**2025 SMA Access Promo Messaging Qual**

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**Moderator:** Hello. How are you today? Thank you so much for joining. I really appreciate it. My name is Nancy. I'm the person who's going to ask you questions today, so thanks for putting up with me. I have lots of material and communication to show you about SMA. I didn't write any of it, so I don't care if you love it or hate it. I just want you to feel comfortable giving your completely honest opinion. I'll be asking you for your preferences about different things I show you, but I'm most interested in why you prefer one thing over another. In terms of the things I'm required to tell you, don't tell me your last name because I have no idea who you are. Let's keep it that way. I want to protect your confidentiality. I have a few colleagues listening in. None of us know who you are. We're recording for research purposes. If you mention that your patient has had an adverse event on a medication, I have to write up a report about that. Sometimes I need to hustle people along to get through all the material. Are you good with all that?

**Respondent:** Yeah. Okay. Great.

**Moderator:** Can you tell me a little bit about your practice, please?

**Respondent:** Yeah. I'm a pediatric neurologist, and I work in an academic medical center. I also specialize in pediatric neuromuscular disease. I've been working in this job for over twenty years. Today, we're talking about SMA, right?

**Moderator:** Yes. So, I think you see about fifteen to eighteen patients with SMA?

**Respondent:** Yes, about fifteen, maybe eighteen. They're all pediatric patients. We do have a couple who are over eighteen, but I would say fourteen are pediatric to teenager.

**Moderator:** Of those fifteen, do you have any who are not being treated, or are they all being treated?

**Respondent:** They're all being treated. I have two patients who got Zolgensma before they were two years old. I have seven patients on Spinraza and four on Evrysdi. I think one patient was on Spinraza and discontinued, and we're going to start Evrysdi.

**Moderator:** Can I ask why that patient discontinued Spinraza?

**Respondent:** He was having trouble with intrathecal injections. He's an older kid with deformity, and we have trouble doing the injection. Every time it's a hassle, and he has some type of meningitis or something right after. So, they want to switch to PO meds.

**Moderator:** With the Evrysdi, of your five patients and potentially six, how many are on the liquid versus the tablet?

**Respondent:** I think one patient is switching to the tablet. There are several requests, so we're working on prescriptions. The patient is able to swallow, and I think they prefer the tablet.

**Moderator:** Has the interest in the tablet come more from the patient than you as the doctor encouraging them to switch?

**Respondent:** Yes. They have preferred it.

**Moderator:** In general, do you have a preferred SMA treatment?

**Respondent:** Theoretically, I thought new patients would want to start on PO, but some prefer injections because they don't have to take a pill every day. Some patients prefer the pill because they find the procedure invasive. Some patients started on Spinraza before Evrysdi was approved, so they've continued with it.

**Moderator:** Do you have a preference for Spinraza over Evrysdi or vice versa?

**Respondent:** Efficacy seems similar to me. I haven't switched many patients to see if one fails and the other works better. The one case we switched was more about tolerability and the infusion procedure.

**Moderator:** Are you aware of any products or treatments in development for SMA?

**Respondent:** There's a move to extend Spinraza to older ages, and people are talking about combinations. All treatments are more about neuroprotection, protecting the neuron and getting SMN protein. There's also potential targeting within the muscle.

**Moderator:** I have brought up five themes on the screen related to treating SMA. Could you pick two that are most important to you when making treatment decisions?

**Respondent:** Efficacy and safety are always our ultimate goals. I would also choose broader indication to see the treatment having more range.

**Moderator:** Why is broad indication important?

**Respondent:** It makes sense if the mechanism of action is directly increasing SMN expression. If we're talking about gene transfer, it's more fundamental.

**Moderator:** Is the idea of five years of long-term control of SMA progression motivating to you?

**Respondent:** Yes, it's important. When the treatment came out, we observed that it's a long-term progressive disease. You need treatment to have a continuous long-term impact. The five-year experience shows good data and summarizes the milestone.

**Moderator:** I have five groups of messages to show you about Evrysdi. For each group, rank them from most to least preferred.

**Respondent:** I prefer messages that emphasize efficacy and long-term control. The biomarker level is key for biological effect, and maintaining SMN protein levels is important.

**Moderator:** What about the mention of SMN protein levels in the first two messages is more compelling?

**Respondent:** It's straightforward. The first two are direct, and you don't have to read the entire sentence to get the idea.

**Moderator:** I have an alternative headline for the message you liked most. Instead of "Evrysdi is the most chosen treatment for SMA," what if it said "Evrysdi is the number one prescribed treatment for SMA"?

**Respondent:** The number one prescribed treatment is more appealing.

**Moderator:** I have two more buckets to go. Which of these two do you prefer more?

**Respondent:** I like the first one, "reliable choice," because it applies to every patient regardless of condition.

**Moderator:** What do you think about the word "reliable"?

**Respondent:** It means the treatment is consistent and not affected by other factors like injection procedures.

**Moderator:** Last group of messages. Rank them for me.

**Respondent:** Systemic disease requires systemic treatment. It's important to have a constant supply of SMN protein.

**Moderator:** Is the word "systemic" important in this message?

**Respondent:** Yes, systemic treatment ensures a continuous supply of protein to the CNS.

**Moderator:** I need to move us along to our last thing. Look through all the messages and tell me which five impact you the most.

**Respondent:** The number one choice and systemic treatment messages are most important. Long-term control and maintaining protein levels are also key.

**Moderator:** Stepping back, what are your thoughts on everything you saw today?

**Respondent:** People look at data from different groups. The key is to see which treatment is more predictable with better outcomes. I learned that Evrysdi is becoming a more preferred treatment, and systemic treatment is important.

**Moderator:** Thank you so much for your time and insights.

**Respondent:** Thank you. Bye.