**2025 SMA Access Promo Messaging Qual**

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**Moderator:** Thank you so much for joining. I really appreciate it. Let me give you a quick introduction to what we're gonna do today. My name is Nancy, and I'm the person who's gonna ask you lots of questions. We're obviously gonna be talking about treating spinal muscular atrophy. I have some material to show you that was given to me. I didn't write any of the material. I don't work for the company that made it, so I don't care if you love it or hate it. Honestly, I just want to get your honest reactions to it. Part of my job is to protect your confidentiality, so just don't tell me your last name or the name of where you work or anything like that. I do have a couple of colleagues listening in, and we are recording, but it's all for research purposes. Your name doesn't get attached, and none of us have any idea who you are. Are you okay with all that?

**Respondent:** Yeah. Alright. Cool.

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

**Respondent:** Yeah. I'm a neurologist, adult and pediatric, specializing in neuromuscular. I've been in practice for twenty-five years in an urban area. I'm the director of MDM.

**Moderator:** Great. About how many SMA patients would you say you're managing right now?

**Respondent:** I think now it's about seven or ten.

**Moderator:** Are they a mix of pediatric and adult patients?

**Respondent:** Yeah, a mix of pediatric and adult.

**Moderator:** Are all your SMA patients on treatment?

**Respondent:** No. Not all are on treatment.

**Moderator:** What types of patients are not on treatment?

**Respondent:** All the adult ones, I think four of them are not on treatment.

**Moderator:** Tell me about that. Why are they untreated?

**Respondent:** Patient's choice.

**Moderator:** Can you tell me a little bit about what went into their choice or what they were thinking about not being treated?

**Respondent:** They don't want to be treated. So instead of pushing it, I leave them alone. If you push and they develop side effects, you'll be responsible.

**Moderator:** I guess I'm just trying to understand if you have any sense of why they are reluctant to go on treatment.

**Respondent:** It happens all the time. I have patients who have cancer and don't want to be treated, so you don't push them. If you push them and something happens, they're gonna say it's because of you. So you follow the patient's reach.

**Moderator:** Do you have a preferred SMA treatment?

**Respondent:** Probably, yeah, Spinraza, but not all my patients are on Spinraza.

**Moderator:** Why is that your preferred treatment?

**Respondent:** It's the first medication for spinal muscular atrophy, and everybody uses it as a benchmark for treatment.

**Moderator:** Of your six patients who are being treated, do you have any sense of how many are on Spinraza and how many are on something else?

**Respondent:** I think four are on Spinraza and two on Evrysdi.

**Moderator:** Do you have any patients on Zolgensma, the gene therapy?

**Respondent:** I had one that took Zolgensma, but I haven't seen the patient in over a year, so I don't include them.

**Moderator:** In general, how would you compare Evrysdi versus Spinraza?

**Respondent:** Spinraza goes direct to the motor neuron. SMA is a motor neuron disease, so Spinraza goes direct.

**Moderator:** Are you aware of any other treatments for SMA coming down the pipeline?

**Respondent:** I know the higher dose Spinraza, and there's a company, I don't know if it's UCB, that is talking about some medicine, but it hasn't been approved.

**Moderator:** Any thoughts about a higher dose of Spinraza being available for your patients?

**Respondent:** That's because you're going to decrease the time for the loading dose since you're given a higher dose.

**Moderator:** How likely do you think you will be using the higher dose of Spinraza?

**Respondent:** Likely, yeah, likely we will use the higher dose.

**Moderator:** Which patients do you think you would use the higher dose for?

**Respondent:** Any patient that requires Spinraza. I don't think there's gonna be a restriction for pediatric or adult. Spinraza is dosed by weight.

**Moderator:** I want to bring something up on the screen for you. Can you see what I brought up on the screen?

**Respondent:** Yeah. Welcome.

**Moderator:** Great. So I have been given a summary of a few SMA treatments that are in the pipeline. We talked about this higher dose of Spinraza. Have you heard that Zolgensma is being looked at for older kids as opposed to now just being up to two years of age?

**Respondent:** Yeah, they've talked about it, but I haven't seen it come out.

**Moderator:** Any thoughts about that?

**Respondent:** The question is gonna be why am I going to Zolgensma? I might as well just go to Spinraza.

**Moderator:** Very helpful. And then I don't know if you've heard about this anti-Myostatin.

**Respondent:** Yeah, I've heard about it. It's gonna be combined with Spinraza or Evrysdi.

**Moderator:** What are your thoughts about that?

**Respondent:** It may be helpful, but I know I will prescribe it because for SMA, anything that is out there, I'll prescribe it one way or the other.

**Moderator:** Next, I want to show you five themes about SMA treatment. If you could help me understand which maybe two are the most important to you when you're making choices about SMA treatment.

**Respondent:** L and M.

**Moderator:** Talk to me about those. Why are those the most important to you?

**Respondent:** Leading treatment, that's important. The mechanism of action is important. Like I said before, Spinraza goes direct to the motor neuron where the problem is. It doesn't go through the GI tract. It goes direct.

**Moderator:** And why is being a leading treatment important to you?

**Respondent:** Because it's the benchmark for treatment of SMA.

**Moderator:** Interesting. So when you see L, which treatment does that make you think they're talking about?

**Respondent:** Spinraza.

**Moderator:** I have a series of messages to show you. I have five buckets or groups of messages to show you. This is the first group of messages. For each of them, my first question is always going to be to rank the messages from the one you prefer the most to the one that you prefer the least. Does that make sense?

**Respondent:** Yeah. M is one, S is two, W is three, and D is four.

**Moderator:** Talk to me about your ranking. Why did you rank them this way?

**Respondent:** M helps increase the production of SMN protein. That's a way of saying it does something on the motor neuron. S obtained impact on the SMN protein level delivered daily. That's another way of saying it goes direct to the motor neuron to help it act. W means the patient is getting results because they're at a steady state. D doesn't really tell me much.

**Moderator:** To what extent are any of these messages actually motivating to you?

**Respondent:** M helps increase the production of SMN protein. The main thing in SMA is you don't have enough survival motor neuron. This helps to improve the daily survival motor neuron so that the muscles can move.

**Moderator:** Is there any new information for you among these messages?

**Respondent:** Not really.

**Moderator:** Why did you rank D the lowest?

**Respondent:** Consistent impact that lasts dose to dose doesn't really tell me much.

**Moderator:** Are you also reading the subheader too, not just the header?

**Respondent:** I've read everything.

**Moderator:** What do you think about this phrase in W? No waning, no wondering.

**Respondent:** Basically, it's telling you that this one dose fits all.

**Moderator:** And what's your reaction to that?

**Respondent:** That's great because if the dose for a seventy-year-old is ten ml, and for a two hundred-pound person, you're giving two ml, it's not gonna work because the volume distribution is larger on the bigger person.

**Moderator:** How believable do you think that statement is?

**Respondent:** I believe that. That's mostly how long-term extended-release medicines work.

**Moderator:** Is there anything on this page that differentiates Evrysdi from Spinraza in your mind?

**Respondent:** Not really.

**Moderator:** Let's look at the next group of messages, the S messages. If you could read these all, the bolded and the unbolded, and then rank them for me in terms of your preference, that'd be great.

**Respondent:** N will be one. W will be two, O will be three, C will be four, and E will be five.

**Moderator:** Walk me through your ranking, if you don't mind.

**Respondent:** Given the natural history, you need to achieve stability. That's the main aim of treating SMA. Two, it has safety within five years, so you can feel more confident. Three, it has a sustained effect. Four and five are almost the same, talking about long-term control of disease progression.

**Moderator:** I'm not understanding why you ranked these the way you did. Could you help me understand?

**Respondent:** Preserving function is clinically significant. The most important reason to treat SMA is to prevent progression and stabilize the patient.

**Moderator:** Is there any new information for you on this page of messages about Evrysdi?

**Respondent:** Not really.

**Moderator:** Anything that makes an impact on how you think about Evrysdi?

**Respondent:** Long-term efficacy makes an impact. You have long-term control of the disease.

**Moderator:** These messages use phrases like manageable tolerability or established safety. Any preference for either one of those phrases?

**Respondent:** Established safety is probably more important. No person has died from taking it.

**Moderator:** Any reason why you ranked E the lowest?

**Respondent:** Four and five are almost the same, talking about long-term control of SMA progression.

**Moderator:** One of the headlines we looked at here was your third most preferred message had this headline, sustained SMA stabilization observed over five years with Evrysdi. Instead of that headline, they could write curb instability observed over five years with Evrysdi or sustained efficacy observed. Do you have a preference for which language?

**Respondent:** Sustained efficacy is more specific.

**Moderator:** Here's our next bucket. It's only two messages. You can just tell me which one you prefer.

**Respondent:** I'll probably take B.

**Moderator:** Why is that?

**Respondent:** Because it came out openly and told you that it's indicated for newborn children and adults across different disease states. YR is saying it has proven efficacy in patients spanning a broad range of ages. It did not tell me if I'm talking with a newborn, a baby, or an adult.

**Moderator:** Does this matter to you when you're thinking about SMA treatments?

**Respondent:** Yeah, because you have to know if it's gonna take care of an eighteen-year-old or a five-year-old.

**Moderator:** Does that differentiate Evrysdi from others to you?

**Respondent:** Not really. It's recently approved for all ages.

**Moderator:** How do you feel about this word reliable choice?

**Respondent:** Reliable is a good way to put it. It's a reliable choice, which is true.

**Moderator:** Let's look at this next bucket.

**Respondent:** B is one.

**Moderator:** Why is that?

**Respondent:** It comes out plainly. It's clear for everybody to understand that it's effective in presymptomatic and type one, type two, type three SMA. This statement is plain.

**Moderator:** What does this mean to you, a foundational treatment?

**Respondent:** Probably the drug of choice or a baseline treatment.

**Moderator:** Does it feel like they're saying it's the drug of choice here?

**Respondent:** Yes.

**Moderator:** How do you feel about them saying that?

**Respondent:** It's correct. If you use it in presymptomatic, type one, type two, and type three.

**Moderator:** I'm confused because I thought you feel like Spinraza is really the benchmark.

**Respondent:** Yes, because Spinraza was the first. But Evrysdi is the first oral.

**Moderator:** This was the message that you didn't like as much. What if it instead had said Evrysdi is the number one prescribed treatment for SMA as opposed to it's the most chosen treatment for SMA?

**Respondent:** If you put number one oral treatment, you have to be specific. I think it's the only oral treatment.

**Moderator:** Do you have a preference for either LR or LS as a headline?

**Respondent:** Probably, in this case, I will say it's the most chosen treatment for SMA, but with the caveat that it's oral.

**Moderator:** The fact that it's oral is important, you're saying?

**Respondent:** It should be highlighted because a lot of people don't want to take intrathecal therapy.

**Moderator:** This is our fifth and final group of messages. If you could rank them for me.

**Respondent:** G is one. O is two.

**Moderator:** Why is G your most preferred statement?

**Respondent:** Increasing SMN protein level everywhere is important. The underlying cause of spinal muscular atrophy is less SMN protein.

**Moderator:** How motivating is that to you?

**Respondent:** It's very motivating, which means this medicine works at the root of the disease.

**Moderator:** To what extent does this distinguish Evrysdi from Spinraza in your mind?

**Respondent:** Spinraza goes directly where the problem is. Evrysdi works there, but everything has to go through a lot of channels to get there.

**Moderator:** Any reactions about these other statements?

**Respondent:** All designed to show up to put SMN everywhere, but one takes care of the deficiency, trying to fix it immediately.

**Moderator:** Message MR says, this is a systemic disease that calls for systemic treatment. I guess an option would be systemic disease requires systemic treatment. Do you prefer calls for or requires?

**Respondent:** Requires. You have to be plain. If not, they will ask you so many questions.

**Moderator:** Here's the biggest job I'm gonna ask you to do. I know this is a slightly overwhelming and busy slide because it's all the messages I showed you today. I'm gonna ask you to pick the five messages that you preferred the most.

**Respondent:** MO one, C five, CS, SC, BR.

**Moderator:** Talk to me about how you came up with these choices. Why were these the most important to you?

**Respondent:** CM tells you that it increases the protein, and that's what you want to know because there's a protein deficiency. CS tells you that once it's increased, you have sustained control. MO helps to put motor neuron in every part of the body. DR is a good choice for every living SMA.

**Moderator:** One thing I'm a little confused about is MO. Because on the one hand, you're talking about you like this statement. But I've only heard you talk about it affecting more than the central nervous system.

**Respondent:** Spinraza goes direct and then spreads its wings. Evrysdi goes through a lot of ways.

**Moderator:** If you had to pick two out of those five, which are the best?

**Respondent:** CM and DR.

**Moderator:** Is there anything you saw today that made you feel like maybe I'll push up Evrysdi a little bit more in the consideration set?

**Respondent:** No. Spinraza is first. But Evrysdi has other parts where you need motor neuron.

**Moderator:** Was there anything that made an impact on how you think about Evrysdi?

**Respondent:** Five years, no big adverse effect, which is a plus.

**Moderator:** Was that new information to you?

**Respondent:** Yeah. This is the first time I've seen it in print.

**Moderator:** Anything else you want to say?

**Respondent:** Evrysdi is a good medicine, but it's like a second choice for those who don't like injection.

**Moderator:** Thank you so very much for your time. I really appreciate you making the time to do this. Thank you for the work that you do, and I hope you have a great rest of your day.

**Respondent:** You do the same. Have a nice day. Bye-bye.