

*September 2025*

**SMA ATU Q2 2025**

**PATIENT QUESTIONNAIRE**

Drug Safety Note:

* A number of questions in this questionnaire use a 1-7 sliding scale. We consider ratings of 1-3 as negative/weak (potentially reportable), 4 as neutral, and 5-7 as strong/positive.
* One question in this questionnaire uses a 1-10 sliding scale. We consider ratings of 1-4 as negative/weak (potentially reportable), 5-6 as neutral, and 7-10 as strong/positive.
* All open-ended responses will be reviewed for adverse events, regardless of whether the question is noted as “POTENTIAL AE”

|  |  |
| --- | --- |
| **RECRUITMENT QUOTAS** | **TOTAL N=50** |
| **PRIMARY QUOTA:** |  |
| 0-2 years old  *PATIENT AGE=1* | **N=5-10** |
| 3-5 years old  *PATIENT AGE=2* | **N=15-20**  **(minimum of n=5 3-5 and n=5 6-17)** |
| 6-17 years old  *PATIENT AGE=3* |
| 18+ years old  *PATIENT AGE=4 OR 5 OR 6* | **N=25** |
| **SECONDARY QUOTA:** |  |
| **SMA Treatment Naïve**  *TREATMENT STATUS=3* | **N=10 (minimum of n=5)** |
| **Discontinued DMT**  *TREATMENT STATUS=2* | **N=10 (minimum of n=5)** |
| **Currently on Evrysdi**  *CURRENT TREATMENT=1 OR 2 OR 6 OR 7* | **N=15 (minimum n=15)** |
| **Currently on Spinraza**  *CURRENT TREATMENT=3 OR 4* | **N=15 (minimum n=15)** |
| **Currently on Zolgensma Only**  *CURRENT TREATMENT=5* | **N=5 (cap of n=5)** |

**SOFT QUOTAS**

|  |  |
| --- | --- |
| **NON-COMPLIANT ON EVRYSDI**  *(COMPLIANT=2 OR S12=1-8)* | **At least n=5 (start with natural fallout)** |
| **EVRYSDI DISCONTINUED**  *(S9r1=2)* | **At least n=5 (start with natural fallout)** |
| **EVRYSDI SWITCH**  *(S9r1=2 AND S9r2=1)* | **At least n=5 (start with natural fallout)** |
| **TABLET USER**  *(S9r3=1)* | **--** |

**SCREENING QUESTIONNAIRE:**

We are conducting interviews among individuals like yourself, and we would like to include your opinions. If you qualify for and complete this interview, you will receive an honorarium in exchange for your time and valued feedback. We will now ask you a few questions to determine whether you are qualified for the interview. We want to assure you that your responses will be kept completely confidential.

S1. For the purposes of this study, you may be asked to provide personal information, including health information. Providing this information is voluntary.

Are you happy to proceed with this screening survey on this basis?

|  |  |  |
| --- | --- | --- |
| 1 | Yes |  |
| 2 | No | **TERMINATE IMMEDIATELY** |

**POTENTIAL AE IF EVRYSDI USER**

S2. Have you or someone you care for been diagnosed with any of the following conditions? *Please select all that apply.*

|  |  |  |
| --- | --- | --- |
| 1 | Amyotrophic lateral sclerosis (ALS) |  |
| 2 | Multiple Sclerosis (MS) |  |
| 3 | Duchenne muscular dystrophy (DMD) |  |
| 4 | Scoliosis |  |
| 5 | Spinal muscular atrophy (SMA) |  |
| 6 | Myasthenia gravis (MG) |  |
| 7 | Cardiomyopathy |  |
| 8 | Kennedy’s disease |  |
| 99 | None of the above apply *[EXCLUSIVE, ANCHOR]* | **TERMINATE** |
| **TERMINATE IMMEDIATELY IF RESPONDENT DOES NOT SELECT CODE 5**  **TERMINATE IMMEDIATELY IF RESPONDENT SELECTS ALL CODES (1-8)** | | |

S3. Please select the option that most accurately describes your experience with SMA:

**Spinal muscular atrophy (SMA)** is a progressive neurodegenerative disease that affects the motor nerve cells in the spinal cord and impacts the muscles used for activities such as breathing, eating, crawling, and walking. It is caused by a mutation in the survival motor neuron gene 1 (SMN1).

|  |  |  |
| --- | --- | --- |
| 1 | I have been diagnosed with SMA | **CLASSIFY AS PATIENT** |
| 2 | I am a caregiver for a child who has been diagnosed with SMA, and I play an active role in their treatment | **CLASSIFY AS CAREGIVER** |
| 99 | None of the above apply *[EXCLUSIVE, ANCHOR]* | **TERMINATE IMMEDIATELY** |

GROUP(Hidden Variable)

|  |  |  |
| --- | --- | --- |
| 1 | Patient | *S3=1* |
| 2 | Caregiver | *S3=2* |

*ASK IF CAREGIVER*

S4. Please indicate the extent of your involvement with the following tasks.

|  |  |  |  |  |
| --- | --- | --- | --- | --- |
|  | *RANDOMIZE* | **I am primarily involved in this task** | **Both the individual living with SMA and I are equally involved in this task** | **I have no role in this task** |
| 1 | Performing independent research for SMA treatment options |  |  |  |
| 2 | Interacting with the person’s doctor |  |  |  |
| 3 | Supporting or guiding the person in treatment decisions along with their doctor |  |  |  |
| 4 | Providing emotional or logistical support (e.g., scheduling, transportation) |  |  |  |
| **TERMINATE IF RESPONDENT SELECTS “I HAVE NO ROLE IN THIS TASK”**  **FOR CODES 1, 2, AND 3** | | | | |

S5**.** How old *[IF PATIENT:* are you*]* *[IF CAREGIVER:* is the person you care for*]*?

*[IF CAREGIVER:* If the person you care for is less than 1 year old, leave the years blank and fill in the person’s age in months.*]*

|  |  |  |
| --- | --- | --- |
| 1 | \_\_\_\_\_\_ years old | *FOR PATIENTS RANGE: 1-99*  *FOR CAREGIVERS RANGE: 1-17* |
| 2 | Less than 1 year old  (enter age:) \_\_\_\_\_\_ months old | *SHOW FOR CAREGIVER ONLY. RANGE: 0-11*  *ONLY REQUIRED TO FILL IN EITHER S5 \_1 OR S5\_2* |
| **IF PATIENT AND S5\_1 <18, TERMINATE**  **IF CAREGIVER AND S5\_1>17, TERMINATE** | | |

PATIENT\_AGE(Hidden Variable)

|  |  |  |
| --- | --- | --- |
| 1 | 0-2 | *S5\_1=1-2 OR S5\_2>0* |
| 2 | 3-5 | *S5\_1=3-5* |
| 3 | 6-17 | *S5\_1=6-17* |
| 4 | 18-24 | *S5\_1=18-24* |
| 5 | 25-39 | *S5\_1= 25-39* |
| 6 | 40+ | *S5\_1>= 40* |

S6. Which medicines are you aware of that treat **spinal muscular atrophy** (SMA)?

|  |  |
| --- | --- |
| *OPEN END: REQUIRE AT LEAST 4 CHARACTERS* | |
|  | |
|  | |
|  | |
|  | |
| Don’t know | *[EXCLUSIVE]* |
| *OPEN END: REQUIRE MINIMUM OF 4 CHARACTERS* | |

S7. Which of the following medicines have you heard of that treat SMA?

*Please select all that apply.*

|  |  |
| --- | --- |
|  | *RANDOMIZE* |
| 1 | Evrysdi (risdiplam) liquid formulation |
| 4 | Evrysdi (risdiplam) Tablet |
| 2 | Spinraza (nusinersen) |
| 3 | Zolgensma (onasemnogene abeparvovec-xioi) |
| 98 | Other (specify) *[ANCHOR, SPECIFY]* |
| 99 | None of these *[ANCHOR, EXCLUSIVE]* |
| **TERMINATE IF S7r1<>1 AND S7r4<>1** | |

*ASK IF CAREGIVER AND AWARE OF ZOLGENSMA (S7r3=1)*

S8. Has the person you care forreceived **Zolgensma (onasemnogene abeparvovec-xioi)** as a treatment for SMA?

|  |  |
| --- | --- |
| 1 | Yes |
| 2 | No |
| 3 | Don’t Know |

**POTENTIAL AE IF PAST EVRYSDI USER**

S9. For each of the following SMA treatments, please indicate which *[IF PATIENT:* you are*]* *[IF CAREGIVER:* the person you care for is*]* currently taking and have taken in the past, if any.

|  |  |  |  |  |
| --- | --- | --- | --- | --- |
|  | *RANDOMIZE. REQUIRE ONE RESPONSE FOR ROWS 1-3, DO NOT FORCE RESPONSE FOR ROW 98.* | **Currently Taking**  *[ONLY ALLOW 1 RESPONSE]* | **Taken in the Past, but no longer** | **Never Taken** |
| 1 | Evrysdi (risdiplam) liquid formulation |  |  |  |
| 3 | Evrysdi (risdiplam) Tablet |  | *~~DO NOT ALLOW RESPONSE~~* |  |
| 2 | Spinraza (nusinersen) |  |  |  |
| 98 | Other (specify)  *[ANCHOR, SPECIFY]* |  |  |  |

TREATMENT STATUS (Hidden Variable)

|  |  |  |
| --- | --- | --- |
| 1 | Current DMT User | *S9r1c1=1 OR S9r2c1=1 OR S9r3c1=1 OR S8=1* |
| 2 | Discontinued DMT User | *(S9r1c2=1 OR S9r2c2=1 OR S9r3c2=1) AND S9r1c1<>1 AND S9r2c1<>1 AND S8<>1 AND S9r3c1<>1 AND S9r3c2<>1* |
| 3 | Treatment Naïve | *S9r1c3=1 AND S9r2c3=1 AND S8<>1*  *AND S9r3c3=1* |

CURRENT TREATMENT (Hidden Variable)

|  |  |  |
| --- | --- | --- |
| 1 | Evrysdi Liquid Formulation Only | *(S8=2 OR S8=3 OR S8 IS NOT ANSWERED) AND S9r1c1=1* |
| 2 | Evrysdi Liquid Formulation & Zolgensma | *S8=1 AND S9r1c1=1* |
| 6 | Evrysdi Tablet Only | *(S8=2 OR S8=3 OR S8 IS NOT ANSWERED) S9r3c1=1* |
| 7 | Evrysdi Tablet & Zolgensma | *S8=1 AND S9r3c1=1* |
| 3 | Spinraza Only | *(S8=2 OR S8=3 OR S8 IS NOT ANSWERED) AND S9r2c1=1* |
| 4 | Spinraza & Zolgensma | *S8=1 AND S9r2c1=1* |
| 5 | Zolgensma Only | *S8=1 AND S9r1c1<>1 AND S9r2c1<>1* |

EVRYSDI USER OR NON-USER (Hidden Variable)

|  |  |  |
| --- | --- | --- |
| 1 | User | *S9r1c1=1 OR S9r3c1=1* |
| 2 | Non-User | *S9r1c1<>1 AND S9r3c1<>1* |

**POTENTIAL AE IF EVRYSDI USER**

ASK IF CURRENT TREATMENT=1,2,3 4, 6, OR 7

S10. You mentioned that *[IF PATIENT:* you are*]* *[IF CAREGIVER:* the person you care for is*]* currently taking *[IF CURRENT TREATMENT=1 OR 2 ~~OR 6 OR 7~~:* theEvrysdi liquid formulation*]* *[IF CURRENT TREATMENT=6 OR 7:* the Evrysdi Tablet*] [IF CURRENT TREATMENT=3 OR 4:* Spinraza*]*. Did *[IF PATIENT:* you*]* *[IF CAREGIVER:* the person you care for*]* discontinue taking *[IF CURRENT TREATMENT=1, 2, 6, OR 7:* Evrysdi*]* *[IF CURRENT TREATMENT=3 OR 4:* Spinraza*]* at any point in the past?

|  |  |
| --- | --- |
| 1 | Yes |
| 2 | No |

RESTART (Hidden Variable)

|  |  |  |
| --- | --- | --- |
| 1 | Evrysdi Restart | *S10=1 AND CURRENT TREATMENT=1 OR 2 OR 6 OR 7* |
| 2 | Spinraza Restart | *S10=1 AND CURRENT TREATMENT=3 OR 4* |
| 3 | No Restart | *S10=2* |

*ASK IF RESTART*

S10A. Please indicate when *[IF PATIENT:* you*]* *[IF CAREGIVER:* the person you care for*]* **first started and stopped** taking the treatment below.

*Please select the month and year. If unsure, please indicate as closely as you can remember.*

|  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- |
|  | | **INITIAL TREATMENT START DATE**  *(date you started treatment the first time)* | | **DISCONTINUATION DATE**  *(date you previously stopped using treatment)* | |
|  |  | **Month**  *[SHOW DROP DOWN MENU OF MONTHS]* | **Year**  *[SHOW DROP DOWN MENU]* | **Month**  *[SHOW DROP DOWN MENU OF MONTHS]* | **Year**  *[SHOW DROP DOWN MENU]* |
| 1 | Evrysdi (risdiplam)  *[SHOW IF EVRYSDI RESTART]* |  | *Show years*  *2020-2024* |  |  |
| 2 | Spinraza (nusinersen)  *[SHOW IF SPINRAZA RESTART]* |  | *Show years*  *2016-2024* |  |  |

INITIAL TIME ON TREATMENT RESTARTED (Hidden Variable)

|  |  |  |
| --- | --- | --- |
| 1 | 1-3 months | *DATES FROM C3-C4 ARE 1-3 MONTHS MORE THAN C1-C2* |
| 2 | 4-6 months | *DATES FROM C3-C4 ARE 4-6 MONTHS MORE THAN C1-C2* |
| 3 | 7-12 months | *DATES FROM C3-C4 ARE 7-12 MONTHS MORE THAN C1-C2* |
| 4 | More than 12 months | *S10Ar1c4>S10Ar1c2* |

**POTENTIAL AE**

*ASK IF EVRYSDI USER=1*

S11. *[IF PATIENT* Have you*] [IF CAREGIVER* Has the person you care for*]* ever missed a dose of Evrysdi (risdiplam)?

*Please select one option that best describes your situation.*

|  |  |
| --- | --- |
| 1 | Yes, frequently miss a dose |
| 2 | Yes, sometimes miss a dose |
| 3 | Yes, rarely miss a dose |
| 4 | No, never miss a dose |
| 99 | Don’t know/Don’t Remember |

COMPLIANT (Hidden Variable)

|  |  |  |
| --- | --- | --- |
| 1 | Compliant | *S11=4* |
| 2 | Non-Compliant | *S11=1-3* |

**POTENTIAL AE**

*ASK IF S11=1-3 (MISSED A DOSE)*

S12. In the last month, how often did *[IF PATIENT* you*] [IF CAREGIVER* the person you care for*]* miss a dose of Evrysdi (risdiplam)?

*Please select one option.*

|  |  |
| --- | --- |
| 1 | Missed 1 to 2 doses of Evrysdi (risdiplam) in the last month |
| 2 | Missed 3 to 4 doses of Evrysdi (risdiplam) in the last month |
| 3 | Missed 5 to 6 doses of Evrysdi (risdiplam) in the last month |
| 4 | Missed 7 to 8 doses of Evrysdi (risdiplam) in the last month |
| 5 | Missed 9 to 10 doses of Evrysdi (risdiplam) in the last month |
| 6 | Missed 11 to 12 doses of Evrysdi (risdiplam) in the last month |
| 7 | Missed 13 to 14 doses of Evrysdi (risdiplam) in the last month |
| 8 | Missed 15 or more doses of Evrysdi (risdiplam) in the last month |
| 99 | Don’t know/Don’t remember |

*ASK IF PREVIOUSLY TAKEN OR CURRENTLY TAKING SMA MEDICINE (S8=1 OR S9r1c1=1 OR S9r1c2=1 OR S9r2c1=1 OR S9r2c2=1 OR S9r3c1=1)*

S13. Please indicate when *[IF PATIENT:* you*]* *[IF CAREGIVER:* the person you care for*]* **started** taking the treatment(s) below.

*Please select the month and year. If unsure, please indicate as closely as you can remember.*

*[IF RESTART:* Note: If you stopped using a treatment and then started using that treatment again, please respond using the date you **restarted** the treatment*]*

|  |  |  |  |
| --- | --- | --- | --- |
|  |  | **Month**  *[SHOW DROP DOWN MENU OF MONTHS]* | **Year**  *[SHOW DROP DOWN MENU]* |
| 1 | Evrysdi (risdiplam) liquid formulation  *[SHOW IF S9r1c1=1 OR S9r1c2=1]* |  | *Show years*  *2020-2025* |
| 4 | Evrysdi (risdiplam) Tablet  *[SHOW IF S9r3c1=1]* |  | *Only show year*  *2025* |
| 2 | Spinraza (nusinersen)  *[SHOW IF S9r2c1=1 OR S9r2c2=1]* |  | *Show years*  *2016-2025* |
| 3 | Zolgensma (onasemnogene abeparvovec-xioi)  *[SHOW IF S8=1]* |  | *Show years*  *2019-2025* |

TIME ON CURRENT TREATMENT (Hidden Variable)

|  |  |  |
| --- | --- | --- |
| 1 | 1-3 months | *S9r1c1=1 AND S13c1 and S13c2 date is 1-3 months ago from current date* |
| 2 | 4-6 months | *S9r1c1=1 AND S13c1 and S13c2 date is 4-6 months ago from current date* |
| 3 | 7-12 months | *S9r1c1=1 AND S13c1 and S13c2 date is 7-12 months ago from current date* |
| 4 | More than 12 months | *S9r1c1=1 AND S13c1 and S13c2 date is more than 12 months ago from current date* |

EVRYSDI RESTART GAP (Hidden Variable)

|  |  |  |
| --- | --- | --- |
| 1 | 1-3 months | *IF RESTART=1 AND S9r2c3=1 (NEVER TAKEN SPIN) AND S13 DATE IS 1-3 MONTHS MORE THAN S10A C3-C4 DATE* |
| 2 | 4-6 months | *IF RESTART=1 AND S9r2c3=1 (NEVER TAKEN SPIN) AND S13 DATE IS 4-6 MONTHS MORE THAN S10A C3-C4 DATE* |
| 3 | 7-12 months | *IF RESTART=1 AND S9r2c3=1 (NEVER TAKEN SPIN) AND S13 DATE IS 7-12 MONTHS MORE THAN S10A C3-C4 DATE* |
| 4 | More than 12 months | *IF RESTART=1 AND S9r2c3=1 (NEVER TAKEN SPIN) AND S13 DATE IS 1 YEAR OR MORE THAN S10A C3-C4 DATE* |

SPINRAZA RESTART GAP (Hidden Variable)

|  |  |  |
| --- | --- | --- |
| 1 | 1-3 months | *IF RESTART=2 AND S9r1c3=1 (NEVER TAKEN EVRYSDI) AND S13 DATE IS 1-3 MONTHS MORE THAN S10A C3-C4 DATE* |
| 2 | 4-6 months | *IF RESTART=2 AND S9r1c3=1 (NEVER TAKEN EVRYSDI) AND S13 DATE IS 4-6 MONTHS MORE THAN S10A C3-C4 DATE* |
| 3 | 7-12 months | *IF RESTART=2 AND S9r1c3=1 (NEVER TAKEN EVRYSDI) AND S13 DATE IS 7-12 MONTHS MORE THAN S10A C3-C4 DATE* |
| 4 | More than 12 months | *IF RESTART=2 AND S9r1c3=1 (NEVER TAKEN EVRYSDI) AND S13 DATE IS 1 YEAR OR MORE THAN S10A C3-C4 DATE* |

*ASK IF PREVIOUSLY TAKEN SMA MEDICINE (S9r1c2=1 OR S9r2c2=1)*

S14. Please indicate when *[IF PATIENT:* you*]* *[IF CAREGIVER:* the person you care for*]* **stopped** taking the treatment(s) below.

*Please select the month and year. If unsure, please indicate as closely as you can remember.*

|  |  |  |  |
| --- | --- | --- | --- |
|  |  | **Month**  *[SHOW DROP DOWN MENU OF MONTHS]* | **Year**  *[SHOW DROP DOWN MENU]* |
| 1 | Evrysdi (risdiplam) liquid formulation  *[SHOW IF S9r1c2=1]* |  | *Show years*  *2020-2025* |
| 2 | Spinraza (nusinersen)  *[SHOW IF S9r2c2=1]* |  | *Show years*  *2016-2025* |

TIME ON PAST TREATMENT (Hidden Variable)

|  |  |  |
| --- | --- | --- |
| 1 | Less than 1 month | *S14c1 and S14c2 date is less than 1 month from S13c1 and S13c2 date* |
| 2 | 1-3 months | *S14c1 and S14c2 date is 1-3 months from S13c1 and S13c2 date* |
| 3 | 4-6 months | *S14c1 and S14c2 date is 4-6 months from S13c1 and S13c2 date* |
| 4 | 7-12 months | *S14c1 and S14c2 date is 7-12 months from S13c1 and S13c2 date* |
| 5 | More than 12 months | *S14c1 and S14c2 date is more than 12 months from S13c1 and S13c2 date* |

EVRYSDI TABLET CONVERSION

|  |  |  |
| --- | --- | --- |
| 1 | Conversion from Liquid | *IF CURRENT TABLET USER (CURRENT TREATMENT=6 OR 7) AND DISCONTINUED EVRYSDI LIQUID WITHIN 6 MONTHS OF TABLET START* |
| 2 | Start from Naïve | *IF CURRENT TABLET USER (CURRENT TREATMENT=6 OR 7) AND NO PAST TREATMENT USE* |
| 3 | Switch from Spinraza | *IF CURRENT TABLET USER (CURRENT TREATMENT=6 OR 7) AND DISCONTINUED SPINRAZA WITHIN 6 MONTHS OF TABLET START* |
| 4 | Switch/Start from Zolgensma only | *IF CURRENT TABLET USER (CURRENT TREATMENT=6 OR 7) AND TAKEN ONLY ZOLGENSMA IN THE PAST* |
| 5 | Switch from Clinical Trial | *IF CURRENT TABLET USER (CURRENT TREATMENT=6 OR 7) AND DISCONTINUED SPINRAZA CLINICAL TRIAL WITHIN 6 MONTHS OF TABLET START* |

I**F QUALIFIED, INVITE:**

Thank you for answering these questions. Based on your responses, we would like to invite you to participate in our study. The survey will take approximately **30 minutes** to complete.

We will not ask you to disclose any information that would identify you, such as your name, telephone, email address, etc.

Are you interested in participating?

|  |  |  |
| --- | --- | --- |
| 1 | Yes |  |
| 2 | No | **TERMINATE** |

*SHOW ON SEPARATE SCREEN:*

Thank you for your interest in participating in this research study. **Cognitive Consulting** wishes to confirm your interest in participating in this research. This Participant Agreement (“Agreement”) sets forth the terms and conditions of your participation.

1. Research Intent. Any information provided to you is for market research purposes only and is not intended to recommend or promote a therapeutic approach, medical procedure, specific product, or class of products, or to be a representation of approved product labeling. You have no obligation to use, purchase, recommend or arrange for the use of any therapeutic approach, medical procedure, or product, based on your participation in this market research. You represent that the facts and information that you provide in the course of your participation are true and accurate.
2. Confidentiality. You agree to keep in confidence for a period of three (3) years from the expiration or termination of this Agreement any confidential information disclosed to you during this discussion. “Confidential Information” includes, but is not limited to, information about the Client (if applicable) or the subject of the research that is either proprietary in nature or is not known to the general public.

Your individual responses and any other information you provide in the context of your participation in this research will be kept confidential and will only be used as described in this document and will not be disclosed to any third party (other than those described in this document) without your approval or unless required by regulation or by court order.

1. Payment. As consideration for the time you are taking to participate in this market research and for your performance of the obligations under this Agreement, we shall pay you the amount that was previously communicated in your invitation email.

By checking “I agree” below, you indicate your acceptance of the terms of this Market Research Participant Agreement.

|  |  |  |
| --- | --- | --- |
| 1 | I agree |  |
| 2 | I do not agree | **TERMINATE** |

Thank you for agreeing to participate in this market research survey. Please be assured that all of your responses will be kept in strict confidence. The data from the survey will be blinded, and we will ensure that the strictest standards of privacy are maintained with the content of your responses. No identifying information will be collected, and all responses will remain anonymous. As such, please make every effort to be open and honest when responding to the questions. The information you provide in this survey is greatly appreciated and valuable.

*SHOW ON SEPARATE SCREEN:*

Different patients sometimes respond in different ways to the same medicine, and some side effects may not be discovered until many people have used a medicine over a period of time. For this reason, we are now required to pass on to our client, who is a pharmaceutical company, details of any side effects/product complaints related to their own products that are mentioned during the course of market research. Although what you say will, of course, be treated in confidence, should you mention during the discussion a side effect when you, or someone you know, became ill after taking one of our client’s medicines, or a problem you have had with one of our client’s medicines we will need to report this, so that they can learn more about the safety of their medicines. Everything else you say during the course of the interview will continue to remain confidential, and you will still have the option to remain anonymous if you so wish.

Are you happy to proceed with the interview on this basis? Please indicate your response by selecting the appropriate option below:

|  |  |  |
| --- | --- | --- |
| 1 | I would like to proceed and give permission for my contact details to be passed on to the product surveillance department of the company if an adverse event or product technical complaint is mentioned by me during the survey. |  |
| 2 | I would like to proceed but do not wish for my contact details to be passed on to the product surveillance department of the company if an adverse event or product technical complaint is mentioned by me during the survey. |  |
| 3 | I don’t want to proceed and wish to end the interview here. | **TERMINATE** |

**MAIN QUESTIONNAIRE**

**SECTION A: PRODUCT PERCEPTIONS**

To begin, we would like to learn your opinions about medicines for the treatment of SMA.

**POTENTIAL AE (IF EVRYSDI USER)**

A1. Using a 7-point scale where 1=Extremely Negative and 7=Extremely Positive, what is your overall impression of the following **spinal muscular atrophy (SMA)** medicines? *Please select one response per row.*

|  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- |
| *RANDOMIZE* | | **Extremely Negative**  **1** | **2** | **3** | **4** | **5** | **6** | **Extremely**  **Positive**  **7** |
| 1 | Evrysdi (risdiplam) liquid formulation  *SHOW IF S7r1=1* |  |  |  |  |  |  |  |
| 4 | Evrysdi (risdiplam) Tablet  *SHOW IF S7r4=1* |  |  |  |  |  |  |  |
| 2 | Spinraza (nusinersen)  *SHOW IF S7r2=1* |  |  |  |  |  |  |  |
| 3 | Zolgensma (onasemnogene abeparvovec-xioi)  *SHOW IF S7r3=1* |  |  |  |  |  |  |  |

**POTENTIAL AE (IF EVRYSDI USER)**

*ASK IF TREATMENT STATUS (Hidden) = 1*

A2. Using a 7-point scale, where 1=Not At All Satisfied and 7=Extremely Satisfied, what is your level of satisfaction with the following **spinal muscular atrophy (SMA)** medicines that *[IF PATIENT* you currently use*]* *[IF CAREGIVER* the person you care for currently uses*]*?

|  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- |
| *RANDOMIZE* | | **Not At All Satisfied**  **1** | **2** | **3** | **4** | **5** | **6** | **Extremely Satisfied**  **7** |
| 1 | Evrysdi (risdiplam) liquid formulation  *SHOW IF S9r1c1=1* |  |  |  |  |  |  |  |
| 4 | Evrysdi (risdiplam) Tablet  *SHOW IF S9r3c1=1* |  |  |  |  |  |  |  |
| 2 | Spinraza (nusinersen)  *SHOW IF S9r2c1=1* |  |  |  |  |  |  |  |
| 3 | Zolgensma (onasemnogene abeparvovec-xioi)  *SHOW IF S8=1* |  |  |  |  |  |  |  |

A3. Using a 7-point scale, where 1=Not At All Interested and 7=Extremely Interested, how interested are you in learning more about the following medicines?

|  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- |
| *RANDOMIZE* | | **Not At All Interested**  **1** | **2** | **3** | **4** | **5** | **6** | **Extremely Interested**  **7** |
| 1 | Evrysdi (risdiplam) liquid formulation  *SHOW IF S7r1=1* |  |  |  |  |  |  |  |
| 4 | Evrysdi (risdiplam) Tablet  *SHOW IF S7r4=1* |  |  |  |  |  |  |  |
| 2 | Spinraza (nusinersen)  *SHOW IF S7r2=1* |  |  |  |  |  |  |  |
| 3 | Zolgensma (onasemnogene abeparvovec-xioi)  *SHOW IF S7r3=1* |  |  |  |  |  |  |  |

A4. When thinking about medicines for SMA, how important is each of the following to you?

Please rate the level of importance using a 7-point scale, where 1=Not At All Important and 7=Extremely Important.

*PROGRAMMING NOTE: If respondents’ straight line (select the same answer for all codes) at*

*A4 - show this error message: "You rated all items equally. Please review." If they proceed*

*without changing anything after seeing this message, proceed to the next screen (they can*

*proceed after seeing the message 1x).*

|  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- |
| *RANDOMIZE*  *DO NOT SHOW HEADERS* | | **Not At All Important**  **1** | **2** | **3** | **4** | **5** | **6** | **Extremely Important**  **7** |
| **EFFICACY** | | | | | | | | |
| 1 | Works well between doses / no loss of efficacy in between doses |  |  |  |  |  |  |  |
| 2 | Improves bulbar function (e.g., chewing, swallowing, speaking, etc.) |  |  |  |  |  |  |  |
| 3 | Efficacy in overall motor function (e.g., lifting head, picking up pencil, climbing stairs, supported standing etc.) |  |  |  |  |  |  |  |
| **SAFETY** | | | | | | | | |
| 4 | Is safe long-term |  |  |  |  |  |  |  |
| 5 | Is well tolerated/able to continue with treatment |  |  |  |  |  |  |  |
| **CONVENIENT** | | | | | | | | |
| 6 | Convenient administration |  |  |  |  |  |  |  |
| 7 | Is easy to start the medicine |  |  |  |  |  |  |  |
| 8 | Is easy to be compliant with (e.g., easy dosing schedule, low risk of missed doses) |  |  |  |  |  |  |  |
| **MISC** | | | | | | | | |
| 9 | Good access and affordability |  |  |  |  |  |  |  |
| 10 | Is recommended by the doctor |  |  |  |  |  |  |  |
| 11 | Designed to reach the brain, spinal cord, and other areas of the body |  |  |  |  |  |  |  |
| 14 | Designed to increase SMN protein production in both the central nervous system and peripheral tissues and organs |  |  |  |  |  |  |  |
| 12 | Suitable for combination therapy |  |  |  |  |  |  |  |
| 13 | Is FDA approved for multiple SMA patient types (e.g., age, severity, SMA type, functional capability) |  |  |  |  |  |  |  |

*ASK ONCE FOR EVRYSDI (RISDIPLAM) IF S7r1=1 OR S7r4=1 AND ONCE FOR SPINRAZA (NUSINERSEN) IF S7=r2=1*

**POTENTIAL AE (IF EVRYSDI USER)**

A5. Using a 7-point scale, where 1=Not At All Associated and 7=Highly Associated, to what extent do you associate each of the following with [INSERT Evrysdi (Risdiplam) / Spinraza (Nusinersen)]?

*PROGRAMMING NOTE: If respondents’ straight line (select the same answer for all codes) at*

*A5 - show this error message: "You rated all items equally. Please review." If they proceed*

*without changing anything after seeing this message, proceed to the next screen (they can*

*proceed after seeing the message 1x).*

|  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- |
| *RANDOMIZE*  *DO NOT SHOW HEADERS* | | **Not At All Associated**  **1** | **2** | **3** | **4** | **5** | **6** | **Highly Associated**  **7** |
| **EFFICACY** | | | | | | | | |
| 1 | Works well between doses / no loss of efficacy in between doses |  |  |  |  |  |  |  |
| 2 | Improves bulbar function (e.g., chewing, swallowing, speaking, etc.) |  |  |  |  |  |  |  |
| 3 | Efficacy in overall motor function (e.g., lifting head, picking up pencil, climbing stairs, supported standing etc.) |  |  |  |  |  |  |  |
| **SAFETY** | | | | | | | | |
| 4 | Is safe long-term |  |  |  |  |  |  |  |
| 5 | Is well tolerated/able to continue with treatment |  |  |  |  |  |  |  |
| **CONVENIENT** | | | | | | | | |
| 6 | Convenient administration |  |  |  |  |  |  |  |
| 7 | Is easy to start the medicine |  |  |  |  |  |  |  |
| 8 | Is easy to be compliant with (e.g., easy dosing schedule, low risk of missed doses) |  |  |  |  |  |  |  |
| **MISC** | | | | | | | | |
| 9 | Good access and affordability |  |  |  |  |  |  |  |
| 10 | Is recommended by the doctor |  |  |  |  |  |  |  |
| 11 | Designed to reach the brain, spinal cord, and other areas of the body |  |  |  |  |  |  |  |
| 14 | Designed to increase SMN protein production in both the central nervous system and peripheral tissues and organs |  |  |  |  |  |  |  |
| 12 | Suitable for combination therapy |  |  |  |  |  |  |  |
| 13 | Is FDA approved for multiple SMA patient types (e.g., age, severity, SMA type, functional capability) |  |  |  |  |  |  |  |

**POTENTIAL AE (IF EVRYSDI USER)**

*ASK IF CURRENTLY OR PREVIOUSLY TREATED (TREATMENT STATUS=1 OR 2)*

A6. Based on your overall experience, how likely would you be to recommend the below medicines for the treatment of spinal muscular atrophy (SMA) to other persons living with SMA?

|  |  |  |  |  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
|  | *RANDOMIZE* | **Not at All Likely to Recommend**  **0** | **1** | **2** | **3** | **4** | **5** | **6** | **7** | **8** | **9** | **Extremely Likely to Recommend**  **10** |
| 1 | Evrysdi (risdiplam) liquid formulation  *SHOW IF S9r1c1=1 OR S9r1c2=1* |  |  |  |  |  |  |  |  |  |  |  |
| 4 | Evrysdi (risdiplam) Tablet  *SHOW IF S9r3c1=1* |  |  |  |  |  |  |  |  |  |  |  |
| 2 | Spinraza (nusinersen) *SHOW IF S9r2c1=1 OR S9r2c2=1* |  |  |  |  |  |  |  |  |  |  |  |
| 3 | Zolgensma (onasemnogene abeparvovec-xioi) *SHOW IF S8=1* |  |  |  |  |  |  |  |  |  |  |  |

**SECTION B: PRODUCT USE**

In the next section of this survey, we would like to learn more about *[IF PATIENT* your SMA treatment journey*] [IF CAREGIVER* the SMA treatment journey of the person you care for*]*.

*[IF CAREGIVER* **Please provide the information on behalf of the person with SMA, who you provide care for***.]*

*ASK IF CURRENTLY OR PREVIOUSLY TREATED (TREATMENT STATUS=1 OR 2)*

B1. When choosingthe followingmedicine(s), did you specifically request the medicine from your doctor or was your doctor the first to recommend it?

|  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- |
|  |  | *SHOW IF S9r1c1=1 OR S9r1c2=1* | *SHOW IF S9r3c1=1* | *SHOW IF S9r2c1=1 OR S9r2c2=1* | *SHOW IF S8=1* |
| Evrysdi  (risdiplam) liquid formulation | Evrysdi  (risdiplam) Tablet | Spinraza  (nusinersen) | Zolgensma (onasemnogene abeparvovec-xioi) |
| 1 | I requested the medicine the doctor |  |  |  |  |
| 2 | The doctorrecommended the medicine first |  |  |  |  |
| 3 | It was a mutual discussion/decision |  |  |  |  |
| 99 | Don’t know/can’t remember |  |  |  |  |

*ASK IF S9r1=3 OR S9r2=3 OR S9r3=3 OR S8=2 OR 3*

B2. Using a 7-point scale, where 1=Not At All Likely and 7=Extremely Likely, how likely would you be to ask *[IF PATIENT* your doctor*]* [*IF CAREGIVER* the doctor of the person you care for*]* to try the following medicine?

|  |  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- | --- |
|  | *RANDOMIZE* | **Not At All Likely**  **1** | **2** | **3** | **4** | **5** | **6** | **Extremely Likely**  **7** | **Have Already Talked to Doctor**  **99** |
| 1 | Evrysdi (risdiplam) liquid formulation  *SHOW IF S9r1=3* |  |  |  |  |  |  |  |  |
| 4 | Evrysdi (risdiplam) Tablet  *SHOW IF S9r3=3* |  |  |  |  |  |  |  |  |
| 2 | Spinraza (nusinersen)  *SHOW IF S9r2=3* |  |  |  |  |  |  |  |  |
| 3 | Zolgensma (onasemnogene abeparvovec-xioi)  *SHOW IF S8=2 OR 3* |  |  |  |  |  |  |  |  |

*ASK IF HAVE NEVER TAKEN EVRYSDI, TABLET, SPINRAZA, OR ZOLGENSMA (S9r1=3 OR S9r2=3 OR S8=2 OR S8=3 OR S9r3=3)*

B3. For which of the following reasons *[IF PATIENT* have you*]* *[IF CAREGIVER* has the person, you care for*]* **never used the following** medicine(s) for the treatment of spinal muscular atrophy (SMA)?

*Please select all that apply.*

|  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- |
|  | *RANDOMIZE*  *DO NO SHOW HEADERS* | *SHOW IF S9r1=3* | *SHOW IF S9r3=3* | *SHOW IF S9r2=3* | *SHOW IF S8=2 OR 3 AND PATIENT AGE=1 OR 2* |
| Evrysdi  (risdiplam) liquid formulation | Evrysdi  (risdiplam) Tablet | Spinraza  (nusinersen) | Zolgensma (onasemnogene abeparvovec-xioi) |
| **EFFICACY** | | | | | |
| 1 | Not as effective as desired with **regard to improving overall movement** (e.g., lifting head, picking up pencil, climbing stairs, supported standing, etc.) |  |  |  |  |
| 2 | Not as effective as desired with **regard to bulbar function** (e.g., chewing, swallowing, speaking, etc.) |  |  |  |  |
| 3 | Worried aboutlosing effectiveness over time |  |  |  |  |
| 4 | Does not have enough long-term efficacy/safety data |  |  |  |  |
| **SAFETY** | | | | | |
| 5 | Worried aboutside effects from this medicine |  |  |  |  |
| 6 | Worried about being able to tolerate/continue with treatment |  |  |  |  |
| 20 | Worried about being able to administer this treatment |  |  |  |  |
| **PATIENT/PHYSICIAN FACTORS** | | | | | |
| 7 | Was notrecommended the doctor |  |  |  |  |
| 8 | Prefer a medicine that is taken orally (by mouth) | *Do not allow response* | *Do not allow response* |  |  |
| 9 | Prefer an injection into the spinal cord by a healthcare professional |  |  | *Do not allow response* | *Do not allow response* |
| 10 | Was not available when first started treatment |  |  |  |  |
| 11 | Is not easy to start the medicine |  |  |  |  |
| 18 | Not eligible for this medicine |  |  |  |  |
| 19 | Was not aware of this treatment |  |  |  |  |
| **ACCESS** | | | | | |
| 12 | Cost issues or concerns |  |  |  |  |
| 13 | Medicine is/was not covered by insurance |  |  |  |  |
| 14 | Worried to lose insurance approval for current treatment if I want to go back |  |  |  |  |
| 15 | Worried about the logistical issues with accessing medicine (e.g., at home medicine delivery/ travel to injection site etc.) |  |  |  |  |
| 16 | Do not have access to a neurologist/medical facility for treatment |  |  |  |  |
| **COMPLIANCE** | | | | | |
| 17 | Worried it would be too difficult to be compliant with |  |  |  | *Do not allow response* |
| **OTHER** | | | | | |
| 98 | Other *[ANCHOR]* |  |  |  |  |

*ASK IF CURRENTLY TREATED (CURRENT TREATMENT=1,2,3,4,6 OR 7)*

B4. Using a 7-point scale, where 1=Not At All Likely and 7=Extremely Likely, how likely are you to change *[IF PATIENT:* your current SMA treatment*] [IF CAREGIVER:* the current SMA treatment of the person you care for*]* in the next 12 months?

*Note: This includes [IF S8=2 OR 3: receiving Zolgensma or] discontinuing use of disease-modifying therapies altogether.*

|  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- |
| **Not At All Likely**  **1** | **2** | **3** | **4** | **5** | **6** | **Extremely Likely**  **7** |

**POTENTIAL AE (IF EVRYSDI USER)**

*ASK IF LIKELY TO SWITCH (B4=5-7)*

B5. For which of the following reasons *[IF PATIENT* are you*]* *[IF CAREGIVER* is the person

you care for*]* likely to change *[IF PATIENT:* your current SMA treatment*] [IF CAREGIVER:* the current SMA treatment of the person you care for*]* in the next 12 months?

*Please select all that apply.*

|  |  |
| --- | --- |
| *RANDOMIZE*  *DO NOT SHOW HEADERS.* | |
| **EFFICACY** | |
| 1 | Not as effective as desired with **regard to improving overall movement** (e.g., lifting head, picking up pencil, climbing stairs, supported standing, etc.) |
| 2 | Not as effective as desired with **regard to bulbar function** (e.g., chewing, swallowing, speaking, etc.) |
| 3 | The medicine is starting to lose effectiveness between doses *[HIDE IF CURRENT TREATMENT=1 OR 2 OR 6 OR 7]* |
| 4 | I am progressing and feel like the medication is not working |
| **TOLERABILITY** | |
| 5 | Side effect/tolerability issues with this medicine |
| 6 | Want to stop for fertility/family planning reasons |
| **ACCESS** | |
| 7 | Cost issues or concerns |
| 8 | Changes with insurance coverage |
| 9 | Worried aboutthe logistical issues with accessing medicine (e.g., at home medicine delivery/ travel to injection site etc.) |
| 10 | In order to receive Zolgensma treatment *[SHOW IF S8=1]* |
| 11 | Attending college made/will make treatment access/logistics challenging |
| 12 | Moving off the insurance plan of parent/guardian/etc. and treatment is no longer affordable |
| 13 | Difficulty with reauthorizations |
| **COMPLIANCE** | |
| 14 | Having to take the medicine every day is becoming too burdensome *[HIDE IF CURRENT TREATMENT=3 OR 4]* |
| 15 | There are difficulties opening the bottle on a daily basis *[HIDE IF CURRENT TREATMENT=3 OR 4]* |
| 16 | The intrathecal injections are becoming too burdensome *[HIDE IF CURRENT TREATMENT=1 OR 2 OR 6 OR 7]* |
| 17 | Traveling to the doctor’s office/hospital to receive the injection is becoming too burdensome *[HIDE IF CURRENT TREATMENT=1 OR 2 OR 6 OR 7]* |
| 18 | Forget to take the medicine too often *[HIDE IF CURRENT TREATMENT=3 OR 4]* |
| **MISC** | |
| 19 | Difficulties swallowing the medicine *[HIDE IF CURRENT TREATMENT=3 OR 4]* |
| 20 | Want to discontinue treatment altogether (no longer want to be treated) |
| 21 | Need or want to take a break from treatment |
| 22 | Enrolling in a clinical trial and have to stop taking the medicine |
| 23 | Transitioning care from a pediatric provider to an adult provider |
| 98 | Other (specify) *[ANCHOR] [SPECIFY]* |

*ASK IF LIKELY TO SWITCH (B4=5-7)*

B6. Which treatment would you be most likely to switch to *[IF S8=2 OR 3:*/add on*]* in the next 12 months?

|  |  |
| --- | --- |
| *RANDOMIZE* | |
| 1 | Evrysdi (risdiplam) liquid formulation  *SHOW IF S9r1c1<>1* |
| 5 | Evrysdi (risdiplam) Tablet  *SHOW IF S9r3c1<>1* |
| 2 | Spinraza (nusinersen)  *SHOW IF S9r2c1<>1* |
| 3 | Zolgensma (onasemnogene abeparvovec)  *SHOW IF S8=2 OR 3* |
| 4 | Would go untreated (no Disease Modifying Therapy) *[ANCHOR] [Do not show if S8=1]* |
| 98 | Other (specify) *[ANCHOR] [SPECIFY]* |
| 99 | Don’t know *[ANCHOR]* |

*ASK IF B6=1*

B7. Why would you switch to Evrysdi (risdiplam) liquid formulation?

*Please be as specific as possible in your response.*

|  |
| --- |
| *OPEN END: REQUIRE AT LEAST 4 CHARACTERS* |

**POTENTIAL AE (IF EVRYSDI USER)**

*ASK IF B6=5*

B7A. Why would you switch to Evrysdi (risdiplam) Tablet?

*Please be as specific as possible in your response.*

|  |
| --- |
| *OPEN END: REQUIRE AT LEAST 4 CHARACTERS* |

**POTENTIAL AE (IF EVRYSDI USER)**

*ASK IF B6=2*

B8. Why would you switch to Spinraza (nusinersen)?

*Please be as specific as possible in your response.*

|  |
| --- |
| *OPEN END: REQUIRE AT LEAST 4 CHARACTERS* |

**POTENTIAL AE (IF EVRYSDI USER)**

*ASK IF RESTART (Hidden) = 1 OR 2*

B9. You mentioned that you are currently on *[IF CURRENT TREATMENT=1 OR 2 OR 6 OR 7: “Evrysdi (risdiplam)”, IF CURRENT TREATMENT=3 OR 4:* *“Spinraza (nusinersen)”]* but have also discontinued it in the past. Why did you decide to restart *[IF CURRENT TREATMENT=1 OR 2 OR 6 OR 7: “Evrysdi (risdiplam)”, IF CURRENT TREATMENT=3 OR 4:* *“Spinraza (nusinersen)”]* after discontinuing?

*Please be as specific as possible in your response..*

|  |
| --- |
| *OPEN END: REQUIRE AT LEAST 4 CHARACTERS* |

**POTENTIAL AE (IF EVRYSDI USER)**

*ASK IF TABLET CONVERTER*

B10. You mentioned that you are currently on the Evrysdi Tablet and you were previously on the Evrysdi Liquid formulation. Which of the following best describe the reason you decided to convert from the Liquid to the Tablet?

*Please select all that apply.*

|  |  |
| --- | --- |
| *RANDOMIZE* | |
| 1 | More convenient administration |
| 2 | More precise dosing/easier to be compliant |
| 3 | Flexible administration (option to swallow whole or disperse in water) |
| 4 | Comparable or better tolerability than the liquid formulation |
| 5 | Comparable or better safety profile than the liquid formulation |
| 6 | Comparable or better efficacy than the liquid formulation |
| 7 | No isomalt (E953) or sucralose |
| 98 | Other (specify) *[ANCHOR] [SPECIFY]* |

**POTENTIAL AE (IF EVRYSDI USER)**

*ASK IF TABLET ~~CONVERTER~~ USER (CURRENT TREATMENT=6 OR 7)*

B11. How has your experience with the Evrysdi Tablet been overall? How has it compared to your initial expectations of the treatment?

*Please be as specific as possible in your response.*

|  |
| --- |
| *OPEN END: REQUIRE AT LEAST 4 CHARACTERS* |

**SECTION C: DISCONTINUATIONS, COMPLIANCE & INSURANCE**

**POTENTIAL AE**

*ASK IF DISCONTINUED OR RESTARTED EVRYSDI (S9r1c2=1 OR RESTART (HIDDEN)=1)*

C1. Earlier you mentioned that you have stopped using **Evrysdi (risdiplam) liquid formulation** in the past. Why *[IF PATIENT* did you*] [IF CAREGIVER* did the person you care for*]* stop usingEvrysdi (risdiplam) liquid formulation?

*Please be as specific as possible in your response.*

|  |
| --- |
| *OPEN TEXT – REQUIRE AT LEAST 4 CHARACTERS* |

*ASK IF DISCONTINUED OR RESTARTED SPINRAZA (S9r2c2=1 OR RESTART*

*(HIDDEN)=2)*

C2. Earlier you mentioned that you have stopped using **Spinraza (nusinersen)** in the past. Why *[IF PATIENT* did you*] [IF CAREGIVER* did the person you care for*]* stop usingSpinraza (nusinersen)?

*Please be as specific as possible in your response.*

|  |
| --- |
| *OPEN TEXT – REQUIRE AT LEAST 4 CHARACTERS* |

**POTENTIAL AE (IF EVRYSDI USER)**

*ASK IF DISCONTINUED OR RESTARTED EVRYSDI OR SPINRAZA (S9r1c2=1 OR S9r2c2=1 OR RESTART=1 OR 2)*

C3. For which of the following reasons *[IF PATIENT* did you*]* *[IF CAREGIVER* did the person

you care for*]* **stop using the following** medicine(s) for the treatment of spinal muscular atrophy (SMA)?

*Please select all that apply.*

|  |  |  |  |
| --- | --- | --- | --- |
|  | *RANDOMIZE*  *DO NOT SHOW HEADERS.* | *SHOW IF S9r1c2=1 OR RESTART=1* | *SHOW IF S9r2c2=1*  *OR RESTART=2* |
| Evrysdi  (risdiplam) liquid formulation | Spinraza  (nusinersen) |
| **EFFICACY** | | | |
| 1 | Not as effective as desired with **regard to improving overall movement** (e.g., lifting head, picking up pencil, climbing stairs, supported standing, etc.) |  |  |
| 2 | Not as effective as desired with **regard to bulbar function** (e.g., chewing, swallowing, speaking, etc.) |  |  |
| 3 | The medicine started to lose effectiveness between doses | *Do not allow response* |  |
| 4 | I progressed and felt like the medication was not working |  |  |
| **TOLERABILITY** | | | |
| 5 | Side effect/tolerability issues with this medicine |  |  |
| 6 | Wanted to stop for fertility/family planning reasons |  |  |
| **ACCESS** | | | |
| 7 | Cost issues or concerns |  |  |
| 8 | Changes with insurance coverage |  |  |
| 9 | Worried aboutthe logistical issues with accessing medicine (e.g., at home medicine delivery/ travel to injection site etc.) |  |  |
| 10 | In order to receive Zolgensma treatment *[SHOW IF S8=1]* |  |  |
| 25 | In order to start on the Evrysdi Tablet |  | *Do not allow response* |
| 11 | Went to college and treatment access/logistics became challenging |  |  |
| 12 | Moved off the insurance plan of parent/guardian/etc. and treatment was no longer affordable |  |  |
| 13 | Difficulty with reauthorizations |  |  |
| **COMPLIANCE** | | | |
| 14 | Having to take the medicine every day became too burdensome |  | *Do not allow response* |
| 15 | There were difficulties opening the bottle on a daily basis |  | *Do not allow response* |
| 16 | The intrathecal injections became too burdensome | *Do not allow response* |  |
| 17 | Traveling to the doctor’s office/hospital to receive the injection became too burdensome | *Do not allow response* |  |
| 18 | Would too often forget to take the medicine |  | *Do not allow response* |
| 24 | Storage/portability of this medicine became too burdensome |  | *Do not allow response* |
| **MISC** | | | |
| 19 | Difficulties swallowing the medicine |  | *Do not allow response* |
| 20 | Decided to discontinue treatment altogether (no longer wanted to be treated) |  |  |
| 21 | Needed or wanted to take a break from treatment |  |  |
| 22 | Enrolled in a clinical trial and had to stop taking the medicine |  |  |
| 23 | Transitioned care from a pediatric provider to an adult provider |  |  |
| 98 | Other (specify) *[ANCHOR] [SPECIFY]* |  |  |

**POTENTIAL AE**

*ASK IF DISCONTINUED EVRYSDI (S9r1c2=1) OR EVRYSDI USER (S9r1c1=1 OR S9r3c1=1) AND LIKELY TO SWITCH (B4=5-7). DO NOT ASK IF CURRENT TREATMENT=1,2, 6 OR 7 AND B6=1 OR 5. DO NOT ASK IF DISCONTINUED EVRYSDI LIQUID FORMULATION AND SWITCHED TO THE EVRYSDI TABLET (S9r1c2=1 AND S9r3c1=1)*

C4. What, if anything, would *[IF DISCONTINUED EVRYSDI:* have helped*]* *[IF EVRYSDI USER LIKELY TO SWITCH:* help*]* *[IF PATIENT* you*]* *[IF CAREGIVER* the person you care for*]* **stay on**Evrysdi?

*Please be as specific as possible in your response.*

|  |
| --- |
| *OPEN END: REQUIRE AT LEAST 4 CHARACTERS* |

**POTENTIAL AE**

*ASK IF DISCONTINUED EVRYSDI (S9r1c2=1) AND NOT CURRENTLY ON EVRYSDI TABLET (S9r3c3=1)*

C5. What, if anything, would motivate *[IF PATIENT* you*]* *[IF CAREGIVER* the person you care for*]* to consider **restarting** Evrysdi?

*Please be as specific as possible in your response.*

|  |
| --- |
| *OPEN END: REQUIRE AT LEAST 4 CHARACTERS* |

*ASK IF DISCONTINUED SPINRAZA (S9r2c2=1) OR SPINRAZA USER (S9r2c1=1) AND LIKELY TO SWITCH (B4=5-7)*

C6. What, if anything, would *[IF DISCONTINUED SPINRAZA:* have helped*]* *[IF SPINRAZA USER LIKELY TO SWITCH:* help*]* *[IF PATIENT* you*]* *[IF CAREGIVER* the person you care for*]* **stay on**Spinraza?

*Please be as specific as possible in your response.*

|  |
| --- |
| *OPEN END: REQUIRE AT LEAST 4 CHARACTERS* |

*ASK IF DISCONTINUED SPINRAZA (S9r2c2=1)*

C7. What, if anything, would motivate *[IF PATIENT* you*]* *[IF CAREGIVER* the person you care for*]* to consider **restarting** Spinraza?

*Please be as specific as possible in your response.*

|  |
| --- |
| *OPEN END: REQUIRE AT LEAST 4 CHARACTERS* |

*ASK IF DISCONTINUED OR RESTARTED EVRYSDI OR SPINRAZA (S9r1c2=1 OR S9r2c2=1 OR RESTART=1 OR 2)*

C8. Was the decision to **stop using the following** medicine(s) your decision, your doctor’s decision, or a mutual decision?

|  |  |  |  |
| --- | --- | --- | --- |
|  |  | *SHOW IF S9r1c2=1 OR RESTART=1. DO NOT SHOW IF S9r1c2=1 AND S9r3c1=1* | *SHOW IF S9r2c2=1*  *OR RESTART=2* |
| Evrysdi  (risdiplam) | Spinraza  (nusinersen) |
| 1 | My decision |  |  |
| 2 | Doctor’s decision |  |  |
| 3 | It was a mutual discussion/decision |  |  |
| 99 | Don’t know/can’t remember |  |  |

*ASK IF DISCONTINUED OR RESTARTED EVRYSDI OR SPINRAZA (S9r1c2=1 OR S9r2c2=1 OR RESTART=1 OR 2)*

C9. Did you discuss your concerns with your doctor before *[IF PATIENT* you*]* *[IF CAREGIVER* the person you care for*]* **stopped using the following** medicine(s)?

|  |  |  |  |
| --- | --- | --- | --- |
|  |  | *SHOW IF S9r1c2=1 OR RESTART=1. DO NOT SHOW IF S9r1c2=1 AND S9r3c1=1* | *SHOW IF S9r2c2=1*  *OR RESTART=2* |
| Evrysdi  (risdiplam) | Spinraza  (nusinersen) |
| 1 | Yes, discussed with the doctor before stopping |  |  |
| 2 | No, did not discuss with the doctor before stopping |  |  |
| 99 | Don’t know/can’t remember |  |  |

***INSURANCE SECTION***

C15. Which of the following best describes *[IF PATIENT* your insurance coverage*] [IF CAREGIVER* the insurance coverage of the person you care for*]*?

*Please select all that apply.*

|  |  |
| --- | --- |
| 1 | Commercial Insurance (HMO, PPO, including employer, union, private or health insurance exchange) |
| 2 | Medicaid (State or Managed Medicaid) |
| 3 | Medicare Part D/Medicare Advantage |
| 4 | Veterans’ Health Administration (VA) |
| 5 | Other Government (TRICARE/Department of Defense) |
| 6 | Self-Pay/No Insurance |
| 98 | Other (specify) *[SPECIFY]* |

C16. Has your insurance company ever denied *[IF PATIENT* you*]* *[IF CAREGIVER* the person you care for*]* access to an SMA medicine?

|  |  |
| --- | --- |
| 1 | Yes |
| 2 | No |
| 99 | Not applicable |

**POTENTIAL AE**

*ASK IF C16=1*

C17. Which medicine(s) did your insurance company deny access to?

*Please select all that apply.*

|  |  |
| --- | --- |
| 1 | Evrysdi (risdiplam) liquid formulation  *SHOW IF S7r1=1* |
| 4 | Evrysdi (risdiplam) Tablet  *SHOW IF S7r4=1* |
| 2 | Spinraza (nusinersen)  *SHOW IF S7r2=1* |
| 3 | Zolgensma (onasemnogene aberparvovec)  *SHOW IF S7r3=1 AND PATIENT AGE (Hidden)=1-3* |
| 98 | Other (specify) *[SPECIFY]* |

*PROGRAMMING NOTE: ASK C18-C23 IN A LOOP IF C17r1- 4=1. IF C16=2 OR C16=99 OR C17=98, SKIP TO D1.*

**POTENTIAL AE**

C18. Why did your insurance company deny access to *[INSERT THERAPY FROM C17]*? *Please select all that apply.*

|  |  |
| --- | --- |
| *RANDOMIZE* | |
| 1 | Over the age of 25  *[SHOW IF PATIENT AGE=5-6]* |
| 2 | Ambulatory status |
| 3 | On permanent ventilation support |
| 4 | SMN2 copy number |
| 5 | Documentation and/or required paperwork was missing |
| 6 | No stabilization/not enough improvement in condition |
| 7 | Not approved to be used with Zolgensma, which had already been administered *[SHOW IF S8=1]* |
| 8 | Medication utilization limit |
| 9 | Required to try a different medication first |
| 10 | Had already received and discontinued medication in the past |
| 98 | Other (specify) *[SPECIFY] [ANCHOR]* |
| 99 | Don’t know *[EXCLUSIVE] [ANCHOR]* |

*ASK IF C17=1 OR 4*

C19. Did you contact your Genentech PAL for help regarding the insurance denial(s)?

|  |  |
| --- | --- |
| 1 | Yes |
| 2 | No, I did not contact my Genentech PAL for help with the insurance denial |
| 3 | Did not have an assigned Genentech PAL to discuss with |
| 4 | Don’t know what a Genentech PAL is |
| 5 | Don’t know/can’t remember |

C20. Did you try to appeal the insurance denial for *[INSERT THERAPY FROM C17]*?

|  |  |
| --- | --- |
| 1 | Yes |
| 2 | No |
| 3 | Don’t know |

*ASK IF C20=2*

C21. Why did you NOT appeal the insurance denial for *[INSERT THERAPY FROM C17]*?

|  |
| --- |
| *OPEN END: REQUIRE AT LEAST 4 CHARACTERS* |

C22. How many times did you appeal the insurance denial for *[INSERT THERAPY FROM C17]*?

|  |  |
| --- | --- |
| \_\_\_\_\_\_ # of times appealed | *RANGE: 1-10* |

**POTENTIAL AE**

C23. Did your insurance company ultimately approve *[INSERT THERAPY FROM C17]* after

you appealed?

|  |  |
| --- | --- |
| 1 | Yes |
| 2 | No |

C24. Which of the following best describes your annual out of pocket costs for *[IF PATIENT:*

your current SMA treatment*] [IF CAREGIVER:* the current SMA treatment of the person you care for*]*?

*Costs can include deductibles, copayments, coinsurance, the cost of medications, medical supplies, and any other medical services or treatments that are not fully covered by your insurance plan. Please do not include your monthly insurance premiums in this amount.*

|  |  |
| --- | --- |
| 1 | $0 |
| 2 | $1-$100 |
| 3 | $101-$200 |
| 4 | $201-$300 |
| 5 | $301-$400 |
| 6 | $401-$500 |
| 7 | $501+ |
| 99 | Don’t know/prefer not to answer |

*ASK IF CURRENT EVRYSDI OR SPINRAZA USER (CURRENT TREATMENT=1,2,3,4,6 OR 7)*

C25. Using a 7-point scale, where 1=Not At All Likely and 7=Extremely Likely, how likely are

you to change *[IF PATIENT:* your current SMA treatment*] [IF CAREGIVER:* the current SMA treatment of the person you care for*]* if each of the following scenarios occurs?

|  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- |
|  |  | **Not At All Likely**  **1** | **2** | **3** | **4** | **5** | **6** | **Extremely Likely**  **7** |
| 1 | The out of pocket cost of Evrysdi **increases** |  |  |  |  |  |  |  |
| 2 | The out of pocket cost of Evrysdi **decreases** |  |  |  |  |  |  |  |
| 3 | The out of pocket cost of Spinraza **increases** |  |  |  |  |  |  |  |
| 4 | The out of pocket cost of Spinraza **decreases** |  |  |  |  |  |  |  |

*ASK IF COMMERCIAL PATIENT (C15=1)*

C26. Do you currently use a copay card to help pay for *[IF PATIENT:* your SMA treatment*] [IF*

*CAREGIVER:* the SMA treatment of the person you care for*]*?

|  |  |
| --- | --- |
| 1 | Yes |
| 2 | No |
| 3 | Don’t know/prefer not to answer |

*ASK IF C26=2*

C27. Why do you **not** use a copay card to help pay for *[IF PATIENT:* your SMA treatment*]*

*[IF CAREGIVER:* the SMA treatment of the person you care for*]*?

|  |
| --- |
| *OPEN END: REQUIRE AT LEAST 4 CHARACTERS* |

**SECTION E: AWARENESS & PERCEPTIONS OF UPCOMING FORMULATIONS**

*– PRODUCTS IN DEVELOPMENT*

E1. Have you heard of any new formulation or dosing options for existing SMA treatments that are being developed?

|  |  |
| --- | --- |
| 1 | Yes |
| 2 | No |

*ASK IF E1=1*

E2. Please list any new formulation or dosing options for existing SMA treatments that you have heard of that are being developed. *Enter your response below.*

|  |  |
| --- | --- |
| *OPEN END: REQUIRE AT LEAST 4 CHARACTERS* | |
|  | |
|  | |
|  | |
|  | |
| Don’t know | *[EXCLUSIVE]* |

E3. Which of the following new formulation or dosing options that are being developed for spinal muscular atrophy, if any, have you ever heard of?

*Please select all that apply.*

|  |  |
| --- | --- |
| *RANDOMIZE* | |
| 2 | Spinraza (nusinersen) high dose |
| 3 | Zolgensma (onasemnogene abeparvovec-xioi) for patients ages 2-17 years old |
| 4 | Taldefgrobep alfa (myostatin inhibitor) |
| 98 | Other (specify) *[SPECIFY] [ANCHOR]* |
| 99 | None of the above *[EXCLUSIVE] [ANCHOR]* |

*ASK IF E3r2=1*

E5. What have you heard about Spinraza (nusinersen) high dose?

|  |
| --- |
| *OPEN END: REQUIRE AT LEAST 4 CHARACTERS* |

*ASK IF E3r3=1*

E6. What have you heard about Zolgensma (onasemnogene abeparvovec-xioi) for patients ages 2-17 years old?

|  |
| --- |
| *OPEN END: REQUIRE AT LEAST 4 CHARACTERS* |

*ASK IF E3r4=1*

E6A. What have you heard about taldefgrobep alfa (myostatin inhibitor)?

|  |
| --- |
| *OPEN END: REQUIRE AT LEAST 4 CHARACTERS* |

E7. Using a 7-point scale, where 1=Not At All Excited and 7=Extremely Excited, how excited are you about the following new formulation, dosing, or indication options?

|  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- |
|  | *HOLD RANDOMIZATION ORDER FROM E3* | **Not At All Excited**  **1** | **2** | **3** | **4** | **5** | **6** | **Extremely Excited**  **7** |
| 2 | High Dose Spinraza (nusinersen)  *SHOW IF E3r2=1* |  |  |  |  |  |  |  |
| 3 | Zolgensma (onasemnogene abeparvovec-xioi) for patients ages 2-17 years old  *SHOW IF E3r3=1 AND CAREGIVER AND S8<>1* |  |  |  |  |  |  |  |
| 4 | Taldefgrobep alfa (myostatin inhibitor)  *SHOW IF E3r4=1* |  |  |  |  |  |  |  |

*ASK IF AWARE OF ANY FROM E3 (E3r2=1 OR E3r3=1 OR E3r4=1)*

E8. If the following new formulation or dosing options became available, how likely would you be to ask *[IF PATIENT* your doctor*] [IF CAREGIVER* the doctor of the person you care for*]* to *[TREATMENT STATUS=1*: switch to*] [OTHERWISE:* try*]* the following medicine? Please use a 7-point scale where 1=Not At All Likely and 7=Extremely Likely.

|  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- |
|  | *HOLD RANDOMIZATION ORDER FROM E3* | **Not At All Likely**  **1** | **2** | **3** | **4** | **5** | **6** | **Extremely Likely**  **7** |
| 2 | High Dose Spinraza (nusinersen)  *SHOW IF E3r2=1* |  |  |  |  |  |  |  |
| 3 | Zolgensma (onasemnogene abeparvovec-xioi) for patients ages 2-17 years old  *SHOW IF E3r3=1 AND CAREGIVER AND S8<>1* |  |  |  |  |  |  |  |
| 4 | Taldefgrobep alfa (myostatin inhibitor)  *SHOW IF E3r4=1* |  |  |  |  |  |  |  |

**SECTION F: MINDSHARE**

*ASK IF AWARE OF ALL BRANDS (S7r1=1 OR S7r4=1) AND S7r2=1 AND S7r3=1*

F1. To what extent do you feel each of these products is a **Scientific Leader** in spinal muscular atrophy (SMA)? Please allocate 100 points across the products below. The more points you allocate, the stronger your perception of that product as a scientific leader.

Definition of Scientific Leader: Performance of company’s existing products across multiple varied conditions, which inspires patient’s confidence in product manufacturer.

|  |  |  |
| --- | --- | --- |
|  | *RANDOMIZE* | **100-Points** |
| 1 | Evrysdi (risdiplam) |  |
| 2 | Spinraza (nusinersen) |  |
| 3 | Zolgensma (onasemnogene abeparvovec) |  |
|  |  | **MUST = 100** |

*ASK IF AWARE OF ALL BRANDS (S7r1=1 OR S7r4=1) AND S7r2=1 AND S7r3=1*

F2. To what extent do you feel each of these products is **Committed to Access/Affordability** in spinal muscular atrophy? Please allocate 100 points across the products below. The more points you allocate, the stronger your perception of that product’s Commitment to Access/Affordability.

Definition of Commitment to Access/Affordability: Patient support services provided (e.g. co-pay, free drug) and different payment plans offered ensure patients receive necessary treatment.

|  |  |  |
| --- | --- | --- |
|  | *RANDOMIZE* | **100-Points** |
| 1 | Evrysdi (risdiplam) |  |
| 2 | Spinraza (nusinersen) |  |
| 3 | Zolgensma (onasemnogene abeparvovec) |  |
|  |  | **MUST = 100** |

*ASK IF AWARE OF ALL BRANDS (S7r1=1 OR S7r4=1) AND S7r2=1 AND S7r3=1*

F3. To what extent do you feel each of these products is **Patient Centric** in spinal muscular atrophy**?** Please allocate 100 points across the products below. The more points you allocate, the stronger your perception of that product’s Patient Centricity.

Definition of Patient Centricity: Manufacturers provide effective and tolerable treatments which directly impact patient quality of life, enhance the patient experience through patient education and support, and contribute to decreasing health disparities.

|  |  |  |
| --- | --- | --- |
|  | *RANDOMIZE* | **100-Points** |
| 1 | Evrysdi (risdiplam) |  |
| 2 | Spinraza (nusinersen) |  |
| 3 | Zolgensma (onasemnogene abeparvovec) |  |
|  |  | **MUST = 100** |

PRODUCT\_SCORE(Hidden Variable)

|  |  |  |
| --- | --- | --- |
| 1 | Evrysdi | *(F1r1 + F2r1 + F3r1) / 3* |
| 2 | Spinraza | *(F1r2 + F2r2 + F3r2) / 3* |
| 3 | Zolgensma | *(F1r3 + F2r3 + F3r3) / 3* |

*ASK IF AWARE OF ALL BRANDS (S7r1=1 OR S7r4=1) AND S7r2=1 AND S7r3=1*

F4. Please allocate 100 points across the criteria below based on the **impact** these criteria have on your perception of **products** in spinal muscular atrophy. The more points you allocate, the greater the influence on the perception of the product.

|  |  |  |
| --- | --- | --- |
|  | RANDOMIZE | **100-Points** |
| 1 | **Scientific Leadership**  Defined as a function of company’s robust drug pipeline and performance of existing products across multiple varied indications, which inspires HCP’s confidence in manufacturers |  |
| 2 | **Commitment to Access/Affordability**  Defined as the manufacturer pricing decisions and support with patient assistance (e.g., co-pay, free drug) and different payment plans they offer to patients to ensure they receive necessary treatment |  |
| 3 | **Patient Centricity**  Defined as how manufacturers provide efficacious and tolerable treatments which directly impact patient quality of life, enhance the patient experience through patient education and support, and contribute to decreasing health disparities |  |
|  |  | **MUST = 100** |

WEIGHTED\_PRODUCT\_SCORE(Hidden Variable)

|  |  |  |
| --- | --- | --- |
| 1 | Evrysdi | *(F4r1/100 x F1r1) + (F4r2/100 x F2r1) + (F4r3/100 x F3r1)* |
| 2 | Spinraza | *(F4r1/100 x F1r2) + (F4r2/100 x F2r2) + (F4r3/100 x F3r2)* |
| 3 | Zolgensma | *(F4r1/100 x F1r3) + (F4r2/100 x F2r3) + (F4r3/100 x F3r3)* |

F5. When interacting with pharmaceutical manufacturers in the last three years, which communication method had the most impact on you staying up to date/informed on a product?

*Please select up to three.*

|  |  |
| --- | --- |
| *RANDOMIZE. ALLOW 1-3 SELECTIONS.* | |
| 1 | Manufacturer press releases |
| 2 | Manufacturer websites |
| 3 | Digital advertising by manufacturers |
| 4 | Social media from manufacturers (e.g., Twitter, LinkedIn, YouTube) |
| 5 | Patient-directed educational material or advertising |
| 6 | Presentations and booths by the manufacturer at SMA conferences |
| 99 | None of the above *[ANCHOR]* |

**SECTION G: DEMOGRAPHICS**

Thank you. The final set of questions are focused on understanding your general background.

G1. What sex *[IF PATIENT:* were you*] [IF CAREGIVER:* wasthe person you care for*]* assigned at birth on *[IF PATIENT:* your*] [IF CAREGIVER:* their*]* original birth certificate?

|  |  |
| --- | --- |
| 1 | Male |
| 2 | Female |
| 99 | Prefer not to answer |

G2. Which type of SMA *[IF PATIENT* do you*]* *[IF CAREGIVER* does the person you care for*]* have?

*Click here for additional information.*

*[THIS TEXT SHOULD BE AVAILABLE IF RESPONDENT CLICKS]*

SMA is sub-classified into four clinical types: Types 1, 2, 3, and 4, defined by age of symptom onset and highest motor milestone achieved.

* SMA Type 1 is usually diagnosed during an infant’s first 6 months. Individuals with Type 1 SMA face physical challenges, including muscle weakness and the inability to sit independently.
* SMA Type 2 is typically diagnosed between 6 months and 2 years of age. Individuals with SMA Type 2 can typically sit up without help, but they are unable to walk independently.
* SMA Type 3 is usually diagnosed after 18 months of age. Individuals with SMA Type 3 are initially able to walk but have increasingly limited mobility as they grow and eventually, many need to use a wheelchair.
* SMA Type 4 usually surfaces in adulthood, and it leads to mild motor impairment.

|  |  |
| --- | --- |
| 1 | Type 1 |
| 2 | Type 2 |
| 3 | Type 3 |
| 4 | Type 4 |

G3. How many copies of the SMN2 gene do you have?

|  |  |
| --- | --- |
| 1 | 1 copy |
| 2 | 2 copies |
| 3 | 3 copies |
| 4 | 4 copies |
| 5 | 4+ copies |
| 99 | Don’t Know |

**POTENTIAL AE (IF EVRYSDI USER)**

G4. Which of the following best describes *[IF PATIENT:* your ability*] [IF CAREGIVER:* the ability of the person you care for*]* to walk (ambulatory status)?

*Please select one option.*

|  |  |
| --- | --- |
| 1 | Unable to walk at all |
| 2 | A cane or some other assistive device is always needed to walk |
| 3 | A cane or some other assistive device is sometimes needed to walk |
| 4 | No problem with walking, but it is difficult to run |
| 5 | No problem with walking or running |
| 6 | Too young to assess *[SHOW IF CAREGIVER]* |
| 98 | Other (specify) *[SPECIFY]* |
| 99 | Prefer not to answer |

**POTENTIAL AE (IF EVRYSDI USER)**

G5. Which of the following best describes *[IF PATIENT:* your*] [IF CAREGIVER:* the person you care for’s*]* ability to swallow a pill? 

|  |  |
| --- | --- |
| 1 | Completely unable to swallow a pill |
| 2 | Very difficult to swallow a pill |
| 3 | Moderately difficult to swallow a pill |
| 4 | Somewhat difficult to swallow a pill |
| 5 | No problem at all swallowing a pill |

**POTENTIAL AE (IF EVRYSDI USER)**

G6. *[IF PATIENT:* Do you*] [IF CAREGIVER:* Does the person you care for*]* use a nasogastric tube/gastrostomy tube?

|  |  |
| --- | --- |
| 1 | Yes |
| 2 | No |
| 99 | Not sure |

G7. *[IF PATIENT* Do you*] [IF CAREGIVER* Does the person you care for]receive primary SMA care from a center of excellence?

Centers of excellence are specialized programs within healthcare institutions that have a concentration in SMA expertise and related resources.

|  |  |
| --- | --- |
| 1 | Yes |
| 2 | No |
| 99 | Not sure |

*ASK IF G7=1*

G8. At which of the following centers of excellence *[IF PATIENT* do you*] [IF CAREGIVER* does the person you care for*]* receive SMA care?

|  |  |
| --- | --- |
| 1 | Advocate Children’s Hospital: Park Ridge, IL |
| 2 | Arkansas Children’s Hospital: Little Rock, AR |
| 3 | Baylor College of Medicine: Houston, TX |
| 4 | Boston Children’s Hospital: Boston, MA |
| 5 | Children’s Healthcare of Atlanta: Atlanta, GA |
| 6 | Children’s of Alabama: Birmingham, AL |
| 7 | Children’s National Medical Center: Washington, DC |
| 8 | Columbia University: New York, NY |
| 9 | Connecticut Children’s Medical Center: Hartford, CT |
| 10 | Duke University Medical Center: Durham, NC |
| 11 | Gillette Children’s Specialty Healthcare: St. Paul, MN |
| 12 | Northwestern University: Evanston, IL |
| 13 | Ohio State University, Wexner Medical Center: Columbus, OH |
| 14 | Phoenix Children’s Hospital: Phoenix, AZ |
| 15 | Stanford Health or Stanford Children’s Health: Palo Alto, CA |
| 16 | Seattle Children’s Hospital: Seattle, WA |
| 25 | St. Louis Children’s Hospital: St. Louis, MO |
| 17 | University of California, Los Angeles (UCLA): Los Angeles, CA |
| 18 | University of Miami: Miami, FL |
| 19 | University of Michigan: Ann Arbor, MI |
| 20 | University of New Mexico: Albuquerque, NM |
| 21 | University of Rochester Medical Center: Rochester, NY |
| 22 | University of Utah, Program for Inherited Neuromuscular Disorders: Salt Lake City, UT |
| 23 | University of Texas Southwestern/Children’s Health: Dallas, TX |
| 24 | Vanderbilt University Medical Center: Nashville, TN |
| 26 | Yale Pediatric Neuromuscular Clinic: New Haven, CT |
| 98 | None of these *[ANCHOR]* |

G9. Which of these best describes *[IF PATIENT:* your racial or ethnic background *[IF CAREGIVER:* the racial or ethnic background of the person you care for?

*Please select all that apply.*

|  |  |
| --- | --- |
| *SHOW IN ALPHABETICAL ORDER* | |
| 1 | Caucasian / White |
| 2 | Black or African-American |
| 3 | Native American or Alaskan Native |
| 4 | Asian (including Asian Indian, Chinese, Filipino, Japanese, Korean, Vietnamese) |
| 5 | Native Hawaiian or other Pacific Islander (including Samoan, Guamanian, or Chamorro) |
| 6 | Middle Eastern or North African |
| 7 | Hispanic, Latino or Spanish origin |
| 98 | Other ethnic background *[ANCHOR]* |
| 99 | Prefer not to answer *[EXCLUSIVE] [ANCHOR]* |

G11. How would you describe the area in which *[if Patient]* “you reside*” [if Caregiver] “*the person you care for resides*”*?

|  |  |
| --- | --- |
| 1 | Urban (within city) |
| 2 | Suburban (residential area on the outskirts of a city) |
| 3 | Rural (settled areas outside towns and cities) |
| 4 | Prefer not to answer |

G12. What category best describes your annual total household income before taxes?

|  |  |
| --- | --- |
| 1 | Less than $25,000 |
| 2 | Between $25,000 and $34,999 |
| 3 | Between $35,000 and $49,999 |
| 4 | Between $50,000 and $74,999 |
| 5 | Between $75,000 and $99,999 |
| 6 | Between $100,000 and $149,999 |
| 7 | Between $150,000 and $199,999 |
| 8 | $200,000 and above |
| 99 | Prefer not to answer |

G13. What is your employment status?

|  |  |
| --- | --- |
| 1 | Employed full-time |
| 2 | Employed part-time |
| 3 | Retired |
| 4 | Student |
| 5 | Homemaker |
| 6 | Unemployed |
| 98 | Other (specify) |
| 99 | Prefer not to answer |

*ASK IF G13=4*

G14. What type of degree are you currently pursuing?

|  |  |
| --- | --- |
| 1 | High School Diploma |
| 2 | Equivalent of High School Degree (e.g. GED) |
| 3 | Post-High School Vocational/Trade School Training |
| 4 | Associate’s Degree |
| 5 | Bachelor’s Degree |
| 6 | Master’s Degree |
| 7 | Professional Degree (e.g. MD, DDS, DVM) |
| 8 | Doctorate |
| 99 | Prefer not to answer |

*ASK IF G13<>4*

G15. What is the highest degree or level of education you have completed?

|  |  |
| --- | --- |
| 1 | Less than a High School Diploma |
| 2 | High School Degree or Equivalent (e.g. GED) |
| 3 | Post-High School Vocational/Trade School Training |
| 4 | Some College |
| 5 | Associate’s Degree |
| 6 | Bachelor’s Degree |
| 7 | Master’s Degree |
| 8 | Professional Degree (e.g. MD, DDS, DVM) |
| 9 | Doctorate |
| 99 | Prefer not to answer |

*ASK IF CAREGIVER*

G16. What type of school system, if any, does the person you care for attend?

|  |  |
| --- | --- |
| 1 | Mainstream school system (with or without an IEP/special needs services) |
| 2 | Special needs school system |
| 3 | Homeschooling system |
| 4 | No schooling system |
| 98 | Other (specify) |
| 99 | Prefer not to answer |

*ASK IF G16r=1-3*

G17. In what type of grade level is the person you care for?

|  |  |
| --- | --- |
| 1 | Preschool |
| 2 | Elementary school |
| 3 | Middle school |
| 4 | High school |
| 5 | College |
| 98 | Other |
| 99 | Prefer not to answer |

G18. Are you or someone you care forcurrently navigating any of the following situations? *Please select all that apply.*

|  |  |
| --- | --- |
| *RANDOMIZE* | |
| 1 | Attending a college/university in the near future |
| 2 | Transitioning care from a pediatric provider to an adult provider |
| 3 | Moving off the insurance plan of parent/guardian/etc. |
| 99 | None of the above *[ANCHOR] [EXCLUSIVE]* |

G19. What is your marital status?

|  |  |
| --- | --- |
| 1 | Single (not in a long-term relationship) |
| 2 | Single (in a long-term relationship) |
| 3 | Married |
| 4 | Separated |
| 5 | Divorced |
| 6 | Widowed |
| 99 | Prefer not to answer |

**Thank you for taking this survey. We value your feedback and are grateful for your time**

**and effort! Please click the button below to submit your responses.**

**~ END OF QUESTIONNAIRE ~**