2025 Community Update Survey

Survey launch: April 2025

Intended audience: Individuals (or primary caregivers of individuals) who have been diagnosed with SMA and reside in the U.S.

If you have any questions, please feel free to contact communityupdatesurvey@curesma.org.

Contents

Information on person completing survey

1. **First Name:***
2. **Last Name:***
3. **Street Address:**
4. **City:**

5. **State/Province:**
6. **Zip Code:**
7. **Country:***
8. **Email:**
9. **Phone Number:**
10. **Date of Birth:*** ***mm/dd/yyyy***
11. **Your relationship to affected child or individual***
    - **Self** ***If selected skip to question 24***
    - **Parent**
    - **Grand parent**
    - **Relative**
    - **Spouse**
    - **Friend**
    - **Other**
12. **Are you the primary caregiver (A person who takes primary responsibility for someone who cannot care fully for themselves. May be a family member, a trained professional or another individual)? ***
    - **Yes**
    - **No**
13. **Are you completing the survey on behalf of an individual that has passed away? ***
    - **Yes**
    - **No**

Affected Individual

14. **First Name:***
15. **Last Name:***
16. **Street Address:**
17. **City:**
18. **State/Province:**
19. **Zip Code:**
20. **Country:**
21. **Email:**
22. **Primary Phone Number:**
23. **Birthdate:*** ***mm/dd/yyyy***
24. ***For those over the age of 18:*** **Marital Status***
    - **Married**
    - **In a civil union, or in a domestic partnership (permanent living arrangement with a partner)**
    - **Widowed**
    - **Divorced**
    - **Single (never married)**
25. ***For those that chose in a civil union or single:*** **Do you fear or worry about getting married due to its impact on your public benefits (i.e. SSI, caregiving)?**
    - **Yes**
    - **No**
    - **Don't know**

- Prefer not to answer

26. **Sex assigned at birth***
    - Male
    - Female
    - Prefer not to answer
27. **Gender identity***
    - Male
    - Female
    - Transgender Man/Transgender Male
    - Transgender Woman/Transgender Female
    - Genderqueer, neither exclusively male nor female
    - Additional gender category or other, please specify _____
    - Prefer not to answer
28. ***For affected adults only:*** **Do you think of yourself as**
    - Straight or heterosexual
    - Gay or lesbian, or homosexual
    - Bisexual
    - Something else (e.g., queer, pansexual, asexual.) Please specify: _____
    - Don't know
    - Prefer not to answer
29. ***For female affected adults only:*** **Have you ever been pregnant?**
    - Yes
    - No
    - Prefer not to answer
30. ***For male affected adults only:*** **Have you ever had a pregnancy with your current or previous partner?**
    - Yes
    - No
    - Prefer not to answer
    - Not applicable
31. ***If 'Yes' selected in question 28 or 29 ask:*** **How many pregnancies have you had?**

Repeat questions below for each pregnancy

32. **What was the date at the start of the first pregnancy? Please estimate the date if unsure of exact date.____**
33. **What was the date at the start of the second pregnancy? Please estimate the date if unsure of the exact date.____**
34. **What was the date at the start of the third pregnancy? Please estimate the date if unsure of the exact date.____**
35. **What was the date at the start of the fourth pregnancy? Please estimate the date if unsure of the exact date.____**
36. **What was the date at the start of the fifth pregnancy? Please estimate the date if unsure of the exact date.____**
37. **What was the date at the start of the sixth pregnancy? Please estimate the date if unsure of the exact date.____**

38. How many pregnancies resulted in a live birth? _____
39. *For affected adults only and if yes to either question 29 or 30:* Was pregnancy achieved through fertility treatments such as intrauterine insemination (IUI), in vitro fertilization (IVF), intracytoplasmic sperm injection (ICSI)?
    - ○ Yes, fertility treatments were used in previous pregnancies
    - ○ No, fertility treatments were not used in previous pregnancies
    - ○ If history of multiple pregnancies, both natural conception and fertility treatments resulted in previous pregnancies
    - ○ Prefer not to answer
    - ○ Not applicable
40. *If no to either question 29 or 30:* Are you and your partner currently trying to conceive?
    - ○ Yes, trying to conceive naturally
    - ○ Yes, trying to conceive and utilizing fertility treatments
    - ○ No, not trying to conceive
    - ○ Not applicable
    - ○ Prefer not to answer
41. What is your/your child's racial identity? If you/your child identify with more than one, please choose 'Two or more races'*
    - ○ American Indian or Alaska Native
    - ○ Asian
    - ○ Black or African American
    - ○ Middle Eastern or North African
    - ○ Native Hawaiian or Other Pacific Islander
    - ○ White
    - ○ Two or more races
    - ○ Prefer not to answer
42. *If 'Two or more races' selected in question above:* Please select all that apply to you/your child:*
    - ☐ American Indian or Alaska Native
    - ☐ Asian
    - ☐ Black or African American
    - ☐ Middle Eastern or North African
    - ☐ Native Hawaiian or Other Pacific Islander
    - ☐ White
    - ☐ Other, please specify _____
43. Do you/your child identify as Hispanic or Latino?*
    - ○ Yes
    - ○ No
    - ○ Prefer not to answer
44. Is English the primary language spoken at home?*
    - ○ Yes
    - ○ No
45. *If No in question above*: What language is primarily spoken at home?* (Drop down menu) _____

46. **What is the highest degree or level of school you/your child has completed? (If currently enrolled, highest degree received. If homeschooled, choose the grade equivalent.)***
    - **No formal schooling completed**
    - **Pre-school**
    - **Kindergarten**
    - **First Grade**
    - **Second Grade**
    - **Third Grade**
    - **Fourth Grade**
    - **Fifth Grade**
    - **Sixth Grade**
    - **Seventh Grade**
    - **Eighth Grade**
    - **Some high school, no diploma**
    - **High school graduate, diploma or the equivalent (for example: GED)**
    - **Some college credit, no degree**
    - **Trade/technical/vocational training**
    - **Associate degree (for example: AA, AS)**
    - **Bachelor's degree (for example: BA, BS)**
    - **Master's degree (for example: MA, MS)**
    - **Doctorate degree (for example: PhD, EdD, MD, DDS)**
47. **What is your total household annual pretax income?**
    - **Under $20,000**
    - **$21,000-$40,000**
    - **$41,000-$70,000**
    - **$71,000-$100,000**
    - **$101,000-$150,000**
    - **$151,000-$200,000**
    - **Greater than $200,000**
    - **Don't know**
    - **Prefer not to answer**
48. ***For those 18 years and older and do not ask for those completing a survey on behalf of a deceased individual*: Are your earnings limited to remain eligible for public programs?**
    - **Yes**
    - **No**
    - **Don't know**
    - **Prefer not to answer**
49. **Has anyone in your immediate family ever served in any of the United States Armed Forces? This does not include the Reserves of the National Guard.**
    - **Yes, I have a family member who is currently on active-duty**
    - **Yes, I have a family member who is a Veteran who has served active-duty in the past**
    - **No one in this house has ever served in the military**
    - **Don't know**

- o Prefer not to answer

50. *If question 48 is answered with either of the 'yes' options:* **What branch of the Armed Forces has your family served? Choose all that apply.**
    - ☐ The Army
    - ☐ The Marine Corps
    - ☐ Navy
    - ☐ Air Force
    - ☐ Space Force
    - ☐ Coast Guard
51. ***If question 48 is answered with either of the 'yes' options:*** **What family member has or is serving in the Armed Forces? Choose all that apply.**
    - ☐ Parent
    - ☐ Spouse
    - ☐ Sibling
    - ☐ Grandparent
    - ☐ Child
    - ☐ Other, please specify:
52. ***For those answering the survey as 'self':*** **Which of the following best describes your current living situation?**
    - ○ Own
    - ○ Rent
    - ○ Living with parents/family members
    - ○ Living in dorm/campus housing
    - ○ Temporarily staying in a shelter or homeless
    - ○ Other, please specify
53. **Do you know your/your child's current height and weight?***
    - ○ Yes
    - ○ No
    - ○ Prefer not to answer
54. ***If yes to question 52:*** **What is your/your child's height and weight?**
55. **Height (Select the unit: feet/inches/meters/centimeters):**

Acceptable values: 18-84 inches, 50-215 cm, 1-7 ft, or 0-2 meters

56. **Weight (Select the unit: pounds/ounces/kilograms/grams):**

Acceptable vales: Acceptable values: 4-400lbs, 64-6400 oz, 1-181 kg, 1814-180000grams

57. **What type of SMA were you/ your child diagnosed with?**
    - ○ Type 0
    - ○ Type 1
    - ○ Type 2
    - ○ Type 3
    - ○ Type 4
    - ○ Distal
    - ○ Kennedy's
    - ○ SMARD (distal hereditary motor, HMN6)
    - ○ SMALED
    - ○ Unknown because diagnosed < 6 months of age before symptom onset

- Unknown
- Other, please specify

58. How many copies of the SMN2 gene do you/ your child have?
    - 1
    - 2
    - 3
    - 4
    - 5 or more
    - Don't know because a genetic test was never done
    - Don't know
59. ***For parents only and do not ask for those completing a survey on behalf of a deceased individual:*** Do you consider your child to be symptomatic or asymptomatic (not showing any signs of SMA-related symptoms).
    - Symptomatic
    - Asymptomatic
    - Don't know
    - Prefer not to answer
60. ***For parents only and asymptomatic was not chosen in the question above:*** At what age (in months) did you first notice when SMA-related symptoms started? ***Drop down menu of <1 month to > 24 months***
    Don't know
    Not applicable because he/she was diagnosed through newborn screening or other testing prior to onset of symptoms.
61. On what date were you/your child diagnosed? mm/dd/yyyy
62. Is your/your child's diagnosis date estimated?
    - Yes
    - No

How were you/your child diagnosed?

	Yes	No	Don't know
63. Genetic Testing (also known as SMA blood test, SMA Diagnostic Test, or SMN Diagnostics Test)			
64. Diagnosed by a physician by the symptoms and did not get tested with a genetic test			
65. Electromyography (EMG)			
66. Muscle Biopsy			
67. Other			

68. ***If yes to genetic test:*** When was the genetic test? mm/dd/yyyy
Acceptable dates: January 1st, 1995 to current date at time of survey
69. Is your/your child's genetic testing date estimated?
    - Yes
    - No
    - Don't know
70. ***If yes to question 62 on genetic testing and parents only:*** Was your child diagnosed through prenatal screening?
    - Yes

- **No**
- **Don't know**

71. ***If yes to prenatal screening:*** **What was the reason for screening for SMA prenatally?**
    - **I have another child with SMA**
    - **I am and/or my partner are carriers of the SMA gene mutation**
    - **Fetal ultrasounds showed abnormal findings such as reduced fetal movement or contractures**
    - **Prenatal screening was recommended by our ob/gyn and/or fertility specialist**
    - **Other, please specify_____**
    - **Don't know**
72. ***If yes to question 63 on genetic testing*****: Were you/your child identified with possible SMA through statewide Newborn screening**
    - **Yes**
    - **No**
    - **Don't know**

If birthdate ≤ 2016 and answer "yes" chosen for Newborn screening, then Error Message appears: "Statewide newborn screening began in 2016" Please check response and birthdate to confirm.

73. ***If yes to question 72 on newborn screening:*** **In what state were you/your child screened for SMA through newborn screening?**
    _____*(add dropdown menu of states)*
74. ***If yes to question 72 on newborn screening*****: Were they referred to a specific center of care following the SMA diagnosis?**
    - **Yes**
    - **No**
    - **Don't know**
75. ***If yes to diagnosed by a physician by the symptoms and did not get tested with a genetic test:*** **On what date were you/your child diagnosed by a physician by the symptoms? mm/dd/yyyy**
76. ***If yes to diagnosed by a physician by the symptoms and did not get tested with a genetic test:*** **Is this diagnosis date estimated?**
    - **Yes**
    - **No**
    - **Don't know**
77. ***If yes to electromyography*****: On what date was the electromyography done? mm/dd/yyyy**
78. ***If yes to electromyography:*** **Is the date of the electromyography estimated?**
    - **Yes**
    - **No**
    - **Don't know**
79. ***If yes to muscle biopsy*****: On what date was the muscle biopsy done? mm/dd/yyyy**
80. ***If yes to muscle biopsy:*** **Is the date of the muscle biopsy estimated?**
    - **Yes**
    - **No**
    - **Don't know**
81. ***For caregivers completing a survey on behalf of an individual that has passed away*****: Please provide the deceased date: *mm/dd/yyyy***

Acceptable dates: < Date of survey

82. ***For caregivers completing a survey on behalf of an individual that has passed away:*** **Was the deceased date estimated?**
    - **Yes**
    - **No**
    - **Don't know**

Treatment Questions

Have you/your child ever been treated with any of the following?

	Yes	No	Don't know
83. Spinraza (nusinersen)			
84. Zolgensma (onasemnogene abeparvovec-xioi)			
85. Evrysdi (risdiplam)			
86. Apitegromab (also known as SRK-015)			
87. Taldefgrobep			
88. RO7204239 (also known as GYM329, treatment used in the Manatee clinical trial)			
89. NMD-670 (treatment used in the NMD Pharma clinical trials)			

How did they receive treatment?

	Yes	No	Don't know
90. ***If yes to Spinraza (nusinersen):*** **They were/are in the Spinraza (nusinersen) clinical trial sponsored by Biogen**			
91. ***If yes to Spinraza (nusinersen):*** **They received Spinraza (nusinersen) treatment through the early access program (EAP)**			
92. ***If yes to Spinraza (nusinersen):*** **They are receiving Spinraza (nusinersen) that their doctor has prescribed for them**			
93. ***If yes to Zolgensma (onasemnogene abeparvovec-xioi):*** **They were in the Zolgensma (onasemnogene abeparvovec-xioi)/AVXS-101 clinical trial sponsored by Novartis Gene Therapies**			
94. ***If yes to Zolgensma (onasemnogene abeparvovec-xioi):*** **They received Zolgensma (onasemnogene abeparvovec-xioi) treatment through the managed access program (MAP)**			
95. ***If yes to Zolgensma (onasemnogene abeparvovec-xioi):*** **They have been treated with Zolgensma (onasemnogene abeparvovec-xioi) that their doctor has prescribed for them**			

96. *If yes to Evrysdi (risdiplam) or RO7204239:* They were in a clinical trial sponsored by Roche-Genentech			
97. *If yes to Evrysdi (risdiplam):* They are receiving Evrysdi (risdiplam) that their doctor has prescribed for them			
98. *If yes to Apitegromab*: They were in a clinical trial sponsored by Scholar Rock			
99. *If yes to Apitegromab*: They received apitegromab treatment through the early access program (EAP)			
100. *If yes to Taldefgrobep:* They were in a clinical trial sponsored by Biohaven			
101. *If yes to NMD-670:* They were in a clinical trial sponsored by NMD Pharma			

94. *If yes to Question 83 (treated with Spinraza) and yes to Question 90 (receiving Spinraza in a clinical trial sponsored by Biogen) ask:* Did you receive the high dose of Spinraza (50/28 mg) in a clinical trial such as Biogen's DEVOTE Clinical Trial?

- **Yes**
- **No**
- **Don't know**

95. *If yes to Question 84 (treated with Zolgensma) and yes to question 93 (receiving Zolgensma in a clinical trial sponsored by Novartis) ask:* How was Zolgensma administered?

- ***Intravenous (approved by the FDA in 2019)***
- ***Intrathecal via spinal lumbar puncture (evaluated as part of the STRENGTH and STEER clinical trial)***
- ***Unknown***

96. *If yes to question 85 (treated with Evrysdi):* What formulation of Evrysdi was received?

- **Liquid (approved by the FDA in 2020)**
- **Tablet**
- **Unknown**

102. *If yes to Spinraza (nusinersen):* When did he/she receive their first dose of Spinraza (nusinersen)?

103. *If yes to Spinraza (nusinersen) – 50/28 mg higher regimen dose:* When did he/she receive their first dose of Spinraza (nusinersen) – 50/28mg higher regimen dose?

104. *If yes to Zolgensma (onasemnogene abeparvovec-xioi):* When did he/she receive their first dose of Zolgensma (onasemnogene abeparvovec-xioi)?

105. *If yes to Zolgensma (onasemnogene abeparvovec-xioi) – Intrathecal via spinal lumbar puncture:* When did he/she receive their first dose of Zolgensma – Intrathecal via spinal lumbar puncture?

106. *If yes to Evrysdi (risdiplam)- Liquid:* When did he/she receive their first dose of Evrysdi (risdiplam) - Liquid?

107. ***If yes to Evrysdi (risdiplam)- Tablet:*** **When did he/she receive their first dose of Evrysdi (risdiplam) - Tablet?**
108. ***If yes to Apitegromab (also known as SRK-015):*** **When did he/she receive their first dose of Apitegromab (also known as SRK-015)?**
109. ***If yes to Taldefgrobep:*** **When did he/she receive their first dose of Taldefgrobep?**
110. ***If yes to RO7204239:*** **When did he/she receive their first dose of RO7204239?**
111. ***If yes to NMD-670:*** **When did he/she receive their first dose of NMD-670?**

112. ***If yes to Evrysdi (risdiplam)- Tablet:*** **What are the main reasons he/she tried the Evrysdi tablet (select maximum of TWO reasons)?**

- ☐ **Convenience (e.g. easier to carry, portability for travel)**
- ☐ **Easier to store (e.g. no need for refrigeration, less mess)**
- ☐ **Confidence in accuracy of dosage**
- ☐ **Easier to remember if medication taken**
- ☐ **Easier to administer**
- ☐ **Ability to get a 30-day supply**
- ☐ **Other (please specify)**

113. ***If yes to Evrysdi (risdiplam)- Tablet:*** **How often do you/they take the Evrysdi tablet by dispersing it in water?**

- ○ **Never**
- ○ **Rarely**
- ○ **Sometimes**
- ○ **Most of the time**
- ○ **Always**

114. ***If yes to Evrysdi (risdiplam)- Tablet:*** **When thinking about the Evrysdi tablet, how confident are you/they that the tablet is effectively treating your/their SMA?**

- ○ **Not at all**
- ○ **A little bit**
- ○ **Somewhat**
- ○ **Quite a bit**
- ○ **Very much**

115. ***If yes to Evrysdi (risdiplam)- Liquid:*** **When thinking about the Evrysdi liquid, how confident are you/they that the liquid is effectively treating your/their SMA?**

- ○ **Not at all**
- ○ **A little bit**
- ○ **Somewhat**
- ○ **Quite a bit**
- ○ **Very much**

116. ***If yes to Evrysdi (risdiplam)-Liquid & Evrysdi (risdiplam)-Tablet*****: When thinking about Evyrsdi treatment, which do/they prefer?**

- ○ **Liquid**
- ○ **Tablet**
- ○ **No preference**

117. ***For those with a preference to the Evrysdi tablet in question above:*** **How strong is his/her preference for the Evrysdi tablet?**

- **Very strong preference for the tablet**
- **Fairly strong preference for the tablet**
- **Not very strong preference for the tablet**

118. ***If yes to Evrysdi (risdiplam)-Liquid & Evrysdi (risdiplam)-Tablet*****: When thinking about how effective the Evrysdi tablet is in treating your SMA compared to the liquid, you are**

- **Doing better with the tablet**
- **Doing the same with the tablet**
- **Doing worse with the tablet**

119. ***If yes to Evrysdi (risdiplam)-Liquid & Evrysdi (risdiplam)-Tablet*****: When thinking about how you are tolerating the Evrysdi tablet compared to the liquid, you are**

- **Tolerating the tablet better**
- **Tolerating the tablet the same**
- **Tolerating the tablet worse**

120. ***If yes to Evrysdi (risdiplam)-Liquid & Evrysdi (risdiplam)-Tablet*****: When thinking about Evrysdi, the side effects of the tablet are _____ what you/they experienced with the liquid**

- **Much better than**
- **Somewhat better than**
- **Similar as**
- **Somewhat worse than**
- **Much worse than**

Are you/your child still receiving any of the following?

	Yes	No	Don't know
121. ***If yes to treated with Spinraza (nusinersen):*** **Spinraza (nusinersen)**			
122. ***If yes to treated with Spinraza (nusinersen) 50/28 mg: Spinraza (nusinersen) -*** **50/28 mg higher regimen dose**			
123. ***If yes to treated with Evrysdi (risdiplam) - Liquid:*** **Evrysdi (risdiplam)- Liquid**			
124. ***If yes to treated with Evrysdi (risdiplam) - Tablet:*** **Evrysdi (risdiplam)- Tablet**			
125. ***If yes to treated with Apitegromab*****: Apitegromab (also known as SRK-015)**			
126. ***If yes to treated with Taldefgrobep:*** **Taldefgrobep**			
127. ***If yes to treated with RO7204239:*** **RO7204239**			
128. ***If yes to treated with NMD-670:*** **NMD-670**			

129. ***If no to still receiving Spinraza (nusinersen), question 121:*** **When did he/she receive their last dose of Spinraza (nusinersen)?**

130. ***If no to still receiving Spinraza (nusinersen) 50/28 mg higher regimen dose, question 122:*** **When did he/she receive their last dose of Spinraza (nusinersen) – 50/28 mg higher regimen dose?**
131. ***If no to still receiving Evrysdi (risdiplam) - Liquid, question 123:*** **When did he/she receive their last dose of Evrysdi (risdiplam) - Liquid?**
132. ***If no to still receiving Evrysdi (risdiplam) - Tablet, question 124:*** **When did he/she receive their last dose of Evrysdi (risdiplam) - Tablet?**
133. ***If no to still receiving Apitegromab (also known as SRK-015), question 125:*** **When did he/she receive their last dose of Apitegromab (also known as SRK-015)?**
134. ***If no to still receiving Taldefgrobep, question 126:*** **When did he/she receive their last dose of Taldefgrobep?**
135. ***If no to still receiving RO7204239, question 127:*** **When did he/she receive their last dose of RO7204239?**
136. ***If no to still receiving NMD-670, question 128:*** **When did he/she receive their last dose of NMD-670?**
137. ***If yes to received Spinraza (nusinersen) that their doctor has prescribed for them, question 92:*** **Have you/your child had to appeal an insurance denial for Spinraza (nusinersen)?**
    - **Yes**
    - **I/They received an insurance denial, and I/they did not appeal it**
    - **No**
    - **Don't know**
    - **Not applicable, I did not access Spinraza (nusinersen) through my insurance**
138. ***If yes to appealing an insurance denial for Spinraza (nusinersen), question 137:*** **Was the denial of Spinraza (nusinersen) due to treatment initiation or treatment renewal?**
    - **Treatment initiation**
    - **Treatment renewal**
    - **Both initiation and renewal have been denied**
    - **Don't know**

If yes or 'yes and did not appeal it' to receiving an insurance denial for Spinraza (nusinersen), question 136: **What reason(s) were you given for the denial of Spinraza (nusinersen)?**

	Yes	No	Don't know
139. Policy cancelled for lack of premium payment	**( )**	**( )**	**( )**
140. Deductible had not been met	**( )**	**( )**	**( )**
141. Provider out-of-network	**( )**	**( )**	**( )**

142. Not eligible for the requested benefit	**( )**	**( )**	**( )**
143. Service was not preauthorized	**( )**	**( )**	**( )**
144. Medication not covered	**( )**	**( )**	**( )**
145. Services were not deemed medically necessary	**( )**	**( )**	**( )**
146. The effectiveness of the medical treatment had not been proven	**( )**	**( )**	**( )**
147. Other			

148. If other, please specify:

_____

149. ***If yes to received Zolgensma (onasemnogene abeparvovec-xioi) that their doctor has prescribed for them, question 95:*** **Have you/your child had to appeal an insurance denial prior to receiving Zolgensma (onasemnogene abeparvovec-xioi)?**

- **Yes**
- **No**
- **Don't know**
- **Not applicable, I did not access Zolgensma (onasemnogene abeparvovec-xioi) through my insurance**

If yes to receiving an insurance denial for Zolgensma (onasemnogene abeparvovec-xioi, question 149: **What reason(s) were you given for the denial of Zolgensma (onasemnogene abeparvovec-xioi)?***

	Yes	**No**	**Don't know**
150. Policy cancelled for lack of premium payment	**( )**	**( )**	**( )**
151. Deductible had not been met	**( )**	**( )**	**( )**
152. Provider out-of-network	**( )**	**( )**	**( )**
153. Not eligible for the requested benefit	**( )**	**( )**	**( )**
154. Service was not preauthorized	**( )**	**( )**	**( )**
155. Medication not covered	**( )**	**( )**	**( )**

156. Services were not deemed medically necessary	**( )**	**( )**	**( )**
157. The effectiveness of the medical treatment had not been proven	**( )**	**( )**	**( )**
158. Nonspecific or non-eligible *SMN2* copy numbers	**( )**	**( )**	**( )**
159. Anti-AAV9 antibody titers	**( )**	**( )**	**( )**
160. Other			

161. *If other, please specify:*

_____

162. *If yes to received Evrysdi (risdiplam) that their doctor has prescribed for them, question 97:* Have you/your child had to appeal an insurance denial for Evrysdi (risdiplam)?

- **Yes**
- **I/They received an insurance denial, and I/they did not appeal it**
- **No**
- **Don't know**
- **Not applicable, I did not access Evrysdi (risdiplam) through my insurance**

163. *If yes to appealing an insurance denial for Evrydi (risdiplam), question 162:* Was the denial of Evrydi (risdiplam) due to treatment initiation or treatment renewal? Chose all that apply

- ☐ **Treatment initiation of Evrysdi liquid**
- ☐ **Treatment initiation of Evrysdi tablet**
- ☐ **Treatment renewal of Evrysdi liquid**
- ☐ **Treatment renewal of Evrysdi tablet**
- ☐ **Don't know**

***If yes or 'yes and did not appeal it' to receiving an insurance denial for Evrysdi (risdiplam), question 162:* What reason(s) were you given for the denial of Evrysdi (ridiplam)?**

	Yes	**No**	**Don't know**
164. Policy cancelled for lack of premium payment			
165. Deductible had not been met			
166. Provider out-of-network			

167. Not eligible for the requested benefit			
168. Service was not preauthorized			
169. Medication not covered			
170. Services were not deemed medically necessary			
171. The effectiveness of the medical treatment had not been proven			
172. Other			

173. If other, please specify: _____

If treated with more than 1 treatment: **What were the reasons for being treated with more than one SMA therapy?**

	Yes	No	Don't know
174. They were not gaining function as expected			
175. They were losing function			
176. They wanted all possible treatments			
177. Physician recommended more than one SMA treatment			
178. They wanted to try a different SMA treatment			
179. They had persistent SMA symptoms			
180. They had worsening SMA symptoms			
181. Their first treatment was not well tolerated			
182. They were no longer able to access their first treatment due to insurance coverage			
183. First treatment was meant to be a temporary treatment while wanting to be treated with another			
184. Other, please specify_____			

185. ***If treated with only 1 treatment:*** **Are you interested in receiving more than 1 therapy for SMA?**

☐ Yes, I would be interested in receiving an additional genetic modifying therapy (e.g. Zolgensma, Evrysdi, Spinraza)

☐ Yes, I would be interested in receiving an additional muscle enhancing therapy (e.g. Apitegromab, Taldefgrobep, RO7204239, NMD-670)

☐ No

☐ Don't Know

If not still receiving Spinraza (nusinersen): **If they have used Spinraza (nusinersen) but discontinued treatment, what were the reasons?**

	Yes	No	Don't know
186. They stopped using Spinraza (nusinersen) to use Zolgensma (onasemnogene abeparvovec) as prescribed by a doctor			
187. They stopped using Spinraza (nusinersen) to enter the Zolgensma (onasemnogene abeparvovec) clinical trial			
188. They stopped using Spinraza (nusinersen) to enter the Zolgensma (onasemnogene abeparvovec) Managed Access Program (MAP)			
189. They stopped using Spinraza (nusinersen) to enter the Evrysdi/risdiplam clinical trial			
190. They stopped using Spinraza (nusinersen) to use Evrysdi (risdiplam) as prescribed by a doctor			
191. They stopped using Spinraza (nusinersen) due to insurance denial			
192. They stopped using Spinraza (nusinersen) due to complications from treatment			
193. They stopped using Spinraza (nusinersen) due to complications from a procedure			
194. They stopped using Spinraza (nusinersen) due to persistent or worsening of SMA symptoms			
195. They stopped using Spinraza (nusinersen) due to route of administration			
196. They stopped using Spinraza (nusinersen) because it was not well tolerated			
197. They stopped using Spinraza (nusinersen) because of high out of pocket expenses			
198. Other, please specify: _____			

If not treated with Spinraza (nusinersen): **What are the reasons for not being treated with Spinraza (nusinersen)?**

	Yes	No	Don't know
199. They are not familiar with Spinraza (nusinersen)			
200. It's too expensive			
201. Their doctor/healthcare center does not administer Spinraza (nusinersen)			
202. Their insurance has denied coverage for Spinraza (nusinersen)			

203. They are unable to receive Spinraza (nusinersen) due to their scoliosis surgery			
204. They don't want to be treated with Spinraza (nusinersen) right now			
205. They were told they were not a candidate for Spinraza (nusinersen)			
206. They were treated with Zolgensma (onasemnogene abeparvovec)			
207. They were treated with Evrysdi (risdiplam)			
208. Other, please specify: _____			

If not treated with Zolgensma (onasemnogene abeparvovec): **What are the reasons for not being treated with Zolgensma (onasemnogene abeparvovec)?**

	Yes	No	Don't know
209. They are not familiar with Zolgensma (onasemnogene abeparvovec)			
210. It's too expensive			
211. Their doctor/healthcare center does not administer Zolgensma (onasemnogene abeparvovec)			
212. Their insurance has denied coverage for Zolgensma (onasemnogene abeparvovec)			
213. They don't want to be treated with Zolgensma (onasemnogene abeparvovec) right now			
214. They were told they were not a candidate for Zolgensma (onasemnogene abeparvovec)			
215. They were treated with Spinraza (nusinersen)			
216. They were treated with Evrysdi (risdiplam)			
217. They are concerned about the safety of Zolgensma (onasemnogene abeparvovec)			
218. Other, please specify: _____			

219. ***If yes to being told they are not a candidate for Zolgensma (onasemnogene abeparvovec):*** **Why were they told they were not a candidate for Zolgensma *(onasemnogene abeparvovec)*?**

- ☐ They did not meet the age requirements
- ☐ They had high anti-AAV9 antibodies
- ☐ Other, please specify_____

If not treated with Evrysdi (risdiplam): **What are the reasons for not being treated with Evrysdi (risdiplam)?**

	Yes	No	Don't know
220. They are not familiar with Evrysdi (risdiplam)			
221. It's too expensive			

222. Their doctor/healthcare center does not prescribe Evrysdi (risdiplam)			
223. Their insurance has denied coverage for Evrysdi (risdiplam)			
224. They don't want to be treated with Evrysdi (risdiplam) right now			
225. They are concerned about the safety/side effects of Evrysdi (risdiplam)			
226. They were told they were not a candidate for Evrysdi (risdiplam)			
227. They were treated with Spinraza (nusinersen)			
228. They were treated with Zolgensma (onasemnogene abeparvovec)			
229. Other, please specify: _____			

If not still receiving Evrysdi (risdiplam): **If they have used Evrysdi (risdiplam) but discontinued treatment, what were the reasons?**

	Yes	No	Don't know
230. They stopped using Evrysdi (risdiplam) to use Zolgensma (onasemnogene abeparvovec) as prescribed by a doctor			
231. They stopped using Evrysdi (risdipam) to use Spinraza (nusinersen) as prescribed by a doctor			
232. They stopped using Evrysdi (risdiplam) to enter into a clinical trial			
233. They stopped using Evrysdi (risdiplam) due to insurance denial			
234. They stopped using Evrysdi (risdiplam) due to complications from treatment			
235. They stopped using Evrysdi (risdiplam) due to safety concerns or side effects			
236. They stopped using Evrysdi (risdiplam) due to persistent or worsening of SMA symptoms			
237. They stopped using Evrysdi (risdiplam) because it was not well tolerated			
238. They stopped using Evrysdi (risdiplam) because of high out of pocket expenses			
239. Other, please specify: _____			

240. What are the key factors in making your choice for you/your child's SMA treatment? Please order the factors the most important at the top to the least important at the bottom

Cost and/or payer coverage
Safety
Efficacy
Dosing Schedule
Route of Administration

For caregivers only: Unmet Needs of the Caregiver

What are the 5 most significant unmet needs that you currently face caring for an individual with SMA?

	1	2	3	4	5
241. Reducing my fatigue					
242. Flexible work schedule/arrangements					
243. Financial assistance					
244. Emotional/mental health care for self					
245. Nursing support for affected individual					
246. Physical therapy for affected individual					
247. Improved communication with affected individual's healthcare team					
248. Improved wait times for affected individual's appointments					
249. Help navigating healthcare insurance					
250. Access to treatment for SMA					
251. Adaptability of public places for disability					
252. Assistance with receiving durable medical equipment for the affected individual					
253. Other, please specify_____					

Unmet Needs

Skip this question for those that answered they consider their child to be asymptomatic, question 59: **What are your 5 most significant current unmet needs that you/your child hope new therapies would address?**

	1	2	3	4	5
254. Reducing fatigue					
255. Improving respiratory (breathing) function					

256. Maintaining respiratory (breathing) function					
257. Improving swallowing					
258. Gaining muscle strength					
259. Achieving new motor function					
260. Improving fine motor skills (i.e. handling small objects, brushing teeth, putting on shoes)					
261. Stabilize motor function					
262. Improving communication through speech and/or technology					
263. Improving your incontinence and/or bowel movements					
264. Improving mental health/wellbeing					
265. Decreasing dependency on using assistive devices for mobility (i.e. wheelchairs, leg braces, standers, etc)					
266. Decreasing number of hospital visits					
267. Decreasing dependency on using assistive devices for breathing and feeding (i.e. feeding tubes, cough assist machines, etc)					
268. Improving fine motor skills to get dressed					
269. Improving fine motor skills to get undressed					
270. Other, please specify_____					

Motor Function

Which of the following motor functions have you/ your child ever achieved?

	Yes	No	Don't know
271. Head control (able to hold head up on own)			
272. Voluntary grasping			
273. Voluntary kicking			
274. Roll over completely			
275. Sitting without support			
276. Hands and knees crawling			
277. Standing with assistance			
278. Walking with assistance			
279. Standing alone			
280. Walking alone			

Can you/your child currently perform the following motor functions?

	Yes	No	Don't know

281. Head control (able to hold head up on own)			
282. Voluntary grasping			
283. Voluntary kicking			
284. Roll over completely			
285. Sitting without support			
286. Hands and knees crawling			
287. Standing with assistance			
288. Walking with assistance			
289. Standing alone			
290. Walking alone			

For affected adults only: Can you currently and/or previously been able to do the following functions?

	Can currently perform	Could previously perform, but no longer able to perform	Don't know	Not applicable, never able to perform
291. Brush your teeth				
292. Brush your hair				
293. Use/transfer to the toilet independently				
294. Hold a cup to drink				
295. Feed yourself with utensils				
296. Lift your legs				
297. Lift arms over head				
298. Grip small objects				
299. Lift a glass of water				
300. Open a door				
301. Close a door				
302. Lift a mobile phone				
303. Put shoes on				
304. Take shoes off				

For affected adults only and did not select *Not applicable, never able to perform* question above: In the last 12 months, have you noticed a change in your ability to perform the following tasks?

	There has been an improvement	There has been a decline	There has been no change	Not applicable, unable to perform this task in the last 12 months	Don't know

305. Brush your teeth					
306. Brush your hair					
307. Use/transfer to the toilet independently					
308. Hold a cup to drink					
309. Feed yourself with utensils					
310. Lift your legs					
311. Lift arms over head					
312. Grip small objects					
313. Lift a glass of water					
314. Open a door					
315. Close a door					
316. Lift a mobile phone					
317. Put shoes on					
318. Take shoes off					

Health Information

319. Have you/ your child ever had a surgery related to SMA including for scoliosis, gastrostomy, tracheostomy and others? Please do not count surgeries due to receiving SMA treatment/therapy.
   - ○ Yes
   - ○ No – *Skip to hospitalization question*
   - ○ Don't know – *Skip to hospitalization question*

What type of surgeries have you/ your child EVER had related to SMA? Please do not count surgeries due to receiving SMA treatment/therapy.

	Yes	No	Don't know
320. Spinal rods and fusion for scoliosis			
321. Growing rods placement for scoliosis			
322. Hip surgery			
323. Ankle/Foot surgery			
324. Gastrointestinal surgery (e.g. gastrostomy, Nissen fundoplication, ileostomy, colostomy)			
325. Surgery for joint contracture(s)			
326. Tracheotomy			
327. Other, please specify_____			

328. *If yes to spinal rods and fusion for scoliosis:* At what age, in years, did you/they have first surgery done? _____
329. *If yes to growing rods placement for scoliosis:* At what age, in years, did you/they have first surgery done? _____

330. **If yes to growing rods placement for scoliosis: What type of growing rods did you/ they have placed?**

☐ MAGEC rods

☐ Vertical expandable prosthetic titanium rib (VEPTR) surgery

☐ Other type of growing rod

☐ Don't know

331. **Have you/ your child ever been hospitalized in the last 12 months for a reason related to SMA?**

☐ Yes

☐ No - *Skip to questions on in-person appointments*

☐ Don't know - *Skip to questions on in-person appointments*

332. *If they answer yes being hospitalized:* **How many times have you/ they been hospitalized in the last 12 months? ____**

If yes to being hospitalized: **Why were you/they hospitalized over the last 12 months for a reason related to SMA?**

	Yes	No	Don't know
333. Respiratory distress			
334. Respiratory failure			
335. Pneumonia			
336. RSV (Respiratory syncytial virus)			
337. Infection other than pneumonia or RSV			
338. Gastrointestinal (GI)/Bowel Issues			
339. Dehydration or malnutrition			
340. Feeding tube problems			
341. Cardiomyopathy (problems with heart muscle) or arrhythmia (irregular heartbeat)			
342. Trauma, fracture or external injury			
343. Seizure			
344. Elective / planned surgery or other procedure (e.g., spinal fusion)			
345. Non-planned surgery or other procedure (e.g., appendectomy)			
346. Complications from receiving SMA-specific treatments			
347. Other, please specify_____			

348. **In the last 12 months, have you/ your child had an in-person appointment with a physician or specialist for SMA related care?**

○ Yes

○ No

○ Don't know

If yes to above ask: **What type of specialist do you/your child currently have a relationship with?**

	Yes	No	Don't know
349. Pediatric neurologist			
350. Adult neurologist			
351. Pulmonologist			
352. Physical therapist			

353. Occupational therapist			
354. Cardiologist			
355. Orthopedic surgeon			
356. Gastroenterologist			
357. Speech therapist			
358. Nutritionist			
359. Social worker			
360. Psychologist			
361. Other, please specify_____			

362. How far do you travel one-way on average for SMA-related healthcare services?

- o Less than 10 miles
- o 10-29 miles
- o 30-59 miles
- ○ 60-99 miles
- ○ 100-199 miles
- ○ 200-299 miles
- ○ 300 or more miles
- o Internationally
- o Don't know
- o Not applicable

Have you/ they EVER been diagnosed or been told by a doctor that they have any of the following conditions?

	Yes	No	Don't know	If yes, at what age (in years) were you first diagnosed?
363. Osteoporosis (bone loss)				
364. Scoliosis				
365. Dysphagia (difficulty in swallowing)				
366. Asthma				
367. Tonsillitis				
368. Contractures				
369. Hip Dysplasia				
370. Depression				
371. Anxiety				
372. Sleep apnea (interrupted breathing while sleeping)/hypoventilation (shallow or slow breathing)/sleep disordered breathing				
373. Hydrocephalus (increased fluid around the brain)				
374. Gastroesophageal reflux disease (GERD) (heartburn, acid reflux)				
375. Chronic respiratory failure				
376. Elevated liver enzymes				
377. Pneumonia				

378. Cardiomyopathy				
379. Neurocognitive delay or deficit other than autism				
380. Arachnoiditis (rare pain disorder caused by inflammation or irritation of the arachnoid, one of the membranes that surrounds the nerves of the spinal cord)				
381. Chronic Pain				
382. Sarcopenia				
383. Other, please specify				

384. *If yes to depression:* Were you/ your child treated for depression in the last 12 months?

- o Yes
- o No
- o Don't know
- o Prefer not to answer

385. *If yes to anxiety*: Were you/ your child treated for anxiety in the last 12 months?

- ○ Yes
- ○ No
- ○ Don't know
- ○ Prefer not to answer

Nutrition

386. Do you/ your child currently have trouble swallowing?

- ○ Always (all the time) (100%)
- ○ Usually (80-99%)
- ○ Often (60-79%)
- ○ About half the time (40-59%)
- ○ Sometimes (20-39%)
- ○ Rarely (1-19%)
- ○ Never (0%)

387. How often is your / your child's voice soft, weak or hard to be heard?

- ○ Always (all the time) (100%)
- ○ Usually (80-99%)
- ○ Often (60-79%)
- ○ About half the time (40-59%)
- ○ Sometimes (20-39%)
- ○ Rarely (1-19%)
- ○ Never (0%)

388. During the past month, which of the following describes your / your child's feeding routine on a typical day?

- ○ Nothing by mouth
- ○ Tube dependent with minimal attempts of food or liquid.
- ○ Tube dependent with consistent oral intake of food or liquid.
- ○ Total oral diet of a single consistency.

- Total oral diet with multiple consistencies, but requiring special preparation or compensations.
- Total oral diet with multiple consistencies without special preparation, but with specific food limitations.
- Total oral diet with no restrictions.

For caregivers of those 18 years and younger only: **Does your child currently receive any of the following for nutrition?**

	Yes	No	Don't know
389. Whole Protein/Standard (e.g. Enfamil NeuroPro, Similac ProAdvance, Nestle Gerber Good Start, Kate Farms, Boost Kids Essentials			
390. Semi-elemental/Peptides (e.g. Nutramigen, Peptamen Jr, Pediasure Peptide)			
391. Elemental/Amino acids (e.g. Elecare, Pediatric Vivonex, Tolerex)			
392. Home blenderized formula			
393. Breast Milk			

Breathing

394. Do you/ your child currently use a breathing machine?
   - Yes
   - No

If yes, then ask: **Do you/ your child currently use any of the following?**

	Yes	No	Don't know
395. Supplemental oxygen			
396. BiPAP (Bilevel positive airway pressure non-invasive ventilation delivered through a mask or nasal pillows)			
397. CPAP with mask (Single continuous air pressure level through a mask or nasal pillows)			
398. Cough Assist Machine			
399. Tracheostomy, on a ventilator			
400. Tracheostomy only for suctioning, not on a ventilator			
401. Other, please specify_____			

402. ***If yes to supplemental oxygen:*** **At what age, in years, did you/your child start to use supplemental oxygen?**
403. ***If yes to BiPAP:*** **At what age, in years, did you / your child start to use BiPAP?**
404. ***If yes to CPAP:*** **At what age, in years, did you/your child start to use CPAP?**
405. ***If yes to cough assist machine:*** **At what age, in years, did you/your child start to use a cough assist machine?**
406. ***If yes to tracheostomy with ventilator:*** **At what age, in years, did you/your child have a tracheostomy with ventilator?**

407. ***If yes to tracheostomy for suctioning:*** **At what age, in years, did you/your child have a tracheostomy for suctioning?**

408. ***If yes to using any of the breathing machines:*** **How many hours per day do you/ your child use oxygen or a breathing machine?**

- **Less than 8 hours per day**
- **8-16 hours per day**
- **More than 16 hours per day**

Home Life

409. How many caregivers care for you/ your child (include both paid and non-paid caretakers)?

What type of caregivers (e.g., family members, nurses, personal care worker, attendant, in-home aide) care for you / your child?

	Yes	No	Don't know
410. My/Their parent(s)/legal guardian(s)			
411. My/Their spouse/partner			
412. My/Their sibling(s)			
413. Other family member(s)			
414. Nurse(s)			
415. Home Health Aide(s)			
416. Personal Care Aide(s)			
417. Friend(s)			
418. Other, please specify_____			

419. About how many hours per week do you/ your child have a paid caregiver, other than a family member?

- **1-5 hours per week**
- **6-10 hours per week**
- **11-20 hours per week**
- **21-40 hours per week**
- **41-60 hours per week**
- **61-80 hours per week**
- **81-100 hours per week**
- **>100 hours per week**
- **I do not have a caregiver**

420. Do you/your child qualify for paid caregiving through Medicaid or other state programs?

- **Yes**
- **No**
- **Do not know**
- **Prefer not to answer**

421. If yes to question above: How many paid caregiving hours do you/your child receive through Medicaid or other state programs?

- **1-5 hours per week**
- **6-10 hours per week**
- **11-20 hours per week**

- 21-40 hours per week
- 41-60 hours per week
- 61-80 hours per week
- 81-100 hours per week
- >100 hours per week
- Not applicable. Although I qualify for caregiving support through Medicaid, I currently do not utilize those services

422. *If yes, to having paid caregiving support, question 419:* How difficult is it to find and retain paid caregivers?

- Extremely difficult
- A little difficult
- A little easy
- Extremely easy

423. Are you/your child currently employed?

- Yes, I am/they are employed full-time
- Yes, I am/they are employed part-time
- I/They are not employed but seeking full-time employment
- I/They are not employed but seeking part-time employment
- I/They are not employed and not seeking employment

424. *If yes to employed part time:* Why employed part-time?

- I/They work part-time by choice
- I/They work part-time, but seek full-time employment
- I/They work part-time due to SMA health-related complications
- I/They work part-time to stay below an income threshold to qualify for/retain healthcare and other public services (i.e. Medicaid attendant care)

425. *If yes to not employed and not seeking employment, question 423*: Are you on a supplemental security income program?

- Yes
- No
- Don't know
- Prefer not to answer

426. Are you/they currently attending school (in person or virtually)?

- Yes
- No

427. *For those living in the United States*: Do you have health insurance?

- Yes
- No

If yes to having health insurance: What type of health insurance do you/ your child have?

		Yes	No	Don't know
Public	428. Medicare			
	429. Medicaid			
	430. State Children's Health Insurance Program (CHIP)			
	431. Employer-based plan			

Private or Commercial	**432. COBRA plan**			
	433. Marketplace plan (ACA)			
Military Service-Related	**434. Tricare**			
	435. VA			
	436. CHAMP/VA			
	437. Other, Please specify			

438. ***If Yes to Private or Commercial:*** **Which of the following insurance plans do you /your child has?**

- **HMO**
- **PPO**
- **POS**
- **HDHP**
- **Don't know**
- **Prefer not to answer**

439. Does your/your child's health insurance cover physical therapy and/or occupational therapy?

- **Yes, but their medical needs exceed what my insurance will cover**
- **Yes, and their insurance fully covers my medical needs**
- **Yes, but they do not require physical and/or occupational therapy**
- **No**
- **Don't know**

440. ***If 'yes, but they do not require physical and/or occupational therapy' is not chosen in previous question:*** **How many hours of physical therapy do you/your child do, on average, in a week? _____**

Are you/they currently using any of the following equipment?

	Yes	No	Don't know
441. Hospital bed			
442. Hoyer lift			
443. Walker and/or Gait Trainer			
444. Manual Wheelchair			
445. Power Wheelchair			
446. Scooter			
447. Bath/Shower Chair			
448. Toilet Chair			
449. Stander			
450. Activity/Therapy Chair			
451. Adaptive stroller			
452. Car Bed			
453. Brace for scoliosis			
454. Hand or finger splints			
455. Foot or ankle braces			
456. Knee or foot braces			
457. Other, please specify_____			

458. *If yes to hoyer lift:* Have you/your child had to appeal an insurance denial related to coverage for power wheelchair?

- **Yes and they approved coverage**
- **Yes and insurance denied coverage**
- **The appeal is still pending**
- **No appeal was needed for insurance approval**
- **Not applicable, did not utilize insurance**
- **Prefer not to say**

459. *If yes to manual wheelchair:* Have you/your child had to appeal an insurance denial related to coverage of manual wheelchair?

- **Yes and they approved coverage**
- **Yes and insurance denied coverage**
- **The appeal is still pending**
- **No appeal was needed for insurance approval**
- **Not applicable, did not utilize insurance**
- **Prefer not to say**

460. *If yes to power wheelchair:* Have you/your child had to appeal an insurance denial related to coverage of your power wheelchair?

- **Yes and they approved coverage**
- **Yes and insurance denied coverage**
- **The appeal is still pending**
- **No appeal was needed for insurance approval**
- **Not applicable, did not utilize insurance**
- **Prefer not to say**

461. *If yes to stander:* Have you/your child had to appeal an insurance denial related to coverage of your stander?

- **Yes and they approved coverage**
- **Yes and insurance denied coverage**
- **The appeal is still pending**
- **No appeal was needed for insurance approval**
- **Not applicable, did not utilize insurance**
- **Prefer not to say**

462. *If yes to scooter:* Have you/your child had to appeal an insurance denial related to coverage of your scooter?

- **Yes and they approved coverage**
- **Yes and insurance denied coverage**
- **The appeal is still pending**
- **No appeal was needed for insurance approval**
- **Not applicable, did not utilize insurance**
- **Prefer not to say**

463. *If yes to brace for scoliosis:* Have you/your child had to appeal an insurance denial related to coverage of your brace for scoliosis?

- **Yes and they approved coverage**
- **Yes and insurance denied coverage**
- **The appeal is still pending**
- **No appeal was needed for insurance approval**

- **Not applicable, did not utilize insurance**
- **Prefer not to say**

For affected adults only – SMAIS-ULM

SMAIS-ULM not shown in this version.

ACEND - the Assessment of Caregiver Experience With Neuromuscular Disease

ACEND not shown in this version.

Caregiver Experience

549. Please drag and drop 5 of the most challenging aspects of caregiving for your child with SMA.

Managing my child's medical needs (e.g., medications, treatments, doctor appointments)
Assisting with mobility and physical care (e.g., lifting, transfers, positioning)
Managing respiratory care (e.g., breathing treatments, suctioning, ventilator support)
Ensuring proper nutrition and feeding (e.g., meal preparation, tube feeding)
Balancing caregiving with work or other responsibilities
Financial strain due to medical expenses and equipment costs
Navigating insurance and access to necessary treatments
Finding qualified caregivers or respite care
Emotional stress and anxiety about my child's health and future
Social isolation or lack of understanding from others
Advocating for my child's needs in school, healthcare, or the community
Planning for long-term care and my child's independence
Other (please specify)

550. What is one thing you wish others understood about caring for a child with SMA?_____

551. What are your biggest fears regarding your child's future?

- ☐ **My child's health worsening over time**
- ☐ **My child experiencing pain or discomfort**
- ☐ **Access to necessary treatments and medical care**
- ☐ **Financial burden of long-term care and medical expenses**
- ☐ **Lack of independence for my child as they grow older**
- ☐ **Difficulty accessing support and resources for my child's needs**
- ☐ **Social isolation or lack of inclusion for my child**
- ☐ **Not being able to provide the best possible care**
- ☐ **What will happen to my child if I am no longer able to care for them**
- ☐ **Other (please specify)**

552. How much do concerns about your child's health impact your daily stress levels?

- **1 (Not at all)**
- **2**
- **3**
- **4**
- **5 (Extremely)**

553. Do you feel you have adequate support (family, medical, social) to help manage your fears and concerns?

- **Yes**
- **Somewhat**
- **No**
- **Don't know**
- **Prefer not to answer**

554. What resources or support would help ease your caregiving experience?

_____

555. How has knowing your child has SMA, despite being asymptomatic, influenced your daily caregiving approach?

- **No change in daily caregiving**
- **Increased focus on preventive care (e.g., physical therapy, monitoring)**
- **Increased anxiety about potential symptom onset**
- **Other – Write in_____**

556. Do you feel adequately prepared for the possibility of your child developing symptoms in the future?

- **Yes, I feel well-prepared**
- **Somewhat, but I still have concerns**
- **No, I do not feel prepared**

557. How often do you think about or worry about your child developing symptoms?

- **Rarely or never**
- **Occasionally**
- **Frequently**
- **Almost all the time**

558. What kind of support or resources would help you navigate the uncertainty of having an asymptomatic child with SMA?

559. What symptoms have had the greatest impact on your caregiving responsibilities? Please select all that apply.

- **Difficulty with movement or mobility**
- **Difficulty with breathing or respiratory function**
- **Difficulty with feeding or swallowing**
- **Frequent hospitalizations or medical interventions**
- **Other, write it_____**

560. Have you noticed changes in your child's symptoms over time? If so, how has this affected your caregiving routine?

561. Have you had to adjust your expectations or plans for the future based on your child's symptoms?

- **Yes, please describe_____**
- **No**
- **Don't know**

Affected Adults only: Montefiore Social Needs Assessment:

562. Are you worried that in the next 2 months, you may not have a safe or stable place to live? (risk of eviction, being kicked out, homelessness)

- **Yes**
- **No**

563. Are you worried that the place you are living now is making you sick? (has mold, bugs/rodents, water leaks, not enough heat)

- **Yes**
- **No**

564. In the past 12 months, has the electric, gas, oil or water company threatened to shut of services to your home?

- **Yes**
- **No**

565. In the last 12 months, did you worry that your food could run out before you got money to buy more?

- **Yes**
- **No**

566. In the last 12 months, has lack of transportation kept you from medical appointments or getting your medications?

- **Yes**
- **No**

567. In the last 12 months, did you have to skip buying medications or going to doctor's appointments to save money?

- **Yes**
- **No**

568. Do you need help getting child care or care for an elderly or sick adult?

- **Yes**
- **No**

569. Do you need legal help? (child/family services, immigration, housing discrimination, domestic issues, etc)

- **Yes**
- **No**

570. Are you finding it hard to get along with a partner, spouse, or family members?

- **Yes**
- **No**

571. Does anyone in your life hurt you, threaten you, frighten you or make you feel unsafe?

- **Yes**
- **No**

For affected adults only: Mental Health Services

572. In the last 12 months have you felt that you have needed mental health services such as counselling, medications, or support groups to help with feelings of anxiety, depression, grief, or other issues?

- **Yes**
- **No**
- **Don't know**
- **Prefer not to answer**

573. Were you able to get the mental health services that you needed?

- **Yes**
- **No**
- **Don't know**
- **Prefer not to answer**

574. Which of these statements explains why you did not get the mental health services you needed? Check ALL that apply

- ☐ **I couldn't afford the cost**
- ☐ **My health insurance does not cover any type of mental health treatment or counseling**
- ☐ **My health insurance does not pay enough for mental health treatment or counseling**
- ☐ **I did not know where to go to get services**
- ☐ **I was concerned that the information I gave the counselor might not be kept confidential**
- ☐ **I didn't want others to find out that I needed treatment**
- ☐ **I was concerned that I might be committed to a psychiatric hospital**
- ☐ **I was concerned that I might have to take medicine**
- ☐ **I had no transportation, treatment was too far away, or the hours were not convenient**
- ☐ **I didn't have time (because of job, childcare, or other commitments)**
- ☐ **Some other reason. Please tell us:**

For caregivers of affected individuals (ages 0-18) THE SPINAL MUSCULAR ATROPHY CAREGIVER REPORTED HEALTH INDEX (SMACR-HI) FOR AGES 0-18

SMACR-HI not shown in this version.

THE SPINAL MUSCULAR ATROPHY HEALTH INDEX SHORT FORM (SMA-HI-SF)

SMA-HI not shown in this version.

Fatigue Severity Scale (FSS)

The Fatigue Severity Scale (FSS) is a method of evaluating the impact of fatigue on you. The FSS is a short questionnaire that requires you to rate your level of fatigue. The FSS questionnaire contains nine statements that rate the severity of your fatigue symptoms. Read each statement and circle a number from 1 to 7, based on how accurately it reflects your condition during the past week and the extent to which you agree or disagree that the statement applies to you.

● A low value (e.g., 1) indicates strong disagreement with the statement, whereas a high value (e.g., 7) indicates strong agreement.

● It is important that you circle a number (1 to 7) for every question.

During the past week, I have found that:	Disagree	←					Agree
1. My motivation is lower when I am fatigued.	1	2	3	4	5	6	7
2. Exercise brings on my fatigue.	1	2	3	4	5	6	7
3. I am easily fatigued.	1	2	3	4	5	6	7
4. Fatigue interferes with my physical functioning.	1	2	3	4	5	6	7
5. Fatigue causes frequent problems for me.	1	2	3	4	5	6	7
6. My fatigue prevents sustained physical functioning.	1	2	3	4	5	6	7
7. Fatigue interferes with carrying out certain duties and responsibilities.	1	2	3	4	5	6	7
8. Fatigue is among my three most disabling symptoms.	1	2	3	4	5	6	7
9. Fatigue interferes with my work, family, or social life.	1	2	3	4	5	6	7

Wrap up

575. **Cure SMA is trying to connect with more physicians treating SMA to inform them of upcoming SMA programs. If you are willing, please provide the name of the clinic(s) where SMA care is being provided. _____**
576. **How did you learn of this survey?**
    - **I received an email with the survey link**
    - **I received a text message with the survey link**
    - **I received a postcard in the mail with the survey link**
    - **I saw the link to the survey on the Cure SMA website**
    - **I received information on the survey at my physician's office**
    - **I saw the link to the survey on social media (Facebook, Instagram, Twitter)**
    - **Someone shared the survey with me**