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Study No: **3938**

Study Name: **Patient and caregiver preference  
for SMA treatment in LatAm**

Interview Length: **45 mins**

Date: **October 2020**



## **Patient and caregiver Discussion Guide**

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*This research is conducted under a code of conduct and is sponsored by a pharmaceutical company*

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## Introduction (2-3 minutes)

### START RECORDING

My name is <name> from Research Partnership, an independent market research company. We are conducting market research on behalf of a pharmaceutical company.

#### Explain purpose of interview:

The purpose of this interview is to understand from patients and caregivers their personal journey and experiences with Spinal Muscular Atrophy (SMA) and its impact on their quality of life.

The information you provide us with will be treated as confidential, it will be combined with feedback from others like yourself and the information will then be communicated through a **publication to illustrate what patients go through**, the challenges they encounter, what matters most to patients and caregivers, and how SMA affects patient's and caregiver's quality of life. If you have given permission in your recruitment agreement form, your contributions may be shared as part of this, but will remain anonymous and data will only be reported on aggregated level. You will be paid an honorarium for contributing to this valuable piece of patient research. You have the right to refuse to answer questions or withdraw at any time.

Taking this opportunity to have your voice heard would greatly help us further our research, and your participation would be hugely appreciated. If you have any questions, please contact \_\_\_\_\_ at Research Partnership by email at \_\_\_\_\_

Please note that this is for market research purposes only – it is in no way intended to be promotional.

The research will comply with UK Data Protection law, British Healthcare Business Intelligence Association's Legal & Ethical Guidelines, Association of the British Pharmaceutical Industry's (ABPI) as well as MRS codes of practice.

### Adverse Events

To ensure compliance with regulations on Pharmacovigilance, we (the Market Research Agency) are obliged to report to our client (the sponsor of the Market Research Survey) details of adverse events and/or product complaints that are mentioned during market research, even if already reported to the company or the regulatory authorities.

Should this occur, the reporting of such events to the sponsor will be performed by us with two different possible levels of your involvement:

- *You accept to waive the confidentiality of contact details granted to you under the Market Research Code of Conduct*
  - You accept to be contacted by the Pharmacovigilance Department of our client for the express and sole purpose of documentation and follow-up of such report(s), all other information that you provided us with in the context of this study remaining confidential.
  - Your name will be mentioned as reporter of the case, further documentation and follow-up of the report(s) will be processed directly between you and the sponsor if needed
- *You do not accept to waive the confidentiality of contact details granted to you under the Market Research Code of Conduct*

- You accept to be contacted only by us (the Market Research Agency).
- Only your professional category will be mentioned as reporter of the case, further documentation and follow-up of the report(s) will be processed only between you and us if needed

If you agree to waive confidentiality towards the sponsor, your name and contact details will be forwarded to the sponsor for the express and sole purpose of documentation and follow-up of adverse events and/or product complaints report(s), as described above.

1.	I agree to waive confidentiality towards the sponsor	<input type="radio"/>	CONTINUE
2.	I do not agree to waive confidentiality towards the sponsor	<input type="radio"/>	CONTINUE

## Section 1 – Introduction and background (5 minutes)

**Objective:** *To build trust and familiarity with the patient / caregiver and understand their socio-cultural background, disease history and perception of SMA*

**Moderator note:** We have outlined separate questions for patient and caregiver respondents, where appropriate, however, please, adjust the question text where necessary to apply to the respondent type you are talking to.

*Before we begin the interview, throughout the discussion please try to provide answers that are not impacted by the current Covid-19 pandemic, thinking about your condition before the current health crisis unless specifically mentioned.*

### Ask all:

Please tell me about yourself, using your first name only.

- What is your age?
- What is your current occupation? What are your hobbies?
- Whom do you live with? How many children (if any) do you have?

### Patient only:

I understand that you suffer from Spinal Muscular Atrophy (SMA).

- How old were you when you first had symptoms associated with SMA? *Please give an estimate if unsure*
  - How long after noticing these symptoms were you then first diagnosed with SMA? *Please give an estimate if unsure*
- Can you confirm what type of SMA do you have?
  - Prompt:
    - **SMA Type 2, (intermediate, older babies and toddlers):** *onset between 7-18 months of age may be able to sit up without help, but not stand or walk*
    - **SMA Type 3, (mild, children and young adults):** *onset between 18 months and 17 years of age; able to stand and walk without help, although may find walking or getting up from a sitting position difficult and may find walking gets gradually harder over time*

- Do you have any other health conditions apart from Spinal Muscular Atrophy (SMA)? If yes, what?

### Caregiver only:

I understand that you care for a child who is suffering from Spinal Muscular Atrophy (SMA).

- How old is your child?
- How old was your child when he/she first had symptoms associated with SMA? *Please give an estimate if unsure*
  - How old was your child when first diagnosed with SMA? *Please give an estimate if unsure*
- Can you confirm what type of SMA does your child have?
  - Prompt:
    - **SMA Type 1 (severe, young babies):** *onset between 0-6 months of age; unable to ever sit without support*
    - **SMA Type 2 (intermediate, older babies and toddlers):** *onset between 7-18 months of age may be able to sit up without help, but not stand or walk*
  - Do they have any other illnesses or conditions? If yes, which ones?
- How many hours (per week) do you approximately spend caring for your child?

### Ask all:

When I say 'SMA' what words, images, thoughts come to your mind?

- If you had to describe the disease to a friend who has no knowledge and information about it, how would you describe it? What causes it, what are the symptoms, and the consequences of the condition?

### Moderator share image deck with respondents [to conduct exercise, if time]

Please have a look at these images.

- Thinking back to the time when you received the SMA diagnosis, which image best represents what you thought and felt at that time?
  - Why this image?
- Thinking about the time of SMA diagnosis how, if at all, could your experience have been improved? Why would that be important?

## Section 2 – Disease Burden and QoL (15 minutes)

**Objective:** *To understand the SMA burden of the disease, how it has affected the patient and caregiver rationally and emotionally, and the impact on QoL*

I would now like to understand more about how you are living with the disease, the impact it might have on your / your child's daily life and activities and how you are coping with it.

### Symptoms of SMA

#### Patient only:

What are the main symptoms you currently experience as part of your SMA?

- **Probe:**
  - What are the physical symptoms/ restrictions on motor function or mobility?

- What are the symptoms related to breathing?
- Can you describe how the symptoms feel?
- What if any supportive equipment or tools do you use to help you manage your SMA?
  - *Probes: mechanical ventilation, walking frame, wheelchair, etc.*

Currently, what is the frequency in which you experience the symptoms?

- Which symptoms are most painful and bothersome?
- What are regular symptoms, and what are occasional symptoms?

How do the symptoms of this disease impact your daily life?

- Please give me some examples in your daily life.

*Probes: mood, sleep, hobbies, work, etc.*

Thinking specifically of your symptoms, in what areas of your daily life, if any, do you feel restricted?

- What precautions do you take to manage and cope with these symptoms?
  - **Prompts:**
    - Alterations to lifestyle
    - Behaviour changes
    - Hacks/work arounds
  - *For each precaution:* how do these changes affect your life? How does it make you feel?

### Caregiver only:

Please think about your child with SMA, what are their main symptoms they currently experience?

- Please describe their current level of physical (motor) functioning or ability. How would you describe their level of movement?
- Prompt:
  - Do they need support when sitting?
  - Can they sit, stand or walk independently?

Now please think about your child and their breathing, how would you describe their ability to breathe?

- Prompt:
  - Can breathe without mechanical support?

How do the symptoms of this disease impact your child's daily life?

- Please give me some examples of the impact it has on their daily life.

### Quality of life

#### Ask all

Let's look a bit more closely in how you feel SMA overall has impacted your [and your child's] life. What are the main things you enjoy doing or experiencing in your daily life?

- What things are more important to you than others?
  - **Prompt:** What things improve or diminish whether you are having a good day or not?
- In what way do you think this disease has impacted those things?

To what extent does the condition (including the symptoms and treatments) affect your family and social life? Why do you say so? Please give me an example.

### Caregiver only:

In what areas of your daily life, if any, do you feel restricted as a result of caring for your child?

- **Probe:** daily life, relationships, financially, emotionally

- What precautions do you take to manage and cope with these symptoms?
  - **Prompts:**
    - Alterations to lifestyle
    - Behaviour changes
    - Hacks/work arounds
  - *For each precaution:* how do these changes affect your life? How does it make your feel?

### Ask all

When thinking about your/ your child's journey since the SMA diagnosis, how has the impact of the disease changed you, if at all, over time?

- How, if at all, has the burden changed over time?
- What adaptations have you had to make as a result to cope better or improve your/ your child's quality of life?
  - **Probe:** Mental / physical
- Is there a slogan, saying/proverb that best describes you and your approach towards life now?
- What fears, concerns, and anxieties do you have about the condition now?
  - Concerns about health risks?
  - Disease progression/ deterioration in condition?

*Show Stimuli 1.* Reflecting back on all your experiences, select 2 words that reflect how the condition has impacted you personally.

- Why did you chose these words? *Moderator to probe fully to identify deep-rooted feelings and beliefs.*

## Section 3 – Unmet needs and Treatment goals (20 minutes)

**Objective:** To understand unmet needs in the SMA patient journey and for treatments and assess the relative importance of different SMA treatment attributes and decision making

If you consider your entire journey with SMA right from symptoms to where you / your child are today:

- What are the biggest frustrations and challenges you experienced, or currently experience with regards to your journey with SMA?
- What further support, if any, could you have been given to cope with SMA?
- What, if any, are the main areas that could have been improved in your journey? Why?

*(For the next set of questions, Moderator to confirm if respondent is treatment experienced or naïve, Referring to screener S4.)*

### For Treatment experienced\*:

I'd now like to talk about what treatments you have had for SMA.

- Are you/ is your child currently receiving or have previously had any pharmaceutical treatments for SMA?
  - If so, what was the name of this treatment?
    - **Prompt:**
      - Spinaraza (nusinersen)
      - Zolgensma (Onasemnogene abeparvovec-xioi) / gene therapy
        - **For each treatment:**
          - What are/were the advantages?
            - **Prompt:** What aspects did you specifically like about the treatment?
          - What are / were the disadvantages?
            - **Prompt:** What aspects did you specifically dislike about the treatment?
      - *Moderator please refer to and record on MCF1* What is/was your level of satisfaction with this treatment, on a scale of 0-10, where 0 is not at all satisfied and 10 is extremely satisfied? Why?
- How did you come to this decision [to take/have your child take] this treatment?
  - At what stage/point was a decision made to use this treatment?
    - Who was involved in the choice? To what extent were you involved versus the doctor?
      - Did you feel you had a choice at all?
  - What was explained to you about the treatment?
    - **Probe:** administration, side effects, overall effects of the treatment?
- What aspects of the treatment were taken into consideration when the treatment choice was made?
  - **Prompt:** by you, by the physician
  - **Probe on each of the following, ensuring clear understanding of respondent's rationale for why:**
    - Treatment administration
      - If you had a choice, what would be your preference on how the treatment is administered? Why?
        - **Probe:**
          - intrathecal (injection in the lower back using a needle inserted into the space around the spinal cord)?
          - intravenous (infusion administered into the vein via needle)?
          - or orally (liquid e.g. administered through to the mouth, or gastric tube or nasal probe)? Why?
          - Frequency of administration? (e.g. Daily, monthly, one-off treatment)
    - Efficacy
      - What is more important:
        - Motor/Physical functioning impact
        - Breathing functioning impact
    - Potential side effects – which ones in particular would you be concerned about?
    - Improvement in quality of life/ daily activities – what?



- Cost
  - Improved life expectancy – by how much?
- Are there any other features about the treatment that were an important consideration for you that have not been mentioned?
  - If yes, which ones? Why?
- What, if any, difficulties did you encounter when trying to get approval for the treatment and its administration? Why?
  - **Prompt:** time delays, geographical, financial, criteria/classification
- What were the expectations you had for this treatment?
  - What were you hoping to achieve with the treatment [for your child]?
    - **Prompt:** physically, emotionally, hopefulness
- What, if anything, would you want from an SMA treatment that the currently available options do not offer?
  - What would be the minimum expectations you would have for any new treatment?
    - **Prompt:** administration, efficacy, side effects, etc.

\*

### For Treatment naïve\*\*:

I'd now like to talk about what treatments you are aware of / might have heard or read about for SMA.

- Have you/ has your child previously been offered any treatments for your SMA?
  - If so, what was the name of this treatment?
    - **Prompt:**
      - Spinaraza (nusinersen)
      - Zolgensma (Onasemnogene abeparvovec-xioi) / gene therapy
- What was explained to you about the treatment?
  - **Probe:** administration, side effects, overall effects of the treatment?
    - How were the advantages described to you?
    - How were the disadvantages described to you?
- Were you / your child previously assessed for treatments for your SMA by a physician?
  - If so, what did this assessment entail?
  - What was the outcome of this assessment? Why?
- Now more generally speaking, how would you come to a decision on treatment choice [for yourself/for your child]?
  - To what extent do you think you would be involved versus the doctor?
    - How would you then come to a decision on the treatment?
- What aspects would you take into consideration when a treatment choice would be made?
  - **Prompt:** by you, by the physician
  - **Probe on each of the following, ensuring clear understanding of respondents rationale for why between the differences, For each of:**
    - Treatment administration
      - If you had a choice, what would be your preference on how the treatment is administered? Why?
    - **Probe:**
      - intrathecal (injection in the lower back using a needle inserted into the space around the spinal cord)?
      - intravenous (infusion administered into the vein via needle)?
      - or orally (liquid e.g. administered through to the mouth, or gastric tube or nasal probe)? Why?



- Frequency of administration? (e.g. Daily, monthly, one-off treatment)
- Efficacy?
  - What is more important:
    - Motor/Physical functioning impact
    - Breathing functioning impact
    - Speech impact
    - Swallowing/feeding impact
  - Potential side effects – which ones in particular would you be concerned about?
  - Improvement in quality of life/ daily activities – what?
  - Cost
  - Improved life expectancy – by how much?
- What if any other treatment features would be an important consideration for you that have not been mentioned?
- What would your expectations be for a treatment?
  - What would you want to achieve with the treatment [for your child]?
    - **Prompt:** physically, emotionally, hopefulness
- What, if anything, would you want from an SMA treatment that the currently available options do not offer?
  - What would be the minimum expectations you would have for any new treatment?
    - **Prompt:** administration, frequency of administration, efficacy, side effects, etc.

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### For those who have not previously mentioned 'Zolgensma' / gene therapy:

Have you heard of a treatment called Zolgensma or gene therapy?

- **If so**, what are your hopes for this treatment? In the future? Why?
- What would your expectations be for this treatment?

### 100-point allocation exercise:

I would now like to understand how important the different treatment features/ attributes that we talked about are for you when selecting a SMA treatment.

#### Moderator to show Self-Completion Form 1.

I'd like you to allocate 100 points across attributes that a hypothetical SMA treatment could offer, based on what attributes are more or less important to you. Please allocate more points to the attributes/features that are more important to you and less points to attributes you consider less important. You cannot allocate the same points to more than one attribute.

Please review all of the attributes. For the box labeled 'Other', is there anything else not listed that you would like to be included here?

**Moderator to note down any answer in the box labeled 'Other'. Once completed ask for rationale:**

- Why is [attribute] the most important attribute for you in an SMA treatment?
- Why is [attribute] the least important attribute for you in an SMA treatment?

## Section 4 – Conclusion (5 minutes)

**Objective:** *To close out any outstanding questions and discuss any further issues*

How would you sum up the impact on [you/on your child], of having SMA?

- What, if anything, do you do differently as a result of SMA? Why?
- What, if anything, do you do that is the same before the diagnosis? Why?

If there was one thing that has impacted [you/your child], that could change, what would it be?

- Why do you say that?

Above and beyond treatment, what would be the main improvements you would like to see that would make it easier for you/ your child as well as yourself to live with SMA?

That's it for today, thank you very much for your participation. Is there anything else you would like to share with me before we say farewell?

### ***THANK AND CLOSE INTERVIEW (CONTINUE RECORDING)***

Following completion of the interview with the respondent please state on the audio file the respondent number and the number and type of adverse events you have noted and intend to report.

Then please ensure you report these adverse events according to the reporting procedure in which you have received training.

Following completion of an interview in which you believe there to have been NO adverse events, please state clearly on the audio file that you are not reporting any adverse events.

END AUDIO RECORDING

## Appendix

### IMAGE DECK



## STIMULI 1

**Scared**

**Helpless**

**Calm**

**Informed**

**Supported**

**Shocked**

**Relieved**

**Angry**

**Accepting**

**Challenged**

**Confused**

**Worried**

**Anxious**

**Confident**

**Indifferent**

**Sad**

**Overwhelmed**

**Motivated**

**Embarrassed**

**Empowered**

## Self-Completion Form 1

Attributes	Definition	Points
<b>Motor Function</b>	Treatments can vary in terms of their average effectiveness in improving motor function amongst SMA patients	
<b>Breathing</b>	Treatments can vary in terms of their average effectiveness in improving breathing ability amongst SMA patients	
<b>Administration</b>	Treatments can vary in terms of whether they are administered by intrathecal injection, intravenous or orally	
<b>Side Effects</b>	People can experience a reaction to their treatment immediately after taking it or develop side effects at a later stage. These reactions may vary in severity (mild, moderate, severe)	
<b>Life Expectancy</b>	Different treatments vary in terms of their effectiveness in controlling the disease. Less effective treatments will mean on average you can expect to live for a shorter time. More effective treatments will mean you are more likely to live longer.	
<b>Other:</b> Please specify _____		
		<b>Total must SUM to 100 points</b>

**MCF 1**

<b>SMA Treatment satisfaction</b>	<b>Rating (0-10)</b>
1. Spinraza (Nusinersen)	
2. Zolgensma (Onasemnogene abeparvovec-xioi) / gene therapy	
3. Other: Please specify: _____	