SMA Promo Refresh Qual

(Aug 12, 2025 - 5:00pm)

(00:00:02 - 00:00:09)

I'm good. Are you close to the, speaker of your phone?

(00:00:09 - 00:09:24)

**charles:** Yeah. I'm just sitting in front of the you can't hear me? And I just it's a teeny like, I can hear it pretty well. It's, like, just not crystal clear. But you're saying is it coming the the sound just coming right through your computer? Is that Yeah. what's happening? Right. K. I think I can, I think I can make it work? I seem to be the only one who has hearing challenges these days. Alright. Well, 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 pester you with lots of questions. We're obviously gonna be talking about, treating spinal muscular atrophy. I have some material to show you, doctor, 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 wanna get your honest reactions to it. And part of my job is to protect your confidentiality, so just don't tell me your last name or the name of, you know, where you work or anything like that. I do have a couple colleagues listening in, a few 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. As you know, if you mentioned that a patient of yours has had an adverse event on a medication, my client may say I just need to write up a report about that, maybe ask you a couple questions. Finally, sometimes I do have to interrupt folks just to move the conversation along just to make sure that we end on time. Are you okay with all that? Yeah. Alright. Cool. Alright. Can you tell me a little bit about your practice, please? Yeah. I'm a neurologist, adult and pediatric, specialize in neuromuscular. I've been in practice for twenty five years in an urban area. Okay. I'm In the director the what I'm sorry. in an urban area. I'm the director of In MDM. an urban area. Okay. Great. About how many SMA patients would you say, you're managing right now? I think now it's about, seven or ten. Did you say seven or ten? Yeah. Seven or ten. Okay. Are are they are they a mix of pediatric and adult patients? Yeah. of pediatric and adult. Okay. This woman is ten. Are say it again? Ten. Ten. Okay. Are all of them on treatment? Are all your SMA patients on treatment? No. No. Not all on treatment. Who, what types of patients are not on treatment? All the adult, the adult ones, I think, four four of them are not on treatment. Tell me about that. Why are they untreated? Patient's choice. Their their choice? Yeah. Can you tell me a little bit about what went into their choice or, you know, what they were thinking about not being treated? Their chances are they don't want to be treated. So instead You of know putting do you it, know I'll leave them alone. yeah. Any sense about that? Like, any more insight you can offer me into why they're not treated? Why they wanna be left alone? Tell patients there's a medicine, and the patient tells you I don't want to try it. So there's no need pushing. Okay. But Because you do if you you have I'm just I'm because gonna turn off my air conditioner, which the house. Yeah. Keep going. because if you push, and they develop side effect now, you'll be responsible. Yeah. Fair enough. I just think I guess I'm just trying to understand if you have any sense of why they have reluctance why they're reluctant to go on treatment. It happens all the time. I have patients who have cancer and doesn't wanna be treated, so you don't push Yeah. them. Because if Right. you push them Fair enough. if you push them, something happen, they're gonna say that it's because of you. So avoid getting into semantics or creating more problem, you you follow the patient's reach. Okay. Yeah. I mean, it's interesting. Like, with cancer, I know sometimes people are concerned about, like, the the ravages of chemotherapy. I didn't know if you had any sense of what the reluctance was in SMA to treat. No. Well, I have I have Okay. two patients Alright. with Duchenne, and they don't wanna be the parent doesn't want them to be treated. So you leave them alone. You take care of the minor things. Yeah. Okay. I hear you. Do you have a preferred SMA treatment? Probably, we say, yeah, Esperanza, but not all my patients are Esperanza. Why is is that your preferred treatment? That's the first the first medication for spinal muscular atrophy, and everybody uses this as a benchmark for treatment. Interesting. 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? I think four peranza and two, a, the Aviso. On Ebrzebe? Yeah. Okay. Do you have any patients on Zolgensma, the gene therapy? I had one that took Zojejensma, but, she hasn't he hasn't been to the clinic for over a year, so I don't include him. So she did you have one patient who had who did Zolgensma, but you haven't seen the patient since Yeah. getting Yeah. I the haven't treatment? been patient in over a year. So Okay. Alright. In general, what are your how would you compare besides the different mode of administration? Any feelings about Ebrzezinski versus Speranza? I mean Speranza goes direct to the motor neuron. SMA is I a motor see. neuron disease, so Speranza goes direct. Interesting. Like Okay. like, when Okay. you ask Willie Hutton, why did you rob the bank? He said that's where the money is. So the main problem is in the motor neuron, Esperanza goes right there. Okay. That's really, really helpful. Okay. Awesome. Are you aware of any other treatments for SMA coming down the pipeline? I know the higher dose Piranha, and there are some there's a company. I don't know if it's a UCVO that is talking about some medicine, but as of now, other than those two. What's you said high dose Spinraza. What was the second one you said? There's a company that I can't remember if it's UCB or Genent no. Not Genentech that is coming up with a medication. That's probably gonna be either IV, but it hasn't it hasn't been approved. Any thoughts initial thoughts about a higher dose of SPINRAZA being available for your patients, I supposed about potentially to be being available? it's supposed to be out by October or November. Mhmm. And what do you think about that as a potential option? Yeah. That's because you're going to decrease the loading dose, the time for the loading dose since you're given a higher dose. Say that one more time. I'm sorry. You're going to increase the time interval for decrease the time for the loading dose since you're using a Yes. high dose. Yes. And how likely do you think you will be using the higher to be using the higher dose of FINRAZA? Likely, yeah, likely we use the higher dose. Okay. Which patients do you think you would use the higher dose for? Any patient that, requires Esperanza. I don't think Okay. there's gonna be restriction for pediatric or for adult. I think any person because Esperanza is those by weight. Okay. Alright. I wanna bring something up on the screen for you. Can you see what.



Screen Shared @ 00:09:24 by Nancy



(00:09:24 - 00:47:16)

**charles:** I brought up on the screen, doctor? Yeah. Welcome. Okay. 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 there's that Zolgensma is being looked at for older kids as opposed to now just being up to two years of age? Yeah. It's They're, being looked at yeah, yeah. they've talked about it, but I haven't seen it, come out. Okay. And it's an intrathecal. They've been using it administering your intrathecal. Okay. Yeah. But the Any question thoughts is about gonna that? be the question is gonna be why am I going to Zoho? might as well just if it's gonna be particular, I might as well just go to Speranza. Okay. Very helpful. And then I don't know if you've heard about this anti Myostatin. Yeah. It's yeah. I've heard about it. It's gonna It's be an combined with the with the and Speranza. exactly. Exactly. Or Everest Day. But, yeah, what are your thoughts about that? 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. Of course, SMA is not like is that is that a nasty disease, you know, basically. Alright. Next one I wanna show you is I wanna show you five themes about SMA treatment. And if you could help me understand which maybe two are the most important to you when you're making choices about SMA treatment. L L, and l and and m. l and m. Talk to me about those. Why are those the most important to you? Leading treatment, that's important. The mechanism of action is important. Like I said before, Spiranza goes direct to the motor neuron where the problem is it doesn't go through GI tract. It goes direct. That's really why I'm not a fan of RISD. Oh, I see. Because it it affects the GI tract. It affects other parts Yeah. It of goes the body. through the GI track, goes through what, Esperanza goes direct. Okay. And why is it being a leading treatment? Why is that important to you? Because it's the benchmark for treatment of SMA. Interesting. So when you see l, does that what I I know this may seem like an obvious question. Which treatment does that make you think they're talking about? Speranza. Okay. And also m or Adam no? is gonna be Speranza. Alright. I wanna now I have a series of messages to show you actually about. And I remember I didn't write these, so I just been looking for your honest reactions to them. I have kind of 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 my first request of you is to rank the messages from the one you prefer the most to the one that you prefer the least. So one, two, three, four. That make sense? Yeah. S is, m is one, s is two, w is three, and d is four. Okay. Talk to me about your ranking. Why did you rank them this way? Averagely help increase the production of SMN protein. That's a bad way of saying that is doing something on the motor neuron. Mhmm. Obtained impact on the SMN protein level delivered daily. That's another way of saying that it goes direct to the motor neuron to help the motor neuron act. And then three, no warning, no wondering, take take it day. It means patient is getting resolved because he's gonna be at a steady state. Okay. Okay. And then four d, that consistent impact that last dose to dose. Yeah. Because instead of you go to the the level goes up and goes down, While it's coming down, you give another one. You you stay at a steady state. To what extent do are any of these messages actually motivating to you or, you know, make an impact on how you think about everything? Yeah. Yeah. M, it And help it why help increase yeah. the production of SMN protein. What's the what's the main thing in SME? You don't have enough, survival motor neuron. So this help to improve the daily survival motor neuron so that the muscles can move and everything can move. Is there any new information for you among these messages? Not really. I don't understand why you ranked CD the lowest. Anything you can tell me about why that one you preferred the least? Consistent impact, that last dose to dose, it doesn't really tell me much. Okay. And are you also reading the subheader too, not just the header, the unbolded part? Well, I've written everything. Great. Brilliant. It's So designed you're saying to just give this you and your patient a treatment that works every day every day to increase and sustain. That's what I said that instead of the graph falling down complete, while the Yeah. graph is falling down let's say you take it this morning. While the graph is falling down around five AM, by six, seven, you take another one. You build up the graph so that area under the curve will be well situated. What do you think about this phrase in CW? No waning, no wondering. Basically, it's telling you that this one dose fits all. Say that one one dose fits all. Is that what you Right. said? Right. And what's your reaction to that? You've got that grade because let's say that, the dose for a seventy year old is, ten ml. Two hundred pound person. You're giving two ml. They said ten ml. It's not gonna work because Mhmm. the volume distribution is larger on the bigger person. Okay. Okay. How believable I mean, you ranked this fourth. How believable do you think that statement is? C d. Yeah. I believe yeah. I believe that. Okay. Because that's mostly how long term this is an extended risk medicine. That's how they work. Okay. Likewise, like an extended medication extended release medication. Is there anything in on this page that kinda differentiates Edwardsey from Spinraz on your mind? Not Nope. not really. Okay. Okay. 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. Is gonna be n will be one. Mhmm. W will be two, Oh, we betray What I'm sorry. What would be three? s o. Okay. Great. C will be four, Okay. and that will be five. So yeah. So walk me through your ranking, if you don't mind. Given the natural history, you need to achieve stability, so I've raised the does it the function. What's your main aim of treating SMA? We want to prevent more damage so that there will be some improvement. Mhmm. Mhmm. Two, Okay. it has a safety within five years of safety sparing rain, you can feel more confident. Yeah. He's been around for a while. He hasn't killed any person. First. I don't know why they didn't just say it that way. It would have been just kidding. No. That's real. He hasn't he hasn't killed any person. Three, it has a sustained effect. It stabilizes it for at least five years. Four is telling you that it has a long term control of disease progression. And then four and five almost the term, it has a long term efficacy. So, doctor, I'm not understanding, though, why you ranked these the way that you did. So could you help me understand what was it about this message that you why did you prefer that message the most, s n? functions. It's clinical significant. Preserving function. What's the most important thing of reason why you treat the same? One, to prevent progression of the disease. One, stabilize the patient. If he's a baby, you need to prevent the motor neuron from dying so that they don't develop significant delay. My, Mhmm. I Okay. have a patient Great. who receives Piranha, two weeks of life. If you see her, you don't know that she has a survey. Wow. Wow. Is there any new information for you on this page of messages about Evertise? Not really. Right. Anything that makes an impact on how you think about Aversity? Long term efficacy makes an impact. You have a long term control of the disease. There they there's a these messages use phrase like manageable tolerability or established safety. Can't find where that is. Established safety or well characterized safety profile. Where's established safety? I don't know if it's in here. Any preference for either one of those phrases? Manageable tolerability, well characterized safety, established safety? Established safety is probably more more important here. Like I said before, no person has died from taking a. Right. Right. Eleven days Okay. already eleven days, has two people died already. Okay. Any reason why you why you ranked this one the lowest or the least preferred, No. s Because e? four and five are almost the same. Okay. Why were they at the bottom? Four and five. Is there a toss-up between which one will be four and which one will be five? But they Yeah. are talking But what is it about either one of those that were the your least favorite? they are they are talking both of them are talking of a long term control of SMA progression. Okay. So there these are some various headlines. One of the headlines that we looked at here was your third most preferred message had this headline, sustained SMA stabilization observed over five years with Everesty. Instead of that headline, they could write curb instability observed over five years with Everesty or sustained efficacy observed. Do you have a preference for which language sustained stabilization? Efficacy is more specific? Right. Okay. Okay. Here's our next bucket bucket. It's only two messages. You can just tell me which one you prefer. I'll probably take b no. Four second. I'll probably take b p. This one? Yeah. The first one? Yeah. Why is that? Because it came out openly and told you that he's indicated for newborn children and adult across different disease Mhmm. state. YR is saying it has proven efficacy in patients spanning a broad range of ages. It has a broad range of ages. It did not tell me, am I talking with a newborn? Am I talking with a baby? Adults and adult and geriatric. You have to be specific. Does this be more specific. Does this actually matter to you when you're thinking about SMA treatments, you know, that it was indicated for such a wide for for everybody, basically, newborns, children, and adults? Yeah. Because you have to know, is he gonna take care of, eighteen year or is he gonna treat five years old? Yeah. Does that differentiate adversity from all to you? Not true. recently approved for all ages. Okay. So that's a bit different. So it's not really different in your mind? Right. Okay. How do you feel about this word reliable choice? Reliable. Is a good way to put it. Is there a reliable choice, which is true? Or then you go back Alright. and ask what do they mean by reliable? Is there a standard of choice, or is Say that it is it say a that standard again? of of treatment, or is it the drug of choice? It's probably Yeah. And how would you answer that? So what are you saying about that? Is it it's probably the drug of choice. so you'd rather it be the drug of choice rather it being a reliable choice. Is Yeah. that what you're saying? of those in medicine, you have to be specific. Okay. Alright. Let's look at this next bucket. B is one. Okay. Why is that? It comes out plainly. It's clear for everybody to understand that is effective in presynthomatic and type one, type two, type three, SMA. So this statement is plain. It doesn't go round and round, or it doesn't talk like a politician. What does this mean to you, a foundational The treatment? What is that word? What do you think they're saying there with foundational? probably the drug of choice or a baseline treatment for Does it feel like they're saying it's the drug of choice here when they're talking about a foundational Yeah. It's treatment? a of choice. And how do you feel about them saying that? It's correct. If You feel you like can Ebrig use it is the drug of drug yeah. of If choice? you use it in presymptomatic, type one, type two, and type three. I'm confused because I thought you feel like SPINRAZA is really the benchmark. Yes. Because Speranza was the first. So everybody is looking at Speranza, but Evarise is the first oral. So, Okay. Yeah. like, with the way I present to my patient is, one, they put a needle in your back, and you get injection every four months. The other one, you get it once, and that's it. And the other one, you take a pill. Mhmm. Okay. Hold on. Hello? Okay. Can I call you back? I mean, in long distance. Okay? Okay. Go ahead. Okay. This was the message that you didn't like as much. What if it instead had said Avisd is the number one prescribed treatment for SMA as opposed to it's the most chosen treatment for SMA? If you put number one oral treatment, you have to be specific. I think it's the only oral treatment. Yeah. But it's the number one oral treatment. Yeah. Do you have a preference for either LR or LS as a headline? Probably, in this case, I will say is the most chosen treatment for SME. But then I have to the caveat there that is oral. Okay. So the fact that it's oral is important, you're saying? It should be highlighted. Yeah. Right. Because a lot of people don't want to take, intra tickle therapy. Yeah. A lot Okay. of people don't even want spinal Yeah. Okay. tap. Especially This is our final book. that they can I'm be paralyzed. I'm sorry? I say Especially especially American when you people. mention that they can be paralyzed. Oh, they're afraid of being paralyzed. K. This is our third, I mean, our fifth and final group of messages, if you could rank them for me. G is one. O G is is one. yeah. O O is is two. two. Mhmm. So why is g your most preferred statement? Help me understand that. Increasing SMN protein level everywhere is important. What's the underlying cause of, spinal muscular atrophy? Less SMN protein. Okay. What and what is it about that language that why did that why If did you you prefer want that language? if you understand the mechanism of action, or if you understand the mechanism of, span and muscular atrophy, Yeah. you will get this that this medicine will improve SMN protein all over the body. Yeah. How motivating is that to you, that idea? It's very motivating, To what which means extent you tell does it me which means you're telling me that this medicine works is working at the root of the disease. yeah. To what extent does this distinguish Ebruzy from Spenratha at all in your mind? least the what where the problem is, But directly Okay. where the problem is and what's there. What everything works there, but everything has to go through a lot of channels to get there. It's like Okay. if you're going to DC, if you go on a a straight flight, you'll get there. But if you do a stopover, you'll get there, but you delayed. The RAVA is one star is a direct flight and is a a connecting flight? Right. it makes sense. Any reactions about these other statements? Any reactions towards or I don't know. I didn't use the right preposition. All designed to show up to put a seven everywhere, but and the three addresses the symptoms by boosting the production, which is which is true. But one takes care of the deficiency, try to fix it immediately. Mhmm. Yeah. It's interesting. There's more, like, science in this message. Any re anything yeah. Okay. Alright. I have a question for you. Message m r it says, this is systemic disease calls for systemic treatment. I guess an option, an alternative would be systemic disease requires systemic treatment. I Right. don't know if you prefer calls for No. or requires. Require. Requires. You have to Require. you have to put remember, you this is for doctors. Right? Yeah. You have to be plain. If not, they will ask you so many questions. Okay. Alright. Here's the biggest job I'm gonna ask you to do. I know this is a slightly overwhelming and busy slide because there are it's all the messages I showed you today. So I am gonna ask you, doctor, to pick the five messages that you preferred the most. I mean, frankly, it's the five messages that mattered to you the most about Abrisee that really came the closest to make an impact on how you feel about Abrisee. M o one. Wait. Which one? Oh, can I just tell you something? I forgot to say, Charles or doctor. You in picking the five statements, you don't have to pick one from each bucket. Like, you could choose all if there were, you know, you could choose four from one group and two from another. I don't want you to feel like you have to go through each and pick one from each. So don't please don't feel that you Yeah. have to do that. So just five Yeah. Okay. and any from it. Okay. Detail Great. I m one neglected. o two M wait. M which? c c m is one. Oh, c c m. Okay. Yeah. Number two is, m o. Okeydoke. C five is number three. Three five. Yeah. C c s C s. I feel like we're playing bingo. Okay. C s. Okay. What's next? s c Let's see. Okeydoke. b r. Okay. Talk to me about how you came up with these choices. Why were these the most important to you? Centimeters, basically, it tells you that increases the protein, and that's what you want to know Yeah. because there are protein deficiency so it increases it. And s tells you that once it's increased, you have sustained. It goes all through. Then K. That's helpful. c s c, he has a long term control, long term of progression, And then MO, it helps to put motor neuron in every part of the body, so it helps all of them. Then d r is a good choice for every living SMA and in every journey, which may tells you that it Okay. Okay. And did you talk about CS? SMA. Yeah. The c c f. Okay. One thing I'm a little confused about is MO. Because on the one hand, you're talking about you said you like this statement. You preferred it. And this statement talks about how it increases SMN protein production throughout the body. But I've only heard you really talk about it sort of being a negative thing that is affecting more than the central nervous system. Do you know what I mean? Yeah. But Spiranza goes and direct. And then from there, Yeah. it starts spreading its wings. But I see. goes through a lot of ways, Remember my analogy of going straight and then Right. making a stopover. Okay. So you're saying that even though SPINRAZA may initially impact the central nervous system, it doesn't stop there. It spreads. If we continue with our aeronautical metaphors, it spreads its ways after that. Okay. If sorry. This is the last time I'm gonna make you do this. If you had to pick two out of those five, which are are the best? Which are the two best ones out of this five? C m, that's one, and the d r. Yeah. I mean, ultimately, is there anything you saw today that I mean, I'm gonna I'm gonna put this away for a second just so I can see your face a little bit. So you really came in today talking about SPINRAZA being truly the benchmark, the standard of care, like, the longest it's been there, takes a direct flight to the central nervous system. Is there anything at all that was said today that made you feel like, you know what? Maybe I'll push up Efresdi a little bit more towards my in the consideration set a little bit more give a little bit more like, maybe I should rethink a little bit about, feeling about Eversity. Or, frankly, was it like, no. This just reinforced why Spinraza is always my first choice. No. are they are first. But every Yep. guess they are second, but it spreads its wings. It has Right. other parts where you need motor neuron. Yep. But my question is, was there anything that you read today you think that made any kind of impact on how you think about? Yeah. Five years, no If no well, like, they were like they were put inside five years, no person died. That's important. Didn't quite say that, but I hear what you're saying. Right. Right. Right. Was that new information to you, the five years and the safety profile? Yeah. This is the first I know that nothing has happened, but this is the first time I've seen it in print that five years hasn't had a big adverse effect, which is a plus. Okay. That's so interesting. So maybe, like, is it fair to say and tell me if I'm wrong. Like, intuitively, you kinda knew that, but you hadn't it it it's helpful to sort of see it in black and white kind Right. of. Right. Yeah. Okay. Anything else you wanna say? Any other impressions? I know I've asked you so many questions about what I've talked to you about today. Any reactions? Any, you know, anything missing from what was written here that would, you know, No. Ev Everest is a good medicine, but better it's like a second choice for those is a first yeah. choice for those who doesn't like injection. Yes. And in reality, Yes. if it wasn't that it has to go through the GI track and all these things to get to the brand, it would have been the first choice. Yeah. I hear what you're saying about the GI tract. Well, I have one last really important question for you. And it's so are you eating peanuts or almonds? I just wanna know what it is you're eating. Almond. And are they are they, like, roasted and salted kinda thing, or do they have any flavor on them? Unsalted. Okay. Good pro good protein source. Good healthy fat source. Right. Doc 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. You do the same. Have a nice day. Bye bye. Bye.

