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SMA Patient Focused Drug Development – Topic Questions

Topic 1: SMA Symptoms and Daily Impacts

1. What symptoms have the most significant impact on you/your child's day-to-day life (please focus on the top 1-3 symptoms of greatest impact on the life of you/your child)?
2. How does SMA affect you/your child's daily life on best days/on worst days? Describe a best day and worst day for your child and your family.
3. Are there specific activities that are important to you/your child that you/your child cannot do at all or as fully as you would like because of SMA?
4. How have your/your child's symptoms changed over time? How has your/your child's ability to cope with symptoms changed over time? (*may apply to patient or caregiver/family of patient*)
5. What do you fear most as the disease progresses and you/your child get older? What *worries* you most about your/your child's condition? What *frustrates* you the most about your/your child's condition?

Topic 2: Current and Future Approaches to Treatment

1. What are you currently doing to help treat your/your child's SMA/SMA symptoms? (*Examples may include prescription medicines, over-the-counter products and other therapies including non-drug therapies*)
 - a. What specific SMA symptoms do your treatments address?
 - b. How has your/your child's treatment regimen changed over time, and why?
2. How well does your current treatment regime treat the most significant symptoms of your/your child's SMA?
 - a. How well do these treatments improve your/your child's ability to do specific activities that are important to you in your daily life?
 - b. What activities that matter to you/your child are you still unable to do?
3. What are the most significant downsides to your/your child's current treatments and how do they affect your/your child's daily life? (*Examples of downsides may include bothersome side effects, going to the hospital for treatment, restrictions on driving, etc.*)
4. Assuming there is no complete cure for SMA, what specific things would you look for in an ideal treatment for SMA?

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Topic 2/All SMA Types

1) Which of your/your child's symptoms are not addressed as well by your current treatment regimen?

On an ideal treatment

2) What issues/symptoms would you like a potential treatment to address?

3) What would you consider to be a meaningful improvement (for example symptom improvements or functional improvements) in your/your child's condition that a treatment could provide?

4) How well have these treatments worked for you as your condition has changed over time? Examples of downsides may include going to the hospital or clinic for treatment, time devoted to treatment, etc.)

On treatment selection

5) What factors do you take into account when making decisions about selecting a course of treatment?

6) What information on the potential benefits of these treatments factors most into your decision?

7) How do you weigh the potential benefits of these treatments versus the common side effects of the treatments? (Common side effects could include headache, nausea, injection site reactions.)

8) How do you weigh potential benefits of these treatments versus the less common but serious risks associated with the treatments? (Examples of less common but serious risks are infections, cancer, liver damage, kidney damage, birth defects, blood disorders, differences in views on near-term serious risks vs serious risks that may emerge many years after treatment)