | **SMA Type Missing (N = 0)** |
| **Calendar year of registry entry; n (%)** | | | | | | | | |
| 2008 | - | - | - | - | - | - | - | - |
| 2009 | - | - | - | - | - | - | - | - |
| 2010 | - | - | - | - | - | - | - | - |
| 2011 | 12 (3.4%) | 5 (5.2%) | * | * | - | - | - | - |
| 2012 | 22 (6.3%) | 6 (6.2%) | 7 (6.0%) | 9 (7.5%) | - | - | - | - |
| 2013 | 10 (2.9%) | * | 6 (5.1%) | * | - | - | - | - |
| 2014 | 16 (4.6%) | 7 (7.3%) | 6 (5.1%) | * | - | - | - | - |
| 2015 | 16 (4.6%) | 5 (5.2%) | * | 6 (5.0%) | * | - | - | - |
| 2016 | 16 (4.6%) | 5 (5.2%) | 10 (8.5%) | * | - | - | - | - |
| 2017 | 23 (6.6%) | 8 (8.3%) | 10 (8.5%) | 5 (4.2%) | - | - | - | - |
| 2018 | 47 (13.5%) | 21 (21.9%) | 16 (13.7%) | 10 (8.3%) | - | - | - | - |
| 2019 | 31 (8.9%) | 10 (10.4%) | 9 (7.7%) | 10 (8.3%) | * | - | - | - |
| 2020 | 60 (17.2%) | 8 (8.3%) | 23 (19.7%) | 29 (24.2%) | - | - | - | - |
| 2021 | 47 (13.5%) | 8 (8.3%) | 11 (9.4%) | 26 (21.7%) | * | - | * | - |
| 2022 | 34 (9.8%) | 6 (6.2%) | 10 (8.5%) | 13 (10.8%) | * | * | * | - |
| 2023 | * | - | - | * | - | - | * | - |
| Missing | 10 (2.9%) | * | * | * | * | - | - | - |
| **Calendar year of death; n (%)** | | | | | | | | |
| 2009 | * | - | * | - | - | - | - | - |
| 2010 | * | - | - | - | * | - | - | - |
| 2012 | * | * | - | - | - | - | - | - |
| 2013 | * | * | - | - | * | - | - | - |
| 2014 | * | * | - | - | - | - | - | - |
| 2015 | * | * | - | - | - | - | - | - |
| 2016 | * | * | - | - | - | - | - | - |
| 2017 | - | - | - | - | - | - | - | - |
| 2018 | * | - | * | - | - | - | - | - |

| Czech Republic & Slovakia \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 348)** | **SMA Type 1 (N = 96)** | **SMA Type 2 (N = 117)** | **SMA Type 3 (N = 120)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 1)** | **SMA Type Presymptomatic (N = 6)** | **SMA Type Missing (N = 0)** |
| 2019 | * | * | - | - | - | - | - | - |
| 2020 | - | - | - | - | - | - | - | - |
| 2021 | * | * | - | - | - | - | - | - |
| 2022 | - | - | - | - | - | - | - | - |
| 2023 | - | - | - | - | - | - | - | - |
| Missing | 331 (95.1%) | 83 (86.5%) | 115 (98.3%) | 120 (100.0%) | 6 (75.0%) | * | 6 (100.0%) | - |
| **Sex; n (%)** | | | | | | | | |
| Female | 176 (50.6%) | 53 (55.2%) | 61 (52.1%) | 58 (48.3%) | * | - | * | - |
| Male | 172 (49.4%) | 43 (44.8%) | 56 (47.9%) | 62 (51.7%) | 5 (62.5%) | * | 5 (83.3%) | - |
| Missing | - | - | - | - | - | - | - | - |
| **Class of age at symptom onset; n (%)** | | | | | | | | |
| Presymptomatic | 6 (1.7%) | - | - | - | - | - | 6 (100.0%) | - |
| Prenatal | - | - | - | - | - | - | - | - |
| < 1 month | 6 (1.7%) | * | * | * | - | * | - | - |
| [1 - 3 months) | 34 (9.8%) | 29 (30.2%) | 5 (4.3%) | - | - | - | - | - |
| [3 - 6 months) | 38 (10.9%) | 28 (29.2%) | 10 (8.5%) | - | - | - | - | - |
| [6 - 18 months) | 110 (31.6%) | 16 (16.7%) | 75 (64.1%) | 19 (15.8%) | - | - | - | - |
| [1.5 - 2 years) | 20 (5.7%) | * | 13 (11.1%) | 6 (5.0%) | - | - | - | - |
| [2 - 6 years) | 56 (16.1%) | - | * | 53 (44.2%) | - | - | - | - |
| [6 - 11 years) | 18 (5.2%) | - | - | 17 (14.2%) | * | - | - | - |
| [11 - 18 years) | 15 (4.3%) | - | - | 14 (11.7%) | * | - | - | - |
| 18 years + | 12 (3.4%) | - | - | 6 (5.0%) | 6 (75.0%) | - | - | - |
| Missing | 33 (9.5%) | 19 (19.8%) | 10 (8.5%) | * | - | - | - | - |
| **Best functional SMA status; n (%)** | | | | | | | | |
| Non-sitter | 44 (12.6%) | 12 (12.5%) | 22 (18.8%) | 8 (6.7%) | - | - | * | - |
| Sitter | 122 (35.1%) | 27 (28.1%) | 68 (58.1%) | 25 (20.8%) | - | - | * | - |
| Walker | 96 (27.6%) | * | 8 (6.8%) | 80 (66.7%) | 6 (75.0%) | - | - | - |
| Missing | 86 (24.7%) | 55 (57.3%) | 19 (16.2%) | 7 (5.8%) | * | * | * | - |

| Czech Republic & Slovakia \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 348)** | **SMA Type 1 (N = 96)** | **SMA Type 2 (N = 117)** | **SMA Type 3 (N = 120)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 1)** | **SMA Type Presymptomatic (N = 6)** | **SMA Type Missing (N = 0)** |
| **Best achieved motor milestone; n (%)** | | | | | | | | |
| Climb stairs | 74 (21.3%) | - | 5 (4.3%) | 65 (54.2%) | * | - | - | - |
| Walk 10 metres without assistance | * | - | - | * | - | - | - | - |
| Walk without assistance | 20 (5.7%) | * | * | 13 (10.8%) | * | - | - | - |
| Walk with assistance | - | - | - | - | - | - | - | - |
| Stand without assistance | - | - | - | - | - | - | - | - |
| Stand with assistance | - | - | - | - | - | - | - | - |
| Crawl | 45 (12.9%) | 5 (5.2%) | 29 (24.8%) | 9 (7.5%) | - | - | * | - |
| Sit without support | 77 (22.1%) | 22 (22.9%) | 39 (33.3%) | 16 (13.3%) | - | - | - | - |
| Roll onto side | 21 (6.0%) | 11 (11.5%) | 7 (6.0%) | * | - | - | * | - |
| Hold head without support | 23 (6.6%) | * | 15 (12.8%) | 6 (5.0%) | - | - | * | - |
| Unknown | - | - | - | - | - | - | - | - |
| Missing | 86 (24.7%) | 55 (57.3%) | 19 (16.2%) | 7 (5.8%) | * | * | * | - |
| **SMN1 gene mutation type; n (%)** | | | | | | | | |
| Compound heterozygous deletion exon 7 | 12 (3.4%) | * | * | 6 (5.0%) | * | - | - | - |
| Compound heterozygous substitutions | - | - | - | - | - | - | - | - |
| Homozygous deletion exon 7 | 329 (94.5%) | 92 (95.8%) | 114 (97.4%) | 110 (91.7%) | 6 (75.0%) | * | 6 (100.0%) | - |
| Missing | 7 (2.0%) | - | * | * | * | - | - | - |
| **Number of SMN2 copies; n (%)** | | | | | | | | |
| 0 | * | - | - | * | - | - | - | - |
| 1 | 6 (1.7%) | - | * | * | - | * | - | - |
| 2 | 72 (20.7%) | 57 (59.4%) | 9 (7.7%) | * | - | - | * | - |

| Czech Republic & Slovakia \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 348)** | **SMA Type 1 (N = 96)** | **SMA Type 2 (N = 117)** | **SMA Type 3 (N = 120)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 1)** | **SMA Type Presymptomatic (N = 6)** | **SMA Type Missing (N = 0)** |
| 3 | 156 (44.8%) | 27 (28.1%) | 92 (78.6%) | 33 (27.5%) | * | - | * | - |
| 4 | 77 (22.1%) | - | 5 (4.3%) | 65 (54.2%) | 6 (75.0%) | - | * | - |
| >4 | - | - | - | - | - | - | - | - |
| Other | * | - | * | - | - | - | - | - |
| Missing | 35 (10.1%) | 12 (12.5%) | 9 (7.7%) | 13 (10.8%) | * | - | - | - |
| **Methods used for genetic testing; n (%)** | | | | | | | | |
| DNA Sequencing | - | - | - | - | - | - | - | - |
| HRM | - | - | - | - | - | - | - | - |
| MLPA | 93 (26.7%) | 21 (21.9%) | 24 (20.5%) | 39 (32.5%) | * | * | 6 (100.0%) | - |
| RFLP | - | - | - | - | - | - | - | - |
| ddPCR | - | - | - | - | - | - | - | - |
| qRT-PCR | * | * | - | - | - | - | - | - |
| Missing | 254 (73.0%) | 74 (77.1%) | 93 (79.5%) | 81 (67.5%) | 6 (75.0%) | - | - | - |
| **Duration of follow up (months)** | | | | | | | | |
| Mean (SD) | 51.6 (34.3) | 51.9 (33.6) | 61.5 (36.2) | 45.2 (30.7) | 30 (12.3) | * | 12.8 (5.6) | - |
| Median [IQR] | 42 [24, 67] | 47.5 [24, 67] | 54 [31.5, 81.5] | 35 [24, 55.8] | 27 [21, 40.5] | * | 11.5 [8.2, 15.5] | - |
| Missing; n (%) | 10 (2.9%) | * | * | * | * | - | - | - |
| **Duration of SMA (months)** | | | | | | | | |
| Mean (SD) | 209.8 (170.3) | 89.9 (81.8) | 221.6 (164.3) | 276 (174.2) | 294 (212.6) | * | - | - |
| Median [IQR] | 162 [65.5, 324.5] | 68 [39, 107.5] | 191.5 [85.2, 330.2] | 245 [128.5, 410.5] | 241.5 [137.2, 471] | * | - | - |
| Missing; n (%) | 45 (12.9%) | 20 (20.8%) | 11 (9.4%) | 6 (5.0%) | * | - | 6 (100.0%) | - |
| **Duration between two consecutive visits (months)** | | | | | | | | |
| Mean (SD) | 7.1 (9.3) | 8.2 (14.1) | 6.3 (6.2) | 6.9 (7.2) | * | - | * | - |
| Median [IQR] | 4.3 [3, 6.8] | 3.6 [2.8, 5.8] | 4.7 [3.4, 6.6] | 4.5 [3, 7.6] | * | - | * | - |
| Missing; n (%) | 53 (15.2%) | 17 (17.7%) | 12 (10.3%) | 17 (14.2%) | * | * | * | - |

| Czech Republic & Slovakia \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 348)** | **SMA Type 1 (N = 96)** | **SMA Type 2 (N = 117)** | **SMA Type 3 (N = 120)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 1)** | **SMA Type Presymptomatic (N = 6)** | **SMA Type Missing (N = 0)** |
| **Duration between genetic report date and registry entry (months)** | | | | | | | | |
| Mean (SD) | 88.1 (132.8) | 27.8 (65.3) | 105.9 (129.5) | 126.1 (159.8) | 27.7 (33) | * | 0.3 (0.5) | - |
| Median [IQR] | 13 [1, 141] | 1 [-, 17.5] | 44 [1, 168] | 67 [2, 194.2] | 9.5 [3, 46.8] | * | - | - |
| Missing; n (%) | 10 (2.9%) | * | * | * | * | - | - | - |
| **Reason for genetic testing; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Family screening | * | - | * | * | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 22 (16.5%) | * | 6 (10.3%) | 13 (22.4%) | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | * | * | - | * | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | * | * | * | * | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| Family screening | * | - | - | * | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | * | * | - | * | - | - | - | - |

| Czech Republic & Slovakia \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 348)** | **SMA Type 1 (N = 96)** | **SMA Type 2 (N = 117)** | **SMA Type 3 (N = 120)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 1)** | **SMA Type Presymptomatic (N = 6)** | **SMA Type Missing (N = 0)** |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | * | - | * | - | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | - | - | - | - | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | * | - | - | * | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| Family screening | * | - | - | * | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 19 (76.0%) | 7 (87.5%) | * | 8 (80.0%) | * | - | - | - |
| Prenatal screening | * | - | - | - | - | - | * | - |
| **2022** | | | | | | | | |
| Family screening | * | - | - | * | - | - | - | - |
| Newborn screening | * | * | - | - | - | - | * | - |
| No screening | 14 (70.0%) | * | 5 (100.0%) | 5 (71.4%) | - | * | - | - |

| Czech Republic & Slovakia \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 348)** | **SMA Type 1 (N = 96)** | **SMA Type 2 (N = 117)** | **SMA Type 3 (N = 120)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 1)** | **SMA Type Presymptomatic (N = 6)** | **SMA Type Missing (N = 0)** |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | * | - | - | - | - | - | * | - |
| No screening | - | - | - | - | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **Age at onset of SMA symptoms (years)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | 4 (6.2) | 0.5 (0.4) | 0.9 (0.5) | 5.5 (5.5) | 23.8 (11.7) | - | - | - |
| Median [IQR] | 1.5 [0.8, 4] | 0.4 [0.2, 0.5] | 0.8 [0.5, 1.1] | 3 [2, 7] | 19 [15, 35] | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | 5.7 (12.2) | 0.3 (0.2) | 0.8 (0.3) | 7.8 (12.2) | * | - | - | - |
| Median [IQR] | 0.7 [0.4, 2] | 0.3 [0.1, 0.5] | 0.6 [0.6, 0.9] | 2.5 [2, 3] | * | - | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | 1.8 (3.8) | 0.3 (0.2) | 0.9 (0.3) | 6.8 (6.6) | - | - | - | - |
| Median [IQR] | 0.6 [0.3, 1.2] | 0.3 [0.3, 0.5] | 0.7 [0.6, 1.2] | 3 [1.1, 12.5] | - | - | - | - |
| **2017** | | | | | | | | |
| Mean (SD) | 0.7 (0.5) | 0.3 (0.2) | * | * | - | - | - | - |
| Median [IQR] | 0.5 [0.3, 1] | 0.3 [0.2, 0.5] | * | * | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | 0.7 (0.7) | 0.3 (0.1) | 0.8 (0.5) | * | - | - | - | - |
| Median [IQR] | 0.5 [0.3, 0.9] | 0.3 [0.2, 0.3] | 0.8 [0.5, 0.9] | * | - | - | - | - |
| **2019** | | | | | | | | |
| Mean (SD) | 3.5 (6.3) | 0.1 (0.1) | * | * | * | - | - | - |
| Median [IQR] | 0.5 [0.1, 1] | 0.1 [0.1, 0.1] | * | * | * | - | - | - |
| **2020** | | | | | | | | |
| Mean (SD) | 1.3 (2.5) | 0.1 (0.1) | * | * | - | - | - | - |

| Czech Republic & Slovakia \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 348)** | **SMA Type 1 (N = 96)** | **SMA Type 2 (N = 117)** | **SMA Type 3 (N = 120)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 1)** | **SMA Type Presymptomatic (N = 6)** | **SMA Type Missing (N = 0)** |
| Median [IQR] | 0.2 [0.1, 0.8] | 0.1 [0.1, 0.2] | * | * | - | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | 1.2 (1.8) | 0.3 (0.2) | 0.6 (0.2) | * | - | - | - | - |
| Median [IQR] | 0.5 [0.4, 0.9] | 0.2 [0.2, 0.4] | 0.5 [0.5, 0.6] | * | - | - | - | - |
| **2022** | | | | | | | | |
| Mean (SD) | 3.2 (6.6) | * | * | * | - | * | - | - |
| Median [IQR] | 0.1 [-, 0.6] | * | * | * | - | * | - | - |
| **2023** | | | | | | | | |
| Mean (SD) | * | - | - | * | - | - | - | - |
| Median [IQR] | * | - | - | * | - | - | - | - |
| **Age at genetic report date (years)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | 7 (9.4) | 1.5 (2.3) | 3.9 (7.1) | 10.1 (8.6) | * | - | - | - |
| Median [IQR] | 2.5 [1.3, 8.4] | 0.8 [0.5, 1.2] | 1.6 [1.2, 2.6] | 7.5 [2.9, 14.7] | * | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | 8 (11.6) | 0.9 (1.3) | 2 (1.9) | 20.3 (12.1) | - | - | - | - |
| Median [IQR] | 1.5 [0.7, 13.5] | 0.7 [0.4, 0.8] | 1.6 [1, 2] | 17 [13.5, 29.1] | - | - | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | 7 (13.7) | 0.6 (0.4) | 4.2 (9) | 20.5 (17.3) | * | - | - | - |
| Median [IQR] | 1.2 [0.5, 2.9] | 0.5 [0.3, 0.8] | 1.6 [1.2, 2.4] | 7.9 [7.8, 35.8] | * | - | - | - |
| **2017** | | | | | | | | |
| Mean (SD) | 6.4 (8.1) | 2.9 (5.7) | 5 (8.8) | 12.8 (6.2) | - | - | - | - |
| Median [IQR] | 1.4 [1.1, 12.9] | 0.7 [0.5, 1.2] | 1.4 [1.3, 1.7] | 14.6 [10.1, 16.4] | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | 9.9 (14.2) | 0.8 (0.5) | 9.9 (15.4) | 17.8 (14.6) | - | - | - | - |
| Median [IQR] | 1.9 [0.8, 12.4] | 0.7 [0.5, 1] | 1.8 [1.1, 8.8] | 12.4 [6.8, 26.1] | - | - | - | - |
| **2019** | | | | | | | | |
| Mean (SD) | 10.5 (15.8) | 0.4 (0.4) | 9.9 (13.7) | 17.2 (14.4) | * | - | - | - |
| Median [IQR] | 1.4 [0.4, 15] | 0.3 [0.2, 0.4] | 1.5 [1.1, 14.7] | 13.3 [6.2, 26.4] | * | - | - | - |

| Czech Republic & Slovakia \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 348)** | **SMA Type 1 (N = 96)** | **SMA Type 2 (N = 117)** | **SMA Type 3 (N = 120)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 1)** | **SMA Type Presymptomatic (N = 6)** | **SMA Type Missing (N = 0)** |
| **2020** | | | | | | | | |
| Mean (SD) | 6.9 (15.4) | 0.3 (0.2) | 9.9 (17.4) | 13.2 (19.9) | - | - | - | - |
| Median [IQR] | 1 [0.3, 3.3] | 0.2 [0.2, 0.4] | 1.1 [1, 1.9] | 4.8 [2.8, 7] | - | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | 12.4 (18.5) | 0.5 (0.3) | 10.7 (19.4) | 19.3 (16.8) | * | - | * | - |
| Median [IQR] | 1.4 [0.6, 15.8] | 0.5 [0.2, 0.8] | 1.1 [1, 1.4] | 12.3 [5, 31.5] | * | - | * | - |
| **2022** | | | | | | | | |
| Mean (SD) | 9.1 (15.6) | * | 7.9 (13.3) | 20 (19) | - | * | * | - |
| Median [IQR] | 1.4 [0.1, 8.7] | * | 1.4 [1.3, 1.5] | 11.2 [5.8, 32] | - | * | * | - |
| **2023** | | | | | | | | |
| Mean (SD) | * | - | - | - | - | - | * | - |
| Median [IQR] | * | - | - | - | - | - | * | - |
| **Age at registry entry (years)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | 11.6 (10.7) | 4.1 (4.6) | 9.7 (6.8) | 21.2 (11.7) | - | - | - | - |
| Median [IQR] | 8.2 [2.7, 15.1] | 1.5 [0.7, 6.6] | 7 [4.6, 13.7] | 16 [12.8, 31.8] | - | - | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | 9.5 (11.1) | 1.9 (4) | 9 (8.7) | 20.4 (10.4) | * | - | - | - |
| Median [IQR] | 3.7 [1.1, 15.9] | 0.9 [0.4, 1.1] | 5.1 [1.9, 13.4] | 18.3 [14, 23] | * | - | - | - |
| **2017** | | | | | | | | |
| Mean (SD) | 10.7 (12) | 7.1 (6.3) | 10.2 (14.1) | 17.5 (11.4) | - | - | - | - |
| Median [IQR] | 7.8 [1.5, 14.9] | 5.5 [1.1, 11.9] | 3.8 [1.6, 11.7] | 16.4 [12.9, 19.8] | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | 6.6 (7.9) | 3.4 (4.1) | 6.2 (6) | 13.8 (11.2) | - | - | - | - |
| Median [IQR] | 3.1 [1.7, 9.9] | 1.8 [0.9, 2.8] | 3.5 [2, 9.6] | 10.5 [6.9, 14.3] | - | - | - | - |
| **2019** | | | | | | | | |

| Czech Republic & Slovakia \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 348)** | **SMA Type 1 (N = 96)** | **SMA Type 2 (N = 117)** | **SMA Type 3 (N = 120)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 1)** | **SMA Type Presymptomatic (N = 6)** | **SMA Type Missing (N = 0)** |
| Mean (SD) | 9.6 (13.2) | 0.5 (0.5) | 10 (13.6) | 12.2 (5.5) | * | - | - | - |
| Median [IQR] | 1.7 [0.9, 14.9] | 0.3 [0.2, 0.8] | 1.5 [1.2, 14.4] | 14.1 [10.9, 15.5] | * | - | - | - |
| **2020** | | | | | | | | |
| Mean (SD) | 24.6 (18.2) | 0.3 (0.2) | 23.5 (13.5) | 32.1 (18) | - | - | - | - |
| Median [IQR] | 23.9 [5, 38.6] | 0.2 [0.2, 0.4] | 24.6 [16.2, 31.5] | 35.2 [21.1, 45.4] | - | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | 23.6 (17.7) | 8.6 (14.4) | 21.7 (18) | 29.1 (15) | * | - | * | - |
| Median [IQR] | 27.8 [1.8, 38.7] | 0.7 [0.2, 7.5] | 27.8 [1.3, 35.5] | 28.7 [20.6, 39.4] | * | - | * | - |
| **2022** | | | | | | | | |
| Mean (SD) | 20.6 (20.4) | 0.9 (0.8) | 23.4 (18.7) | 27.1 (17.5) | * | * | * | - |
| Median [IQR] | 14.1 [1.4, 40.7] | 0.5 [0.2, 1.6] | 30.6 [1.5, 38.4] | 21.6 [11.2, 45.9] | * | * | * | - |
| **2023** | | | | | | | | |
| Mean (SD) | * | - | - | * | - | - | * | - |
| Median [IQR] | * | - | - | * | - | - | * | - |
| **Age at death (years)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | * | - | * | - | * | - | - | - |
| Median [IQR] | * | - | * | - | * | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | * | * | - | - | * | - | - | - |
| Median [IQR] | * | * | - | - | * | - | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | 1 (1) | 1 (1) | - | - | - | - | - | - |
| Median [IQR] | 0.6 [0.4, 0.9] | 0.6 [0.4, 0.9] | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | * | - | * | - | - | - | - | - |

| Czech Republic & Slovakia \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 348)** | **SMA Type 1 (N = 96)** | **SMA Type 2 (N = 117)** | **SMA Type 3 (N = 120)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 1)** | **SMA Type Presymptomatic (N = 6)** | **SMA Type Missing (N = 0)** |
| Median [IQR] | * | - | * | - | - | - | - | - |
| **2019** | | | | | | | | |
| Mean (SD) | * | * | - | - | - | - | - | - |
| Median [IQR] | * | * | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | * | * | - | - | - | - | - | - |
| Median [IQR] | * | * | - | - | - | - | - | - |
| **2022** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **Lost to follow-up; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 41 (93.2%) | 13 (92.9%) | 16 (100.0%) | 12 (85.7%) | - | - | - | - |
| Yes | * | * | - | * | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 77 (89.5%) | 24 (88.9%) | 34 (94.4%) | 18 (81.8%) | * | - | - | - |
| Yes | 9 (10.5%) | * | * | * | - | - | - | - |
| **2017** | | | | | | | | |
| No | 91 (94.8%) | 26 (92.9%) | 43 (97.7%) | 21 (91.3%) | * | - | - | - |
| Yes | 5 (5.2%) | * | * | * | - | - | - | - |

| Czech Republic & Slovakia \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 348)** | **SMA Type 1 (N = 96)** | **SMA Type 2 (N = 117)** | **SMA Type 3 (N = 120)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 1)** | **SMA Type Presymptomatic (N = 6)** | **SMA Type Missing (N = 0)** |
| **2018** | | | | | | | | |
| No | 130 (94.2%) | 44 (93.6%) | 57 (96.6%) | 28 (90.3%) | * | - | - | - |
| Yes | 8 (5.8%) | * | * | * | - | - | - | - |
| **2019** | | | | | | | | |
| No | 154 (96.2%) | 52 (96.3%) | 63 (96.9%) | 37 (97.4%) | * | - | - | - |
| Yes | 6 (3.8%) | * | * | * | * | - | - | - |
| **2020** | | | | | | | | |
| No | 210 (99.1%) | 57 (98.3%) | 86 (100.0%) | 65 (98.5%) | * | - | - | - |
| Yes | * | * | - | * | - | - | - | - |
| **2021** | | | | | | | | |
| No | 254 (98.8%) | 63 (96.9%) | 97 (100.0%) | 91 (100.0%) | * | - | * | - |
| Yes | * | * | - | - | * | - | - | - |
| **2022** | | | | | | | | |
| No | 269 (93.7%) | 65 (95.6%) | 102 (95.3%) | 94 (90.4%) | * | * | * | - |
| Yes | 18 (6.3%) | * | 5 (4.7%) | 10 (9.6%) | - | - | - | - |
| **2023** | | | | | | | | |
| No | 250 (91.6%) | 55 (84.6%) | 94 (92.2%) | 91 (95.8%) | * | * | 6 (100.0%) | - |
| Yes | 23 (8.4%) | 10 (15.4%) | 8 (7.8%) | * | * | - | - | - |
| **Treated with at least one DMT; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 44 (100.0%) | 14 (100.0%) | 16 (100.0%) | 14 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 81 (100.0%) | 26 (100.0%) | 35 (100.0%) | 19 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |

| Czech Republic & Slovakia \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 348)** | **SMA Type 1 (N = 96)** | **SMA Type 2 (N = 117)** | **SMA Type 3 (N = 120)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 1)** | **SMA Type Presymptomatic (N = 6)** | **SMA Type Missing (N = 0)** |
| No | 71 (86.6%) | 18 (78.3%) | 36 (90.0%) | 16 (88.9%) | * | - | - | - |
| Yes | 11 (13.4%) | 5 (21.7%) | * | * | - | - | - | - |
| **2018** | | | | | | | | |
| No | 45 (36.6%) | 10 (23.8%) | 18 (33.3%) | 17 (63.0%) | - | - | - | - |
| Yes | 78 (63.4%) | 32 (76.2%) | 36 (66.7%) | 10 (37.0%) | - | - | - | - |
| **2019** | | | | | | | | |
| No | 43 (28.3%) | 9 (17.6%) | 17 (27.0%) | 15 (41.7%) | * | - | - | - |
| Yes | 109 (71.7%) | 42 (82.4%) | 46 (73.0%) | 21 (58.3%) | - | - | - | - |
| **2020** | | | | | | | | |
| No | 33 (15.9%) | 5 (9.1%) | 13 (15.1%) | 14 (21.5%) | * | - | - | - |
| Yes | 174 (84.1%) | 50 (90.9%) | 73 (84.9%) | 51 (78.5%) | - | - | - | - |
| **2021** | | | | | | | | |
| No | 29 (12.3%) | 5 (8.3%) | 6 (6.5%) | 17 (21.0%) | * | - | - | - |
| Yes | 207 (87.7%) | 55 (91.7%) | 86 (93.5%) | 64 (79.0%) | * | - | * | - |
| **2022** | | | | | | | | |
| No | 21 (8.6%) | * | 5 (5.4%) | 12 (13.5%) | * | * | - | - |
| Yes | 222 (91.4%) | 52 (96.3%) | 88 (94.6%) | 77 (86.5%) | * | - | * | - |
| **2023** | | | | | | | | |
| No | 10 (8.3%) | * | 5 (11.1%) | * | - | - | - | - |
| Yes | 110 (91.7%) | 23 (95.8%) | 40 (88.9%) | 39 (90.7%) | * | - | 6 (100.0%) | - |
| **Treated with more than one DMT; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 44 (100.0%) | 14 (100.0%) | 16 (100.0%) | 14 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 81 (100.0%) | 26 (100.0%) | 35 (100.0%) | 19 (100.0%) | * | - | - | - |

| Czech Republic & Slovakia \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 348)** | **SMA Type 1 (N = 96)** | **SMA Type 2 (N = 117)** | **SMA Type 3 (N = 120)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 1)** | **SMA Type Presymptomatic (N = 6)** | **SMA Type Missing (N = 0)** |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 82 (100.0%) | 23 (100.0%) | 40 (100.0%) | 18 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 123 (100.0%) | 42 (100.0%) | 54 (100.0%) | 27 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 152 (100.0%) | 51 (100.0%) | 63 (100.0%) | 36 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 197 (95.2%) | 50 (90.9%) | 81 (94.2%) | 65 (100.0%) | * | - | - | - |
| Yes | 10 (4.8%) | 5 (9.1%) | 5 (5.8%) | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 218 (92.4%) | 52 (86.7%) | 89 (96.7%) | 74 (91.4%) | * | - | * | - |
| Yes | 18 (7.6%) | 8 (13.3%) | * | 7 (8.6%) | - | - | - | - |
| **2022** | | | | | | | | |
| No | 238 (97.9%) | 52 (96.3%) | 91 (97.8%) | 89 (100.0%) | * | * | * | - |
| Yes | 5 (2.1%) | * | * | - | - | - | * | - |
| **2023** | | | | | | | | |
| No | 118 (98.3%) | 22 (91.7%) | 45 (100.0%) | 43 (100.0%) | * | - | 6 (100.0%) | - |
| Yes | * | * | - | - | - | - | - | - |
| **Treated with nusinersen; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 44 (100.0%) | 14 (100.0%) | 16 (100.0%) | 14 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |

| Czech Republic & Slovakia \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 348)** | **SMA Type 1 (N = 96)** | **SMA Type 2 (N = 117)** | **SMA Type 3 (N = 120)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 1)** | **SMA Type Presymptomatic (N = 6)** | **SMA Type Missing (N = 0)** |
| **2014-2016** | | | | | | | | |
| No | 81 (100.0%) | 26 (100.0%) | 35 (100.0%) | 19 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 71 (86.6%) | 18 (78.3%) | 36 (90.0%) | 16 (88.9%) | * | - | - | - |
| Yes | 11 (13.4%) | 5 (21.7%) | * | * | - | - | - | - |
| **2018** | | | | | | | | |
| No | 45 (36.6%) | 10 (23.8%) | 18 (33.3%) | 17 (63.0%) | - | - | - | - |
| Yes | 78 (63.4%) | 32 (76.2%) | 36 (66.7%) | 10 (37.0%) | - | - | - | - |
| **2019** | | | | | | | | |
| No | 43 (28.3%) | 9 (17.6%) | 17 (27.0%) | 15 (41.7%) | * | - | - | - |
| Yes | 109 (71.7%) | 42 (82.4%) | 46 (73.0%) | 21 (58.3%) | - | - | - | - |
| **2020** | | | | | | | | |
| No | 43 (20.8%) | 7 (12.7%) | 21 (24.4%) | 14 (21.5%) | * | - | - | - |
| Yes | 164 (79.2%) | 48 (87.3%) | 65 (75.6%) | 51 (78.5%) | - | - | - | - |
| **2021** | | | | | | | | |
| No | 85 (36.0%) | 25 (41.7%) | 38 (41.3%) | 20 (24.7%) | * | - | * | - |
| Yes | 151 (64.0%) | 35 (58.3%) | 54 (58.7%) | 61 (75.3%) | * | - | - | - |
| **2022** | | | | | | | | |
| No | 120 (49.4%) | 35 (64.8%) | 49 (52.7%) | 31 (34.8%) | * | * | * | - |
| Yes | 123 (50.6%) | 19 (35.2%) | 44 (47.3%) | 58 (65.2%) | * | - | * | - |
| **2023** | | | | | | | | |
| No | 61 (50.8%) | 12 (50.0%) | 26 (57.8%) | 17 (39.5%) | * | - | 5 (83.3%) | - |
| Yes | 59 (49.2%) | 12 (50.0%) | 19 (42.2%) | 26 (60.5%) | * | - | * | - |
| **Treated with onasemnogene abeparvovec-xioi; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |

| Czech Republic & Slovakia \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 348)** | **SMA Type 1 (N = 96)** | **SMA Type 2 (N = 117)** | **SMA Type 3 (N = 120)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 1)** | **SMA Type Presymptomatic (N = 6)** | **SMA Type Missing (N = 0)** |
| **2011-2013** | | | | | | | | |
| No | 44 (100.0%) | 14 (100.0%) | 16 (100.0%) | 14 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 81 (100.0%) | 26 (100.0%) | 35 (100.0%) | 19 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 82 (100.0%) | 23 (100.0%) | 40 (100.0%) | 18 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 123 (100.0%) | 42 (100.0%) | 54 (100.0%) | 27 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 152 (100.0%) | 51 (100.0%) | 63 (100.0%) | 36 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 197 (95.2%) | 48 (87.3%) | 83 (96.5%) | 65 (100.0%) | * | - | - | - |
| Yes | 10 (4.8%) | 7 (12.7%) | * | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 207 (87.7%) | 43 (71.7%) | 84 (91.3%) | 78 (96.3%) | * | - | - | - |
| Yes | 29 (12.3%) | 17 (28.3%) | 8 (8.7%) | * | - | - | * | - |
| **2022** | | | | | | | | |
| No | 209 (86.0%) | 37 (68.5%) | 83 (89.2%) | 85 (95.5%) | * | * | - | - |
| Yes | 34 (14.0%) | 17 (31.5%) | 10 (10.8%) | * | - | - | * | - |
| **2023** | | | | | | | | |
| No | 100 (83.3%) | 17 (70.8%) | 41 (91.1%) | 39 (90.7%) | * | - | * | - |
| Yes | 20 (16.7%) | 7 (29.2%) | * | * | - | - | 5 (83.3%) | - |
| **Treated with risdiplan; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |

| Czech Republic & Slovakia \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 348)** | **SMA Type 1 (N = 96)** | **SMA Type 2 (N = 117)** | **SMA Type 3 (N = 120)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 1)** | **SMA Type Presymptomatic (N = 6)** | **SMA Type Missing (N = 0)** |
| No | - | - | - | - | - | - | - | - |
| Yes | | | | | | | | |
| **2011-2013** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 44 (100.0%) | 14 (100.0%) | 16 (100.0%) | 14 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 81 (100.0%) | 26 (100.0%) | 35 (100.0%) | 19 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 82 (100.0%) | 23 (100.0%) | 40 (100.0%) | 18 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 123 (100.0%) | 42 (100.0%) | 54 (100.0%) | 27 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 152 (100.0%) | 51 (100.0%) | 63 (100.0%) | 36 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 197 (95.2%) | 55 (100.0%) | 76 (88.4%) | 65 (100.0%) | * | - | - | - |
| Yes | 10 (4.8%) | - | 10 (11.6%) | - | - | - | - | - |
| **2022** | | | | | | | | |
| No | 191 (80.9%) | 49 (81.7%) | 65 (70.7%) | 74 (91.4%) | * | - | * | - |
| Yes | 45 (19.1%) | 11 (18.3%) | 27 (29.3%) | 7 (8.6%) | - | - | - | - |
| **2023** | | | | | | | | |
| No | 172 (70.8%) | 36 (66.7%) | 57 (61.3%) | 74 (83.1%) | * | * | * | - |
| Yes | 71 (29.2%) | 18 (33.3%) | 36 (38.7%) | 15 (16.9%) | * | - | * | - |

| Czech Republic & Slovakia \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 348)** | **SMA Type 1 (N = 96)** | **SMA Type 2 (N = 117)** | **SMA Type 3 (N = 120)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 1)** | **SMA Type Presymptomatic (N = 6)** | **SMA Type Missing (N = 0)** |
| **At least one episode of invasive ventilation; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 38 (86.4%) | 9 (64.3%) | 15 (93.8%) | 14 (100.0%) | - | - | - | - |
| Yes | 6 (13.6%) | 5 (35.7%) | * | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 72 (88.9%) | 18 (69.2%) | 34 (97.1%) | 19 (100.0%) | * | - | - | - |
| Yes | 9 (11.1%) | 8 (30.8%) | * | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 71 (86.6%) | 13 (56.5%) | 39 (97.5%) | 18 (100.0%) | * | - | - | - |
| Yes | 11 (13.4%) | 10 (43.5%) | * | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 105 (85.4%) | 26 (61.9%) | 52 (96.3%) | 27 (100.0%) | - | - | - | - |
| Yes | 18 (14.6%) | 16 (38.1%) | * | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 134 (88.2%) | 35 (68.6%) | 61 (96.8%) | 36 (100.0%) | * | - | - | - |
| Yes | 18 (11.8%) | 16 (31.4%) | * | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 190 (91.8%) | 40 (72.7%) | 84 (97.7%) | 65 (100.0%) | * | - | - | - |
| Yes | 17 (8.2%) | 15 (27.3%) | * | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 219 (92.8%) | 45 (75.0%) | 90 (97.8%) | 81 (100.0%) | * | - | * | - |
| Yes | 17 (7.2%) | 15 (25.0%) | * | - | - | - | - | - |
| **2022** | | | | | | | | |
| No | 229 (94.2%) | 42 (77.8%) | 92 (98.9%) | 89 (100.0%) | * | - | * | - |
| Yes | 14 (5.8%) | 12 (22.2%) | * | - | - | * | - | - |
| **2023** | | | | | | | | |

| Czech Republic & Slovakia \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 348)** | **SMA Type 1 (N = 96)** | **SMA Type 2 (N = 117)** | **SMA Type 3 (N = 120)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 1)** | **SMA Type Presymptomatic (N = 6)** | **SMA Type Missing (N = 0)** |
| No | 113 (94.2%) | 18 (75.0%) | 44 (97.8%) | 43 (100.0%) | * | - | 6 (100.0%) | - |
| Yes | 7 (5.8%) | 6 (25.0%) | * | - | - | - | - | - |
| **At least one episode of non-invasive ventilation; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 38 (86.4%) | 12 (85.7%) | 14 (87.5%) | 12 (85.7%) | - | - | - | - |
| Yes | 6 (13.6%) | * | * | * | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 72 (88.9%) | 22 (84.6%) | 31 (88.6%) | 18 (94.7%) | * | - | - | - |
| Yes | 9 (11.1%) | * | * | * | - | - | - | - |
| **2017** | | | | | | | | |
| No | 70 (85.4%) | 17 (73.9%) | 35 (87.5%) | 17 (94.4%) | * | - | - | - |
| Yes | 12 (14.6%) | 6 (26.1%) | 5 (12.5%) | * | - | - | - | - |
| **2018** | | | | | | | | |
| No | 108 (87.8%) | 33 (78.6%) | 48 (88.9%) | 27 (100.0%) | - | - | - | - |
| Yes | 15 (12.2%) | 9 (21.4%) | 6 (11.1%) | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 134 (88.2%) | 38 (74.5%) | 58 (92.1%) | 36 (100.0%) | * | - | - | - |
| Yes | 18 (11.8%) | 13 (25.5%) | 5 (7.9%) | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 182 (87.9%) | 38 (69.1%) | 78 (90.7%) | 65 (100.0%) | * | - | - | - |
| Yes | 25 (12.1%) | 17 (30.9%) | 8 (9.3%) | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 206 (87.3%) | 41 (68.3%) | 81 (88.0%) | 81 (100.0%) | * | - | * | - |
| Yes | 30 (12.7%) | 19 (31.7%) | 11 (12.0%) | - | - | - | - | - |
| **2022** | | | | | | | | |

| Czech Republic & Slovakia \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 348)** | **SMA Type 1 (N = 96)** | **SMA Type 2 (N = 117)** | **SMA Type 3 (N = 120)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 1)** | **SMA Type Presymptomatic (N = 6)** | **SMA Type Missing (N = 0)** |
| No | 215 (88.5%) | 39 (72.2%) | 80 (86.0%) | 89 (100.0%) | * | * | * | - |
| Yes | 28 (11.5%) | 15 (27.8%) | 13 (14.0%) | - | - | - | - | - |
| **2023** | | | | | | | | |
| No | 105 (87.5%) | 15 (62.5%) | 39 (86.7%) | 43 (100.0%) | * | - | 6 (100.0%) | - |
| Yes | 15 (12.5%) | 9 (37.5%) | 6 (13.3%) | - | - | - | - | - |
| **At least one episode of feeding tube usage; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 40 (90.9%) | 10 (71.4%) | 16 (100.0%) | 14 (100.0%) | - | - | - | - |
| Yes | * | * | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 74 (91.4%) | 19 (73.1%) | 35 (100.0%) | 19 (100.0%) | * | - | - | - |
| Yes | 7 (8.6%) | 7 (26.9%) | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 72 (87.8%) | 14 (60.9%) | 39 (97.5%) | 18 (100.0%) | * | - | - | - |
| Yes | 10 (12.2%) | 9 (39.1%) | * | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 104 (84.6%) | 24 (57.1%) | 53 (98.1%) | 27 (100.0%) | - | - | - | - |
| Yes | 19 (15.4%) | 18 (42.9%) | * | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 127 (83.6%) | 27 (52.9%) | 62 (98.4%) | 36 (100.0%) | * | - | - | - |
| Yes | 25 (16.4%) | 24 (47.1%) | * | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 183 (88.4%) | 33 (60.0%) | 84 (97.7%) | 65 (100.0%) | * | - | - | - |
| Yes | 24 (11.6%) | 22 (40.0%) | * | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 209 (88.6%) | 35 (58.3%) | 90 (97.8%) | 81 (100.0%) | * | - | * | - |

| Czech Republic & Slovakia \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 348)** | **SMA Type 1 (N = 96)** | **SMA Type 2 (N = 117)** | **SMA Type 3 (N = 120)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 1)** | **SMA Type Presymptomatic (N = 6)** | **SMA Type Missing (N = 0)** |
| Yes | 27 (11.4%) | 25 (41.7%) | * | - | - | - | - | - |
| **2022** | | | | | | | | |
| No | 218 (89.7%) | 33 (61.1%) | 90 (96.8%) | 89 (100.0%) | * | - | * | - |
| Yes | 25 (10.3%) | 21 (38.9%) | * | - | - | * | - | - |
| **2023** | | | | | | | | |
| No | 107 (89.2%) | 12 (50.0%) | 44 (97.8%) | 43 (100.0%) | * | - | 6 (100.0%) | - |
| Yes | 13 (10.8%) | 12 (50.0%) | * | - | - | - | - | - |
| **At least one episode of wheelchair usage; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 19 (43.2%) | 7 (50.0%) | * | 11 (78.6%) | - | - | - | - |
| Yes | 25 (56.8%) | 7 (50.0%) | 15 (93.8%) | * | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 25 (30.9%) | 10 (38.5%) | - | 14 (73.7%) | * | - | - | - |
| Yes | 56 (69.1%) | 16 (61.5%) | 35 (100.0%) | 5 (26.3%) | - | - | - | - |
| **2017** | | | | | | | | |
| No | 21 (25.6%) | 5 (21.7%) | * | 12 (66.7%) | * | - | - | - |
| Yes | 61 (74.4%) | 18 (78.3%) | 37 (92.5%) | 6 (33.3%) | - | - | - | - |
| **2018** | | | | | | | | |
| No | 33 (26.8%) | 12 (28.6%) | * | 17 (63.0%) | - | - | - | - |
| Yes | 90 (73.2%) | 30 (71.4%) | 50 (92.6%) | 10 (37.0%) | - | - | - | - |
| **2019** | | | | | | | | |
| No | 51 (33.6%) | 17 (33.3%) | 9 (14.3%) | 23 (63.9%) | * | - | - | - |
| Yes | 101 (66.4%) | 34 (66.7%) | 54 (85.7%) | 13 (36.1%) | - | - | - | - |
| **2020** | | | | | | | | |
| No | 68 (32.9%) | 17 (30.9%) | 12 (14.0%) | 38 (58.5%) | * | - | - | - |

| Czech Republic & Slovakia \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 348)** | **SMA Type 1 (N = 96)** | **SMA Type 2 (N = 117)** | **SMA Type 3 (N = 120)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 1)** | **SMA Type Presymptomatic (N = 6)** | **SMA Type Missing (N = 0)** |
| Yes | 139 (67.1%) | 38 (69.1%) | 74 (86.0%) | 27 (41.5%) | - | - | - | - |
| **2021** | | | | | | | | |
| No | 79 (33.5%) | 17 (28.3%) | 12 (13.0%) | 48 (59.3%) | * | - | * | - |
| Yes | 157 (66.5%) | 43 (71.7%) | 80 (87.0%) | 33 (40.7%) | * | - | - | - |
| **2022** | | | | | | | | |
| No | 85 (35.0%) | 13 (24.1%) | 10 (10.8%) | 57 (64.0%) | * | * | * | - |
| Yes | 158 (65.0%) | 41 (75.9%) | 83 (89.2%) | 32 (36.0%) | * | - | - | - |
| **2023** | | | | | | | | |
| No | 41 (34.2%) | * | * | 27 (62.8%) | - | - | 6 (100.0%) | - |
| Yes | 79 (65.8%) | 20 (83.3%) | 41 (91.1%) | 16 (37.2%) | * | - | - | - |
| **At least one reported measure by available motor function scale or test and by at least one PRO; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 44 (100.0%) | 14 (100.0%) | 16 (100.0%) | 14 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 81 (100.0%) | 26 (100.0%) | 35 (100.0%) | 19 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 82 (100.0%) | 23 (100.0%) | 40 (100.0%) | 18 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 123 (100.0%) | 42 (100.0%) | 54 (100.0%) | 27 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |

| Czech Republic & Slovakia \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 348)** | **SMA Type 1 (N = 96)** | **SMA Type 2 (N = 117)** | **SMA Type 3 (N = 120)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 1)** | **SMA Type Presymptomatic (N = 6)** | **SMA Type Missing (N = 0)** |
| No | 152 (100.0%) | 51 (100.0%) | 63 (100.0%) | 36 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 207 (100.0%) | 55 (100.0%) | 86 (100.0%) | 65 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 236 (100.0%) | 60 (100.0%) | 92 (100.0%) | 81 (100.0%) | * | - | * | - |
| Yes | - | - | - | - | - | - | - | - |
| **2022** | | | | | | | | |
| No | 243 (100.0%) | 54 (100.0%) | 93 (100.0%) | 89 (100.0%) | * | * | * | - |
| Yes | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| No | 120 (100.0%) | 24 (100.0%) | 45 (100.0%) | 43 (100.0%) | * | - | 6 (100.0%) | - |
| Yes | - | - | - | - | - | - | - | - |
| **At least three reported measures by available motor function scale or test and by at least one PRO; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 44 (100.0%) | 14 (100.0%) | 16 (100.0%) | 14 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 81 (100.0%) | 26 (100.0%) | 35 (100.0%) | 19 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 82 (100.0%) | 23 (100.0%) | 40 (100.0%) | 18 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |

| Czech Republic & Slovakia \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 348)** | **SMA Type 1 (N = 96)** | **SMA Type 2 (N = 117)** | **SMA Type 3 (N = 120)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 1)** | **SMA Type Presymptomatic (N = 6)** | **SMA Type Missing (N = 0)** |
| **2018** | | | | | | | | |
| No | 123 (100.0%) | 42 (100.0%) | 54 (100.0%) | 27 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 152 (100.0%) | 51 (100.0%) | 63 (100.0%) | 36 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 207 (100.0%) | 55 (100.0%) | 86 (100.0%) | 65 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 236 (100.0%) | 60 (100.0%) | 92 (100.0%) | 81 (100.0%) | * | - | * | - |
| Yes | - | - | - | - | - | - | - | - |
| **2022** | | | | | | | | |
| No | 243 (100.0%) | 54 (100.0%) | 93 (100.0%) | 89 (100.0%) | * | * | * | - |
| Yes | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| No | 120 (100.0%) | 24 (100.0%) | 45 (100.0%) | 43 (100.0%) | * | - | 6 (100.0%) | - |
| Yes | - | - | - | - | - | - | - | - |
| **Available number of records of each motor function scale by patient** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | * | - | - | * | - | - | - | - |
| Median [IQR] | * | - | - | * | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | * | * | - | * | - | - | - | - |
| Median [IQR] | * | * | - | * | - | - | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |

| Czech Republic & Slovakia \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 348)** | **SMA Type 1 (N = 96)** | **SMA Type 2 (N = 117)** | **SMA Type 3 (N = 120)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 1)** | **SMA Type Presymptomatic (N = 6)** | **SMA Type Missing (N = 0)** |
| **2017** | | | | | | | | |
| Mean (SD) | 1.1 (0.3) | * | * | 1 (-) | - | - | - | - |
| Median [IQR] | 1 [1, 1] | * | * | 1 [1, 1] | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | 2.1 (1.6) | 1.8 (1) | 2 (1.4) | 2.8 (2.1) | - | - | - | - |
| Median [IQR] | 1 [1, 3] | 1 [1, 2] | 2 [1, 2.5] | 1.5 [1, 5] | - | - | - | - |
| **2019** | | | | | | | | |
| Mean (SD) | - | 1.9 (0.9) | 1.7 (0.9) | 2 (0.9) | - | - | - | - |
| Median [IQR] | 2 [1, 3] | 2 [1, 2] | 1.5 [1, 2] | 2 [1, 3] | - | - | - | - |
| **2020** | | | | | | | | |
| Mean (SD) | 1.6 (0.8) | 1.9 (1) | 1.5 (0.7) | 1.6 (0.7) | - | - | - | - |
| Median [IQR] | 1 [1, 2] | 2 [1, 2] | 1 [1, 2] | 1 [1, 2] | - | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | 1.8 (0.9) | 1.6 (0.9) | 1.8 (0.8) | 1.9 (1) | * | - | * | - |
| Median [IQR] | 2 [1, 2] | 1 [1, 2] | 2 [1, 2] | 2 [1, 2] | * | - | * | - |
| **2022** | | | | | | | | |
| Mean (SD) | 1.7 (0.8) | 1.4 (0.6) | 1.7 (0.8) | 1.7 (0.9) | * | * | 1.5 (0.8) | - |
| Median [IQR] | 1 [1, 2] | 1 [1, 2] | 2 [1, 2] | 1 [1, 2] | * | * | 1 [1, 1.8] | - |
| **2023** | | | | | | | | |
| Mean (SD) | 1 (0.2) | 1.1 (0.3) | 1 (-) | 1.1 (0.3) | 1 (-) | - | * | - |
| Median [IQR] | 1 [1, 1] | 1 [1, 1] | 1 [1, 1] | 1 [1, 1] | 1 [1, 1] | - | * | - |
| **Available number of records of PRO** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |

| Czech Republic & Slovakia \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 348)** | **SMA Type 1 (N = 96)** | **SMA Type 2 (N = 117)** | **SMA Type 3 (N = 120)** | **SMA Type 4 (N = 8)** | **SMA Type Other (N = 1)** | **SMA Type Presymptomatic (N = 6)** | **SMA Type Missing (N = 0)** |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2022** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |

| Germany & Austria \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 697)** | **SMA Type 1 (N = 134)** | **SMA Type 2 (N = 291)** | **SMA Type 3 (N = 269)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 1)** | **SMA Type Missing (N = 0)** |
| **Calendar year of registry entry; n (%)** | | | | | | | | |
| 2008 | * | * | * | - | - | - | - | - |
| 2009 | 13 (1.9%) | * | * | 5 (1.9%) | - | - | - | - |
| 2010 | 8 (1.1%) | * | * | * | - | - | - | - |
| 2011 | 11 (1.6%) | 6 (4.5%) | * | * | - | - | - | - |
| 2012 | 117 (16.8%) | 14 (10.4%) | 54 (18.6%) | 49 (18.2%) | - | - | - | - |
| 2013 | 83 (11.9%) | 12 (9.0%) | 40 (13.7%) | 31 (11.5%) | - | - | - | - |
| 2014 | 123 (17.6%) | 14 (10.4%) | 51 (17.5%) | 58 (21.6%) | - | - | - | - |
| 2015 | 75 (10.8%) | 15 (11.2%) | 31 (10.7%) | 29 (10.8%) | - | - | - | - |
| 2016 | 51 (7.3%) | 20 (14.9%) | 18 (6.2%) | 13 (4.8%) | - | - | - | - |
| 2017 | 68 (9.8%) | 10 (7.5%) | 25 (8.6%) | 33 (12.3%) | - | - | - | - |
| 2018 | 55 (7.9%) | 10 (7.5%) | 21 (7.2%) | 24 (8.9%) | - | - | - | - |
| 2019 | 36 (5.2%) | 11 (8.2%) | 12 (4.1%) | 12 (4.5%) | * | - | - | - |
| 2020 | 24 (3.4%) | 7 (5.2%) | 14 (4.8%) | * | - | - | - | - |
| 2021 | 13 (1.9%) | * | 7 (2.4%) | * | - | - | - | - |
| 2022 | 12 (1.7%) | * | * | 5 (1.9%) | * | - | * | - |
| 2023 | * | * | - | - | - | - | - | - |
| Missing | * | * | - | - | - | - | - | - |
| **Calendar year of death; n (%)** | | | | | | | | |
| 2009 | - | - | - | - | - | - | - | - |
| 2010 | - | - | - | - | - | - | - | - |
| 2012 | - | - | - | - | - | - | - | - |
| 2013 | - | - | - | - | - | - | - | - |
| 2014 | - | - | - | - | - | - | - | - |
| 2015 | - | - | - | - | - | - | - | - |
| 2016 | - | - | - | - | - | - | - | - |
| 2017 | - | - | - | - | - | - | - | - |
| 2018 | - | - | - | - | - | - | - | - |

| | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| 2019 | - | - | - | - | - | - | - | - |
| 2020 | - | - | - | - | - | - | - | - |
| 2021 | - | - | - | - | - | - | - | - |
| 2022 | - | - | - | - | - | - | - | - |
| 2023 | - | - | - | - | - | - | - | - |
| Missing | 697 (100.0%) | 134 (100.0%) | 291 (100.0%) | 269 (100.0%) | * | - | * | - |
| **Sex; n (%)** | | | | | | | | |
| Female | 341 (48.9%) | 65 (48.5%) | 156 (53.6%) | 119 (44.2%) | * | - | - | - |
| Male | 356 (51.1%) | 69 (51.5%) | 135 (46.4%) | 150 (55.8%) | * | - | * | - |
| Missing | - | - | - | - | - | - | - | - |
| **Class of age at symptom onset; n (%)** | | | | | | | | |
| Presymptomatic | * | - | - | - | - | - | * | - |
| Prenatal | * | * | - | - | - | - | - | - |
| < 1 month | 10 (1.4%) | 6 (4.5%) | * | * | - | - | - | - |
| [1 - 3 months) | 14 (2.0%) | 13 (9.7%) | * | - | - | - | - | - |
| [3 - 6 months) | 24 (3.4%) | 17 (12.7%) | 6 (2.1%) | * | - | - | - | - |
| [6 - 18 months) | 153 (22.0%) | 15 (11.2%) | 119 (40.9%) | 19 (7.1%) | - | - | - | - |
| [1.5 - 2 years) | 18 (2.6%) | - | 5 (1.7%) | 13 (4.8%) | - | - | - | - |
| [2 - 6 years) | 71 (10.2%) | - | 7 (2.4%) | 64 (23.8%) | - | - | - | - |
| [6 - 11 years) | 11 (1.6%) | - | - | 11 (4.1%) | - | - | - | - |
| [11 - 18 years) | 40 (5.7%) | * | - | 39 (14.5%) | - | - | - | - |
| 18 years + | 7 (1.0%) | - | - | 6 (2.2%) | * | - | - | - |
| Missing | 347 (49.8%) | 81 (60.4%) | 152 (52.2%) | 113 (42.0%) | * | - | - | - |
| **Best functional SMA status; n (%)** | | | | | | | | |
| Non-sitter | 30 (4.3%) | 21 (15.7%) | 9 (3.1%) | - | - | - | - | - |
| Sitter | 111 (15.9%) | 16 (11.9%) | 93 (32.0%) | * | - | - | - | - |
| Walker | 210 (30.1%) | 9 (6.7%) | 41 (14.1%) | 158 (58.7%) | * | - | - | - |
| Missing | 346 (49.6%) | 88 (65.7%) | 148 (50.9%) | 109 (40.5%) | - | - | * | - |
| **Best achieved motor milestone; n (%)** | | | | | | | | |
| Climb stairs | 147 (21.1%) | * | * | 139 (51.7%) | * | - | - | - |

| | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| Walk 10 metres without assistance | 24 (3.4%) | * | 6 (2.1%) | 16 (5.9%) | - | - | - | - |
| Walk without assistance | 5 (0.7%) | * | * | * | - | - | - | - |
| Walk with assistance | 34 (4.9%) | * | 29 (10.0%) | * | - | - | - | - |
| Stand without assistance | * | - | * | * | - | - | - | - |
| Stand with assistance | 30 (4.3%) | 8 (6.0%) | 22 (7.6%) | - | - | - | - | - |
| Crawl | 27 (3.9%) | - | 27 (9.3%) | - | - | - | - | - |
| Sit without support | 52 (7.5%) | 8 (6.0%) | 43 (14.8%) | * | - | - | - | - |
| Roll onto side | 18 (2.6%) | 14 (10.4%) | * | - | - | - | - | - |
| Hold head without support | 12 (1.7%) | 7 (5.2%) | 5 (1.7%) | - | - | - | - | - |
| Unknown | - | - | - | - | - | - | - | - |
| Missing | 346 (49.6%) | 88 (65.7%) | 148 (50.9%) | 109 (40.5%) | - | - | * | - |
| **SMN1 gene mutation type; n (%)** | | | | | | | | |
| Compound heterozygous deletion exon 7 | 22 (3.2%) | * | 8 (2.7%) | 12 (4.5%) | - | - | - | - |
| Compound heterozygous substitutions | - | - | - | - | - | - | - | - |
| Homozygous deletion exon 7 | 672 (96.4%) | 131 (97.8%) | 282 (96.9%) | 256 (95.2%) | * | - | * | - |
| Missing | * | * | * | * | - | - | - | - |
| **Number of SMN2 copies; n (%)** | | | | | | | | |
| 0 | 13 (1.9%) | * | 7 (2.4%) | 5 (1.9%) | - | - | - | - |
| 1 | * | * | * | - | - | - | - | - |
| 2 | 94 (13.5%) | 65 (48.5%) | 15 (5.2%) | 14 (5.2%) | - | - | - | - |
| 3 | 214 (30.7%) | 33 (24.6%) | 124 (42.6%) | 57 (21.2%) | - | - | - | - |
| 4 | 99 (14.2%) | - | 19 (6.5%) | 77 (28.6%) | * | - | * | - |
| >4 | 10 (1.4%) | * | * | 7 (2.6%) | - | - | - | - |
| Other | * | - | - | * | - | - | - | - |
| Missing | 263 (37.7%) | 32 (23.9%) | 123 (42.3%) | 108 (40.1%) | - | - | - | - |

| | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| **Methods used for genetic testing; n (%)** | | | | | | | | |
| DNA Sequencing | - | - | - | - | - | - | - | - |
| HRM | - | - | - | - | - | - | - | - |
| MLPA | 82 (11.8%) | 9 (6.7%) | 33 (11.3%) | 39 (14.5%) | * | - | - | - |
| RFLP | - | - | - | - | - | - | - | - |
| ddPCR | - | - | - | - | - | - | - | - |
| qRT-PCR | 6 (0.9%) | * | * | * | - | - | - | - |
| Missing | 609 (87.4%) | 123 (91.8%) | 256 (88.0%) | 228 (84.8%) | * | - | * | - |
| **Duration of follow up (months)** | | | | | | | | |
| Mean (SD) | 86.9 (35.4) | 71 (33.9) | 90.3 (35) | 91.8 (33.8) | * | - | * | - |
| Median [IQR] | 92 [61, 115] | 70 [44, 93] | 96 [65, 118] | 100 [67, 117] | * | - | * | - |
| Missing; n (%) | * | * | - | - | - | - | - | - |
| **Duration of SMA (months)** | | | | | | | | |
| Mean (SD) | 300.7 (215.2) | 112.7 (84.6) | 251.3 (165.2) | 409.6 (225) | * | - | - | - |
| Median [IQR] | 237 [113, 456] | 82 [62, 119] | 210 [112.5, 358] | 416.5 [198.8, 615.5] | * | - | - | - |
| Missing; n (%) | 348 (49.9%) | 81 (60.4%) | 152 (52.2%) | 113 (42.0%) | * | - | * | - |
| **Duration between two consecutive visits (months)** | | | | | | | | |
| Mean (SD) | 25.2 (19.8) | 24.9 (20.7) | 26.9 (20.9) | 23.6 (17.8) | * | - | * | - |
| Median [IQR] | 18.2 [13, 29.8] | 18 [11.1, 32.5] | 19.3 [13.7, 32] | 17.8 [13.2, 26.5] | * | - | * | - |
| Missing; n (%) | 37 (5.3%) | 10 (7.5%) | 14 (4.8%) | 13 (4.8%) | - | - | - | - |
| **Duration between genetic report date and registry entry (months)** | | | | | | | | |
| Mean (SD) | 55.1 (65.4) | 18.7 (43.7) | 59 (67.2) | 65.9 (65.7) | * | - | * | - |
| Median [IQR] | 30 [3, 90] | 2 [1, 11] | 33 [2, 101.5] | 44.5 [10.8, 101.8] | * | - | * | - |
| Missing; n (%) | 160 (23.0%) | 48 (35.8%) | 63 (21.6%) | 49 (18.2%) | - | - | - | - |
| **Reason for genetic testing; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |

| | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 20 (9.6%) | * | 7 (7.2%) | 12 (12.0%) | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | * | * | - | * | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | * | * | * | - | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| Family screening | * | - | - | * | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 9 (17.0%) | * | * | * | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 10 (26.3%) | - | * | 6 (42.9%) | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 7 (33.3%) | - | * | * | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |

| | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| **2020** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | * | * | - | - | - | - | - | - |
| No screening | 9 (52.9%) | * | * | * | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 18 (94.7%) | * | 7 (100.0%) | 8 (88.9%) | * | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2022** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | * | * | - | - | - | - | * | - |
| No screening | 6 (75.0%) | * | * | * | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | - | - | - | - | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **Age at onset of SMA symptoms (years)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | 4.2 (5.5) | 1.8 (4) | 0.9 (0.5) | 6.6 (6.2) | - | - | - | - |
| Median [IQR] | 1.5 [0.9, 4] | 0.5 [0.3, 0.5] | 0.9 [0.5, 1] | 3 [2, 12.8] | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | 4.5 (10.9) | 0.4 (0.2) | 0.8 (0.3) | 9.5 (14.5) | * | - | - | - |
| Median [IQR] | 0.9 [0.5, 1.3] | 0.5 [0.4, 0.5] | 0.8 [0.5, 1] | 2 [1.2, 12] | * | - | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | 1.2 (1.7) | 0.3 (0.2) | 0.9 (0.2) | 3.3 (2.6) | - | - | - | - |

| | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| Median [IQR] | 0.8 [0.5, 1] | 0.2 [0.1, 0.4] | 1 [0.8, 1] | 2.8 [2, 3] | - | - | - | - |
| **2017** | | | | | | | | |
| Mean (SD) | 0.8 (0.8) | 0.2 (0.2) | 1 (0.5) | * | - | - | - | - |
| Median [IQR] | 0.7 [0.2, 1] | 0.2 [0.1, 0.2] | 0.8 [0.7, 1] | * | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | 0.4 (0.4) | 0.2 (0.1) | * | - | - | - | - | - |
| Median [IQR] | 0.2 [0.1, 0.7] | 0.2 [0.1, 0.2] | * | - | - | - | - | - |
| **2019** | | | | | | | | |
| Mean (SD) | 0.6 (0.5) | * | * | * | - | - | - | - |
| Median [IQR] | 0.3 [0.2, 1] | * | * | * | - | - | - | - |
| **2020** | | | | | | | | |
| Mean (SD) | 1.2 (0.7) | * | * | * | - | - | - | - |
| Median [IQR] | 1.2 [0.7, 1.8] | * | * | * | - | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | * | - | - | * | - | - | - | - |
| Median [IQR] | * | - | - | * | - | - | - | - |
| **2022** | | | | | | | | |
| Mean (SD) | * | * | - | - | - | - | - | - |
| Median [IQR] | * | * | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **Age at genetic report date (years)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | 15.3 (16.1) | 1.7 (2.9) | 8.5 (12.1) | 23.3 (16.3) | - | - | - | - |
| Median [IQR] | 8.1 [1.6, 28.2] | 0.5 [0.3, 1.2] | 1.8 [1.2, 11.8] | 23.1 [7.7, 36.2] | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | 14.3 (17.8) | 0.6 (0.3) | 7.1 (10.4) | 26.2 (19.5) | - | - | - | - |
| Median [IQR] | 2.4 [1.2, 26.1] | 0.5 [0.4, 0.8] | 1.7 [1.2, 3.3] | 25 [4.7, 42.1] | - | - | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | 9.5 (15.6) | 2.2 (8.6) | 4.9 (8.5) | 24.8 (19.2) | - | - | - | - |
| Median [IQR] | 1.6 [0.9, 10] | 0.6 [0.2, 0.8] | 1.6 [1.2, 2] | 23 [5.5, 39.2] | - | - | - | - |
| **2017** | | | | | | | | |

| | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| Mean (SD) | 24 (19.1) | 5.9 (10.4) | 21.6 (14.1) | 32.3 (19.8) | - | - | - | - |
| Median [IQR] | 24.9 [1.7, 37.2] | 1 [0.5, 1.1] | 24.9 [9.2, 29.5] | 35.2 [15.8, 47.2] | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | 16 (18.9) | 0.5 (0.8) | 16.8 (17.5) | 29.7 (17.7) | - | - | - | - |
| Median [IQR] | 2.9 [0.5, 32.5] | 0.2 [0.2, 0.6] | 2.7 [1.5, 31] | 33.5 [15.6, 43.4] | - | - | - | - |
| **2019** | | | | | | | | |
| Mean (SD) | 23 (20.7) | 1.4 (2.2) | 19.7 (16.6) | 41.5 (13) | * | - | - | - |
| Median [IQR] | 25.7 [1.3, 43.7] | 0.5 [0.2, 0.7] | 25.7 [1.7, 31.9] | 44.8 [33.8, 52.4] | * | - | - | - |
| **2020** | | | | | | | | |
| Mean (SD) | 23.9 (19.1) | * | 22.4 (14.5) | * | - | - | - | - |
| Median [IQR] | 23.2 [1.4, 41.4] | * | 23.2 [15.9, 30.2] | * | - | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | 34.9 (19) | * | 29.5 (13.9) | 39 (21.4) | * | - | - | - |
| Median [IQR] | 39.6 [22.5, 45.7] | * | 35.5 [22.5, 39.7] | 41.5 [38.9, 56.3] | * | - | - | - |
| **2022** | | | | | | | | |
| Mean (SD) | 26 (22.8) | * | * | * | - | - | * | - |
| Median [IQR] | 29.3 [0.1, 38.5] | * | * | * | - | - | * | - |
| **2023** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **Age at registry entry (years)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | 10.4 (12.4) | 1.2 (1.8) | 15.6 (14.8) | 13.5 (9.5) | - | - | - | - |
| Median [IQR] | 5.9 [1.3, 13.7] | 0.5 [0.2, 1.3] | 13.2 [4.1, 17.4] | 11.1 [7.3, 15] | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | 19.5 (17.3) | 3 (5.4) | 15.1 (13.1) | 31.3 (17.1) | - | - | - | - |
| Median [IQR] | 14.8 [3.6, 33.1] | 0.9 [0.6, 3] | 10.6 [4.3, 23.7] | 31.5 [18.2, 45.3] | - | - | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | 17.1 (18.4) | 3 (7.8) | 10.5 (11.5) | 30.6 (18.9) | - | - | - | - |
| Median [IQR] | 9 [1.8, 28.2] | 0.8 [0.3, 1.2] | 6.1 [1.8, 14.1] | 31.3 [14, 47.4] | - | - | - | - |
| **2017** | | | | | | | | |
| Mean (SD) | 25.7 (18.8) | 3.5 (5.6) | 21.9 (15.1) | 35.2 (17.3) | - | - | - | - |
| Median [IQR] | 25.5 [4.7, 39.7] | 0.9 [0.6, 1.3] | 24.8 [3, 30.3] | 36.5 [18.6, 47.2] | - | - | - | - |

| | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| **2018** | | | | | | | | |
| Mean (SD) | 20.3 (20.4) | 0.8 (0.9) | 16.6 (15.1) | 31.6 (21.4) | - | - | - | - |
| Median [IQR] | 12.9 [1.6, 37.8] | 0.4 [0.2, 0.7] | 12.9 [1.8, 29.2] | 37.8 [6, 51.3] | - | - | - | - |
| **2019** | | | | | | | | |
| Mean (SD) | 19.1 (19.9) | 1.4 (1.7) | 14.4 (15.9) | 37.6 (14.8) | * | - | - | - |
| Median [IQR] | 8.4 [1.3, 41.2] | 1 [0.6, 1.3] | 5.8 [1.6, 24] | 43.2 [26.2, 49.7] | * | - | - | - |
| **2020** | | | | | | | | |
| Mean (SD) | 15.4 (15.8) | 7.6 (10.4) | 15 (11.5) | * | - | - | - | - |
| Median [IQR] | 9.2 [1.6, 25.4] | 1.4 [0.6, 12.8] | 13.1 [4.7, 25.8] | * | - | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | 24.8 (17.1) | * | 28.4 (12.3) | * | - | - | - | - |
| Median [IQR] | 30.8 [2.3, 34.8] | * | 32.8 [25.6, 36.6] | * | - | - | - | - |
| **2022** | | | | | | | | |
| Mean (SD) | 30.6 (23.6) | * | * | 42.2 (21.8) | * | - | * | - |
| Median [IQR] | 25.8 [9.9, 54.6] | * | * | 52.9 [21.1, 59.6] | * | - | * | - |
| **2023** | | | | | | | | |
| Mean (SD) | * | * | - | - | - | - | - | - |
| Median [IQR] | * | * | - | - | - | - | - | - |
| **Age at death (years)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |

| | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2022** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **Lost to follow-up; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 25 (100.0%) | 8 (100.0%) | 10 (100.0%) | 7 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 236 (100.0%) | 40 (100.0%) | 108 (100.0%) | 88 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 445 (91.8%) | 72 (80.9%) | 197 (94.7%) | 176 (93.6%) | - | - | - | - |
| Yes | 40 (8.2%) | 17 (19.1%) | 11 (5.3%) | 12 (6.4%) | - | - | - | - |
| **2017** | | | | | | | | |
| No | 497 (96.9%) | 74 (90.2%) | 217 (97.7%) | 206 (98.6%) | - | - | - | - |
| Yes | 16 (3.1%) | 8 (9.8%) | 5 (2.3%) | * | - | - | - | - |
| **2018** | | | | | | | | |
| No | 550 (99.6%) | 83 (98.8%) | 237 (99.6%) | 230 (100.0%) | - | - | - | - |
| Yes | * | * | * | - | - | - | - | - |
| **2019** | | | | | | | | |

| | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| No | 581 (99.1%) | 93 (98.9%) | 246 (98.8%) | 241 (99.6%) | * | - | - | - |
| Yes | 5 (0.9%) | * | * | * | - | - | - | - |
| **2020** | | | | | | | | |
| No | 594 (98.2%) | 95 (95.0%) | 256 (98.5%) | 242 (99.2%) | * | - | - | - |
| Yes | 11 (1.8%) | 5 (5.0%) | * | * | - | - | - | - |
| **2021** | | | | | | | | |
| No | 601 (99.0%) | 93 (95.9%) | 262 (99.6%) | 245 (99.6%) | * | - | - | - |
| Yes | 6 (1.0%) | * | * | * | - | - | - | - |
| **2022** | | | | | | | | |
| No | 356 (58.1%) | 50 (53.2%) | 145 (54.5%) | 158 (63.2%) | * | - | * | - |
| Yes | 257 (41.9%) | 44 (46.8%) | 121 (45.5%) | 92 (36.8%) | - | - | - | - |
| **2023** | | | | | | | | |
| No | 293 (81.6%) | 47 (88.7%) | 116 (80.0%) | 127 (80.4%) | * | - | * | - |
| Yes | 66 (18.4%) | 6 (11.3%) | 29 (20.0%) | 31 (19.6%) | - | - | - | - |
| **Treated with at least one DMT; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 25 (100.0%) | 8 (100.0%) | 10 (100.0%) | 7 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 236 (100.0%) | 40 (100.0%) | 108 (100.0%) | 88 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 442 (96.9%) | 67 (89.3%) | 193 (97.5%) | 182 (99.5%) | - | - | - | - |
| Yes | 14 (3.1%) | 8 (10.7%) | 5 (2.5%) | * | - | - | - | - |
| **2017** | | | | | | | | |
| No | 422 (85.3%) | 51 (69.9%) | 186 (86.1%) | 185 (89.8%) | - | - | - | - |
| Yes | 73 (14.7%) | 22 (30.1%) | 30 (13.9%) | 21 (10.2%) | - | - | - | - |
| **2018** | | | | | | | | |
| No | 400 (73.4%) | 54 (65.9%) | 183 (78.2%) | 163 (71.2%) | - | - | - | - |
| Yes | 145 (26.6%) | 28 (34.1%) | 51 (21.8%) | 66 (28.8%) | - | - | - | - |
| **2019** | | | | | | | | |
| No | 381 (66.8%) | 54 (61.4%) | 174 (71.9%) | 152 (63.6%) | * | - | - | - |

| | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| Yes | 189 (33.2%) | 34 (38.6%) | 68 (28.1%) | 87 (36.4%) | - | - | - | - |
| **2020** | | | | | | | | |
| No | 372 (63.3%) | 47 (51.6%) | 172 (67.5%) | 153 (63.5%) | - | - | - | - |
| Yes | 216 (36.7%) | 44 (48.4%) | 83 (32.5%) | 88 (36.5%) | * | - | - | - |
| **2021** | | | | | | | | |
| No | 127 (36.9%) | 12 (24.5%) | 51 (36.2%) | 64 (41.8%) | - | - | - | - |
| Yes | 217 (63.1%) | 37 (75.5%) | 90 (63.8%) | 89 (58.2%) | * | - | - | - |
| **2022** | | | | | | | | |
| No | 127 (44.1%) | 14 (31.8%) | 53 (46.1%) | 58 (46.0%) | * | - | * | - |
| Yes | 161 (55.9%) | 30 (68.2%) | 62 (53.9%) | 68 (54.0%) | * | - | - | - |
| **2023** | | | | | | | | |
| No | 28 (36.4%) | 5 (33.3%) | 6 (33.3%) | 16 (37.2%) | * | - | - | - |
| Yes | 49 (63.6%) | 10 (66.7%) | 12 (66.7%) | 27 (62.8%) | - | - | - | - |
| **Treated with more than one DMT; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 25 (100.0%) | 8 (100.0%) | 10 (100.0%) | 7 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 236 (100.0%) | 40 (100.0%) | 108 (100.0%) | 88 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 456 (100.0%) | 75 (100.0%) | 198 (100.0%) | 183 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 495 (100.0%) | 73 (100.0%) | 216 (100.0%) | 206 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 545 (100.0%) | 82 (100.0%) | 234 (100.0%) | 229 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 570 (100.0%) | 88 (100.0%) | 242 (100.0%) | 239 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |

| | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| **2020** | | | | | | | | |
| No | 583 (99.1%) | 87 (95.6%) | 254 (99.6%) | 241 (100.0%) | * | - | - | - |
| Yes | 5 (0.9%) | * | * | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 331 (96.2%) | 46 (93.9%) | 135 (95.7%) | 149 (97.4%) | * | - | - | - |
| Yes | 13 (3.8%) | * | 6 (4.3%) | * | - | - | - | - |
| **2022** | | | | | | | | |
| No | 281 (97.6%) | 41 (93.2%) | 112 (97.4%) | 125 (99.2%) | * | - | * | - |
| Yes | 7 (2.4%) | * | * | * | - | - | - | - |
| **2023** | | | | | | | | |
| No | 77 (100.0%) | 15 (100.0%) | 18 (100.0%) | 43 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **Treated with nusinersen; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 25 (100.0%) | 8 (100.0%) | 10 (100.0%) | 7 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 236 (100.0%) | 40 (100.0%) | 108 (100.0%) | 88 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 442 (96.9%) | 67 (89.3%) | 193 (97.5%) | 182 (99.5%) | - | - | - | - |
| Yes | 14 (3.1%) | 8 (10.7%) | 5 (2.5%) | * | - | - | - | - |
| **2017** | | | | | | | | |
| No | 424 (85.7%) | 51 (69.9%) | 187 (86.6%) | 186 (90.3%) | - | - | - | - |
| Yes | 71 (14.3%) | 22 (30.1%) | 29 (13.4%) | 20 (9.7%) | - | - | - | - |
| **2018** | | | | | | | | |
| No | 403 (73.9%) | 54 (65.9%) | 185 (79.1%) | 164 (71.6%) | - | - | - | - |
| Yes | 142 (26.1%) | 28 (34.1%) | 49 (20.9%) | 65 (28.4%) | - | - | - | - |
| **2019** | | | | | | | | |
| No | 385 (67.5%) | 54 (61.4%) | 177 (73.1%) | 153 (64.0%) | * | - | - | - |
| Yes | 185 (32.5%) | 34 (38.6%) | 65 (26.9%) | 86 (36.0%) | - | - | - | - |
| **2020** | | | | | | | | |
| No | 391 (66.5%) | 55 (60.4%) | 182 (71.4%) | 154 (63.9%) | - | - | - | - |

| | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| Yes | 197 (33.5%) | 36 (39.6%) | 73 (28.6%) | 87 (36.1%) | * | - | - | - |
| **2021** | | | | | | | | |
| No | 193 (56.1%) | 27 (55.1%) | 87 (61.7%) | 79 (51.6%) | - | - | - | - |
| Yes | 151 (43.9%) | 22 (44.9%) | 54 (38.3%) | 74 (48.4%) | * | - | - | - |
| **2022** | | | | | | | | |
| No | 207 (71.9%) | 32 (72.7%) | 91 (79.1%) | 82 (65.1%) | * | - | * | - |
| Yes | 81 (28.1%) | 12 (27.3%) | 24 (20.9%) | 44 (34.9%) | * | - | - | - |
| **2023** | | | | | | | | |
| No | 57 (74.0%) | 12 (80.0%) | 16 (88.9%) | 28 (65.1%) | * | - | - | - |
| Yes | 20 (26.0%) | * | * | 15 (34.9%) | - | - | - | - |
| **Treated with onasemnogene abeparvovec-xioi; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 25 (100.0%) | 8 (100.0%) | 10 (100.0%) | 7 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 236 (100.0%) | 40 (100.0%) | 108 (100.0%) | 88 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 456 (100.0%) | 75 (100.0%) | 198 (100.0%) | 183 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 495 (100.0%) | 73 (100.0%) | 216 (100.0%) | 206 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 545 (100.0%) | 82 (100.0%) | 234 (100.0%) | 229 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 570 (100.0%) | 88 (100.0%) | 242 (100.0%) | 239 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 578 (98.3%) | 83 (91.2%) | 253 (99.2%) | 241 (100.0%) | * | - | - | - |

| | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| Yes | 10 (1.7%) | 8 (8.8%) | * | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 330 (95.9%) | 40 (81.6%) | 136 (96.5%) | 153 (100.0%) | * | - | - | - |
| Yes | 14 (4.1%) | 9 (18.4%) | 5 (3.5%) | - | - | - | - | - |
| **2022** | | | | | | | | |
| No | 276 (95.8%) | 35 (79.5%) | 112 (97.4%) | 126 (100.0%) | * | - | * | - |
| Yes | 12 (4.2%) | 9 (20.5%) | * | - | - | - | - | - |
| **2023** | | | | | | | | |
| No | 72 (93.5%) | 11 (73.3%) | 17 (94.4%) | 43 (100.0%) | * | - | - | - |
| Yes | 5 (6.5%) | * | * | - | - | - | - | - |
| **Treated with risdiplan; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | | | | | | | | |
| **2011-2013** | | | | | | | | |
| No | 25 (100.0%) | 8 (100.0%) | 10 (100.0%) | 7 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 236 (100.0%) | 40 (100.0%) | 108 (100.0%) | 88 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 456 (100.0%) | 75 (100.0%) | 198 (100.0%) | 183 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 493 (99.6%) | 73 (100.0%) | 215 (99.5%) | 205 (99.5%) | - | - | - | - |
| Yes | * | - | * | * | - | - | - | - |
| **2019** | | | | | | | | |
| No | 542 (99.4%) | 82 (100.0%) | 232 (99.1%) | 228 (99.6%) | - | - | - | - |
| Yes | * | - | * | * | - | - | - | - |
| **2020** | | | | | | | | |
| No | 566 (99.3%) | 88 (100.0%) | 239 (98.8%) | 238 (99.6%) | * | - | - | - |
| Yes | * | - | * | * | - | - | - | - |
| **2021** | | | | | | | | |

| | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| No | 574 (97.6%) | 87 (95.6%) | 246 (96.5%) | 240 (99.6%) | * | - | - | - |
| Yes | 14 (2.4%) | * | 9 (3.5%) | * | - | - | - | - |
| **2022** | | | | | | | | |
| No | 279 (81.1%) | 40 (81.6%) | 104 (73.8%) | 134 (87.6%) | * | - | - | - |
| Yes | 65 (18.9%) | 9 (18.4%) | 37 (26.2%) | 19 (12.4%) | - | - | - | - |
| **2023** | | | | | | | | |
| No | 213 (74.0%) | 32 (72.7%) | 77 (67.0%) | 101 (80.2%) | * | - | * | - |
| Yes | 75 (26.0%) | 12 (27.3%) | 38 (33.0%) | 25 (19.8%) | - | - | - | - |
| **At least one episode of invasive ventilation; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 25 (100.0%) | 8 (100.0%) | 10 (100.0%) | 7 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 234 (99.2%) | 38 (95.0%) | 108 (100.0%) | 88 (100.0%) | - | - | - | - |
| Yes | * | * | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 452 (99.1%) | 71 (94.7%) | 198 (100.0%) | 183 (100.0%) | - | - | - | - |
| Yes | * | * | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 484 (97.8%) | 65 (89.0%) | 213 (98.6%) | 206 (100.0%) | - | - | - | - |
| Yes | 11 (2.2%) | 8 (11.0%) | * | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 534 (98.0%) | 75 (91.5%) | 230 (98.3%) | 229 (100.0%) | - | - | - | - |
| Yes | 11 (2.0%) | 7 (8.5%) | * | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 558 (97.9%) | 81 (92.0%) | 238 (98.3%) | 238 (99.6%) | * | - | - | - |
| Yes | 12 (2.1%) | 7 (8.0%) | * | * | - | - | - | - |
| **2020** | | | | | | | | |
| No | 568 (96.6%) | 77 (84.6%) | 250 (98.0%) | 240 (99.6%) | * | - | - | - |
| Yes | 20 (3.4%) | 14 (15.4%) | 5 (2.0%) | * | - | - | - | - |
| **2021** | | | | | | | | |
| No | 336 (97.7%) | 44 (89.8%) | 139 (98.6%) | 152 (99.3%) | * | - | - | - |

| | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| Yes | 8 (2.3%) | 5 (10.2%) | * | * | - | - | - | - |
| **2022** | | | | | | | | |
| No | 282 (97.9%) | 41 (93.2%) | 113 (98.3%) | 125 (99.2%) | * | - | * | - |
| Yes | 6 (2.1%) | * | * | * | - | - | - | - |
| **2023** | | | | | | | | |
| No | 77 (100.0%) | 15 (100.0%) | 18 (100.0%) | 43 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **At least one episode of non-invasive ventilation; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 25 (100.0%) | 8 (100.0%) | 10 (100.0%) | 7 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 231 (97.9%) | 39 (97.5%) | 104 (96.3%) | 88 (100.0%) | - | - | - | - |
| Yes | 5 (2.1%) | * | * | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 432 (94.7%) | 63 (84.0%) | 187 (94.4%) | 182 (99.5%) | - | - | - | - |
| Yes | 24 (5.3%) | 12 (16.0%) | 11 (5.6%) | * | - | - | - | - |
| **2017** | | | | | | | | |
| No | 467 (94.3%) | 60 (82.2%) | 202 (93.5%) | 205 (99.5%) | - | - | - | - |
| Yes | 28 (5.7%) | 13 (17.8%) | 14 (6.5%) | * | - | - | - | - |
| **2018** | | | | | | | | |
| No | 506 (92.8%) | 67 (81.7%) | 211 (90.2%) | 228 (99.6%) | - | - | - | - |
| Yes | 39 (7.2%) | 15 (18.3%) | 23 (9.8%) | * | - | - | - | - |
| **2019** | | | | | | | | |
| No | 526 (92.3%) | 70 (79.5%) | 219 (90.5%) | 236 (98.7%) | * | - | - | - |
| Yes | 44 (7.7%) | 18 (20.5%) | 23 (9.5%) | * | - | - | - | - |
| **2020** | | | | | | | | |
| No | 464 (78.9%) | 53 (58.2%) | 178 (69.8%) | 232 (96.3%) | * | - | - | - |
| Yes | 124 (21.1%) | 38 (41.8%) | 77 (30.2%) | 9 (3.7%) | - | - | - | - |
| **2021** | | | | | | | | |
| No | 291 (84.6%) | 28 (57.1%) | 114 (80.9%) | 148 (96.7%) | * | - | - | - |

| | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| Yes | 53 (15.4%) | 21 (42.9%) | 27 (19.1%) | 5 (3.3%) | - | - | - | - |
| **2022** | | | | | | | | |
| No | 243 (84.4%) | 28 (63.6%) | 89 (77.4%) | 123 (97.6%) | * | - | * | - |
| Yes | 45 (15.6%) | 16 (36.4%) | 26 (22.6%) | * | - | - | - | - |
| **2023** | | | | | | | | |
| No | 68 (88.3%) | 10 (66.7%) | 15 (83.3%) | 42 (97.7%) | * | - | - | - |
| Yes | 9 (11.7%) | 5 (33.3%) | * | * | - | - | - | - |
| **At least one episode of feeding tube usage; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 25 (100.0%) | 8 (100.0%) | 10 (100.0%) | 7 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 216 (91.5%) | 28 (70.0%) | 100 (92.6%) | 88 (100.0%) | - | - | - | - |
| Yes | 20 (8.5%) | 12 (30.0%) | 8 (7.4%) | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 417 (91.4%) | 50 (66.7%) | 185 (93.4%) | 182 (99.5%) | - | - | - | - |
| Yes | 39 (8.6%) | 25 (33.3%) | 13 (6.6%) | * | - | - | - | - |
| **2017** | | | | | | | | |
| No | 470 (94.9%) | 58 (79.5%) | 206 (95.4%) | 206 (100.0%) | - | - | - | - |
| Yes | 25 (5.1%) | 15 (20.5%) | 10 (4.6%) | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 499 (91.6%) | 53 (64.6%) | 218 (93.2%) | 228 (99.6%) | - | - | - | - |
| Yes | 46 (8.4%) | 29 (35.4%) | 16 (6.8%) | * | - | - | - | - |
| **2019** | | | | | | | | |
| No | 529 (92.8%) | 58 (65.9%) | 231 (95.5%) | 239 (100.0%) | * | - | - | - |
| Yes | 41 (7.2%) | 30 (34.1%) | 11 (4.5%) | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 546 (92.9%) | 62 (68.1%) | 242 (94.9%) | 241 (100.0%) | * | - | - | - |
| Yes | 42 (7.1%) | 29 (31.9%) | 13 (5.1%) | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 309 (89.8%) | 27 (55.1%) | 128 (90.8%) | 153 (100.0%) | * | - | - | - |
| Yes | 35 (10.2%) | 22 (44.9%) | 13 (9.2%) | - | - | - | - | - |

| | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| **2022** | | | | | | | | |
| No | 261 (90.6%) | 27 (61.4%) | 105 (91.3%) | 126 (100.0%) | * | - | * | - |
| Yes | 27 (9.4%) | 17 (38.6%) | 10 (8.7%) | - | - | - | - | - |
| **2023** | | | | | | | | |
| No | 72 (93.5%) | 11 (73.3%) | 17 (94.4%) | 43 (100.0%) | * | - | - | - |
| Yes | 5 (6.5%) | * | * | - | - | - | - | - |
| **At least one episode of wheelchair usage; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 11 (44.0%) | 7 (87.5%) | * | * | - | - | - | - |
| Yes | 14 (56.0%) | * | 9 (90.0%) | * | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 99 (41.9%) | 34 (85.0%) | 21 (19.4%) | 44 (50.0%) | - | - | - | - |
| Yes | 137 (58.1%) | 6 (15.0%) | 87 (80.6%) | 44 (50.0%) | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 164 (36.0%) | 55 (73.3%) | 35 (17.7%) | 74 (40.4%) | - | - | - | - |
| Yes | 292 (64.0%) | 20 (26.7%) | 163 (82.3%) | 109 (59.6%) | - | - | - | - |
| **2017** | | | | | | | | |
| No | 178 (36.0%) | 47 (64.4%) | 43 (19.9%) | 88 (42.7%) | - | - | - | - |
| Yes | 317 (64.0%) | 26 (35.6%) | 173 (80.1%) | 118 (57.3%) | - | - | - | - |
| **2018** | | | | | | | | |
| No | 206 (37.8%) | 51 (62.2%) | 55 (23.5%) | 100 (43.7%) | - | - | - | - |
| Yes | 339 (62.2%) | 31 (37.8%) | 179 (76.5%) | 129 (56.3%) | - | - | - | - |
| **2019** | | | | | | | | |
| No | 265 (46.5%) | 54 (61.4%) | 89 (36.8%) | 121 (50.6%) | * | - | - | - |
| Yes | 305 (53.5%) | 34 (38.6%) | 153 (63.2%) | 118 (49.4%) | - | - | - | - |
| **2020** | | | | | | | | |
| No | 297 (50.5%) | 57 (62.6%) | 115 (45.1%) | 124 (51.5%) | * | - | - | - |
| Yes | 291 (49.5%) | 34 (37.4%) | 140 (54.9%) | 117 (48.5%) | - | - | - | - |
| **2021** | | | | | | | | |
| No | 105 (30.5%) | 13 (26.5%) | 29 (20.6%) | 62 (40.5%) | * | - | - | - |
| Yes | 239 (69.5%) | 36 (73.5%) | 112 (79.4%) | 91 (59.5%) | - | - | - | - |

| | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| **2022** | | | | | | | | |
| No | 104 (36.1%) | 15 (34.1%) | 34 (29.6%) | 52 (41.3%) | * | - | * | - |
| Yes | 184 (63.9%) | 29 (65.9%) | 81 (70.4%) | 74 (58.7%) | - | - | - | - |
| **2023** | | | | | | | | |
| No | 29 (37.7%) | 9 (60.0%) | 5 (27.8%) | 14 (32.6%) | * | - | - | - |
| Yes | 48 (62.3%) | 6 (40.0%) | 13 (72.2%) | 29 (67.4%) | - | - | - | - |
| **At least one reported measure by available motor function scale or test and by at least one PRO; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 25 (100.0%) | 8 (100.0%) | 10 (100.0%) | 7 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 236 (100.0%) | 40 (100.0%) | 108 (100.0%) | 88 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 456 (100.0%) | 75 (100.0%) | 198 (100.0%) | 183 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 495 (100.0%) | 73 (100.0%) | 216 (100.0%) | 206 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 545 (100.0%) | 82 (100.0%) | 234 (100.0%) | 229 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 570 (100.0%) | 88 (100.0%) | 242 (100.0%) | 239 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 588 (100.0%) | 91 (100.0%) | 255 (100.0%) | 241 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 344 (100.0%) | 49 (100.0%) | 141 (100.0%) | 153 (100.0%) | * | - | - | - |

| | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| Yes | - | - | - | - | - | - | - | - |
| **2022** | | | | | | | | |
| No | 288 (100.0%) | 44 (100.0%) | 115 (100.0%) | 126 (100.0%) | * | - | * | - |
| Yes | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| No | 77 (100.0%) | 15 (100.0%) | 18 (100.0%) | 43 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **At least three reported measures by available motor function scale or test and by at least one PRO; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 25 (100.0%) | 8 (100.0%) | 10 (100.0%) | 7 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 236 (100.0%) | 40 (100.0%) | 108 (100.0%) | 88 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 456 (100.0%) | 75 (100.0%) | 198 (100.0%) | 183 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 495 (100.0%) | 73 (100.0%) | 216 (100.0%) | 206 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 545 (100.0%) | 82 (100.0%) | 234 (100.0%) | 229 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 570 (100.0%) | 88 (100.0%) | 242 (100.0%) | 239 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 588 (100.0%) | 91 (100.0%) | 255 (100.0%) | 241 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |

| | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| No | 344 (100.0%) | 49 (100.0%) | 141 (100.0%) | 153 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2022** | | | | | | | | |
| No | 288 (100.0%) | 44 (100.0%) | 115 (100.0%) | 126 (100.0%) | * | - | * | - |
| Yes | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| No | 77 (100.0%) | 15 (100.0%) | 18 (100.0%) | 43 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **Available number of records of each motor function scale by patient** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |

| | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2022** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **Available number of records of PRO** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |

| | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2022** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |

| Spain \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 319)** | **SMA Type 1 (N = 71)** | **SMA Type 2 (N = 153)** | **SMA Type 3 (N = 93)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 0)** |
| **Calendar year of registry entry; n (%)** | | | | | | | | |
| 2008 | - | - | - | - | - | - | - | - |
| 2009 | - | - | - | - | - | - | - | - |
| 2010 | - | - | - | - | - | - | - | - |
| 2011 | - | - | - | - | - | - | - | - |
| 2012 | - | - | - | - | - | - | - | - |
| 2013 | - | - | - | - | - | - | - | - |
| 2014 | - | - | - | - | - | - | - | - |
| 2015 | 7 (2.2%) | * | * | * | - | - | - | - |
| 2016 | 25 (7.8%) | 6 (8.5%) | 15 (9.8%) | * | - | - | - | - |
| 2017 | 80 (25.1%) | 12 (16.9%) | 41 (26.8%) | 26 (28.0%) | * | - | - | - |
| 2018 | 85 (26.6%) | 14 (19.7%) | 46 (30.1%) | 25 (26.9%) | - | - | - | - |
| 2019 | 44 (13.8%) | 13 (18.3%) | 16 (10.5%) | 15 (16.1%) | - | - | - | - |
| 2020 | 33 (10.3%) | 8 (11.3%) | 18 (11.8%) | 7 (7.5%) | - | - | - | - |
| 2021 | 20 (6.3%) | 9 (12.7%) | 5 (3.3%) | 6 (6.5%) | - | - | - | - |
| 2022 | 21 (6.6%) | 8 (11.3%) | 8 (5.2%) | 5 (5.4%) | - | - | - | - |
| 2023 | * | - | * | * | * | - | - | - |
| Missing | - | - | - | - | - | - | - | - |
| **Calendar year of death; n (%)** | | | | | | | | |
| 2009 | - | - | - | - | - | - | - | - |
| 2010 | - | - | - | - | - | - | - | - |
| 2012 | - | - | - | - | - | - | - | - |
| 2013 | - | - | - | - | - | - | - | - |
| 2014 | - | - | - | - | - | - | - | - |
| 2015 | - | - | - | - | - | - | - | - |
| 2016 | - | - | - | - | - | - | - | - |
| 2017 | * | - | - | * | - | - | - | - |
| 2018 | - | - | - | - | - | - | - | - |

| Spain \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 319)** | **SMA Type 1 (N = 71)** | **SMA Type 2 (N = 153)** | **SMA Type 3 (N = 93)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 0)** |
| 2019 | * | * | - | * | - | - | - | - |
| 2020 | * | - | * | - | - | - | - | - |
| 2021 | * | * | - | - | - | - | - | - |
| 2022 | * | - | * | - | - | - | - | - |
| 2023 | - | - | - | - | - | - | - | - |
| Missing | 310 (97.2%) | 69 (97.2%) | 148 (96.7%) | 91 (97.8%) | * | - | - | - |
| **Sex; n (%)** | | | | | | | | |
| Female | 145 (45.5%) | 31 (43.7%) | 68 (44.4%) | 46 (49.5%) | - | - | - | - |
| Male | 174 (54.5%) | 40 (56.3%) | 85 (55.6%) | 47 (50.5%) | * | - | - | - |
| Missing | - | - | - | - | - | - | - | - |
| **Class of age at symptom onset; n (%)** | | | | | | | | |
| Presymptomatic | - | - | - | - | - | - | - | - |
| Prenatal | - | - | - | - | - | - | - | - |
| < 1 month | 7 (2.2%) | 7 (9.9%) | - | - | - | - | - | - |
| [1 - 3 months) | 30 (9.4%) | 30 (42.3%) | - | - | - | - | - | - |
| [3 - 6 months) | 31 (9.7%) | 29 (40.8%) | * | - | - | - | - | - |
| [6 - 18 months) | 154 (48.3%) | 5 (7.0%) | 138 (90.2%) | 11 (11.8%) | - | - | - | - |
| [1.5 - 2 years) | 26 (8.2%) | - | 9 (5.9%) | 17 (18.3%) | - | - | - | - |
| [2 - 6 years) | 34 (10.7%) | - | - | 34 (36.6%) | - | - | - | - |
| [6 - 11 years) | 6 (1.9%) | - | - | 6 (6.5%) | - | - | - | - |
| [11 - 18 years) | 13 (4.1%) | - | - | 13 (14.0%) | - | - | - | - |
| 18 years + | * | - | - | - | * | - | - | - |
| Missing | 16 (5.0%) | - | * | 12 (12.9%) | - | - | - | - |
| **Best functional SMA status; n (%)** | | | | | | | | |
| Non-sitter | 11 (3.4%) | 11 (15.5%) | - | - | - | - | - | - |
| Sitter | 116 (36.4%) | 23 (32.4%) | 92 (60.1%) | * | - | - | - | - |
| Walker | 163 (51.1%) | 19 (26.8%) | 55 (35.9%) | 87 (93.5%) | * | - | - | - |
| Missing | 29 (9.1%) | 18 (25.4%) | 6 (3.9%) | 5 (5.4%) | - | - | - | - |

| Spain \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 319)** | **SMA Type 1 (N = 71)** | **SMA Type 2 (N = 153)** | **SMA Type 3 (N = 93)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 0)** |
| **Best achieved motor milestone; n (%)** | | | | | | | | |
| Climb stairs | 21 (6.6%) | - | * | 20 (21.5%) | - | - | - | - |
| Walk 10 metres without assistance | 81 (25.4%) | * | 9 (5.9%) | 67 (72.0%) | * | - | - | - |
| Walk without assistance | 9 (2.8%) | * | 6 (3.9%) | - | - | - | - | - |
| Walk with assistance | 52 (16.3%) | 13 (18.3%) | 39 (25.5%) | - | - | - | - | - |
| Stand without assistance | * | - | * | - | - | - | - | - |
| Stand with assistance | 7 (2.2%) | * | * | - | - | - | - | - |
| Crawl | - | - | - | - | - | - | - | - |
| Sit without support | 108 (33.9%) | 20 (28.2%) | 87 (56.9%) | * | - | - | - | - |
| Roll onto side | 11 (3.4%) | 11 (15.5%) | - | - | - | - | - | - |
| Hold head without support | - | - | - | - | - | - | - | - |
| Unknown | - | - | - | - | - | - | - | - |
| Missing | 29 (9.1%) | 18 (25.4%) | 6 (3.9%) | 5 (5.4%) | - | - | - | - |
| **SMN1 gene mutation type; n (%)** | | | | | | | | |
| Compound heterozygous deletion exon 7 | 9 (2.8%) | * | 7 (4.6%) | * | - | - | - | - |
| Compound heterozygous substitutions | * | - | * | * | - | - | - | - |
| Homozygous deletion exon 7 | 307 (96.2%) | 70 (98.6%) | 144 (94.1%) | 91 (97.8%) | * | - | - | - |
| Missing | - | - | - | - | - | - | - | - |
| **Number of SMN2 copies; n (%)** | | | | | | | | |
| 0 | - | - | - | - | - | - | - | - |
| 1 | - | - | - | - | - | - | - | - |
| 2 | 73 (22.9%) | 56 (78.9%) | 13 (8.5%) | * | - | - | - | - |

| Spain \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 319)** | **SMA Type 1 (N = 71)** | **SMA Type 2 (N = 153)** | **SMA Type 3 (N = 93)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 0)** |
| 3 | 183 (57.4%) | 14 (19.7%) | 112 (73.2%) | 57 (61.3%) | - | - | - | - |
| 4 | 32 (10.0%) | - | 6 (3.9%) | 25 (26.9%) | * | - | - | - |
| >4 | - | - | - | - | - | - | - | - |
| Other | - | - | - | - | - | - | - | - |
| Missing | 31 (9.7%) | * | 22 (14.4%) | 7 (7.5%) | * | - | - | - |
| **Methods used for genetic testing; n (%)** | | | | | | | | |
| DNA Sequencing | - | - | - | - | - | - | - | - |
| HRM | - | - | - | - | - | - | - | - |
| MLPA | - | - | - | - | - | - | - | - |
| RFLP | - | - | - | - | - | - | - | - |
| ddPCR | - | - | - | - | - | - | - | - |
| qRT-PCR | - | - | - | - | - | - | - | - |
| Missing | 319 (100.0%) | 71 (100.0%) | 153 (100.0%) | 93 (100.0%) | * | - | - | - |
| **Duration of follow up (months)** | | | | | | | | |
| Mean (SD) | 58.3 (22.2) | 51.6 (23.1) | 61.5 (20.8) | 58.4 (22.1) | * | - | - | - |
| Median [IQR] | 66 [45, 71.5] | 54 [29.5, 69.5] | 67 [47, 72] | 66 [48, 73] | * | - | - | - |
| Missing; n (%) | - | - | - | - | - | - | - | - |
| **Duration of SMA (months)** | | | | | | | | |
| Mean (SD) | 232 (186) | 83.4 (69.6) | 234.9 (160.8) | 349.9 (205.8) | * | - | - | - |
| Median [IQR] | 177 [76, 328.5] | 63 [46, 95] | 201 [112, 286] | 334 [165, 514] | * | - | - | - |
| Missing; n (%) | 16 (5.0%) | - | * | 12 (12.9%) | - | - | - | - |
| **Duration between two consecutive visits (months)** | | | | | | | | |
| Mean (SD) | 9.5 (6) | 8.8 (5.8) | 10.4 (6.6) | 8.5 (4.6) | * | - | - | - |
| Median [IQR] | 7.6 [6.1, 10.8] | 7.2 [5.8, 9.7] | 8.5 [6.2, 12.5] | 7.5 [6.3, 9.7] | * | - | - | - |
| Missing; n (%) | 8 (2.5%) | * | * | * | - | - | - | - |

| Spain \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 319)** | **SMA Type 1 (N = 71)** | **SMA Type 2 (N = 153)** | **SMA Type 3 (N = 93)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 0)** |
| **Duration between genetic report date and registry entry (months)** | | | | | | | | |
| Mean (SD) | 80 (87.2) | 26.4 (47.3) | 98.1 (85) | 89.2 (94.8) | * | - | - | - |
| Median [IQR] | 44 [9, 145] | 7 [3, 21] | 81 [20.5, 160.5] | 53 [11.5, 148] | * | - | - | - |
| Missing; n (%) | 10 (3.1%) | * | 6 (3.9%) | * | - | - | - | - |
| **Reason for genetic testing; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Family screening | * | - | * | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 113 (99.1%) | 6 (100.0%) | 74 (98.7%) | 32 (100.0%) | * | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 34 (100.0%) | 6 (100.0%) | 13 (100.0%) | 15 (100.0%) | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| Family screening | * | - | - | * | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 38 (97.4%) | 9 (100.0%) | 18 (100.0%) | 10 (90.9%) | * | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 18 (100.0%) | 8 (100.0%) | 7 (100.0%) | * | - | - | - | - |

| Spain \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 319)** | **SMA Type 1 (N = 71)** | **SMA Type 2 (N = 153)** | **SMA Type 3 (N = 93)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 0)** |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 32 (100.0%) | 10 (100.0%) | 13 (100.0%) | 9 (100.0%) | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 42 (100.0%) | 14 (100.0%) | 14 (100.0%) | 14 (100.0%) | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 5 (100.0%) | * | * | * | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 18 (100.0%) | 11 (100.0%) | 5 (100.0%) | * | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2022** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 7 (100.0%) | * | * | * | - | - | - | - |

| Spain \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 319)** | **SMA Type 1 (N = 71)** | **SMA Type 2 (N = 153)** | **SMA Type 3 (N = 93)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 0)** |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | - | - | - | - | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **Age at onset of SMA symptoms (years)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | 2.5 (4.3) | 0.3 (0.2) | 0.9 (0.3) | 4.3 (4.4) | * | - | - | - |
| Median [IQR] | 1.2 [0.8, 2] | 0.3 [0.1, 0.4] | 1 [0.7, 1.1] | 2 [1.7, 3.8] | * | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | 2 (3.7) | 0.4 (0.1) | 0.8 (0.2) | 6.1 (6) | - | - | - | - |
| Median [IQR] | 0.8 [0.5, 1.2] | 0.5 [0.3, 0.5] | 0.7 [0.5, 1] | 2.2 [1.5, 11.2] | - | - | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | 1 (1.4) | 0.2 (0.2) | 0.8 (0.3) | * | - | - | - | - |
| Median [IQR] | 0.8 [0.4, 1.1] | 0.2 [0.1, 0.3] | 0.8 [0.6, 1.1] | * | - | - | - | - |
| **2017** | | | | | | | | |
| Mean (SD) | 1.3 (2.9) | 0.2 (0.1) | 0.9 (0.3) | * | - | - | - | - |
| Median [IQR] | 0.7 [0.2, 1.1] | 0.2 [0.2, 0.2] | 0.9 [0.7, 1.1] | * | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | 0.5 (0.5) | 0.1 (0.1) | 0.8 (0.3) | * | - | - | - | - |
| Median [IQR] | 0.2 [0.2, 0.7] | 0.2 [0.1, 0.2] | 0.7 [0.5, 1] | * | - | - | - | - |
| **2019** | | | | | | | | |
| Mean (SD) | 0.4 (0.4) | 0.2 (0.1) | 0.7 (0.3) | * | - | - | - | - |
| Median [IQR] | 0.2 [0.1, 0.5] | 0.2 [-, 0.2] | 0.7 [0.5, 0.7] | * | - | - | - | - |
| **2020** | | | | | | | | |
| Mean (SD) | 0.8 (0.5) | * | * | * | - | - | - | - |

| Spain \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 319)** | **SMA Type 1 (N = 71)** | **SMA Type 2 (N = 153)** | **SMA Type 3 (N = 93)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 0)** |
| Median [IQR] | 1.2 [0.4, 1.2] | * | * | * | - | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | 1.5 (3.9) | 0.2 (0.1) | * | * | - | - | - | - |
| Median [IQR] | 0.2 [0.2, 0.3] | 0.2 [0.2, 0.3] | * | * | - | - | - | - |
| **2022** | | | | | | | | |
| Mean (SD) | * | * | - | - | - | - | - | - |
| Median [IQR] | * | * | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **Age at genetic report date (years)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | 7.5 (11.6) | 0.8 (0.3) | 3.9 (7.5) | 15.9 (13.4) | * | - | - | - |
| Median [IQR] | 1.8 [1.2, 6.7] | 0.8 [0.7, 0.8] | 1.5 [1.2, 2.3] | 12.8 [3.1, 27.4] | * | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | 11.6 (18.2) | 0.7 (0.2) | 1.4 (0.8) | 24.9 (20.8) | - | - | - | - |
| Median [IQR] | 1.5 [1, 14.1] | 0.7 [0.6, 0.8] | 1.2 [1, 1.3] | 18.8 [5.6, 38.4] | - | - | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | 11.8 (17.4) | 2.9 (7.1) | 5.8 (11.8) | 25.1 (18.7) | * | - | - | - |
| Median [IQR] | 1.7 [1, 17.2] | 0.4 [0.3, 0.5] | 1.5 [1.3, 1.9] | 34.6 [6.1, 38.9] | * | - | - | - |
| **2017** | | | | | | | | |
| Mean (SD) | 5.3 (10.4) | 0.3 (0.1) | 6.1 (11.2) | * | - | - | - | - |
| Median [IQR] | 1.4 [0.3, 1.8] | 0.3 [0.2, 0.3] | 1.7 [1.4, 1.8] | * | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | 14.3 (19.9) | 0.2 (0.1) | 19.9 (20.3) | 21.8 (22.2) | - | - | - | - |
| Median [IQR] | 2 [0.3, 27.5] | 0.2 [0.1, 0.3] | 5.5 [1.7, 36.8] | 10.9 [2.2, 36.1] | - | - | - | - |
| **2019** | | | | | | | | |
| Mean (SD) | 14.2 (18) | 0.3 (0.2) | 8.6 (14.4) | 33.6 (13) | - | - | - | - |
| Median [IQR] | 1.5 [0.5, 32] | 0.3 [0.1, 0.5] | 1.5 [1, 1.9] | 35 [28.9, 42.6] | - | - | - | - |

| Spain \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 319)** | **SMA Type 1 (N = 71)** | **SMA Type 2 (N = 153)** | **SMA Type 3 (N = 93)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 0)** |
| **2020** | | | | | | | | |
| Mean (SD) | 15.8 (18.1) | * | * | * | - | - | - | - |
| Median [IQR] | 2.5 [1.6, 31.7] | * | * | * | - | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | 5.6 (13.5) | 0.4 (0.1) | 18.4 (20.8) | * | - | - | - | - |
| Median [IQR] | 0.5 [0.4, 1.9] | 0.4 [0.3, 0.5] | 1.9 [1.8, 38.8] | * | - | - | - | - |
| **2022** | | | | | | | | |
| Mean (SD) | 6.6 (10.9) | * | * | * | - | - | - | - |
| Median [IQR] | 0.6 [0.3, 6] | * | * | * | - | - | - | - |
| **2023** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **Age at registry entry (years)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | 13.4 (11.6) | 4 (3.9) | 13.8 (8.7) | 21.6 (15.6) | - | - | - | - |
| Median [IQR] | 10.3 [3.5, 18.4] | 2.2 [1.8, 3.7] | 12.5 [8.2, 18.1] | 24.5 [8.2, 27.8] | - | - | - | - |
| **2017** | | | | | | | | |
| Mean (SD) | 20.1 (16.8) | 8.8 (7.3) | 14.2 (11.5) | 33.3 (17.3) | * | - | - | - |
| Median [IQR] | 13.8 [6.9, 26.7] | 6.6 [2.1, 14.1] | 12.2 [6.7, 18.2] | 31.7 [20.8, 45.7] | * | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | 17.7 (16.8) | 1.3 (1.4) | 15.8 (12.6) | 30.4 (18.6) | - | - | - | - |
| Median [IQR] | 13.1 [3.5, 26.5] | 0.6 [0.6, 1.3] | 13.2 [5.4, 20.1] | 26.5 [13.9, 42.7] | - | - | - | - |
| **2019** | | | | | | | | |

| Spain \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 319)** | **SMA Type 1 (N = 71)** | **SMA Type 2 (N = 153)** | **SMA Type 3 (N = 93)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 0)** |
| Mean (SD) | 16.4 (17.9) | 0.8 (0.5) | 16.6 (16.5) | 29.6 (16.3) | - | - | - | - |
| Median [IQR] | 6.4 [1.1, 34.6] | 0.7 [0.4, 0.8] | 8.8 [2.2, 29.6] | 34 [15.9, 40.8] | - | - | - | - |
| **2020** | | | | | | | | |
| Mean (SD) | 12.2 (14.6) | 3.3 (6.1) | 12 (13) | 22.9 (18.2) | - | - | - | - |
| Median [IQR] | 3.7 [1.2, 19.2] | 0.9 [0.4, 1.8] | 5.5 [2.5, 17.7] | 22.9 [3.8, 40.5] | - | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | 15.2 (18.3) | 0.7 (0.4) | 27.3 (21) | 26.7 (13.2) | - | - | - | - |
| Median [IQR] | 2 [0.6, 31.9] | 0.5 [0.5, 0.8] | 39.4 [2.1, 41.7] | 31.4 [21.5, 32.4] | - | - | - | - |
| **2022** | | | | | | | | |
| Mean (SD) | 13.7 (18.4) | 1.1 (0.7) | 22.3 (21.2) | 20.2 (16.7) | - | - | - | - |
| Median [IQR] | 3.7 [1.1, 16.7] | 1 [0.6, 1.1] | 11.4 [5, 39.9] | 15.3 [5.8, 27.3] | - | - | - | - |
| **2023** | | | | | | | | |
| Mean (SD) | * | _ | * | * | * | _ | _ | _ |
| Median [IQR] | * | _ | * | * | * | _ | _ | _ |
| **Age at death (years)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| Mean (SD) | * | - | - | * | - | - | - | - |
| Median [IQR] | * | - | - | * | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |

| Spain \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 319)** | **SMA Type 1 (N = 71)** | **SMA Type 2 (N = 153)** | **SMA Type 3 (N = 93)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 0)** |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| Mean (SD) | * | * | - | * | - | - | - | - |
| Median [IQR] | * | * | - | * | - | - | - | - |
| **2020** | | | | | | | | |
| Mean (SD) | * | - | * | - | - | - | - | - |
| Median [IQR] | * | - | * | - | - | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | * | * | - | - | - | - | - | - |
| Median [IQR] | * | * | - | - | - | - | - | - |
| **2022** | | | | | | | | |
| Mean (SD) | * | - | * | - | - | - | - | - |
| Median [IQR] | * | - | * | - | - | - | - | - |
| **2023** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **Lost to follow-up; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 32 (100.0%) | 7 (100.0%) | 18 (100.0%) | 7 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 112 (100.0%) | 19 (100.0%) | 59 (100.0%) | 33 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |

| Spain \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 319)** | **SMA Type 1 (N = 71)** | **SMA Type 2 (N = 153)** | **SMA Type 3 (N = 93)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 0)** |
| **2018** | | | | | | | | |
| No | 196 (100.0%) | 33 (100.0%) | 105 (100.0%) | 57 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 240 (100.0%) | 46 (100.0%) | 121 (100.0%) | 72 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 271 (100.0%) | 53 (100.0%) | 139 (100.0%) | 78 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 288 (99.7%) | 62 (100.0%) | 141 (99.3%) | 84 (100.0%) | * | - | - | - |
| Yes | * | - | * | - | - | - | - | - |
| **2022** | | | | | | | | |
| No | 307 (99.7%) | 69 (100.0%) | 149 (100.0%) | 88 (98.9%) | * | - | - | - |
| Yes | * | - | - | * | - | - | - | - |
| **2023** | | | | | | | | |
| No | 308 (100.0%) | 69 (100.0%) | 147 (100.0%) | 90 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **Treated with at least one DMT; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 30 (93.8%) | 5 (71.4%) | 18 (100.0%) | 7 (100.0%) | - | - | - | - |
| Yes | * | * | - | - | - | - | - | - |
| **2017** | | | | | | | | |

| Spain \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 319)** | **SMA Type 1 (N = 71)** | **SMA Type 2 (N = 153)** | **SMA Type 3 (N = 93)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 0)** |
| No | 90 (81.1%) | * | 52 (88.1%) | 33 (100.0%) | * | - | - | - |
| Yes | 21 (18.9%) | 14 (77.8%) | 7 (11.9%) | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 84 (42.9%) | * | 44 (41.9%) | 37 (63.8%) | * | - | - | - |
| Yes | 112 (57.1%) | 30 (93.8%) | 61 (58.1%) | 21 (36.2%) | - | - | - | - |
| **2019** | | | | | | | | |
| No | 81 (33.9%) | * | 41 (33.9%) | 36 (50.0%) | * | - | - | - |
| Yes | 158 (66.1%) | 42 (93.3%) | 80 (66.1%) | 36 (50.0%) | - | - | - | - |
| **2020** | | | | | | | | |
| No | 66 (24.4%) | - | 38 (27.5%) | 27 (34.6%) | * | - | - | - |
| Yes | 204 (75.6%) | 53 (100.0%) | 100 (72.5%) | 51 (65.4%) | - | - | - | - |
| **2021** | | | | | | | | |
| No | 60 (20.8%) | * | 37 (26.1%) | 21 (25.3%) | * | - | - | - |
| Yes | 228 (79.2%) | 61 (98.4%) | 105 (73.9%) | 62 (74.7%) | - | - | - | - |
| **2022** | | | | | | | | |
| No | 66 (21.6%) | * | 37 (24.8%) | 26 (29.5%) | * | - | - | - |
| Yes | 240 (78.4%) | 66 (97.1%) | 112 (75.2%) | 62 (70.5%) | - | - | - | - |
| **2023** | | | | | | | | |
| No | 53 (29.8%) | - | 31 (33.7%) | 21 (43.8%) | * | - | - | - |
| Yes | 125 (70.2%) | 37 (100.0%) | 61 (66.3%) | 27 (56.2%) | - | - | - | - |
| **Treated with more than one DMT; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 32 (100.0%) | 7 (100.0%) | 18 (100.0%) | 7 (100.0%) | - | - | - | - |

| Spain \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 319)** | **SMA Type 1 (N = 71)** | **SMA Type 2 (N = 153)** | **SMA Type 3 (N = 93)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 0)** |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 111 (100.0%) | 18 (100.0%) | 59 (100.0%) | 33 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 196 (100.0%) | 32 (100.0%) | 105 (100.0%) | 58 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 238 (99.6%) | 44 (97.8%) | 121 (100.0%) | 72 (100.0%) | * | - | - | - |
| Yes | * | * | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 262 (97.0%) | 52 (98.1%) | 131 (94.9%) | 78 (100.0%) | * | - | - | - |
| Yes | 8 (3.0%) | * | 7 (5.1%) | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 284 (98.6%) | 59 (95.2%) | 141 (99.3%) | 83 (100.0%) | * | - | - | - |
| Yes | * | * | * | - | - | - | - | - |
| **2022** | | | | | | | | |
| No | 300 (98.0%) | 62 (91.2%) | 149 (100.0%) | 88 (100.0%) | * | - | - | - |
| Yes | 6 (2.0%) | 6 (8.8%) | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| No | 174 (97.8%) | 33 (89.2%) | 92 (100.0%) | 48 (100.0%) | * | - | - | - |
| Yes | * | * | - | - | - | - | - | - |
| **Treated with nusinersen; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |

| Spain \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 319)** | **SMA Type 1 (N = 71)** | **SMA Type 2 (N = 153)** | **SMA Type 3 (N = 93)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 0)** |
| **2014-2016** | | | | | | | | |
| No | 30 (93.8%) | 5 (71.4%) | 18 (100.0%) | 7 (100.0%) | - | - | - | - |
| Yes | * | * | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 92 (82.9%) | * | 54 (91.5%) | 33 (100.0%) | * | - | - | - |
| Yes | 19 (17.1%) | 14 (77.8%) | 5 (8.5%) | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 99 (50.5%) | * | 56 (53.3%) | 39 (67.2%) | * | - | - | - |
| Yes | 97 (49.5%) | 29 (90.6%) | 49 (46.7%) | 19 (32.8%) | - | - | - | - |
| **2019** | | | | | | | | |
| No | 98 (41.0%) | 5 (11.1%) | 54 (44.6%) | 38 (52.8%) | * | - | - | - |
| Yes | 141 (59.0%) | 40 (88.9%) | 67 (55.4%) | 34 (47.2%) | - | - | - | - |
| **2020** | | | | | | | | |
| No | 94 (34.8%) | 8 (15.1%) | 56 (40.6%) | 29 (37.2%) | * | - | - | - |
| Yes | 176 (65.2%) | 45 (84.9%) | 82 (59.4%) | 49 (62.8%) | - | - | - | - |
| **2021** | | | | | | | | |
| No | 97 (33.7%) | 7 (11.3%) | 66 (46.5%) | 23 (27.7%) | * | - | - | - |
| Yes | 191 (66.3%) | 55 (88.7%) | 76 (53.5%) | 60 (72.3%) | - | - | - | - |
| **2022** | | | | | | | | |
| No | 107 (35.0%) | 12 (17.6%) | 66 (44.3%) | 28 (31.8%) | * | - | - | - |
| Yes | 199 (65.0%) | 56 (82.4%) | 83 (55.7%) | 60 (68.2%) | - | - | - | - |
| **2023** | | | | | | | | |
| No | 70 (39.3%) | 6 (16.2%) | 41 (44.6%) | 22 (45.8%) | * | - | - | - |
| Yes | 108 (60.7%) | 31 (83.8%) | 51 (55.4%) | 26 (54.2%) | - | - | - | - |
| **Treated with onasemnogene abeparvovec-xioi; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |

| Spain \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 319)** | **SMA Type 1 (N = 71)** | **SMA Type 2 (N = 153)** | **SMA Type 3 (N = 93)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 0)** |
| **2011-2013** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 32 (100.0%) | 7 (100.0%) | 18 (100.0%) | 7 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 111 (100.0%) | 18 (100.0%) | 59 (100.0%) | 33 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 195 (99.5%) | 31 (96.9%) | 105 (100.0%) | 58 (100.0%) | * | - | - | - |
| Yes | * | * | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 236 (98.7%) | 42 (93.3%) | 121 (100.0%) | 72 (100.0%) | * | - | - | - |
| Yes | * | * | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 267 (98.9%) | 50 (94.3%) | 138 (100.0%) | 78 (100.0%) | * | - | - | - |
| Yes | * | * | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 284 (98.6%) | 58 (93.5%) | 142 (100.0%) | 83 (100.0%) | * | - | - | - |
| Yes | * | * | - | - | - | - | - | - |
| **2022** | | | | | | | | |
| No | 295 (96.4%) | 57 (83.8%) | 149 (100.0%) | 88 (100.0%) | * | - | - | - |
| Yes | 11 (3.6%) | 11 (16.2%) | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| No | 172 (96.6%) | 31 (83.8%) | 92 (100.0%) | 48 (100.0%) | * | - | - | - |
| Yes | 6 (3.4%) | 6 (16.2%) | - | - | - | - | - | - |
| **Treated with risdiplan; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |

| Spain \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 319)** | **SMA Type 1 (N = 71)** | **SMA Type 2 (N = 153)** | **SMA Type 3 (N = 93)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 0)** |
| No | - | - | - | - | - | - | - | - |
| Yes | | | | | | | | |
| **2011-2013** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 32 (100.0%) | 7 (100.0%) | 18 (100.0%) | 7 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 109 (98.2%) | 18 (100.0%) | 57 (96.6%) | 33 (100.0%) | * | - | - | - |
| Yes | * | - | * | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 182 (92.9%) | 32 (100.0%) | 93 (88.6%) | 56 (96.6%) | * | - | - | - |
| Yes | 14 (7.1%) | - | 12 (11.4%) | * | - | - | - | - |
| **2020** | | | | | | | | |
| No | 224 (93.7%) | 45 (100.0%) | 108 (89.3%) | 70 (97.2%) | * | - | - | - |
| Yes | 15 (6.3%) | - | 13 (10.7%) | * | - | - | - | - |
| **2021** | | | | | | | | |
| No | 237 (87.8%) | 47 (88.7%) | 113 (81.9%) | 76 (97.4%) | * | - | - | - |
| Yes | 33 (12.2%) | 6 (11.3%) | 25 (18.1%) | * | - | - | - | - |
| **2022** | | | | | | | | |
| No | 251 (87.2%) | 57 (91.9%) | 112 (78.9%) | 81 (97.6%) | * | - | - | - |
| Yes | 37 (12.8%) | 5 (8.1%) | 30 (21.1%) | * | - | - | - | - |
| **2023** | | | | | | | | |
| No | 270 (88.2%) | 63 (92.6%) | 120 (80.5%) | 86 (97.7%) | * | - | - | - |
| Yes | 36 (11.8%) | 5 (7.4%) | 29 (19.5%) | * | - | - | - | - |

| Spain \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 319)** | **SMA Type 1 (N = 71)** | **SMA Type 2 (N = 153)** | **SMA Type 3 (N = 93)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 0)** |
| **At least one episode of invasive ventilation; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 31 (96.9%) | 6 (85.7%) | 18 (100.0%) | 7 (100.0%) | - | - | - | - |
| Yes | * | * | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 103 (92.8%) | 10 (55.6%) | 59 (100.0%) | 33 (100.0%) | * | - | - | - |
| Yes | 8 (7.2%) | 8 (44.4%) | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 186 (94.9%) | 23 (71.9%) | 104 (99.0%) | 58 (100.0%) | * | - | - | - |
| Yes | 10 (5.1%) | 9 (28.1%) | * | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 226 (94.6%) | 33 (73.3%) | 120 (99.2%) | 72 (100.0%) | * | - | - | - |
| Yes | 13 (5.4%) | 12 (26.7%) | * | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 255 (94.4%) | 39 (73.6%) | 137 (99.3%) | 78 (100.0%) | * | - | - | - |
| Yes | 15 (5.6%) | 14 (26.4%) | * | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 271 (94.1%) | 46 (74.2%) | 141 (99.3%) | 83 (100.0%) | * | - | - | - |
| Yes | 17 (5.9%) | 16 (25.8%) | * | - | - | - | - | - |
| **2022** | | | | | | | | |
| No | 290 (94.8%) | 53 (77.9%) | 148 (99.3%) | 88 (100.0%) | * | - | - | - |
| Yes | 16 (5.2%) | 15 (22.1%) | * | - | - | - | - | - |
| **2023** | | | | | | | | |

| Spain \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 319)** | **SMA Type 1 (N = 71)** | **SMA Type 2 (N = 153)** | **SMA Type 3 (N = 93)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 0)** |
| No | 172 (96.6%) | 31 (83.8%) | 92 (100.0%) | 48 (100.0%) | * | - | - | - |
| Yes | 6 (3.4%) | 6 (16.2%) | - | - | - | - | - | - |
| **At least one episode of non-invasive ventilation; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 14 (43.8%) | * | 6 (33.3%) | 7 (100.0%) | - | - | - | - |
| Yes | 18 (56.2%) | 6 (85.7%) | 12 (66.7%) | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 56 (50.5%) | 7 (38.9%) | 18 (30.5%) | 30 (90.9%) | * | - | - | - |
| Yes | 55 (49.5%) | 11 (61.1%) | 41 (69.5%) | * | - | - | - | - |
| **2018** | | | | | | | | |
| No | 115 (58.7%) | 15 (46.9%) | 47 (44.8%) | 52 (89.7%) | * | - | - | - |
| Yes | 81 (41.3%) | 17 (53.1%) | 58 (55.2%) | 6 (10.3%) | - | - | - | - |
| **2019** | | | | | | | | |
| No | 142 (59.4%) | 17 (37.8%) | 58 (47.9%) | 66 (91.7%) | * | - | - | - |
| Yes | 97 (40.6%) | 28 (62.2%) | 63 (52.1%) | 6 (8.3%) | - | - | - | - |
| **2020** | | | | | | | | |
| No | 159 (58.9%) | 22 (41.5%) | 65 (47.1%) | 71 (91.0%) | * | - | - | - |
| Yes | 111 (41.1%) | 31 (58.5%) | 73 (52.9%) | 7 (9.0%) | - | - | - | - |
| **2021** | | | | | | | | |
| No | 168 (58.3%) | 24 (38.7%) | 68 (47.9%) | 75 (90.4%) | * | - | - | - |
| Yes | 120 (41.7%) | 38 (61.3%) | 74 (52.1%) | 8 (9.6%) | - | - | - | - |
| **2022** | | | | | | | | |

| Spain \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 319)** | **SMA Type 1 (N = 71)** | **SMA Type 2 (N = 153)** | **SMA Type 3 (N = 93)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 0)** |
| No | 177 (57.8%) | 28 (41.2%) | 69 (46.3%) | 79 (89.8%) | * | - | - | - |
| Yes | 129 (42.2%) | 40 (58.8%) | 80 (53.7%) | 9 (10.2%) | - | - | - | - |
| **2023** | | | | | | | | |
| No | 97 (54.5%) | 14 (37.8%) | 40 (43.5%) | 42 (87.5%) | * | - | - | - |
| Yes | 81 (45.5%) | 23 (62.2%) | 52 (56.5%) | 6 (12.5%) | - | - | - | - |
| **At least one episode of feeding tube usage; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 29 (90.6%) | 5 (71.4%) | 17 (94.4%) | 7 (100.0%) | - | - | - | - |
| Yes | * | * | * | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 105 (94.6%) | 12 (66.7%) | 59 (100.0%) | 33 (100.0%) | * | - | - | - |
| Yes | 6 (5.4%) | 6 (33.3%) | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 189 (96.4%) | 26 (81.2%) | 104 (99.0%) | 58 (100.0%) | * | - | - | - |
| Yes | 7 (3.6%) | 6 (18.8%) | * | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 216 (90.4%) | 23 (51.1%) | 120 (99.2%) | 72 (100.0%) | * | - | - | - |
| Yes | 23 (9.6%) | 22 (48.9%) | * | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 244 (90.4%) | 30 (56.6%) | 135 (97.8%) | 78 (100.0%) | * | - | - | - |
| Yes | 26 (9.6%) | 23 (43.4%) | * | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 263 (91.3%) | 39 (62.9%) | 140 (98.6%) | 83 (100.0%) | * | - | - | - |

| Spain \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 319)** | **SMA Type 1 (N = 71)** | **SMA Type 2 (N = 153)** | **SMA Type 3 (N = 93)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 0)** |
| Yes | 25 (8.7%) | 23 (37.1%) | * | - | - | - | - | - |
| **2022** | | | | | | | | |
| No | 270 (88.2%) | 37 (54.4%) | 145 (97.3%) | 87 (98.9%) | * | - | - | - |
| Yes | 36 (11.8%) | 31 (45.6%) | * | * | - | - | - | - |
| **2023** | | | | | | | | |
| No | 161 (90.4%) | 23 (62.2%) | 90 (97.8%) | 47 (97.9%) | * | - | - | - |
| Yes | 17 (9.6%) | 14 (37.8%) | * | * | - | - | - | - |
| **At least one episode of wheelchair usage; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 8 (25.0%) | * | - | 6 (85.7%) | - | - | - | - |
| Yes | 24 (75.0%) | 5 (71.4%) | 18 (100.0%) | * | - | - | - | - |
| **2017** | | | | | | | | |
| No | 42 (37.8%) | 7 (38.9%) | 19 (32.2%) | 15 (45.5%) | * | - | - | - |
| Yes | 69 (62.2%) | 11 (61.1%) | 40 (67.8%) | 18 (54.5%) | - | - | - | - |
| **2018** | | | | | | | | |
| No | 87 (44.4%) | 17 (53.1%) | 36 (34.3%) | 33 (56.9%) | * | - | - | - |
| Yes | 109 (55.6%) | 15 (46.9%) | 69 (65.7%) | 25 (43.1%) | - | - | - | - |
| **2019** | | | | | | | | |
| No | 60 (25.1%) | 14 (31.1%) | 16 (13.2%) | 29 (40.3%) | * | - | - | - |
| Yes | 179 (74.9%) | 31 (68.9%) | 105 (86.8%) | 43 (59.7%) | - | - | - | - |
| **2020** | | | | | | | | |
| No | 69 (25.6%) | 17 (32.1%) | 26 (18.8%) | 25 (32.1%) | * | - | - | - |

| Spain \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 319)** | **SMA Type 1 (N = 71)** | **SMA Type 2 (N = 153)** | **SMA Type 3 (N = 93)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 0)** |
| Yes | 201 (74.4%) | 36 (67.9%) | 112 (81.2%) | 53 (67.9%) | - | - | - | - |
| **2021** | | | | | | | | |
| No | 68 (23.6%) | 18 (29.0%) | 23 (16.2%) | 26 (31.3%) | * | - | - | - |
| Yes | 220 (76.4%) | 44 (71.0%) | 119 (83.8%) | 57 (68.7%) | - | - | - | - |
| **2022** | | | | | | | | |
| No | 56 (18.3%) | 12 (17.6%) | 19 (12.8%) | 24 (27.3%) | * | - | - | - |
| Yes | 250 (81.7%) | 56 (82.4%) | 130 (87.2%) | 64 (72.7%) | - | - | - | - |
| **2023** | | | | | | | | |
| No | 22 (12.4%) | * | 5 (5.4%) | 12 (25.0%) | * | - | - | - |
| Yes | 156 (87.6%) | 33 (89.2%) | 87 (94.6%) | 36 (75.0%) | - | - | - | - |
| **At least one reported measure by available motor function scale or test and by at least one PRO; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 32 (100.0%) | 7 (100.0%) | 18 (100.0%) | 7 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 111 (100.0%) | 18 (100.0%) | 59 (100.0%) | 33 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 196 (100.0%) | 32 (100.0%) | 105 (100.0%) | 58 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |

| Spain \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 319)** | **SMA Type 1 (N = 71)** | **SMA Type 2 (N = 153)** | **SMA Type 3 (N = 93)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 0)** |
| No | 239 (100.0%) | 45 (100.0%) | 121 (100.0%) | 72 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 270 (100.0%) | 53 (100.0%) | 138 (100.0%) | 78 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 288 (100.0%) | 62 (100.0%) | 142 (100.0%) | 83 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2022** | | | | | | | | |
| No | 306 (100.0%) | 68 (100.0%) | 149 (100.0%) | 88 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| No | 178 (100.0%) | 37 (100.0%) | 92 (100.0%) | 48 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **At least three reported measures by available motor function scale or test and by at least one PRO; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | - | - | - | - | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 32 (100.0%) | 7 (100.0%) | 18 (100.0%) | 7 (100.0%) | - | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 111 (100.0%) | 18 (100.0%) | 59 (100.0%) | 33 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |

| Spain \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 319)** | **SMA Type 1 (N = 71)** | **SMA Type 2 (N = 153)** | **SMA Type 3 (N = 93)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 0)** |
| **2018** | | | | | | | | |
| No | 196 (100.0%) | 32 (100.0%) | 105 (100.0%) | 58 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 239 (100.0%) | 45 (100.0%) | 121 (100.0%) | 72 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 270 (100.0%) | 53 (100.0%) | 138 (100.0%) | 78 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 288 (100.0%) | 62 (100.0%) | 142 (100.0%) | 83 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2022** | | | | | | | | |
| No | 306 (100.0%) | 68 (100.0%) | 149 (100.0%) | 88 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| No | 178 (100.0%) | 37 (100.0%) | 92 (100.0%) | 48 (100.0%) | * | - | - | - |
| Yes | - | - | - | - | - | - | - | - |
| **Available number of records of each motor function scale by patient** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |

| Spain \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 319)** | **SMA Type 1 (N = 71)** | **SMA Type 2 (N = 153)** | **SMA Type 3 (N = 93)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 0)** |
| **2017** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2022** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **Available number of records of PRO** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |

| Spain \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 319)** | **SMA Type 1 (N = 71)** | **SMA Type 2 (N = 153)** | **SMA Type 3 (N = 93)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 0)** |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| Mean (SD) | 1.3 (0.6) | 1.2 (0.4) | 1.3 (0.6) | 1.4 (0.5) | - | - | - | - |
| Median [IQR] | 1 [1, 2] | 1 [1, 1.2] | 1 [1, 2] | 1 [1, 2] | - | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | 1.1 (0.3) | 1.1 (0.3) | 1.1 (0.2) | 1.1 (0.3) | - | - | - | - |
| Median [IQR] | 1 [1, 1] | 1 [1, 1] | 1 [1, 1] | 1 [1, 1] | - | - | - | - |
| **2022** | | | | | | | | |
| Mean (SD) | 1.2 (0.5) | 1.2 (0.4) | 1.1 (0.3) | 1.3 (0.6) | - | - | - | - |
| Median [IQR] | 1 [1, 1] | 1 [1, 1] | 1 [1, 1] | 1 [1, 1] | - | - | - | - |
| **2023** | | | | | | | | |
| Mean (SD) | 1.1 (0.2) | 1 (-) | 1.1 (0.3) | 1 (-) | - | - | - | - |
| Median [IQR] | 1 [1, 1] | 1 [1, 1] | 1 [1, 1] | 1 [1, 1] | - | - | - | - |

| UK & Ireland \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 393)** | **SMA Type 1 (N = 69)** | **SMA Type 2 (N = 183)** | **SMA Type 3 (N = 123)** | **SMA Type 4 (N = 0)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 1)** | **SMA Type Missing (N = 17)** |
| **Calendar year of registry entry; n (%)** | | | | | | | | |
| 2008 | 23 (5.9%) | * | 12 (6.6%) | 5 (4.1%) | - | - | - | * |
| 2009 | 10 (2.5%) | * | 5 (2.7%) | * | - | - | - | * |
| 2010 | * | * | * | * | - | - | - | * |
| 2011 | 7 (1.8%) | * | * | * | - | - | - | - |
| 2012 | * | - | * | - | - | - | - | - |
| 2013 | * | - | * | - | - | - | - | - |
| 2014 | 48 (12.2%) | 11 (15.9%) | 23 (12.6%) | 14 (11.4%) | - | - | - | - |
| 2015 | 85 (21.6%) | 7 (10.1%) | 49 (26.8%) | 28 (22.8%) | - | - | - | * |
| 2016 | 36 (9.2%) | 9 (13.0%) | 12 (6.6%) | 15 (12.2%) | - | - | - | - |
| 2017 | 21 (5.3%) | * | 11 (6.0%) | 8 (6.5%) | - | - | - | - |
| 2018 | 33 (8.4%) | 8 (11.6%) | 13 (7.1%) | 12 (9.8%) | - | - | - | - |
| 2019 | 24 (6.1%) | * | 13 (7.1%) | 7 (5.7%) | - | - | - | * |
| 2020 | 9 (2.3%) | - | 6 (3.3%) | * | - | - | - | * |
| 2021 | 11 (2.8%) | * | 5 (2.7%) | * | - | - | - | - |
| 2022 | 46 (11.7%) | 13 (18.8%) | 13 (7.1%) | 13 (10.6%) | - | - | * | 6 (35.3%) |
| 2023 | 31 (7.9%) | * | 14 (7.7%) | 11 (8.9%) | - | - | - | * |
| Missing | * | - | - | - | - | - | - | * |
| **Calendar year of death; n (%)** | | | | | | | | |
| 2009 | - | - | - | - | - | - | - | - |
| 2010 | - | - | - | - | - | - | - | - |
| 2012 | - | - | - | - | - | - | - | - |
| 2013 | - | - | - | - | - | - | - | - |
| 2014 | - | - | - | - | - | - | - | - |
| 2015 | - | - | - | - | - | - | - | - |
| 2016 | - | - | - | - | - | - | - | - |
| 2017 | - | - | - | - | - | - | - | - |
| 2018 | - | - | - | - | - | - | - | - |

| UK & Ireland \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 393)** | **SMA Type 1 (N = 69)** | **SMA Type 2 (N = 183)** | **SMA Type 3 (N = 123)** | **SMA Type 4 (N = 0)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 1)** | **SMA Type Missing (N = 17)** |
| 2019 | - | - | - | - | - | - | - | - |
| 2020 | - | - | - | - | - | - | - | - |
| 2021 | - | - | - | - | - | - | - | - |
| 2022 | * | * | * | * | - | - | - | - |
| 2023 | - | - | - | - | - | - | - | - |
| Missing | 389 (99.0%) | 68 (98.6%) | 181 (98.9%) | 122 (99.2%) | - | - | * | 17 (100.0%) |
| **Sex; n (%)** | | | | | | | | |
| Female | 179 (45.5%) | 28 (40.6%) | 87 (47.5%) | 54 (43.9%) | - | - | - | 10 (58.8%) |
| Male | 214 (54.5%) | 41 (59.4%) | 96 (52.5%) | 69 (56.1%) | - | - | * | 7 (41.2%) |
| Missing | - | - | - | - | - | - | - | - |
| **Class of age at symptom onset; n (%)** | | | | | | | | |
| Presymptomatic | * | - | - | - | - | - | * | - |
| Prenatal | * | * | * | - | - | - | - | - |
| < 1 month | 5 (1.3%) | * | - | * | - | - | - | - |
| [1 - 3 months) | 7 (1.8%) | 7 (10.1%) | - | - | - | - | - | - |
| [3 - 6 months) | 18 (4.6%) | 12 (17.4%) | 6 (3.3%) | - | - | - | - | - |
| [6 - 18 months) | 88 (22.4%) | 6 (8.7%) | 60 (32.8%) | 20 (16.3%) | - | - | - | * |
| [1.5 - 2 years) | 14 (3.6%) | - | 7 (3.8%) | 7 (5.7%) | - | - | - | - |
| [2 - 6 years) | 35 (8.9%) | - | 6 (3.3%) | 29 (23.6%) | - | - | - | - |
| [6 - 11 years) | 7 (1.8%) | - | * | 6 (4.9%) | - | - | - | - |
| [11 - 18 years) | 11 (2.8%) | - | * | 10 (8.1%) | - | - | - | - |
| 18 years + | * | - | - | * | - | - | - | - |
| Missing | 202 (51.4%) | 39 (56.5%) | 100 (54.6%) | 48 (39.0%) | - | - | - | 15 (88.2%) |
| **Best functional SMA status; n (%)** | | | | | | | | |
| Non-sitter | 18 (4.6%) | 9 (13.0%) | 6 (3.3%) | * | - | - | - | - |
| Sitter | 61 (15.5%) | 13 (18.8%) | 46 (25.1%) | * | - | - | - | * |
| Walker | 103 (26.2%) | - | 34 (18.6%) | 67 (54.5%) | - | - | * | * |
| Missing | 211 (53.7%) | 47 (68.1%) | 97 (53.0%) | 52 (42.3%) | - | - | - | 15 (88.2%) |

| UK & Ireland \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 393)** | **SMA Type 1 (N = 69)** | **SMA Type 2 (N = 183)** | **SMA Type 3 (N = 123)** | **SMA Type 4 (N = 0)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 1)** | **SMA Type Missing (N = 17)** |
| **Best achieved motor milestone; n (%)** | | | | | | | | |
| Climb stairs | 63 (16.0%) | - | 8 (4.4%) | 54 (43.9%) | - | - | - | * |
| Walk 10 metres without assistance | 10 (2.5%) | - | * | 9 (7.3%) | - | - | - | - |
| Walk without assistance | 6 (1.5%) | - | * | * | - | - | - | - |
| Walk with assistance | 24 (6.1%) | - | 22 (12.0%) | * | - | - | * | - |
| Stand without assistance | 6 (1.5%) | - | * | * | - | - | - | * |
| Stand with assistance | 19 (4.8%) | 7 (10.1%) | 12 (6.6%) | - | - | - | - | - |
| Crawl | 17 (4.3%) | - | 17 (9.3%) | - | - | - | - | - |
| Sit without support | 19 (4.8%) | 6 (8.7%) | 13 (7.1%) | - | - | - | - | - |
| Roll onto side | 8 (2.0%) | 7 (10.1%) | * | - | - | - | - | - |
| Hold head without support | 10 (2.5%) | * | 5 (2.7%) | * | - | - | - | - |
| Unknown | - | - | - | - | - | - | - | - |
| Missing | 211 (53.7%) | 47 (68.1%) | 97 (53.0%) | 52 (42.3%) | - | - | - | 15 (88.2%) |
| **SMN1 gene mutation type; n (%)** | | | | | | | | |
| Compound heterozygous deletion exon 7 | 5 (1.3%) | * | - | * | - | - | - | - |
| Compound heterozygous substitutions | - | - | - | - | - | - | - | - |
| Homozygous deletion exon 7 | 237 (60.3%) | 36 (52.2%) | 117 (63.9%) | 81 (65.9%) | - | - | - | * |
| Missing | 151 (38.4%) | 32 (46.4%) | 66 (36.1%) | 38 (30.9%) | - | - | * | 14 (82.4%) |
| **Number of SMN2 copies; n (%)** | | | | | | | | |
| 0 | * | * | - | - | - | - | - | - |
| 1 | - | - | - | - | - | - | - | - |
| 2 | 18 (4.6%) | 13 (18.8%) | * | * | - | - | - | - |

| UK & Ireland \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 393)** | **SMA Type 1 (N = 69)** | **SMA Type 2 (N = 183)** | **SMA Type 3 (N = 123)** | **SMA Type 4 (N = 0)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 1)** | **SMA Type Missing (N = 17)** |
| 3 | 61 (15.5%) | * | 40 (21.9%) | 16 (13.0%) | - | - | - | * |
| 4 | 12 (3.1%) | - | - | 12 (9.8%) | - | - | - | - |
| >4 | - | - | - | - | - | - | - | - |
| Other | - | - | - | - | - | - | - | - |
| Missing | 301 (76.6%) | 51 (73.9%) | 141 (77.0%) | 92 (74.8%) | - | - | * | 16 (94.1%) |
| **Methods used for genetic testing; n (%)** | | | | | | | | |
| DNA Sequencing | - | - | - | - | - | - | - | - |
| HRM | - | - | - | - | - | - | - | - |
| MLPA | 27 (6.9%) | 6 (8.7%) | 11 (6.0%) | 10 (8.1%) | - | - | - | - |
| RFLP | * | - | * | - | - | - | - | - |
| ddPCR | - | - | - | - | - | - | - | - |
| qRT-PCR | * | - | - | * | - | - | - | - |
| Missing | 362 (92.1%) | 63 (91.3%) | 171 (93.4%) | 110 (89.4%) | - | - | * | 17 (100.0%) |
| **Duration of follow up (months)** | | | | | | | | |
| Mean (SD) | 67.8 (43.9) | 58.8 (38) | 74.1 (45.4) | 67.5 (41.3) | - | - | * | 38.3 (49.8) |
| Median [IQR] | 73 [24, 95] | 61 [24, 90] | 80 [31.5, 102.5] | 74 [25, 95] | - | - | * | 19 [11.5, 24] |
| Missing; n (%) | * | - | - | - | - | - | - | * |
| **Duration of SMA (months)** | | | | | | | | |
| Mean (SD) | 311.1 (225.9) | 90 (98.5) | 296.2 (199.7) | 419.4 (219.3) | - | - | - | * |
| Median [IQR] | 261.5 [108, 492.8] | 50 [26, 92] | 256 [129.5, 431.8] | 415 [234, 590] | - | - | - | * |
| Missing; n (%) | 205 (52.2%) | 40 (58.0%) | 101 (55.2%) | 48 (39.0%) | - | - | * | 15 (88.2%) |
| **Duration between two consecutive visits (months)** | | | | | | | | |
| Mean (SD) | 33.2 (38.2) | 28.2 (29.1) | 36.1 (42.3) | 30.1 (32.6) | - | - | * | 58.1 (64.7) |
| Median [IQR] | 18.3 [12, 35] | 20.8 [11.8, 33.5] | 18 [12.4, 35] | 18.3 [12, 30.7] | - | - | * | 28.5 [4.6, 92.5] |
| Missing; n (%) | 75 (19.1%) | 14 (20.3%) | 28 (15.3%) | 22 (17.9%) | - | - | - | 11 (64.7%) |

| UK & Ireland \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 393)** | **SMA Type 1 (N = 69)** | **SMA Type 2 (N = 183)** | **SMA Type 3 (N = 123)** | **SMA Type 4 (N = 0)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 1)** | **SMA Type Missing (N = 17)** |
| **Duration between genetic report date and registry entry (months)** | | | | | | | | |
| Mean (SD) | 83.6 (91.6) | 13.6 (16.1) | 81 (81.1) | 105.8 (102.9) | - | - | - | - |
| Median [IQR] | 45 [10, 156] | 9 [-, 19.5] | 45 [19, 124] | 57 [6, 194] | - | - | - | - |
| Missing; n (%) | 336 (85.5%) | 62 (89.9%) | 158 (86.3%) | 98 (79.7%) | - | - | * | 17 (100.0%) |
| **Reason for genetic testing; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 15 (68.2%) | - | 6 (54.5%) | 9 (81.8%) | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Family screening | * | - | - | * | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | * | - | * | - | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 5 (83.3%) | - | * | * | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | * | * | * | * | - | - | - | - |

| UK & Ireland \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 393)** | **SMA Type 1 (N = 69)** | **SMA Type 2 (N = 183)** | **SMA Type 3 (N = 123)** | **SMA Type 4 (N = 0)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 1)** | **SMA Type Missing (N = 17)** |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 5 (100.0%) | * | * | * | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | * | * | * | * | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | * | * | - | * | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 7 (100.0%) | * | * | * | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2022** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | * | * | - | * | - | - | - | - |

| UK & Ireland \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 393)** | **SMA Type 1 (N = 69)** | **SMA Type 2 (N = 183)** | **SMA Type 3 (N = 123)** | **SMA Type 4 (N = 0)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 1)** | **SMA Type Missing (N = 17)** |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | - | - | - | - | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **Age at onset of SMA symptoms (years)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | 3.2 (5.1) | 0.3 (0.1) | 1.3 (1.9) | 5.2 (6.3) | - | - | - | - |
| Median [IQR] | 1.4 [0.7, 3] | 0.3 [0.2, 0.5] | 1 [0.6, 1.4] | 2.4 [1.4, 6] | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | 1.4 (0.9) | - | 0.9 (0.5) | * | - | - | - | - |
| Median [IQR] | 1 [0.8, 2] | - | 0.9 [0.7, 1] | * | - | - | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | 0.7 (0.3) | * | 0.8 (0.2) | * | - | - | - | * |
| Median [IQR] | 0.6 [0.5, 1] | * | 0.8 [0.6, 0.9] | * | - | - | - | * |
| **2017** | | | | | | | | |
| Mean (SD) | 0.8 (0.4) | * | * | * | - | - | - | - |
| Median [IQR] | 0.8 [0.5, 0.8] | * | * | * | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | 0.5 (0.3) | * | * | - | - | - | - | - |
| Median [IQR] | 0.5 [0.2, 0.6] | * | * | - | - | - | - | - |
| **2019** | | | | | | | | |
| Mean (SD) | * | * | * | * | - | - | - | * |
| Median [IQR] | * | * | * | * | - | - | - | * |
| **2020** | | | | | | | | |
| Mean (SD) | 2 (3.6) | * | * | - | - | - | - | - |

| UK & Ireland \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 393)** | **SMA Type 1 (N = 69)** | **SMA Type 2 (N = 183)** | **SMA Type 3 (N = 123)** | **SMA Type 4 (N = 0)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 1)** | **SMA Type Missing (N = 17)** |
| Median [IQR] | 0.4 [0.3, 0.9] | * | * | - | - | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | 0.3 (0.4) | 0.2 (0.2) | - | * | - | - | - | - |
| Median [IQR] | 0.1 [0.1, 0.6] | 0.1 [0.1, 0.3] | - | * | - | - | - | - |
| **2022** | | | | | | | | |
| Mean (SD) | * | * | - | - | - | - | - | - |
| Median [IQR] | * | * | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **Age at genetic report date (years)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | 23.4 (14) | - | 14.9 (11.9) | 31.9 (10.4) | - | - | - | - |
| Median [IQR] | 25.4 [13, 33] | - | 16.3 [2.6, 25.2] | 33.3 [26.8, 36.8] | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | 6.8 (9.7) | - | * | * | - | - | - | - |
| Median [IQR] | 2.2 [1.7, 2.2] | - | * | * | - | - | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | 3.2 (1.7) | - | * | * | - | - | - | - |
| Median [IQR] | 3.1 [1.8, 3.5] | - | * | * | - | - | - | - |
| **2017** | | | | | | | | |
| Mean (SD) | * | * | * | * | - | - | - | - |
| Median [IQR] | * | * | * | * | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | 25.7 (21) | * | * | * | - | - | - | - |
| Median [IQR] | 31.8 [1.9, 44.7] | * | * | * | - | - | - | - |
| **2019** | | | | | | | | |
| Mean (SD) | * | * | * | * | - | - | - | - |
| Median [IQR] | * | * | * | * | - | - | - | - |

| UK & Ireland \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 393)** | **SMA Type 1 (N = 69)** | **SMA Type 2 (N = 183)** | **SMA Type 3 (N = 123)** | **SMA Type 4 (N = 0)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 1)** | **SMA Type Missing (N = 17)** |
| **2020** | | | | | | | | |
| Mean (SD) | * | * | - | * | - | - | - | - |
| Median [IQR] | * | * | - | * | - | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | 21.3 (19.1) | * | * | * | - | - | - | - |
| Median [IQR] | 20.1 [5.5, 30.4] | * | * | * | - | - | - | - |
| **2022** | | | | | | | | |
| Mean (SD) | * | * | - | * | - | - | - | - |
| Median [IQR] | * | * | - | * | - | - | - | - |
| **2023** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **Age at registry entry (years)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | 15.9 (20) | 1.5 (1.6) | 9.1 (9.1) | 31.3 (23.4) | - | - | - | * |
| Median [IQR] | 6 [3.2, 23.2] | 0.9 [0.6, 1.2] | 5 [3.3, 10.8] | 24.7 [10.5, 53.5] | - | - | - | * |
| **2011-2013** | | | | | | | | |
| Mean (SD) | 7.1 (8.9) | * | 8.9 (9.5) | * | - | - | - | - |
| Median [IQR] | 1.6 [0.6, 11.7] | * | 4.9 [1.6, 11.7] | * | - | - | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | 17 (18.6) | 3.8 (4.8) | 13.3 (13.8) | 28.9 (21.9) | - | - | - | * |
| Median [IQR] | 9.6 [3.4, 25.1] | 1 [0.7, 6.9] | 7.8 [3.5, 16.7] | 21.2 [10, 48.1] | - | - | - | * |
| **2017** | | | | | | | | |
| Mean (SD) | 25.2 (20.6) | * | 22.6 (20.5) | 34.8 (17.1) | - | - | - | - |
| Median [IQR] | 19.8 [2.1, 43.1] | * | 19.8 [2.1, 36.5] | 30.5 [17.7, 54.2] | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | 18.1 (20.6) | 5.5 (9.1) | 9.8 (9.5) | 35.7 (22.8) | - | - | - | - |
| Median [IQR] | 8.9 [1.5, 27.7] | 1.3 [0.4, 4] | 8 [1.9, 14.6] | 38.5 [12.3, 54.7] | - | - | - | - |
| **2019** | | | | | | | | |

| UK & Ireland \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 393)** | **SMA Type 1 (N = 69)** | **SMA Type 2 (N = 183)** | **SMA Type 3 (N = 123)** | **SMA Type 4 (N = 0)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 1)** | **SMA Type Missing (N = 17)** |
| Mean (SD) | 20.9 (18.6) | * | 17.1 (13.7) | 39.1 (16.3) | - | - | - | * |
| Median [IQR] | 13 [2.6, 39.9] | * | 12.2 [9.2, 27.6] | 41.9 [36.4, 51.2] | - | - | - | * |
| **2020** | | | | | | | | |
| Mean (SD) | 23.1 (17.5) | - | 24.2 (14.8) | * | - | - | - | * |
| Median [IQR] | 19.8 [8.9, 34.8] | - | 21.7 [17.9, 32] | * | - | - | - | * |
| **2021** | | | | | | | | |
| Mean (SD) | 23.9 (25.5) | * | 16.5 (13.1) | * | - | - | - | - |
| Median [IQR] | 14.2 [1.1, 41.5] | * | 14.2 [3.8, 27.2] | * | - | - | - | - |
| **2022** | | | | | | | | |
| Mean (SD) | 23.1 (19.2) | 3.2 (5.3) | 28.3 (15.2) | 42 (13.3) | - | - | * | 18 (9.8) |
| Median [IQR] | 22.7 [3, 39.5] | 1.6 [0.9, 2.4] | 27.8 [11.8, 40.1] | 45.5 [31.2, 48.1] | - | - | * | 19.4 [9.6, 24.6] |
| **2023** | | | | | | | | |
| Mean (SD) | 26.3 (16.8) | * | 32.5 (13.9) | 25.4 (15.6) | - | - | - | * |
| Median [IQR] | 23.2 [15.5, 38.5] | * | 31.5 [22.6, 41.8] | 20.5 [15.5, 33.2] | - | - | - | * |
| **Age at death (years)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |

| UK & Ireland \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 393)** | **SMA Type 1 (N = 69)** | **SMA Type 2 (N = 183)** | **SMA Type 3 (N = 123)** | **SMA Type 4 (N = 0)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 1)** | **SMA Type Missing (N = 17)** |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2022** | | | | | | | | |
| Mean (SD) | * | * | * | * | - | - | - | - |
| Median [IQR] | * | * | * | * | - | - | - | - |
| **2023** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **Lost to follow-up; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 31 (83.8%) | 5 (83.3%) | 14 (77.8%) | 8 (88.9%) | - | - | - | * |
| Yes | 6 (16.2%) | * | * | * | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 35 (85.4%) | 6 (75.0%) | 17 (85.0%) | 9 (100.0%) | - | - | - | * |
| Yes | 6 (14.6%) | * | * | - | - | - | - | * |
| **2014-2016** | | | | | | | | |
| No | 198 (97.1%) | 30 (90.9%) | 100 (99.0%) | 65 (98.5%) | - | - | - | * |
| Yes | 6 (2.9%) | * | * | * | - | - | - | * |
| **2017** | | | | | | | | |
| No | 211 (96.3%) | 28 (87.5%) | 109 (98.2%) | 72 (98.6%) | - | - | - | * |
| Yes | 8 (3.7%) | * | * | * | - | - | - | * |

| UK & Ireland \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 393)** | **SMA Type 1 (N = 69)** | **SMA Type 2 (N = 183)** | **SMA Type 3 (N = 123)** | **SMA Type 4 (N = 0)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 1)** | **SMA Type Missing (N = 17)** |
| **2018** | | | | | | | | |
| No | 238 (97.5%) | 35 (97.2%) | 120 (98.4%) | 81 (96.4%) | - | - | - | * |
| Yes | 6 (2.5%) | * | * | * | - | - | - | - |
| **2019** | | | | | | | | |
| No | 254 (96.9%) | 37 (97.4%) | 132 (99.2%) | 83 (94.3%) | - | - | - | * |
| Yes | 8 (3.1%) | * | * | 5 (5.7%) | - | - | - | * |
| **2020** | | | | | | | | |
| No | 259 (98.5%) | 36 (97.3%) | 137 (99.3%) | 83 (97.6%) | - | - | - | * |
| Yes | * | * | * | * | - | - | - | - |
| **2021** | | | | | | | | |
| No | 261 (96.7%) | 39 (100.0%) | 138 (97.2%) | 82 (95.3%) | - | - | - | * |
| Yes | 9 (3.3%) | - | * | * | - | - | - | * |
| **2022** | | | | | | | | |
| No | 286 (93.2%) | 47 (90.4%) | 143 (94.7%) | 88 (92.6%) | - | - | * | 7 (87.5%) |
| Yes | 21 (6.8%) | 5 (9.6%) | 8 (5.3%) | 7 (7.4%) | - | - | - | * |
| **2023** | | | | | | | | |
| No | 306 (97.8%) | 48 (96.0%) | 151 (97.4%) | 97 (99.0%) | - | - | * | 9 (100.0%) |
| Yes | 7 (2.2%) | * | * | * | - | - | - | - |
| **Treated with at least one DMT; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 37 (100.0%) | 6 (100.0%) | 18 (100.0%) | 9 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 38 (100.0%) | 8 (100.0%) | 18 (100.0%) | 9 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 204 (100.0%) | 33 (100.0%) | 101 (100.0%) | 66 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |

| UK & Ireland \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 393)** | **SMA Type 1 (N = 69)** | **SMA Type 2 (N = 183)** | **SMA Type 3 (N = 123)** | **SMA Type 4 (N = 0)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 1)** | **SMA Type Missing (N = 17)** |
| No | 202 (98.5%) | 26 (96.3%) | 106 (99.1%) | 68 (98.6%) | - | - | - | * |
| Yes | * | * | * | * | - | - | - | - |
| **2018** | | | | | | | | |
| No | 222 (96.5%) | 30 (88.2%) | 117 (98.3%) | 74 (97.4%) | - | - | - | * |
| Yes | 8 (3.5%) | * | * | * | - | - | - | - |
| **2019** | | | | | | | | |
| No | 228 (91.2%) | 29 (80.6%) | 121 (92.4%) | 76 (93.8%) | - | - | - | * |
| Yes | 22 (8.8%) | 7 (19.4%) | 10 (7.6%) | 5 (6.2%) | - | - | - | - |
| **2020** | | | | | | | | |
| No | 214 (85.6%) | 28 (77.8%) | 116 (87.2%) | 68 (86.1%) | - | - | - | * |
| Yes | 36 (14.4%) | 8 (22.2%) | 17 (12.8%) | 11 (13.9%) | - | - | - | - |
| **2021** | | | | | | | | |
| No | 168 (70.3%) | 25 (73.5%) | 88 (68.2%) | 54 (72.0%) | - | - | - | * |
| Yes | 71 (29.7%) | 9 (26.5%) | 41 (31.8%) | 21 (28.0%) | - | - | - | - |
| **2022** | | | | | | | | |
| No | 158 (56.8%) | 28 (62.2%) | 85 (61.6%) | 40 (46.0%) | - | - | - | 5 (71.4%) |
| Yes | 120 (43.2%) | 17 (37.8%) | 53 (38.4%) | 47 (54.0%) | - | - | * | * |
| **2023** | | | | | | | | |
| No | 168 (71.5%) | 25 (78.1%) | 83 (71.6%) | 55 (68.8%) | - | - | - | 5 (83.3%) |
| Yes | 67 (28.5%) | 7 (21.9%) | 33 (28.4%) | 25 (31.2%) | - | - | * | * |
| **Treated with more than one DMT; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 37 (100.0%) | 6 (100.0%) | 18 (100.0%) | 9 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 38 (100.0%) | 8 (100.0%) | 18 (100.0%) | 9 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 204 (100.0%) | 33 (100.0%) | 101 (100.0%) | 66 (100.0%) | - | - | - | * |

| UK & Ireland \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 393)** | **SMA Type 1 (N = 69)** | **SMA Type 2 (N = 183)** | **SMA Type 3 (N = 123)** | **SMA Type 4 (N = 0)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 1)** | **SMA Type Missing (N = 17)** |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 205 (100.0%) | 27 (100.0%) | 107 (100.0%) | 69 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 230 (100.0%) | 34 (100.0%) | 119 (100.0%) | 76 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 250 (100.0%) | 36 (100.0%) | 131 (100.0%) | 81 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 249 (99.6%) | 36 (100.0%) | 132 (99.2%) | 79 (100.0%) | - | - | - | * |
| Yes | * | - | * | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 236 (98.7%) | 33 (97.1%) | 127 (98.4%) | 75 (100.0%) | - | - | - | * |
| Yes | * | * | * | - | - | - | - | - |
| **2022** | | | | | | | | |
| No | 276 (99.3%) | 44 (97.8%) | 137 (99.3%) | 87 (100.0%) | - | - | * | 7 (100.0%) |
| Yes | * | * | * | - | - | - | - | - |
| **2023** | | | | | | | | |
| No | 235 (100.0%) | 32 (100.0%) | 116 (100.0%) | 80 (100.0%) | - | - | * | 6 (100.0%) |
| Yes | - | - | - | - | - | - | - | - |
| **Treated with nusinersen; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 37 (100.0%) | 6 (100.0%) | 18 (100.0%) | 9 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 38 (100.0%) | 8 (100.0%) | 18 (100.0%) | 9 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |

| UK & Ireland \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 393)** | **SMA Type 1 (N = 69)** | **SMA Type 2 (N = 183)** | **SMA Type 3 (N = 123)** | **SMA Type 4 (N = 0)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 1)** | **SMA Type Missing (N = 17)** |
| **2014-2016** | | | | | | | | |
| No | 204 (100.0%) | 33 (100.0%) | 101 (100.0%) | 66 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 203 (99.0%) | 26 (96.3%) | 107 (100.0%) | 68 (98.6%) | - | - | - | * |
| Yes | * | * | - | * | - | - | - | - |
| **2018** | | | | | | | | |
| No | 224 (97.4%) | 30 (88.2%) | 119 (100.0%) | 74 (97.4%) | - | - | - | * |
| Yes | 6 (2.6%) | * | - | * | - | - | - | - |
| **2019** | | | | | | | | |
| No | 230 (92.0%) | 29 (80.6%) | 123 (93.9%) | 76 (93.8%) | - | - | - | * |
| Yes | 20 (8.0%) | 7 (19.4%) | 8 (6.1%) | 5 (6.2%) | - | - | - | - |
| **2020** | | | | | | | | |
| No | 221 (88.4%) | 29 (80.6%) | 122 (91.7%) | 68 (86.1%) | - | - | - | * |
| Yes | 29 (11.6%) | 7 (19.4%) | 11 (8.3%) | 11 (13.9%) | - | - | - | - |
| **2021** | | | | | | | | |
| No | 200 (83.7%) | 28 (82.4%) | 117 (90.7%) | 54 (72.0%) | - | - | - | * |
| Yes | 39 (16.3%) | 6 (17.6%) | 12 (9.3%) | 21 (28.0%) | - | - | - | - |
| **2022** | | | | | | | | |
| No | 233 (83.8%) | 42 (93.3%) | 126 (91.3%) | 57 (65.5%) | - | - | * | 7 (100.0%) |
| Yes | 45 (16.2%) | * | 12 (8.7%) | 30 (34.5%) | - | - | - | - |
| **2023** | | | | | | | | |
| No | 206 (87.7%) | 31 (96.9%) | 106 (91.4%) | 63 (78.8%) | - | - | * | 5 (83.3%) |
| Yes | 29 (12.3%) | * | 10 (8.6%) | 17 (21.2%) | - | - | - | * |
| **Treated with onasemnogene abeparvovec-xioi; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 37 (100.0%) | 6 (100.0%) | 18 (100.0%) | 9 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |

| UK & Ireland \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 393)** | **SMA Type 1 (N = 69)** | **SMA Type 2 (N = 183)** | **SMA Type 3 (N = 123)** | **SMA Type 4 (N = 0)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 1)** | **SMA Type Missing (N = 17)** |
| **2011-2013** | | | | | | | | |
| No | 38 (100.0%) | 8 (100.0%) | 18 (100.0%) | 9 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 204 (100.0%) | 33 (100.0%) | 101 (100.0%) | 66 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 205 (100.0%) | 27 (100.0%) | 107 (100.0%) | 69 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 229 (99.6%) | 34 (100.0%) | 118 (99.2%) | 76 (100.0%) | - | - | - | * |
| Yes | * | - | * | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 249 (99.6%) | 36 (100.0%) | 130 (99.2%) | 81 (100.0%) | - | - | - | * |
| Yes | * | - | * | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 249 (99.6%) | 36 (100.0%) | 132 (99.2%) | 79 (100.0%) | - | - | - | * |
| Yes | * | - | * | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 234 (97.9%) | 31 (91.2%) | 127 (98.4%) | 75 (100.0%) | - | - | - | * |
| Yes | 5 (2.1%) | * | * | - | - | - | - | - |
| **2022** | | | | | | | | |
| No | 264 (95.0%) | 34 (75.6%) | 136 (98.6%) | 87 (100.0%) | - | - | - | 7 (100.0%) |
| Yes | 14 (5.0%) | 11 (24.4%) | * | - | - | - | * | - |
| **2023** | | | | | | | | |
| No | 229 (97.4%) | 27 (84.4%) | 116 (100.0%) | 80 (100.0%) | - | - | - | 6 (100.0%) |
| Yes | 6 (2.6%) | 5 (15.6%) | - | - | - | - | * | - |
| **Treated with risdiplan; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |

| UK & Ireland \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 393)** | **SMA Type 1 (N = 69)** | **SMA Type 2 (N = 183)** | **SMA Type 3 (N = 123)** | **SMA Type 4 (N = 0)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 1)** | **SMA Type Missing (N = 17)** |
| No | - | - | - | - | - | - | - | - |
| Yes | | | | | | | | |
| **2011-2013** | | | | | | | | |
| No | 37 (100.0%) | 6 (100.0%) | 18 (100.0%) | 9 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 38 (100.0%) | 8 (100.0%) | 18 (100.0%) | 9 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 204 (100.0%) | 33 (100.0%) | 101 (100.0%) | 66 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 204 (99.5%) | 27 (100.0%) | 106 (99.1%) | 69 (100.0%) | - | - | - | * |
| Yes | * | - | * | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 229 (99.6%) | 34 (100.0%) | 118 (99.2%) | 76 (100.0%) | - | - | - | * |
| Yes | * | - | * | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 249 (99.6%) | 36 (100.0%) | 130 (99.2%) | 81 (100.0%) | - | - | - | * |
| Yes | * | - | * | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 243 (97.2%) | 35 (97.2%) | 127 (95.5%) | 79 (100.0%) | - | - | - | * |
| Yes | 7 (2.8%) | * | 6 (4.5%) | - | - | - | - | - |
| **2022** | | | | | | | | |
| No | 209 (87.4%) | 33 (97.1%) | 100 (77.5%) | 75 (100.0%) | - | - | - | * |
| Yes | 30 (12.6%) | * | 29 (22.5%) | - | - | - | - | - |
| **2023** | | | | | | | | |
| No | 215 (77.3%) | 41 (91.1%) | 98 (71.0%) | 70 (80.5%) | - | - | * | 5 (71.4%) |
| Yes | 63 (22.7%) | * | 40 (29.0%) | 17 (19.5%) | - | - | - | * |

| UK & Ireland \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 393)** | **SMA Type 1 (N = 69)** | **SMA Type 2 (N = 183)** | **SMA Type 3 (N = 123)** | **SMA Type 4 (N = 0)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 1)** | **SMA Type Missing (N = 17)** |
| **At least one episode of invasive ventilation; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 37 (100.0%) | 6 (100.0%) | 18 (100.0%) | 9 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 38 (100.0%) | 8 (100.0%) | 18 (100.0%) | 9 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 202 (99.0%) | 32 (97.0%) | 101 (100.0%) | 65 (98.5%) | - | - | - | * |
| Yes | * | * | - | * | - | - | - | - |
| **2017** | | | | | | | | |
| No | 203 (99.0%) | 25 (92.6%) | 107 (100.0%) | 69 (100.0%) | - | - | - | * |
| Yes | * | * | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 227 (98.7%) | 32 (94.1%) | 119 (100.0%) | 75 (98.7%) | - | - | - | * |
| Yes | * | * | - | * | - | - | - | - |
| **2019** | | | | | | | | |
| No | 248 (99.2%) | 34 (94.4%) | 131 (100.0%) | 81 (100.0%) | - | - | - | * |
| Yes | * | * | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 245 (98.0%) | 32 (88.9%) | 132 (99.2%) | 79 (100.0%) | - | - | - | * |
| Yes | 5 (2.0%) | * | * | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 236 (98.7%) | 31 (91.2%) | 129 (100.0%) | 75 (100.0%) | - | - | - | * |
| Yes | * | * | - | - | - | - | - | - |
| **2022** | | | | | | | | |
| No | 276 (99.3%) | 43 (95.6%) | 138 (100.0%) | 87 (100.0%) | - | - | * | 7 (100.0%) |
| Yes | * | * | - | - | - | - | - | - |
| **2023** | | | | | | | | |

| UK & Ireland \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 393)** | **SMA Type 1 (N = 69)** | **SMA Type 2 (N = 183)** | **SMA Type 3 (N = 123)** | **SMA Type 4 (N = 0)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 1)** | **SMA Type Missing (N = 17)** |
| No | 233 (99.1%) | 31 (96.9%) | 115 (99.1%) | 80 (100.0%) | - | - | * | 6 (100.0%) |
| Yes | * | * | * | - | - | - | - | - |
| **At least one episode of non-invasive ventilation; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 37 (100.0%) | 6 (100.0%) | 18 (100.0%) | 9 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 38 (100.0%) | 8 (100.0%) | 18 (100.0%) | 9 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 193 (94.6%) | 29 (87.9%) | 94 (93.1%) | 66 (100.0%) | - | - | - | * |
| Yes | 11 (5.4%) | * | 7 (6.9%) | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 188 (91.7%) | 22 (81.5%) | 96 (89.7%) | 68 (98.6%) | - | - | - | * |
| Yes | 17 (8.3%) | 5 (18.5%) | 11 (10.3%) | * | - | - | - | - |
| **2018** | | | | | | | | |
| No | 208 (90.4%) | 26 (76.5%) | 107 (89.9%) | 74 (97.4%) | - | - | - | * |
| Yes | 22 (9.6%) | 8 (23.5%) | 12 (10.1%) | * | - | - | - | - |
| **2019** | | | | | | | | |
| No | 223 (89.2%) | 27 (75.0%) | 116 (88.5%) | 78 (96.3%) | - | - | - | * |
| Yes | 27 (10.8%) | 9 (25.0%) | 15 (11.5%) | * | - | - | - | - |
| **2020** | | | | | | | | |
| No | 211 (84.4%) | 24 (66.7%) | 112 (84.2%) | 73 (92.4%) | - | - | - | * |
| Yes | 39 (15.6%) | 12 (33.3%) | 21 (15.8%) | 6 (7.6%) | - | - | - | - |
| **2021** | | | | | | | | |
| No | 210 (87.9%) | 24 (70.6%) | 113 (87.6%) | 72 (96.0%) | - | - | - | * |
| Yes | 29 (12.1%) | 10 (29.4%) | 16 (12.4%) | * | - | - | - | - |
| **2022** | | | | | | | | |

| UK & Ireland \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 393)** | **SMA Type 1 (N = 69)** | **SMA Type 2 (N = 183)** | **SMA Type 3 (N = 123)** | **SMA Type 4 (N = 0)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 1)** | **SMA Type Missing (N = 17)** |
| No | 231 (83.1%) | 31 (68.9%) | 111 (80.4%) | 81 (93.1%) | - | - | * | 7 (100.0%) |
| Yes | 47 (16.9%) | 14 (31.1%) | 27 (19.6%) | 6 (6.9%) | - | - | - | - |
| **2023** | | | | | | | | |
| No | 199 (84.7%) | 20 (62.5%) | 95 (81.9%) | 77 (96.2%) | - | - | * | 6 (100.0%) |
| Yes | 36 (15.3%) | 12 (37.5%) | 21 (18.1%) | * | - | - | - | - |
| **At least one episode of feeding tube usage; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 37 (100.0%) | 6 (100.0%) | 18 (100.0%) | 9 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 38 (100.0%) | 8 (100.0%) | 18 (100.0%) | 9 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 174 (85.3%) | 16 (48.5%) | 89 (88.1%) | 65 (98.5%) | - | - | - | * |
| Yes | 30 (14.7%) | 17 (51.5%) | 12 (11.9%) | * | - | - | - | - |
| **2017** | | | | | | | | |
| No | 184 (89.8%) | 15 (55.6%) | 98 (91.6%) | 69 (100.0%) | - | - | - | * |
| Yes | 21 (10.2%) | 12 (44.4%) | 9 (8.4%) | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 199 (86.5%) | 16 (47.1%) | 107 (89.9%) | 75 (98.7%) | - | - | - | * |
| Yes | 31 (13.5%) | 18 (52.9%) | 12 (10.1%) | * | - | - | - | - |
| **2019** | | | | | | | | |
| No | 228 (91.2%) | 23 (63.9%) | 122 (93.1%) | 81 (100.0%) | - | - | - | * |
| Yes | 22 (8.8%) | 13 (36.1%) | 9 (6.9%) | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 229 (91.6%) | 25 (69.4%) | 124 (93.2%) | 78 (98.7%) | - | - | - | * |
| Yes | 21 (8.4%) | 11 (30.6%) | 9 (6.8%) | * | - | - | - | - |
| **2021** | | | | | | | | |
| No | 219 (91.6%) | 24 (70.6%) | 120 (93.0%) | 74 (98.7%) | - | - | - | * |

| UK & Ireland \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 393)** | **SMA Type 1 (N = 69)** | **SMA Type 2 (N = 183)** | **SMA Type 3 (N = 123)** | **SMA Type 4 (N = 0)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 1)** | **SMA Type Missing (N = 17)** |
| Yes | 20 (8.4%) | 10 (29.4%) | 9 (7.0%) | * | - | - | - | - |
| **2022** | | | | | | | | |
| No | 258 (92.8%) | 31 (68.9%) | 132 (95.7%) | 87 (100.0%) | - | - | * | 7 (100.0%) |
| Yes | 20 (7.2%) | 14 (31.1%) | 6 (4.3%) | - | - | - | - | - |
| **2023** | | | | | | | | |
| No | 223 (94.9%) | 25 (78.1%) | 111 (95.7%) | 80 (100.0%) | - | - | * | 6 (100.0%) |
| Yes | 12 (5.1%) | 7 (21.9%) | 5 (4.3%) | - | - | - | - | - |
| **At least one episode of wheelchair usage; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 10 (27.0%) | 5 (83.3%) | * | * | - | - | - | * |
| Yes | 27 (73.0%) | * | 16 (88.9%) | 7 (77.8%) | - | - | - | * |
| **2011-2013** | | | | | | | | |
| No | 34 (89.5%) | 8 (100.0%) | 15 (83.3%) | 8 (88.9%) | - | - | - | * |
| Yes | * | - | * | * | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 72 (35.3%) | 20 (60.6%) | 25 (24.8%) | 23 (34.8%) | - | - | - | * |
| Yes | 132 (64.7%) | 13 (39.4%) | 76 (75.2%) | 43 (65.2%) | - | - | - | - |
| **2017** | | | | | | | | |
| No | 69 (33.7%) | 12 (44.4%) | 30 (28.0%) | 25 (36.2%) | - | - | - | * |
| Yes | 136 (66.3%) | 15 (55.6%) | 77 (72.0%) | 44 (63.8%) | - | - | - | - |
| **2018** | | | | | | | | |
| No | 77 (33.5%) | 19 (55.9%) | 39 (32.8%) | 18 (23.7%) | - | - | - | * |
| Yes | 153 (66.5%) | 15 (44.1%) | 80 (67.2%) | 58 (76.3%) | - | - | - | - |
| **2019** | | | | | | | | |
| No | 106 (42.4%) | 23 (63.9%) | 51 (38.9%) | 30 (37.0%) | - | - | - | * |
| Yes | 144 (57.6%) | 13 (36.1%) | 80 (61.1%) | 51 (63.0%) | - | - | - | - |
| **2020** | | | | | | | | |
| No | 128 (51.2%) | 24 (66.7%) | 64 (48.1%) | 38 (48.1%) | - | - | - | * |

| UK & Ireland \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 393)** | **SMA Type 1 (N = 69)** | **SMA Type 2 (N = 183)** | **SMA Type 3 (N = 123)** | **SMA Type 4 (N = 0)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 1)** | **SMA Type Missing (N = 17)** |
| Yes | 122 (48.8%) | 12 (33.3%) | 69 (51.9%) | 41 (51.9%) | - | - | - | - |
| **2021** | | | | | | | | |
| No | 134 (56.1%) | 26 (76.5%) | 70 (54.3%) | 37 (49.3%) | - | - | - | * |
| Yes | 105 (43.9%) | 8 (23.5%) | 59 (45.7%) | 38 (50.7%) | - | - | - | - |
| **2022** | | | | | | | | |
| No | 168 (60.4%) | 34 (75.6%) | 80 (58.0%) | 46 (52.9%) | - | - | * | 7 (100.0%) |
| Yes | 110 (39.6%) | 11 (24.4%) | 58 (42.0%) | 41 (47.1%) | - | - | - | - |
| **2023** | | | | | | | | |
| No | 170 (72.3%) | 26 (81.2%) | 80 (69.0%) | 57 (71.2%) | - | - | * | 6 (100.0%) |
| Yes | 65 (27.7%) | 6 (18.8%) | 36 (31.0%) | 23 (28.7%) | - | - | - | - |
| **At least one reported measure by available motor function scale or test and by at least one PRO; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 37 (100.0%) | 6 (100.0%) | 18 (100.0%) | 9 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 38 (100.0%) | 8 (100.0%) | 18 (100.0%) | 9 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 204 (100.0%) | 33 (100.0%) | 101 (100.0%) | 66 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 205 (100.0%) | 27 (100.0%) | 107 (100.0%) | 69 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 230 (100.0%) | 34 (100.0%) | 119 (100.0%) | 76 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |

| UK & Ireland \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 393)** | **SMA Type 1 (N = 69)** | **SMA Type 2 (N = 183)** | **SMA Type 3 (N = 123)** | **SMA Type 4 (N = 0)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 1)** | **SMA Type Missing (N = 17)** |
| No | 250 (100.0%) | 36 (100.0%) | 131 (100.0%) | 81 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 250 (100.0%) | 36 (100.0%) | 133 (100.0%) | 79 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 239 (100.0%) | 34 (100.0%) | 129 (100.0%) | 75 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2022** | | | | | | | | |
| No | 278 (100.0%) | 45 (100.0%) | 138 (100.0%) | 87 (100.0%) | - | - | * | 7 (100.0%) |
| Yes | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| No | 235 (100.0%) | 32 (100.0%) | 116 (100.0%) | 80 (100.0%) | - | - | * | 6 (100.0%) |
| Yes | - | - | - | - | - | - | - | - |
| **At least three reported measures by available motor function scale or test and by at least one PRO; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 37 (100.0%) | 6 (100.0%) | 18 (100.0%) | 9 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 38 (100.0%) | 8 (100.0%) | 18 (100.0%) | 9 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 204 (100.0%) | 33 (100.0%) | 101 (100.0%) | 66 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 205 (100.0%) | 27 (100.0%) | 107 (100.0%) | 69 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |

| UK & Ireland \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 393)** | **SMA Type 1 (N = 69)** | **SMA Type 2 (N = 183)** | **SMA Type 3 (N = 123)** | **SMA Type 4 (N = 0)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 1)** | **SMA Type Missing (N = 17)** |
| **2018** | | | | | | | | |
| No | 230 (100.0%) | 34 (100.0%) | 119 (100.0%) | 76 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 250 (100.0%) | 36 (100.0%) | 131 (100.0%) | 81 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 250 (100.0%) | 36 (100.0%) | 133 (100.0%) | 79 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 239 (100.0%) | 34 (100.0%) | 129 (100.0%) | 75 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2022** | | | | | | | | |
| No | 278 (100.0%) | 45 (100.0%) | 138 (100.0%) | 87 (100.0%) | - | - | * | 7 (100.0%) |
| Yes | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| No | 235 (100.0%) | 32 (100.0%) | 116 (100.0%) | 80 (100.0%) | - | - | * | 6 (100.0%) |
| Yes | - | - | - | - | - | - | - | - |
| **Available number of records of each motor function scale by patient** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |

| UK & Ireland \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 393)** | **SMA Type 1 (N = 69)** | **SMA Type 2 (N = 183)** | **SMA Type 3 (N = 123)** | **SMA Type 4 (N = 0)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 1)** | **SMA Type Missing (N = 17)** |
| **2017** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2022** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **Available number of records of PRO** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |

| UK & Ireland \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 393)** | **SMA Type 1 (N = 69)** | **SMA Type 2 (N = 183)** | **SMA Type 3 (N = 123)** | **SMA Type 4 (N = 0)** | **SMA Type Other (N = 0)** | **SMA Type Presymptomatic (N = 1)** | **SMA Type Missing (N = 17)** |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2022** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |

| Sweden \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 175)** | **SMA Type 1 (N = 28)** | **SMA Type 2 (N = 69)** | **SMA Type 3 (N = 71)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 2)** |
| **Calendar year of registry entry; n (%)** | | | | | | | | |
| 2008 | - | - | - | - | - | - | - | - |
| 2009 | - | - | - | - | - | - | - | - |
| 2010 | 29 (16.6%) | * | 20 (29.0%) | 7 (9.9%) | - | - | - | * |
| 2011 | 29 (16.6%) | - | 10 (14.5%) | 18 (25.4%) | - | * | - | - |
| 2012 | 6 (3.4%) | * | * | * | - | - | - | - |
| 2013 | 7 (4.0%) | - | * | 5 (7.0%) | - | - | - | - |
| 2014 | 16 (9.1%) | - | 8 (11.6%) | 8 (11.3%) | - | - | - | - |
| 2015 | * | * | * | * | - | - | - | - |
| 2016 | 8 (4.6%) | * | - | * | - | * | - | - |
| 2017 | 7 (4.0%) | * | * | - | - | - | - | - |
| 2018 | 18 (10.3%) | * | 6 (8.7%) | 10 (14.1%) | - | - | - | - |
| 2019 | 20 (11.4%) | 5 (17.9%) | 6 (8.7%) | 8 (11.3%) | * | - | - | - |
| 2020 | 9 (5.1%) | 5 (17.9%) | * | - | - | - | - | - |
| 2021 | 12 (6.9%) | * | * | 5 (7.0%) | * | * | - | - |
| 2022 | 8 (4.6%) | 5 (17.9%) | * | - | - | - | - | * |
| 2023 | * | - | - | * | - | - | - | - |
| Missing | * | - | * | - | - | - | - | - |
| **Calendar year of death; n (%)** | | | | | | | | |
| 2009 | - | - | - | - | - | - | - | - |
| 2010 | - | - | - | - | - | - | - | - |
| 2012 | - | - | - | - | - | - | - | - |
| 2013 | - | - | - | - | - | - | - | - |
| 2014 | - | - | - | - | - | - | - | - |
| 2015 | - | - | - | - | - | - | - | - |
| 2016 | * | - | * | - | - | - | - | - |
| 2017 | - | - | - | - | - | - | - | - |
| 2018 | - | - | - | - | - | - | - | - |

| Sweden \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 175)** | **SMA Type 1 (N = 28)** | **SMA Type 2 (N = 69)** | **SMA Type 3 (N = 71)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 2)** |
| 2019 | - | - | - | - | - | - | - | - |
| 2020 | - | - | - | - | - | - | - | - |
| 2021 | * | * | - | - | - | - | - | - |
| 2022 | * | * | * | - | - | - | - | - |
| 2023 | * | * | - | - | - | - | - | - |
| Missing | 168 (96.0%) | 24 (85.7%) | 66 (95.7%) | 71 (100.0%) | * | * | - | * |
| **Sex; n (%)** | | | | | | | | |
| Female | 89 (50.9%) | 11 (39.3%) | 35 (50.7%) | 40 (56.3%) | * | * | - | * |
| Male | 86 (49.1%) | 17 (60.7%) | 34 (49.3%) | 31 (43.7%) | * | * | - | * |
| Missing | - | - | - | - | - | - | - | - |
| **Class of age at symptom onset; n (%)** | | | | | | | | |
| Presymptomatic | - | - | - | - | - | - | - | - |
| Prenatal | - | - | - | - | - | - | - | - |
| < 1 month | * | * | - | - | - | - | - | - |
| [1 - 3 months) | 13 (7.4%) | 12 (42.9%) | * | - | - | - | - | - |
| [3 - 6 months) | 15 (8.6%) | 8 (28.6%) | 6 (8.7%) | * | - | - | - | - |
| [6 - 18 months) | 52 (29.7%) | * | 43 (62.3%) | 8 (11.3%) | - | - | - | - |
| [1.5 - 2 years) | 20 (11.4%) | - | * | 17 (23.9%) | - | - | - | - |
| [2 - 6 years) | 23 (13.1%) | - | - | 23 (32.4%) | - | - | - | - |
| [6 - 11 years) | 7 (4.0%) | - | - | 6 (8.5%) | - | - | - | * |
| [11 - 18 years) | 8 (4.6%) | - | - | 6 (8.5%) | * | * | - | - |
| 18 years + | * | - | - | * | * | * | - | - |
| Missing | 33 (18.9%) | 6 (21.4%) | 16 (23.2%) | 9 (12.7%) | - | * | - | * |
| **Best functional SMA status; n (%)** | | | | | | | | |
| Non-sitter | 9 (5.1%) | 7 (25.0%) | * | - | - | - | - | - |
| Sitter | 37 (21.1%) | 10 (35.7%) | 27 (39.1%) | - | - | - | - | - |
| Walker | 54 (30.9%) | * | 9 (13.0%) | 42 (59.2%) | - | * | - | - |
| Missing | 75 (42.9%) | 9 (32.1%) | 31 (44.9%) | 29 (40.8%) | * | * | - | * |

| Sweden \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 175)** | **SMA Type 1 (N = 28)** | **SMA Type 2 (N = 69)** | **SMA Type 3 (N = 71)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 2)** |
| **Best achieved motor milestone; n (%)** | | | | | | | | |
| Climb stairs | 19 (10.9%) | - | - | 19 (26.8%) | - | - | - | - |
| Walk 10 metres without assistance | 9 (5.1%) | - | * | 6 (8.5%) | - | * | - | - |
| Walk without assistance | 20 (11.4%) | * | * | 17 (23.9%) | - | - | - | - |
| Walk with assistance | 6 (3.4%) | - | 6 (8.7%) | - | - | - | - | - |
| Stand without assistance | * | - | * | - | - | - | - | - |
| Stand with assistance | 6 (3.4%) | * | 5 (7.2%) | - | - | - | - | - |
| Crawl | 7 (4.0%) | - | 7 (10.1%) | - | - | - | - | - |
| Sit without support | 23 (13.1%) | 9 (32.1%) | 14 (20.3%) | - | - | - | - | - |
| Roll onto side | 8 (4.6%) | 7 (25.0%) | * | - | - | - | - | - |
| Hold head without support | * | - | * | - | - | - | - | - |
| Unknown | - | - | - | - | - | - | - | - |
| Missing | 75 (42.9%) | 9 (32.1%) | 31 (44.9%) | 29 (40.8%) | * | * | - | * |
| **SMN1 gene mutation type; n (%)** | | | | | | | | |
| Compound heterozygous deletion exon 7 | * | - | * | - | - | - | - | - |
| Compound heterozygous substitutions | - | - | - | - | - | - | - | - |
| Homozygous deletion exon 7 | 118 (67.4%) | 22 (78.6%) | 45 (65.2%) | 47 (66.2%) | * | * | - | * |
| Missing | 54 (30.9%) | 6 (21.4%) | 21 (30.4%) | 24 (33.8%) | * | * | - | - |
| **Number of SMN2 copies; n (%)** | | | | | | | | |
| 0 | - | - | - | - | - | - | - | - |
| 1 | - | - | - | - | - | - | - | - |
| 2 | 27 (15.4%) | 20 (71.4%) | 5 (7.2%) | * | - | - | - | - |

| Sweden \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 175)** | **SMA Type 1 (N = 28)** | **SMA Type 2 (N = 69)** | **SMA Type 3 (N = 71)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 2)** |
| 3 | 60 (34.3%) | * | 42 (60.9%) | 14 (19.7%) | - | * | - | * |
| 4 | 31 (17.7%) | - | - | 29 (40.8%) | * | - | - | * |
| >4 | - | - | - | - | - | - | - | - |
| Other | - | - | - | - | - | - | - | - |
| Missing | 57 (32.6%) | 6 (21.4%) | 22 (31.9%) | 26 (36.6%) | * | * | - | - |
| **Methods used for genetic testing; n (%)** | | | | | | | | |
| DNA Sequencing | - | - | - | - | - | - | - | - |
| HRM | - | - | - | - | - | - | - | - |
| MLPA | 124 (70.9%) | 22 (78.6%) | 49 (71.0%) | 49 (69.0%) | * | * | - | * |
| RFLP | - | - | - | - | - | - | - | - |
| ddPCR | - | - | - | - | - | - | - | - |
| qRT-PCR | - | - | - | - | - | - | - | - |
| Missing | 51 (29.1%) | 6 (21.4%) | 20 (29.0%) | 22 (31.0%) | * | * | - | - |
| **Duration of follow up (months)** | | | | | | | | |
| Mean (SD) | 96.9 (49.9) | 55.9 (35.9) | 107.9 (49.7) | 104.9 (45) | * | * | - | * |
| Median [IQR] | 104.5 [51.2, 148] | 47 [31, 73] | 117 [59.8, 155] | 117 [60, 147] | * | * | - | * |
| Missing; n (%) | * | - | * | - | - | - | - | - |
| **Duration of SMA (months)** | | | | | | | | |
| Mean (SD) | 211.5 (171.6) | 67.2 (52.7) | 204.7 (153.2) | 252.2 (173.5) | * | * | - | * |
| Median [IQR] | 159.5 [84.2, 272] | 52.5 [28.8, 84.8] | 171 [77, 260] | 197.5 [129.2, 335.5] | * | * | - | * |
| Missing; n (%) | 33 (18.9%) | 6 (21.4%) | 16 (23.2%) | 9 (12.7%) | - | * | - | * |
| **Duration between two consecutive visits (months)** | | | | | | | | |
| Mean (SD) | 11.2 (11.5) | 5.1 (1.8) | 13 (15.9) | 11.8 (7.3) | * | * | - | * |
| Median [IQR] | 8.3 [5.1, 12] | 4.6 [4.1, 6] | 8.8 [5.9, 12.2] | 9.4 [6.5, 15.6] | * | * | - | * |
| Missing; n (%) | * | - | * | * | - | - | - | - |

| Sweden \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 175)** | **SMA Type 1 (N = 28)** | **SMA Type 2 (N = 69)** | **SMA Type 3 (N = 71)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 2)** |
| **Duration between genetic report date and registry entry (months)** | | | | | | | | |
| Mean (SD) | 30.9 (39.8) | 6 (14.8) | 36.8 (38.5) | 36.8 (44.3) | * | * | - | * |
| Median [IQR] | 8 [1, 56.2] | - | 19 [1, 75] | 12 [3, 60] | * | * | - | * |
| Missing; n (%) | 51 (29.1%) | 6 (21.4%) | 20 (29.0%) | 22 (31.0%) | * | * | - | - |
| **Reason for genetic testing; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 27 (100.0%) | * | 13 (100.0%) | 12 (100.0%) | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Family screening | * | - | - | * | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 12 (92.3%) | * | 7 (100.0%) | * | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| Family screening | * | * | * | * | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 20 (83.3%) | * | * | 14 (87.5%) | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| Family screening | * | * | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 8 (88.9%) | * | 5 (100.0%) | - | - | - | - | - |

| Sweden \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 175)** | **SMA Type 1 (N = 28)** | **SMA Type 2 (N = 69)** | **SMA Type 3 (N = 71)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 2)** |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| Family screening | * | - | * | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 10 (83.3%) | * | * | * | - | - | - | * |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| Family screening | * | - | * | * | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 13 (81.2%) | * | 5 (83.3%) | * | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| Family screening | * | * | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 9 (90.0%) | * | * | * | * | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| Family screening | * | - | * | - | - | * | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 6 (75.0%) | * | * | * | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2022** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | 5 (100.0%) | * | * | - | - | - | - | * |

| Sweden \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 175)** | **SMA Type 1 (N = 28)** | **SMA Type 2 (N = 69)** | **SMA Type 3 (N = 71)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 2)** |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| Family screening | - | - | - | - | - | - | - | - |
| Newborn screening | - | - | - | - | - | - | - | - |
| No screening | - | - | - | - | - | - | - | - |
| Prenatal screening | - | - | - | - | - | - | - | - |
| **Age at onset of SMA symptoms (years)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | 4 (6.6) | * | 0.8 (0.4) | 4.9 (6.6) | * | * | - | - |
| Median [IQR] | 1.5 [1, 2.9] | * | 1 [0.5, 1] | 2 [1.5, 4.5] | * | * | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | 1.3 (0.6) | * | 0.8 (0.3) | 1.8 (0.4) | - | - | - | - |
| Median [IQR] | 1.4 [0.9, 1.8] | * | 0.8 [0.5, 1] | 1.8 [1.5, 2] | - | - | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | 2.1 (2.4) | * | * | 3.1 (2.3) | - | - | - | * |
| Median [IQR] | 1.5 [0.4, 2] | * | * | 2 [1.9, 3.1] | - | - | - | * |
| **2017** | | | | | | | | |
| Mean (SD) | 1 (1) | * | * | * | - | - | - | - |
| Median [IQR] | 0.8 [0.3, 1.2] | * | * | * | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | * | - | * | - | - | - | - | - |
| Median [IQR] | * | - | * | - | - | - | - | - |
| **2019** | | | | | | | | |
| Mean (SD) | 1.7 (3.6) | * | 0.7 (0.2) | * | - | - | - | - |
| Median [IQR] | 0.5 [0.2, 1] | * | 0.6 [0.5, 1] | * | - | - | - | - |
| **2020** | | | | | | | | |
| Mean (SD) | 0.5 (0.4) | * | * | * | - | - | - | - |

| Sweden \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 175)** | **SMA Type 1 (N = 28)** | **SMA Type 2 (N = 69)** | **SMA Type 3 (N = 71)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 2)** |
| Median [IQR] | 0.4 [0.2, 0.6] | * | * | * | - | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | 2.2 (3.2) | * | * | * | - | - | - | - |
| Median [IQR] | 0.6 [0.4, 1.1] | * | * | * | - | - | - | - |
| **2022** | | | | | | | | |
| Mean (SD) | * | * | - | - | - | - | - | - |
| Median [IQR] | * | * | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **Age at genetic report date (years)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | 11.5 (15.3) | * | 11.6 (15.7) | 13.2 (15.4) | - | - | - | - |
| Median [IQR] | 2.5 [1.5, 20.8] | * | 1.7 [1.3, 27.1] | 3.3 [2.6, 20.6] | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | 9.1 (12) | * | 8.8 (6.8) | 11.1 (17) | - | - | - | - |
| Median [IQR] | 4 [1.9, 12.2] | * | 6.3 [3.2, 14.7] | 2.4 [2, 4] | - | - | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | 8.6 (13.5) | * | 4.2 (5.5) | 11.5 (15.3) | - | - | - | - |
| Median [IQR] | 2.7 [1.4, 8.5] | * | 1.5 [1.4, 2.2] | 5.6 [2.4, 10.1] | - | - | - | - |
| **2017** | | | | | | | | |
| Mean (SD) | 3.1 (6.3) | * | 5.2 (7.8) | - | - | - | - | - |
| Median [IQR] | 1.1 [0.6, 1.3] | * | 1.3 [1.1, 2.1] | - | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | 16.5 (14.7) | * | 21.3 (18.5) | * | - | - | - | * |
| Median [IQR] | 15.4 [4.1, 17.2] | * | 16 [5.2, 38] | * | - | - | - | * |
| **2019** | | | | | | | | |
| Mean (SD) | 9.8 (14.3) | * | 17.7 (20.1) | 8.2 (4.2) | - | - | - | - |
| Median [IQR] | 2.9 [0.7, 12.1] | * | 9.8 [1.1, 25.8] | 9 [5.4, 10.9] | - | - | - | - |

| Sweden \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 175)** | **SMA Type 1 (N = 28)** | **SMA Type 2 (N = 69)** | **SMA Type 3 (N = 71)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 2)** |
| **2020** | | | | | | | | |
| Mean (SD) | 18.8 (23.8) | * | * | * | * | - | - | - |
| Median [IQR] | 6 [1, 36] | * | * | * | * | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | 8.6 (12.2) | * | * | * | - | * | - | - |
| Median [IQR] | 1.2 [0.5, 12.2] | * | * | * | - | * | - | - |
| **2022** | | | | | | | | |
| Mean (SD) | 3.2 (5.4) | * | * | - | - | - | - | * |
| Median [IQR] | 0.5 [0.5, 0.9] | * | * | - | - | - | - | * |
| **2023** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **Age at registry entry (years)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | 16.4 (12.7) | * | 16.6 (13.3) | 18.2 (11.6) | - | - | - | * |
| Median [IQR] | 11.3 [6.5, 25.9] | * | 11.6 [7, 21.1] | 17.8 [6.2, 28.3] | - | - | - | * |
| **2011-2013** | | | | | | | | |
| Mean (SD) | 20.3 (16.9) | * | 13.8 (13.4) | 22.7 (16.2) | - | * | - | - |
| Median [IQR] | 13 [6.7, 29.9] | * | 9.6 [6.2, 12.7] | 19 [9.3, 32] | - | * | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | 12.3 (16.4) | * | 13.2 (14.3) | 11.5 (14.3) | - | * | - | - |
| Median [IQR] | 3.8 [1.9, 11.9] | * | 5.5 [1.8, 27.5] | 5.8 [3.2, 9.3] | - | * | - | - |
| **2017** | | | | | | | | |
| Mean (SD) | 1 (0.7) | * | * | - | - | - | - | - |
| Median [IQR] | 1 [0.4, 1.2] | * | * | - | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | 7.4 (6.8) | * | 6.8 (9) | 9.1 (4.9) | - | - | - | - |
| Median [IQR] | 5.1 [2.1, 11.9] | * | 1.9 [1.5, 6.7] | 7.2 [5, 13] | - | - | - | - |
| **2019** | | | | | | | | |

| Sweden \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 175)** | **SMA Type 1 (N = 28)** | **SMA Type 2 (N = 69)** | **SMA Type 3 (N = 71)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 2)** |
| Mean (SD) | 10.3 (16.5) | 0.1 (0.1) | 13.2 (24) | 10.1 (4.8) | * | - | - | - |
| Median [IQR] | 4.1 [0.6, 11.3] | 0.1 [-, 0.2] | 2.8 [1.1, 4.6] | 10.3 [8, 12.8] | * | - | - | - |
| **2020** | | | | | | | | |
| Mean (SD) | 2.9 (6) | 0.5 (0.3) | * | - | - | - | - | - |
| Median [IQR] | 1 [0.6, 1.2] | 0.6 [0.2, 0.6] | * | - | - | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | 18.9 (23.6) | * | * | 27.9 (26.4) | * | * | - | - |
| Median [IQR] | 4.3 [1.1, 37.2] | * | * | 14.6 [6.6, 44.4] | * | * | - | - |
| **2022** | | | | | | | | |
| Mean (SD) | 6.6 (11.5) | 0.6 (0.2) | * | - | - | - | - | * |
| Median [IQR] | 0.8 [0.5, 4.3] | 0.5 [0.5, 0.7] | * | - | - | - | - | * |
| **2023** | | | | | | | | |
| Mean (SD) | * | - | - | * | - | - | - | - |
| Median [IQR] | * | - | - | * | - | - | - | - |
| **Age at death (years)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| Mean (SD) | * | - | * | - | - | - | - | - |
| Median [IQR] | * | - | * | - | - | - | - | - |
| **2017** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |

| Sweden \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 175)** | **SMA Type 1 (N = 28)** | **SMA Type 2 (N = 69)** | **SMA Type 3 (N = 71)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 2)** |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | * | * | - | - | - | - | - | - |
| Median [IQR] | * | * | - | - | - | - | - | - |
| **2022** | | | | | | | | |
| Mean (SD) | * | * | * | - | - | - | - | - |
| Median [IQR] | * | * | * | - | - | - | - | - |
| **2023** | | | | | | | | |
| Mean (SD) | * | * | - | - | - | - | - | - |
| Median [IQR] | * | * | - | - | - | - | - | - |
| **Lost to follow-up; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 29 (100.0%) | * | 20 (100.0%) | 7 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 71 (100.0%) | * | 33 (100.0%) | 34 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 99 (100.0%) | 6 (100.0%) | 43 (100.0%) | 47 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 106 (100.0%) | 10 (100.0%) | 46 (100.0%) | 47 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |

| Sweden \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 175)** | **SMA Type 1 (N = 28)** | **SMA Type 2 (N = 69)** | **SMA Type 3 (N = 71)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 2)** |
| **2018** | | | | | | | | |
| No | 124 (100.0%) | 12 (100.0%) | 52 (100.0%) | 57 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 143 (100.0%) | 17 (100.0%) | 57 (100.0%) | 65 (100.0%) | * | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 152 (100.0%) | 22 (100.0%) | 61 (100.0%) | 65 (100.0%) | * | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 164 (100.0%) | 23 (100.0%) | 65 (100.0%) | 70 (100.0%) | * | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2022** | | | | | | | | |
| No | 170 (100.0%) | 26 (100.0%) | 67 (100.0%) | 70 (100.0%) | * | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| No | 169 (100.0%) | 25 (100.0%) | 66 (100.0%) | 71 (100.0%) | * | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **Treated with at least one DMT; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 29 (100.0%) | * | 20 (100.0%) | 7 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 71 (100.0%) | * | 33 (100.0%) | 34 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 96 (97.0%) | * | 42 (97.7%) | 47 (100.0%) | - | * | - | * |
| Yes | * | * | * | - | - | - | - | - |
| **2017** | | | | | | | | |

| Sweden \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 175)** | **SMA Type 1 (N = 28)** | **SMA Type 2 (N = 69)** | **SMA Type 3 (N = 71)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 2)** |
| No | 92 (86.8%) | * | 41 (89.1%) | 47 (100.0%) | - | * | - | * |
| Yes | 14 (13.2%) | 9 (90.0%) | 5 (10.9%) | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 73 (58.9%) | - | 35 (67.3%) | 36 (63.2%) | - | * | - | - |
| Yes | 51 (41.1%) | 12 (100.0%) | 17 (32.7%) | 21 (36.8%) | - | - | - | * |
| **2019** | | | | | | | | |
| No | 62 (43.1%) | - | 25 (43.1%) | 34 (52.3%) | * | * | - | - |
| Yes | 82 (56.9%) | 17 (100.0%) | 33 (56.9%) | 31 (47.7%) | - | - | - | * |
| **2020** | | | | | | | | |
| No | 54 (35.5%) | * | 23 (37.7%) | 26 (40.0%) | * | * | - | - |
| Yes | 98 (64.5%) | 20 (90.9%) | 38 (62.3%) | 39 (60.0%) | - | - | - | * |
| **2021** | | | | | | | | |
| No | 56 (34.1%) | * | 21 (32.3%) | 29 (41.4%) | * | * | - | - |
| Yes | 108 (65.9%) | 21 (91.3%) | 44 (67.7%) | 41 (58.6%) | - | * | - | * |
| **2022** | | | | | | | | |
| No | 64 (37.2%) | * | 26 (38.8%) | 30 (42.9%) | * | * | - | - |
| Yes | 108 (62.8%) | 24 (85.7%) | 41 (61.2%) | 40 (57.1%) | - | * | - | * |
| **2023** | | | | | | | | |
| No | 70 (40.5%) | 5 (17.9%) | 29 (43.3%) | 31 (43.7%) | * | * | - | * |
| Yes | 103 (59.5%) | 23 (82.1%) | 38 (56.7%) | 40 (56.3%) | - | * | - | * |
| **Treated with more than one DMT; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 29 (100.0%) | * | 20 (100.0%) | 7 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 71 (100.0%) | * | 33 (100.0%) | 34 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 99 (100.0%) | 6 (100.0%) | 43 (100.0%) | 47 (100.0%) | - | * | - | * |

| Sweden \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 175)** | **SMA Type 1 (N = 28)** | **SMA Type 2 (N = 69)** | **SMA Type 3 (N = 71)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 2)** |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 106 (100.0%) | 10 (100.0%) | 46 (100.0%) | 47 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 124 (100.0%) | 12 (100.0%) | 52 (100.0%) | 57 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 144 (100.0%) | 17 (100.0%) | 58 (100.0%) | 65 (100.0%) | * | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 152 (100.0%) | 22 (100.0%) | 61 (100.0%) | 65 (100.0%) | * | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 162 (98.8%) | 22 (95.7%) | 64 (98.5%) | 70 (100.0%) | * | * | - | * |
| Yes | * | * | * | - | - | - | - | - |
| **2022** | | | | | | | | |
| No | 123 (71.5%) | 16 (57.1%) | 53 (79.1%) | 48 (68.6%) | * | * | - | * |
| Yes | 49 (28.5%) | 12 (42.9%) | 14 (20.9%) | 22 (31.4%) | - | * | - | - |
| **2023** | | | | | | | | |
| No | 173 (100.0%) | 28 (100.0%) | 67 (100.0%) | 71 (100.0%) | * | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **Treated with nusinersen; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 29 (100.0%) | * | 20 (100.0%) | 7 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 71 (100.0%) | * | 33 (100.0%) | 34 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |

| Sweden \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 175)** | **SMA Type 1 (N = 28)** | **SMA Type 2 (N = 69)** | **SMA Type 3 (N = 71)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 2)** |
| **2014-2016** | | | | | | | | |
| No | 96 (97.0%) | * | 42 (97.7%) | 47 (100.0%) | - | * | - | * |
| Yes | * | * | * | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 92 (86.8%) | * | 41 (89.1%) | 47 (100.0%) | - | * | - | * |
| Yes | 14 (13.2%) | 9 (90.0%) | 5 (10.9%) | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 73 (58.9%) | - | 35 (67.3%) | 36 (63.2%) | - | * | - | - |
| Yes | 51 (41.1%) | 12 (100.0%) | 17 (32.7%) | 21 (36.8%) | - | - | - | * |
| **2019** | | | | | | | | |
| No | 62 (43.1%) | - | 25 (43.1%) | 34 (52.3%) | * | * | - | - |
| Yes | 82 (56.9%) | 17 (100.0%) | 33 (56.9%) | 31 (47.7%) | - | - | - | * |
| **2020** | | | | | | | | |
| No | 54 (35.5%) | * | 23 (37.7%) | 26 (40.0%) | * | * | - | - |
| Yes | 98 (64.5%) | 20 (90.9%) | 38 (62.3%) | 39 (60.0%) | - | - | - | * |
| **2021** | | | | | | | | |
| No | 59 (36.0%) | * | 24 (36.9%) | 29 (41.4%) | * | * | - | - |
| Yes | 105 (64.0%) | 21 (91.3%) | 41 (63.1%) | 41 (58.6%) | - | * | - | * |
| **2022** | | | | | | | | |
| No | 73 (42.4%) | 9 (32.1%) | 28 (41.8%) | 31 (44.3%) | * | * | - | * |
| Yes | 99 (57.6%) | 19 (67.9%) | 39 (58.2%) | 39 (55.7%) | - | * | - | * |
| **2023** | | | | | | | | |
| No | 126 (72.8%) | 20 (71.4%) | 45 (67.2%) | 54 (76.1%) | * | * | - | * |
| Yes | 47 (27.2%) | 8 (28.6%) | 22 (32.8%) | 17 (23.9%) | - | - | - | - |
| **Treated with onasemnogene abeparvovec-xioi; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 29 (100.0%) | * | 20 (100.0%) | 7 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |

| Sweden \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 175)** | **SMA Type 1 (N = 28)** | **SMA Type 2 (N = 69)** | **SMA Type 3 (N = 71)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 2)** |
| **2011-2013** | | | | | | | | |
| No | 71 (100.0%) | * | 33 (100.0%) | 34 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 99 (100.0%) | 6 (100.0%) | 43 (100.0%) | 47 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 106 (100.0%) | 10 (100.0%) | 46 (100.0%) | 47 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 124 (100.0%) | 12 (100.0%) | 52 (100.0%) | 57 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 144 (100.0%) | 17 (100.0%) | 58 (100.0%) | 65 (100.0%) | * | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 152 (100.0%) | 22 (100.0%) | 61 (100.0%) | 65 (100.0%) | * | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 162 (98.8%) | 22 (95.7%) | 64 (98.5%) | 70 (100.0%) | * | * | - | * |
| Yes | * | * | * | - | - | - | - | - |
| **2022** | | | | | | | | |
| No | 167 (97.1%) | 24 (85.7%) | 66 (98.5%) | 70 (100.0%) | * | * | - | * |
| Yes | 5 (2.9%) | * | * | - | - | - | - | - |
| **2023** | | | | | | | | |
| No | 168 (97.1%) | 24 (85.7%) | 66 (98.5%) | 71 (100.0%) | * | * | - | * |
| Yes | 5 (2.9%) | * | * | - | - | - | - | - |
| **Treated with risdiplan; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |

| Sweden \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 175)** | **SMA Type 1 (N = 28)** | **SMA Type 2 (N = 69)** | **SMA Type 3 (N = 71)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 2)** |
| No | - | - | - | - | - | - | - | - |
| Yes | | | | | | | | |
| **2011-2013** | | | | | | | | |
| No | 29 (100.0%) | * | 20 (100.0%) | 7 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 71 (100.0%) | * | 33 (100.0%) | 34 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 99 (100.0%) | 6 (100.0%) | 43 (100.0%) | 47 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 106 (100.0%) | 10 (100.0%) | 46 (100.0%) | 47 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 124 (100.0%) | 12 (100.0%) | 52 (100.0%) | 57 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 144 (100.0%) | 17 (100.0%) | 58 (100.0%) | 65 (100.0%) | * | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 152 (100.0%) | 22 (100.0%) | 61 (100.0%) | 65 (100.0%) | * | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2022** | | | | | | | | |
| No | 161 (98.2%) | 23 (100.0%) | 62 (95.4%) | 70 (100.0%) | * | * | - | * |
| Yes | * | - | * | - | - | - | - | - |
| **2023** | | | | | | | | |
| No | 119 (69.2%) | 15 (53.6%) | 52 (77.6%) | 47 (67.1%) | * | * | - | * |
| Yes | 53 (30.8%) | 13 (46.4%) | 15 (22.4%) | 23 (32.9%) | - | * | - | * |

| Sweden \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 175)** | **SMA Type 1 (N = 28)** | **SMA Type 2 (N = 69)** | **SMA Type 3 (N = 71)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 2)** |
| **At least one episode of invasive ventilation; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 29 (100.0%) | * | 20 (100.0%) | 7 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 71 (100.0%) | * | 33 (100.0%) | 34 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 98 (99.0%) | 6 (100.0%) | 42 (97.7%) | 47 (100.0%) | - | * | - | * |
| Yes | * | - | * | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 106 (100.0%) | 10 (100.0%) | 46 (100.0%) | 47 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 124 (100.0%) | 12 (100.0%) | 52 (100.0%) | 57 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 144 (100.0%) | 17 (100.0%) | 58 (100.0%) | 65 (100.0%) | * | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 152 (100.0%) | 22 (100.0%) | 61 (100.0%) | 65 (100.0%) | * | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 164 (100.0%) | 23 (100.0%) | 65 (100.0%) | 70 (100.0%) | * | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2022** | | | | | | | | |
| No | 171 (99.4%) | 28 (100.0%) | 66 (98.5%) | 70 (100.0%) | * | * | - | * |
| Yes | * | - | * | - | - | - | - | - |
| **2023** | | | | | | | | |

| Sweden \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 175)** | **SMA Type 1 (N = 28)** | **SMA Type 2 (N = 69)** | **SMA Type 3 (N = 71)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 2)** |
| No | 173 (100.0%) | 28 (100.0%) | 67 (100.0%) | 71 (100.0%) | * | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **At least one episode of non-invasive ventilation; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 25 (86.2%) | * | 17 (85.0%) | 7 (100.0%) | - | - | - | - |
| Yes | * | - | * | - | - | - | - | * |
| **2011-2013** | | | | | | | | |
| No | 55 (77.5%) | - | 21 (63.6%) | 33 (97.1%) | - | * | - | - |
| Yes | 16 (22.5%) | * | 12 (36.4%) | * | - | - | - | * |
| **2014-2016** | | | | | | | | |
| No | 77 (77.8%) | * | 27 (62.8%) | 47 (100.0%) | - | * | - | - |
| Yes | 22 (22.2%) | * | 16 (37.2%) | - | - | * | - | * |
| **2017** | | | | | | | | |
| No | 88 (83.0%) | 6 (60.0%) | 33 (71.7%) | 46 (97.9%) | - | * | - | * |
| Yes | 18 (17.0%) | * | 13 (28.3%) | * | - | - | - | - |
| **2018** | | | | | | | | |
| No | 100 (80.6%) | 6 (50.0%) | 37 (71.2%) | 55 (96.5%) | - | * | - | - |
| Yes | 24 (19.4%) | 6 (50.0%) | 15 (28.8%) | * | - | - | - | * |
| **2019** | | | | | | | | |
| No | 118 (81.9%) | 10 (58.8%) | 42 (72.4%) | 64 (98.5%) | * | * | - | - |
| Yes | 26 (18.1%) | 7 (41.2%) | 16 (27.6%) | * | - | * | - | * |
| **2020** | | | | | | | | |
| No | 123 (80.9%) | 13 (59.1%) | 45 (73.8%) | 62 (95.4%) | * | * | - | - |
| Yes | 29 (19.1%) | 9 (40.9%) | 16 (26.2%) | * | - | - | - | * |
| **2021** | | | | | | | | |
| No | 136 (82.9%) | 16 (69.6%) | 47 (72.3%) | 68 (97.1%) | * | * | - | - |
| Yes | 28 (17.1%) | 7 (30.4%) | 18 (27.7%) | * | - | - | - | * |
| **2022** | | | | | | | | |

| Sweden \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 175)** | **SMA Type 1 (N = 28)** | **SMA Type 2 (N = 69)** | **SMA Type 3 (N = 71)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 2)** |
| No | 160 (93.0%) | 27 (96.4%) | 59 (88.1%) | 68 (97.1%) | * | * | - | * |
| Yes | 12 (7.0%) | * | 8 (11.9%) | * | - | - | - | * |
| **2023** | | | | | | | | |
| No | 171 (98.8%) | 28 (100.0%) | 65 (97.0%) | 71 (100.0%) | * | * | - | * |
| Yes | * | - | * | - | - | - | - | - |
| **At least one episode of feeding tube usage; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 29 (100.0%) | * | 20 (100.0%) | 7 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 71 (100.0%) | * | 33 (100.0%) | 34 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 96 (97.0%) | * | 43 (100.0%) | 47 (100.0%) | - | * | - | * |
| Yes | * | * | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 103 (97.2%) | 7 (70.0%) | 46 (100.0%) | 47 (100.0%) | - | * | - | * |
| Yes | * | * | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 120 (96.8%) | 8 (66.7%) | 52 (100.0%) | 57 (100.0%) | - | * | - | * |
| Yes | * | * | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 139 (96.5%) | 12 (70.6%) | 58 (100.0%) | 65 (100.0%) | * | * | - | * |
| Yes | 5 (3.5%) | 5 (29.4%) | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 145 (95.4%) | 16 (72.7%) | 60 (98.4%) | 65 (100.0%) | * | * | - | * |
| Yes | 7 (4.6%) | 6 (27.3%) | * | - | - | - | - | - |
| **2021** | | | | | | | | |
| No | 156 (95.1%) | 15 (65.2%) | 65 (100.0%) | 70 (100.0%) | * | * | - | * |

| Sweden \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 175)** | **SMA Type 1 (N = 28)** | **SMA Type 2 (N = 69)** | **SMA Type 3 (N = 71)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 2)** |
| Yes | 8 (4.9%) | 8 (34.8%) | - | - | - | - | - | - |
| **2022** | | | | | | | | |
| No | 165 (95.9%) | 21 (75.0%) | 67 (100.0%) | 70 (100.0%) | * | * | - | * |
| Yes | 7 (4.1%) | 7 (25.0%) | - | - | - | - | - | - |
| **2023** | | | | | | | | |
| No | 166 (96.0%) | 21 (75.0%) | 67 (100.0%) | 71 (100.0%) | * | * | - | * |
| Yes | 7 (4.0%) | 7 (25.0%) | - | - | - | - | - | - |
| **At least one episode of wheelchair usage; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 19 (65.5%) | * | 13 (65.0%) | 5 (71.4%) | - | - | - | - |
| Yes | 10 (34.5%) | - | 7 (35.0%) | * | - | - | - | * |
| **2011-2013** | | | | | | | | |
| No | 30 (42.3%) | * | 16 (48.5%) | 12 (35.3%) | - | * | - | - |
| Yes | 41 (57.7%) | * | 17 (51.5%) | 22 (64.7%) | - | - | - | * |
| **2014-2016** | | | | | | | | |
| No | 45 (45.5%) | * | 21 (48.8%) | 19 (40.4%) | - | * | - | - |
| Yes | 54 (54.5%) | * | 22 (51.2%) | 28 (59.6%) | - | * | - | * |
| **2017** | | | | | | | | |
| No | 59 (55.7%) | 8 (80.0%) | 22 (47.8%) | 27 (57.4%) | - | * | - | - |
| Yes | 47 (44.3%) | * | 24 (52.2%) | 20 (42.6%) | - | - | - | * |
| **2018** | | | | | | | | |
| No | 66 (53.2%) | 9 (75.0%) | 25 (48.1%) | 30 (52.6%) | - | * | - | - |
| Yes | 58 (46.8%) | * | 27 (51.9%) | 27 (47.4%) | - | - | - | * |
| **2019** | | | | | | | | |
| No | 75 (52.1%) | 10 (58.8%) | 26 (44.8%) | 36 (55.4%) | * | * | - | - |
| Yes | 69 (47.9%) | 7 (41.2%) | 32 (55.2%) | 29 (44.6%) | - | - | - | * |
| **2020** | | | | | | | | |
| No | 74 (48.7%) | 15 (68.2%) | 21 (34.4%) | 35 (53.8%) | * | * | - | - |

| Sweden \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 175)** | **SMA Type 1 (N = 28)** | **SMA Type 2 (N = 69)** | **SMA Type 3 (N = 71)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 2)** |
| Yes | 78 (51.3%) | 7 (31.8%) | 40 (65.6%) | 30 (46.2%) | - | - | - | * |
| **2021** | | | | | | | | |
| No | 57 (34.8%) | 8 (34.8%) | 14 (21.5%) | 31 (44.3%) | * | * | - | - |
| Yes | 107 (65.2%) | 15 (65.2%) | 51 (78.5%) | 39 (55.7%) | - | * | - | * |
| **2022** | | | | | | | | |
| No | 64 (37.2%) | 13 (46.4%) | 10 (14.9%) | 36 (51.4%) | * | * | - | * |
| Yes | 108 (62.8%) | 15 (53.6%) | 57 (85.1%) | 34 (48.6%) | - | * | - | * |
| **2023** | | | | | | | | |
| No | 67 (38.7%) | 13 (46.4%) | 9 (13.4%) | 40 (56.3%) | * | * | - | * |
| Yes | 106 (61.3%) | 15 (53.6%) | 58 (86.6%) | 31 (43.7%) | - | * | - | * |
| **At least one reported measure by available motor function scale or test and by at least one PRO; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 29 (100.0%) | * | 20 (100.0%) | 7 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 71 (100.0%) | * | 33 (100.0%) | 34 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 99 (100.0%) | 6 (100.0%) | 43 (100.0%) | 47 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 106 (100.0%) | 10 (100.0%) | 46 (100.0%) | 47 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| No | 124 (100.0%) | 12 (100.0%) | 52 (100.0%) | 57 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |

| Sweden \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 175)** | **SMA Type 1 (N = 28)** | **SMA Type 2 (N = 69)** | **SMA Type 3 (N = 71)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 2)** |
| No | 144 (100.0%) | 17 (100.0%) | 58 (100.0%) | 65 (100.0%) | * | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 149 (98.0%) | 22 (100.0%) | 60 (98.4%) | 63 (96.9%) | * | * | - | * |
| Yes | * | - | * | * | - | - | - | - |
| **2021** | | | | | | | | |
| No | 155 (94.5%) | 23 (100.0%) | 61 (93.8%) | 67 (95.7%) | * | * | - | * |
| Yes | 9 (5.5%) | - | * | * | * | * | - | - |
| **2022** | | | | | | | | |
| No | 167 (97.1%) | 28 (100.0%) | 66 (98.5%) | 66 (94.3%) | * | * | - | * |
| Yes | 5 (2.9%) | - | * | * | - | - | - | - |
| **2023** | | | | | | | | |
| No | 168 (97.1%) | 27 (96.4%) | 66 (98.5%) | 68 (95.8%) | * | * | - | * |
| Yes | 5 (2.9%) | * | * | * | - | - | - | - |
| **At least three reported measures by available motor function scale or test and by at least one PRO; n (%)** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| No | 29 (100.0%) | * | 20 (100.0%) | 7 (100.0%) | - | - | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| No | 71 (100.0%) | * | 33 (100.0%) | 34 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |
| No | 99 (100.0%) | 6 (100.0%) | 43 (100.0%) | 47 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| No | 106 (100.0%) | 10 (100.0%) | 46 (100.0%) | 47 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |

| Sweden \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 175)** | **SMA Type 1 (N = 28)** | **SMA Type 2 (N = 69)** | **SMA Type 3 (N = 71)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 2)** |
| **2018** | | | | | | | | |
| No | 124 (100.0%) | 12 (100.0%) | 52 (100.0%) | 57 (100.0%) | - | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| No | 144 (100.0%) | 17 (100.0%) | 58 (100.0%) | 65 (100.0%) | * | * | - | * |
| Yes | - | - | - | - | - | - | - | - |
| **2020** | | | | | | | | |
| No | 151 (99.3%) | 22 (100.0%) | 61 (100.0%) | 64 (98.5%) | * | * | - | * |
| Yes | * | - | - | * | - | - | - | - |
| **2021** | | | | | | | | |
| No | 159 (97.0%) | 23 (100.0%) | 61 (93.8%) | 69 (98.6%) | * | * | - | * |
| Yes | 5 (3.0%) | - | * | * | - | - | - | - |
| **2022** | | | | | | | | |
| No | 168 (97.7%) | 28 (100.0%) | 67 (100.0%) | 66 (94.3%) | * | * | - | * |
| Yes | * | - | - | * | - | - | - | - |
| **2023** | | | | | | | | |
| No | 170 (98.3%) | 28 (100.0%) | 67 (100.0%) | 68 (95.8%) | * | * | - | * |
| Yes | * | - | - | * | - | - | - | - |
| **Available number of records of each motor function scale by patient** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | 2 (1.2) | - | 2.1 (1.2) | * | - | - | - | - |
| Median [IQR] | 2 [1, 2] | - | 2 [1, 2.5] | * | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | 1.7 (0.7) | - | 1.9 (0.8) | 1.3 (0.5) | - | - | - | * |
| Median [IQR] | 2 [1, 2] | - | 2 [1, 2.2] | 1 [1, 1.5] | - | - | - | * |
| **2014-2016** | | | | | | | | |
| Mean (SD) | 1.6 (0.7) | 1.2 (0.4) | 1.7 (0.8) | 1.5 (0.7) | - | - | - | * |
| Median [IQR] | 1 [1, 2] | 1 [1, 1] | 2 [1, 2] | 1 [1, 2] | - | - | - | * |

| Sweden \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 175)** | **SMA Type 1 (N = 28)** | **SMA Type 2 (N = 69)** | **SMA Type 3 (N = 71)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 2)** |
| **2017** | | | | | | | | |
| Mean (SD) | 1.1 (0.3) | 1.5 (0.5) | 1.1 (0.3) | 1 (0.2) | - | - | - | - |
| Median [IQR] | 1 [1, 1] | 1 [1, 2] | 1 [1, 1] | 1 [1, 1] | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | 1.3 (0.7) | 2.4 (1.4) | 1.3 (0.7) | 1.1 (0.4) | - | * | - | * |
| Median [IQR] | 1 [1, 1] | 2 [1, 3.8] | 1 [1, 1] | 1 [1, 1] | - | * | - | * |
| **2019** | | | | | | | | |
| Mean (SD) | - | 1.4 (0.5) | 1.2 (0.4) | 1 (0.2) | - | - | - | * |
| Median [IQR] | 1 [1, 1] | 1 [1, 2] | 1 [1, 1] | 1 [1, 1] | - | - | - | * |
| **2020** | | | | | | | | |
| Mean (SD) | 1.2 (0.5) | 1.4 (0.6) | 1.2 (0.5) | 1.2 (0.4) | - | - | - | * |
| Median [IQR] | 1 [1, 1] | 1 [1, 2] | 1 [1, 1] | 1 [1, 1] | - | - | - | * |
| **2021** | | | | | | | | |
| Mean (SD) | 1.2 (0.5) | 1.2 (0.4) | 1.2 (0.5) | 1.3 (0.5) | * | * | - | * |
| Median [IQR] | 1 [1, 1] | 1 [1, 1] | 1 [1, 1] | 1 [1, 1] | * | * | - | * |
| **2022** | | | | | | | | |
| Mean (SD) | 1.3 (0.5) | 1.5 (0.6) | 1.2 (0.5) | 1.2 (0.4) | - | - | - | 1 (-) |
| Median [IQR] | 1 [1, 1] | 1 [1, 2] | 1 [1, 1] | 1 [1, 1] | - | - | - | 1 [1, 1] |
| **2023** | | | | | | | | |
| Mean (SD) | 1 (-) | 1 (-) | 1 (-) | 1 (-) | - | - | - | 1 (-) |
| Median [IQR] | 1 [1, 1] | 1 [1, 1] | 1 [1, 1] | 1 [1, 1] | - | - | - | 1 [1, 1] |
| **Available number of records of PRO** | | | | | | | | |
| **Before 2011** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2011-2013** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2014-2016** | | | | | | | | |

| Sweden \| SMA Type | | | | | | | | |
|---|---|---|---|---|---|---|---|---|
| | **Overall (N = 175)** | **SMA Type 1 (N = 28)** | **SMA Type 2 (N = 69)** | **SMA Type 3 (N = 71)** | **SMA Type 4 (N = 2)** | **SMA Type Other (N = 3)** | **SMA Type Presymptomatic (N = 0)** | **SMA Type Missing (N = 2)** |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2017** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2018** | | | | | | | | |
| Mean (SD) | - | - | - | - | - | - | - | - |
| Median [IQR] | - | - | - | - | - | - | - | - |
| **2019** | | | | | | | | |
| Mean (SD) | * | - | - | * | - | - | - | - |
| Median [IQR] | * | - | - | * | - | - | - | - |
| **2020** | | | | | | | | |
| Mean (SD) | 1 (-) | - | * | * | - | - | - | - |
| Median [IQR] | 1 [1, 1] | - | * | * | - | - | - | - |
| **2021** | | | | | | | | |
| Mean (SD) | 1.2 (0.4) | - | 1.5 (0.5) | 1 (-) | * | * | - | - |
| Median [IQR] | 1 [1, 1] | - | 1.5 [1, 2] | 1 [1, 1] | * | * | - | - |
| **2022** | | | | | | | | |
| Mean (SD) | 1 (-) | - | * | 1 (-) | - | - | - | - |
| Median [IQR] | 1 [1, 1] | - | * | 1 [1, 1] | - | - | - | - |
| **2023** | | | | | | | | |
| Mean (SD) | 1 (-) | * | * | 1 (-) | - | - | - | - |
| Median [IQR] | 1 [1, 1] | * | * | 1 [1, 1] | - | - | - | - |