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*September 2025*

**SMA ADULT ACTIVATION QUAL**

**DISCUSSION GUIDE**

**Objectives:**

Understand treatment barriers, unmet needs, and motivations among untreated adult SMA patients to identify opportunities for targeted engagement and activation.

More specifically:

* Explore awareness and perceptions of available SMA treatments, including perceived benefits and drawbacks
* Identify key motivators and barriers to future treatment consideration, including access to care and availability of specialized SMA care
* Assess current levels of community engagement to uncover potential channels for outreach and activation

**Quotas:**

* n=6 adult untreated patients
  + Treatment naive adults (best efforts and priority target)
  + Discontinued adults who have not been on treatment in the past 12 months

1. **INTRODUCTION** *(2 mins)*

Thank you for agreeing to take part in this interview, which will last approximately 60 minutes. During this discussion, I'd like to obtain your thoughts and feedback on spinal muscular atrophy (SMA).

Review Logistics with Respondent:

* Independent marketing research firm – no vested interest in responses, therefore, be frank
* Information discussed will be kept strictly confidential
* Members of the research team listening (when appropriate)
* No right or wrong answers – opinions from their own perspectives based on individual experiences
* Recording of interview for analysis purposes

Different people 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 wish.

Are you happy to proceed with the interview on this basis? ***(If no, end the interview here)***

**US ADVERSE EVENT DISCLAIMER**

We are required to pass on to our client details of adverse events and product technical complaints that are mentioned during the course of market research. Although what you say will, of course, be treated in confidence, should you raise during the discussion an adverse event or product technical complaint in an individual or group of individuals, we will need to report this.

In such a situation you will be asked whether or not you are willing to waive the confidentiality given to you under the Market Research Codes of Conduct specifically in relation to that adverse event or product technical complaint. Everything else you say during the course of our discussion will continue to remain confidential, and you will still have the option to remain anonymous if you so wish. Are you happy to participate in the interview on this basis? [YES/NO; IF NO, THANK AND END INTERVIEW]

1. **BACKGROUND** (*8 mins)*

*SECTION GOAL: Brief warm-up and introduction. Allow for some discussion but maintain control and do not go into too much detail.*

1. Let’s begin by having you tell me a little bit about yourself.
   1. What is your living situation/who lives in your household?
   2. What do you like to do in your free time (hobbies, activities, etc.)
2. Tell me a little bit about your journey with SMA so far (when you were diagnosed, your day to day, challenges, positive experiences).
3. How would you describe your current health?
4. Are you currently seeing any HCPs (neurologist, PT, PCP) for SMA-related care?
   1. *IF NO:*
      1. Why not?
      2. What (if anything) are you currently doing to manage your SMA?
   2. *IF YES:* How many HCPs are you currently seeing?
      1. How involved are each in guiding your treatment decisions? How much of an impact do each have on your final treatment decision? (probe on weight of HCP suggestions vs. self-advocacy)
      2. *IF MULTIPLE:* Which would you consider your primary SMA doctor?
   3. Who else, if anybody, is involved in your health decisions?
      1. To what extent are they involved (support, information, caregiver)?
5. What goals do you have for your health?
   1. Short-term goals
   2. Long-term goals
   3. Personal aspirations
6. **CURRENT PERCEPTIONS & ATTITUDES** *(20 mins)*

*SECTION GOAL: gauge current awareness, perceptions, experiences, and considerations for SMA treatments.*

1. What treatments are you aware of that are currently available?
   1. *PROBE:* Evrysdi liquid, Evrysdi tablet, Spinraza
   2. What experience do you have with each treatment, if any (taken in the past but not currently, never taken)?

*[FOR TREATMENTS TAKEN IN THE PAST BUT NOT CURRENTLY]*

1. What was your treatment experience with *[DISCONTINUED TREATMENT]*?
   1. What do you see as the benefits?
   2. What do you see as the drawbacks?
   3. What was the reason that you discontinued the treatment? *PROBE on main vs contributing factors.*
      1. Did you experience any life change/event, change in SMA severity/symptoms (i.e. motor function) that motivated you to discontinue the treatment?
      2. Did insurance coverage or access issues contribute? *(If yes, probe)*
      3. Who was involved in your decision to stop the treatment?
         1. *PROBE if HCP involved:* what was that conversation like with the HCP? Who initiated the conversation?
      4. Is there anything that could have kept you on treatment? If so, what?
      5. Would you consider re-starting this treatment in the future? If yes, how likely are you to re-start the treatment in the future?

*[FOR TREATMENTS NEVER TAKEN]*

1. What are your impressions of *[TREATMENT NEVER TAKEN]?*
   1. What do you know about this treatment? *PROBE on specific treatment features (efficacy, safety, ROA, dosing schedule) on their impact on likelihood to try.*
      1. What do you see as the benefit of this treatment option for you?
      2. What do you see as the drawbacks? What concerns or hesitations do you have?
      3. Where did you get your information about this treatment?
   2. Have you ever considered starting on this treatment?
      1. If yes, what made you consider but not end up trying the treatment?
      2. If no, why not?
   3. Did you talk with your HCP about this treatment?
      1. If yes, what was that conversation like?
         1. How much of an impact did your HCP have on your final decision to not initialize this treatment?
      2. Did you talk with anybody else when considering starting this treatment? If so, who? How much of an impact did they have on your final decision?
   4. How likely are you to consider this treatment in the future?
      1. If likely: what would drive you to consider starting the treatment?
      2. If not likely: why not?
   5. Do you consider yourself a good candidate for this treatment?
      1. If yes: why?
      2. If no: why not? Could you see yourself eventually being a good candidate? If yes, why?
2. What treatments are you aware of that are in development/not currently available?
   1. PROBE: HD Spinraza, Anti-myostatin
   2. What are your impressions of each?
      1. What do you see as the benefit of this treatment option for you?
      2. What do you see as the drawbacks? What concerns or hesitations do you have?
      3. Do you consider yourself a good candidate for any? Why/Why not?
         1. Would you consider starting on any of these treatments?
            1. If yes, why? What would drive your consideration? What would you hope to achieve?
            2. If no, why not? What hesitations do you have?
      4. *IF AWARE OF ANTI-MYOSTATINS: Probe on Scholar Rock vs Roche*

**4. BARRIERS TO TREATMENT & UNMET NEEDS** *(10 mins)*

*SECTION GOAL: Identify why they remain untreated and what gaps currently exist in care.*

1. What are the main reasons you are not currently on any SMA treatment?
   1. *PROBE IF NOT MENTIONED:* 
      1. Manageable SMA severity (not severe enough to feel the need for treatment)
         1. What would drive you to consider starting on treatment? New treatment options? SMA progression?
         2. Doesan option like Apitegromab (Scholar Rock) change that thinking (being able to address muscle loss)?
         3. How often do you track your symptoms/progression?
         4. How do you track your symptom maintenance and SMA progression?
         5. Did you discuss this with your HCP? If yes, what was that discussion like? Did you and your HCP agree that treatment is not needed?
      2. Emotional factors (e.g., fear, anxiety, denial, uncertainty about outcomes)
      3. Practical or logistical factors (e.g., scheduling, travel, time, disruption to daily life)
      4. Clinical factors (e.g., concerns about safety, side effects, not seeing enough benefit)
         1. What treatment benefit is needed for you to consider starting on a treatment?
         2. Did you discuss this with your HCP? If yes, what was that discussion like? Did you and your HCP agree that treatment is not needed?
      5. Financial or access-related barriers (e.g., cost, insurance coverage, prior authorization challenges)
2. What challenges have you experienced (or expect to experience) in trying to access treatment?
   1. *PROBE IF NOT MENTIONED:* 
      1. Availability of specialized SMA care near you
      2. Finding an HCP you trust to manage SMA
      3. Managing the logistics (visits, PT, tests, travel, time off work/school)
3. What needs are currently not being met by available treatments?
   1. *PROBE IF NOT MENTIONED:* 
      1. Convenience, ease of administration, impact on daily functioning
      2. Expectations for outcomes or quality of life improvements that aren’t being met
      3. Gaps in support services, education, or community resources
   2. Which of your unmet needs would be most important to be addressed in order for you to consider a new or existing treatment?

**5. MOTIVATIONS & FUTURE CONSIDERATION** *(10 mins)*

*SECTION GOAL: Understand what could drive future interest in treatment.*

1. How likely are you to consider starting any treatment in the future? Why or why not?
2. Are there specific changes or situations that would increase your likelihood to consider treatment?
   1. *PROBE IF NOT MENTIONED:* 
      1. **Worsening symptoms or decline in function**
      2. **Availability of new treatments (*PROBE* on High Dose Spinraza and Scholar Rock if not mentioned and aware)**
      3. **Easier access**
      4. **More information or reassurance about safety/side effects**
3. What would you hope to achieve or improve if you started treatment?
4. Who would be most influential in your decision to consider starting treatment?
   1. Role of family, friends, or caregivers
   2. Role of HCPs, PTs, or other specialists
   3. How would they be involved (encouragement, logistics, advocacy)
5. What would you need to feel confident in making a decision to start treatment?
   1. *PROBE:* Trusted information, hearing from other patients, clear expectations on outcomes, support with logistics/insurance

**6. COMMUNITY ENGAGEMENT & INFO SOURCES** *(9 mins)*

*SECTION GOAL: Understand how untreated patients currently seek support and information and what would feel most helpful/credible.*

1. How connected do you feel to the SMA community?
   1. Do you participate in any in-person groups, online forums, or advocacy organizations?
   2. *PROBES IF NOT INVOLVED:*
      1. Why not?
      2. Were you involved in the SMA community at any point in the past?
      3. *PROBE* on if current communities are too caregiver/pediatric centric
      4. Are there any other people, groups, or networks that you engage in for support?
2. What sources of information about SMA do you currently use and trust?
   1. *PROBE:* HCPs, peers, social media, online resources, advocacy groups, PTs, others
   2. Are there any sources or communities you **don’t** trust (e.g., Cure SMA, neurology clinics, SMA camps)? Why?
3. How do you **typically** find out about new treatments or care options?
   1. How would you **prefer** to receive that type of information?
   2. What format would feel most helpful or credible (patient testimonials, data and stats, doctor-led info, social content, etc.)?
4. What could companies, HCPs, or advocacy groups do to make it easier for people like you to learn about SMA treatment options current available and in development?

**7. THANK AND CONCLUDE** *(1 min)*

*SECTION GOAL: Wrap up and ask any outstanding questions.*

1. Any last comments?
2. Thank and conclude