Patient/Caregiver Survey

Quota: US N=100 SMA Respondents / Caregivers of Individuals with SMA

Statement of informed consent

S01

Purpose of the research: To understand to what extent existing treatments meet SMA individuals’ medical needs; and to understand the perceptions and experiences with existing treatments.

In addition, a separate survey is conducted to assess the amount of assistance required to perform daily activities in individuals with Type 2 and 3 SMA using a novel questionnaire called Spinal Muscular Atrophy Independence Scale (SMAIS)

If you are a caregiver of an individual with SMA, you will also be asked to complete a survey which assess your overall health-related quality of life as a caregiver.

What we will ask you to do in this research: If you agree to participate, we will ask you to answer questions regarding current treatments, symptoms, frequency of doctor visits, and the impact on you and/or the individual for whom you care.

Time required to complete the survey: The survey should take approximately 20 minutes to complete.

Payment for participating: If you are eligible for the study and complete the survey, we will pay you for your time.

Confidentiality: We will protect your privacy by removing your name and email address from the final records. Any personal or private information you provide will be de-identified by giving you an ID number instead of using your name. You are part of a larger study, so the information you provide will be combined with other respondents and assessed as a group.

Voluntary participation and right to withdraw from the research: Taking part in this study is your choice. There is no penalty for not participating. You may decide to stop or you may leave the study at any time. [SINGLE SELECT]

1. I have read the above statement and I consent to participate in this study.

1. I have read the above statement and I do not wish to participate. [TERMINATE

AND SHOW THE “THANK YOU!” SCREEN.]

IF TERMINATE, THEN SHOW TERMINATION SCREEN:

Thank you for your valuable time. Unfortunately, you will not be able to participate at this time due to not meeting the eligibility criteria.

# SCREENER [NOT SHOWN]

S1. Are you an individual or the primary caregiver of an individual with Spinal Muscular Atrophy (SMA)? Please select the option that best describes you.

1. Individual diagnosed with SMA
2. Caregiver of an individual with SMA
3. Other (Specify): \_\_\_\_\_ [TERMINATE]

[IF S1=B SHOW S2; IF S1=1 SKIP TO S9]

S2. Are you a paid or unpaid caregiver of an individual with SMA? [SINGLE SELECT]

1. Paid caregiver [TERMINATE]
2. Unpaid caregiver

S3. Are you routinely involved in the medical management of an individual with SMA for whom you provide care (e.g., medical appointments, treatment decisions, occupational or physical therapy (OT or PT), etc.)?

1. Yes
2. No [TERMINATE]

S4. What is your relationship to the affected individual?

1. Spouse
2. Parent
3. Grandparent
4. Sibling
5. Other relative
6. Other

S5. Please indicate your age.

\_\_\_\_\_\_\_\_[NUMERIC 10-90. IF <18, TERMINATE]

S6. How old is the individual with SMA for whom you are a caregiver?

\_\_\_\_\_\_ [NUMERIC 0-80. IF ≥ 18, TERMINATE]

S7. Which type of SMA is the individual for whom you provide care diagnosed with?

1. Type I
2. Type II
3. Type III
4. Type IV

S8. Is the individual for whom you care currently receiving Spinraza®?

1. Yes
2. No

[IF S1=1 SHOW S9]

S9. Please indicate your age.

\_\_\_\_\_\_ [NUMERIC 10-90. IF <18, TERMINATE]

[IF S1=1 SHOW S10]

S10. Which type of SMA are you diagnosed with?

1. Type I
2. Type II
3. Type III
4. Type IV

[IF S1=1 SHOW S11]

S11. Are you currently receiving Spinraza®?

1. Yes
2. No

[END SCREENER]

# SECTION 1: SURVEY DEMOGRAPHICS- SMA RESPONDENT/CAREGIVER [NOT

## SHOWN]

In this section, we will ask you some basic demographic questions about yourself.

Q12. What is your biological gender?

1. Male
2. Female
3. Prefer not to state

Q13. Please select the US state where you currently reside: [SINGLE SELECT]

Q14. Which category best describes you? [SINGLE SELECT]

1. Non-Hispanic White
2. Hispanic
3. Black or African American
4. Asian
5. Mixed race/ethnicity
6. Other race, ethnicity, or origin
7. I prefer not to state

Q15. Which of the following types of health insurance or health coverage plans are you covered by? You may select all that apply. [MULTI-SELECT, FORCE TO SELECT AT LEAST ONE]

1. Medicare
2. Medicaid
3. Private insurance (HMO)
4. Private insurance (PPO)
5. Veterans Administration/ Department of Defense
6. Long Term Care (LTC) insurance
7. Other
8. Uninsured [EXCLUSIVE]

[IF S1=2 SKIP TO Q20]

Q16. What is your highest degree of level of education?

1. Less than high school
2. High school or GED equivalent
3. Some college
4. Undergraduate degree
5. Graduate degree

Q17. What is your current employment status?

1. Employed full-time
2. Employed part-time
3. Receiving social security disability benefits
4. Homemaker
5. Unemployed
6. Retired
7. Student

[IF Q17=1 OR 2 SHOW Q18, OTHERWISE SKIP TO Q19] Q18. Please indicate your place of work.

1. Home
2. Workplace outside of home

Q19. What is your annual household income?

1. < $20,000
2. $20,000 to $49,999
3. $50,000 to $99,999
4. ≥ $100,000
5. Prefer not to state

[IF S1=1, SKIP TO Q28. IF S1=2, SHOW Q20-Q27]

SECTION 2: DEMOGRAPHICS - CAREGIVER ONLY [NOT SHOWN]

In this section, we will ask you some basic demographic questions about yourself and the individual for whom you care.

Q20. What is the biological gender of the individual for whom you care?

1. Male
2. Female
3. Prefer not to state

Q21. Please select the US state where the individual for whom you care currently resides:

[SINGLE SELECT]

Q22. Which category best describes the individual for whom you care? [SINGLE SELECT]

1. Non-Hispanic White
2. Hispanic
3. Black or African American
4. Asian
5. Mixed race/ethnicity
6. Other race, ethnicity, or origin
7. I prefer not to state

Q23. Which of the following types of health insurance or health coverage plans is the individual for whom you care covered by? You may select all that apply. [MULTI-SELECT, FORCE TO SELECT AT LEAST ONE]

1. Medicare
2. Medicaid
3. Private insurance (HMO)
4. Private insurance (PPO)
5. Veteran’s Administration/ Department of Defense
6. Long Term Care (LTC) insurance
7. Other
8. Uninsured [EXCLUSIVE]

Q24. What is your highest degree of level of education?

1. Less than high school
2. High school or GED equivalent
3. Some college
4. Undergraduate degree
5. Graduate degree

Q25. What is your current employment status?

1. Employed full-time
2. Employed part-time
3. Unemployed
4. Retired
5. Student
6. Full-time caregiver

Q26. Do you have any additional caregiver support (such as a friend, family member, or nurse)?

1. Yes, paid caregiver support
2. Yes, unpaid caregiver support
3. No, I am the sole caregiver

Q27. What is your annual household income?

1. < $20,000
2. $20,000 to $49,999
3. $50,000 to $99,999
4. ≥ $100,000
5. Prefer not to state

[UNIVERSAL NOTE: IF S1=1, INSERT THE FIRST TEXT BRACKET IN EACH SENTENCE (for patient themselves,i.e. “Your”). IF S1=2, INSERT THE SECOND TEXT (for caregiver,i.e. “The individual for whom you care”). Display text in bold.]

SECTION 3: HEALTH HISTORY [NOT SHOWN]

## In this section we will ask questions related to [your health history] [the health history of the individual for whom you care]

Q28. When [were you][was the individual for whom you care] diagnosed with SMA?

1. Less than 6 months ago
2. 6 months to less than 1 year ago
3. 1 year to less than 2 years ago
4. Greater than 2 years ago

Q29. At what age did [your SMA symptoms start][SMA symptoms start for the individual for whom you care]?

1. Less than 6 months ago
2. 6 months to less than 18 months ago
3. 18 months to less than 3 years ago
4. 3 years to less than 18 years ago
5. Greater than 18 years ago

Q30. At what age [were you][was the individual for whom you care] diagnosed with SMA?

1. Less than 6 months old
2. 6 months to less than 18 months old
3. 18 months to less than 3 years old
4. 3 years to less than 18 years old
5. Greater than 18 years old

Q31. What is [your highest level of motor function ability][the highest level of motor function ability of the individual for whom you care]?

1. Sitting without support
2. Standing with assistance
3. Hands and knee crawling
4. Walking with assistance
5. Standing alone
6. Walking alone
7. None of the above

Q32. What is [your current level of motor function ability][the current level of motor function ability of the individual for whom you care]?

1. Sitting without support
2. Standing with assistance
3. Hands and knee crawling
4. Walking with assistance
5. Standing alone
6. Walking alone
7. None of the above

SECTION 4: CURRENT MEDICAL TREATMENT [NOT SHOWN]

In this section we will ask questions related to [your current medical treatment] [the current medical treatment of the individual for whom you care].

[IF S8=1 OR S11=1 SHOW Q33]- These respondents indicated they are currently receiving Spinraza®

Q33. For how long [have you][has the individual for whom you care] received Spinraza®?

1. Less than 2 months
2. 4 months to 6 months
3. 6 months to 12 months
4. Greater than 12 months

[IF S8=2 OR S11=2 SHOW Q34]- These respondents indicated they are not currently receiving Spinraza®

Q34. As you are not currently receiving Spinraza®, [have you][has the individual for whom you care] ever received Spinraza®?

1. Yes, I was in clinical trials or early access program, but not currently
2. Yes, I was treated with Spinraza® in the past, but not currently 3- No

[IF Q34=1 OR 2 SHOW Q35]

Q35. Please indicate the reason [you][the individual for whom you provide care] no longer

[take][takes] Spinraza®. Please select all that apply. [MULTI-SELECT, FORCE TO SELECT

AT LEAST ONE]

* 1. Lack of efficacy
  2. Side effects
  3. Challenging treatment route of administration (intrathecal/spinal)
  4. High out-of-pocket cost
  5. Overall treatment regimen too burdensome
  6. Insurance Ineligibility
  7. [I][He/she] was told by [my][his/her] physician that [I][he/she] was not a candidate for Spinraza®
  8. Clinical trial participation
  9. Lack of administration center nearby
  10. Waiting for more clinical data and clinical experience with Spinraza®
  11. Not sure if Spinraza is the right treatment for [me][the individual for whom I care]
  12. Individual/caregiver decided against it
  13. Other (SPECIFY)

[IF Q35=7 SHOW Q36]

Q36. Please indicate the reason [you are not][the individual for whom you care is not] a candidate for Spinraza®?

* 1. Type 4 SMA
  2. Older age
  3. Scoliosis
  4. Invasive ventilation/tracheostomy
  5. Other (SPECIFY)

Q37. Which of the following surgeries [have you][has the individual for whom you care] received for SMA? Please select all that apply. [MULTI-SELECT, FORCE TO SELECT AT

LEAST ONE]

* 1. Spinal fusion
  2. Spinal rods
  3. Gastrostomy (G-tube)
  4. Joint contracture
  5. Tracheotomy

[IF S8=1 OR S11=1 SHOW Q38 and Q39. ; IF S8=2 OR S11=2 SKIP TO Q40]

Q38. Overall, how satisfied are you with Spinraza®?

|  |  |  |  |  |
| --- | --- | --- | --- | --- |
| ○ | ○ | ○ | ○ | ○ |
| Not at all satisfied | A little satisfied | Somewhat satisfied | Very satisfied | Extremely satisfied |

Q39. Please rate your satisfaction with Spinraza® with respect to the changes you have seen based on the following dimensions

|  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- |
|  | Not at all satisfied | A little satisfied | Somewhat satisfied | Very satisfied | Extremely satisfied |
| Motor function | ○ | ○ | ○ | ○ | ○ |
| Respiratory function | ○ | ○ | ○ | ○ | ○ |
| Bulbar function (e.g., ability to eat and speak) | ○ | ○ | ○ | ○ | ○ |
| Energy and stamina | ○ | ○ | ○ | ○ | ○ |
| Other symptoms (e.g., pain, constipation) | ○ | ○ | ○ | ○ | ○ |
| Activities of daily living (e.g., bathing/showring, dressing,  feeding) | ○ | ○ | ○ | ○ | ○ |
| Physical functioning | ○ | ○ | ○ | ○ | ○ |
| Social functioning | ○ | ○ | ○ | ○ | ○ |
| Emotional well-being | ○ | ○ | ○ | ○ | ○ |
| Caregiver quality of life | ○ | ○ | ○ | ○ | ○ |
| Safety/adverse events | ○ | ○ | ○ | ○ | ○ |
| Treatment effect sustained | ○ | ○ | ○ | ○ | ○ |
| over time (or until next dose) |  |  |  |  |  |
| Administration (including risk and discomfort) | ○ | ○ | ○ | ○ | ○ |
| Time commitment to treatment | ○ | ○ | ○ | ○ | ○ |

SECTION 5: BARRIERS TO CARE [NOT SHOWN]

In this section, we will ask you some questions about barriers to medical care.

Q40. In a typical month, how often do you speak with [your insurance provider][the insurance provider for the individual for whom you care] regarding [your][their] medication/access or [your][their] medication/medical bills?

* 1. Daily
  2. Weekly
  3. Once a month
  4. Less than once a month
  5. Never

[IF S8=1 OR S11=1 SHOW Q41; IF S8=2 OR S11=2 SKIP TO Q46]

Q41. [Are you][Is the individual for whom you care] a member of any copay assistance program for Spinraza®?

* 1. Yes
  2. No

Q42. How much are the out-of-pocket costs for each Spinraza® injection (including drug and its administration)?

$\_\_\_\_\_\_ [NUMERIC 0- 10,000]

Q43. How long did it take for the insurance company to approve Spinraza® treatment?

* 1. Less than 1 month
  2. 2 to 6 months
  3. Greater than 6 months

Q44. How close is the SMA treatment center where [you receive][the individual for whom you care receives] SMA treatment?

* 1. Less than 1-hour drive
  2. 1-2-hour drive
  3. Greater than 2-hour drive

Q45. Please rate [your level of discomfort (0 to 10)][the level of discomfort (0-10) of the individual for whom you provide care] when experiencing an intrathecal administration of Spinraza®? 0 indicates no discomfort and 10 indicates extreme discomfort.

\_\_\_\_\_\_ [NUMERIC, 0 TO 10]

Q46. In a typical month, how much time do you spend doing the following activities related to SMA? Please fill in the table below for each category to the nearest half hour.

NUMERIC, 0-1000

SHOW ERROR IF DECIMAL OTHER THAN 0 OR 5

|  |  |  |
| --- | --- | --- |
|  | Category | Hours |
| 1 | Hours spent **each week** managing  SMA treatment (e.g., doctor office visit, OT, PT etc.) |  |
| 2 | **Total** hours spent working with the individual for whom you care (including managing treatment) (SHOW IF S1=2) |  |

[IF S8=1 OR S11=1 SHOW Q47]

Q47. In a typical month, when [you receive][the individual for whom you provide care receives] Spinraza® treatment, how much time did you spend doing the following activities related to [your][their] SMA treatment? Please fill in the table below for each category to the nearest half hour.

NUMERIC, 0-1000

SHOW ERROR IF DECIMAL OTHER THAN 0 OR 5

|  |  |  |
| --- | --- | --- |
|  | Category | Hours |
| 1 | Meeting with the medical team, such as [my][their] neurologist or physical therapist (not to receive the medication) |  |
| 2 | Driving to an office/treatment center to receive Spinraza® |  |
| 3 | Waiting to receive Spinraza® |  |
| 4 | The procedure to receive Spinraza® |  |
| 5 | Waiting post procedure |  |
| 6 | Working with [my][their] insurance provider |  |

Q48. In a typical month, what proportion of your time spent on SMA management and treatment came from the following areas?

|  |  |  |
| --- | --- | --- |
|  | Category | Proportion |
| 1 | Paid work (such as paid job) | NUMERIC, 0100 |
| 2 | Un-paid work (such as household chores, unpaid obligations, child care) | NUMERIC, 0100 |
| 3 | Social activities, hobbies, or lifestyle activities | NUMERIC, 0100 |
|  | TOTAL | [MUST SUM TO  100] |

[IF S8=1 OR S11=1 SHOW Q49]

Q49. What are your top three concerns about [your Spinraza® treatment][Spinraza® treatment of the individual for whom you care]?

[RANKING EXERCISE: ALLOW SELECTION OF 3 RESPONSES ONLY AND FORCE

RANK 1, 2, 3]

|  |  |  |
| --- | --- | --- |
|  | Category | Rank |
| 1 | Finding a center to administer the medication |  |
| 2 | Time spent working with any insurance provider regarding the medication |  |
| 3 | Total time involved with receiving medication (including travel time) |  |
| 4 | Total time spent with medical team outside of receiving therapy |  |
| 5 | Route of administration |  |
| 6 | Out of pocket costs |  |
| 7 | Treatment effect waning over time |  |
| 8 | Treatment efficacy not strong enough |  |
| 9 | Treatment side effects |  |

SECTION 6: NEW MEDICATION [NOT SHOWN]

In this section, we will ask you some questions about new SMA medications.

Q50. When thinking about a new medication, what factors would stand out as the most important? Please rank the top 3 factors.

[RANKING EXERCISE: ALLOW SELECTION OF 3 RESPONSES ONLY AND FORCE

RANK 1, 2, 3]

|  |  |  |
| --- | --- | --- |
|  | Category | Rank |
| 1 | Efficacy |  |
| 2 | Safety |  |
| 3 | Route of administration |  |
| 4 | Fixed dosing |  |
| 5 | Robust clinical data |  |
| 6 | Proximity to treatment center |  |
| 7 | Good care team |  |
| 8 | Insurance approval process |  |
| 9 | Total time commitment to treatment and care coordination |  |
| 10 | Out of pocket costs |  |
| 11 | Good patient assistance programs |  |

[IF S1=2 SHOW SECTION 9, OTHERWISE SKIP TO END SCREEN]

SECTION 7: EQ-5D [NOT SHOWN] In this section we want to assess your quality of life as a caregiver of an individual with SMA.

Q53.

Under each heading, please tick the ONE box that best describes your health **TODAY**.

[MUST SELECT ONLY ONE PER GROUP]

# MOBILITY

|  |  |
| --- | --- |
| I have no problems in walking about |  |
| I have slight problems in walking about |  |
| I have moderate problems in walking about |  |
| I have severe problems in walking about |  |
| I am unable to walk about    SELF-CARE |  |
| I have no problems washing or dressing myself |  |
| I have slight problems washing or dressing myself |  |

I have moderate problems washing or dressing myself 

I have severe problems washing or dressing myself 

I am unable to wash or dress myself 

USUAL ACTIVITIES (e.g. work, study, housework, family or leisure activities)

I have no problems doing my usual activities 

I have slight problems doing my usual activities 

I have moderate problems doing my usual activities 

I have severe problems doing my usual activities 

I am unable to do my usual activities 

# PAIN / DISCOMFORT

I have no pain or discomfort 

I have slight pain or discomfort 

I have moderate pain or discomfort 

I have severe pain or discomfort 

I have extreme pain or discomfort 

# ANXIETY / DEPRESSION

I am not anxious or depressed 

I am slightly anxious or depressed 

I am moderately anxious or depressed 

I am severely anxious or depressed 

I am extremely anxious or depressed 

[NEW SCREEN]

## END SCREEN [NOT SHOWN]

Thank you for your participation! You have now reached the end of the survey.

[END SURVEY]