**2025 SMA Adult Activation Qual**

*3:00pm EST | R01 Transcript*

SMA Adult Activation Qual

(Oct 3, 2025 - 3:00pm)

**Moderator:** Everything he says is completely confidential. Okay. Now I'm gonna say that same thing, but in earnest to you.

**Respondent:** Yes, ma'am.

**Moderator:** So my name is Nancy. This is what I do for a living. My job today is to ask you a lot of questions about SMA, and I am not an expert. I'm not a medical provider or anything like that. I'm just here to understand. I have a lot of questions, so every once in a while, I interrupt folks just to make sure that we end on time. I'm sure you have other things you wanna do on a Friday afternoon. But, also, part of my job is to protect your confidentiality. So just don't tell me your last name or if you can help with the names of any of your doctors. I have a couple colleagues at this point who are listening in. None of us know who you are, how to reach you, or anything like that. Also, we are recording. It's just for research purposes. Your name doesn't get attached to that. If by any chance you mentioned that you've had a less than ideal experience on a medication my client makes, I just have to write up a report called an adverse event report, and then I would ask you some questions about it. Are you good with all that?

**Respondent:** I am. Thank you.

**Moderator:** Awesome. Can you just tell me a little bit about yourself, like, how old you are, if you live with anybody, how you spend your days?

**Respondent:** So I am 60 years old, and I live with my spouse of thirty-four years. We have a 22-year-old daughter who's in college, and she also lives here. And then our two dogs, only one isn't here with me. I work full time at a university. I oversee a team of researchers in the fundraising arm of the university, and I have been doing that for almost thirteen years. I was at another university in the Midwest for seven years. I love flea markets. I love to travel. I recently had a medical emergency in July, returned from a twelve-day Mediterranean cruise, and got sick when I got home, turned into pneumonia, and I ended up in the ICU and respiratory failure. Now I have a trach. So the trach is new, within the last couple of months, and I'm adjusting to what my new normal is.

**Moderator:** Is it purple? Am I seeing that correctly?

**Respondent:** The speaking valve is purple. In order to speak, you have to have either a cap, which I have not graduated to using, or a speaking valve. So my speaking valve is purple. I have another one that's green. But, yes, it is purple. It's my favorite color, so that was a good thing.

**Moderator:** Well, that is a remarkable story that you just told. You used the word respiratory failure, which is dramatic. Were you intubated?

**Respondent:** They could not intubate. They tried three times in the ICU, and then they had to perform an emergency cric, which is a trach, but not permanently placed. I was sedated for several days. It was very scary for my family and for me when I came out of the sedation. A few days after that emergency trach, I went into surgery, and they put in the permanent trach. I was in the hospital for three and a half weeks. I've been home since early August, and two weeks ago, I was back in the hospital to have my gallbladder removed. I've had no surgeries ever in my life until this year. So I made it sixty years with no surgeries or any major medical issues, other than my spinal muscular atrophy. I consider that a win, and so do the pulmonologists that I see.

**Moderator:** You present as an incredibly joyous human being.

**Respondent:** Thank you. I really am trying. I'm on short-term disability from my job right now, and I'm actually hoping to go back to work in the next couple of weeks, hopefully, because I miss it, and I miss all my people.

**Moderator:** It's a time where universities need you to be doing some fundraising.

**Respondent:** Yes, ma'am.

**Moderator:** I have so much to ask you, and I'm so excited that I got you on this interview. Thank you so much. I'm gonna just do my best to get through all my questions. I have written down here that you're type three SMA. Is that accurate?

**Respondent:** That's accurate. When I was diagnosed, there were no types. In 1967, when I was diagnosed at three years old, I probably would not have been diagnosed that early, but my very young mother was very intuitive. I have a brother who's eleven months older than me. She noticed one of us was walking differently than the other one, and she took us both into the pediatrician. They did a muscle biopsy, and they actually diagnosed me with Weierman Costman Syndrome, which I believe now is considered type one. But there were no types back then, and so it got readjusted later in my life to SMA type three.

**Moderator:** Before the crisis in July, the medical emergency, how would you describe your SMA, like, had it been and the arc of the SMA up until that point?

**Respondent:** Sure. I'm a very active person, and people are often surprised with my SMA, how active I am. Certainly in the last ten to twelve years, probably since we've been here, so twelve to thirteen years, there has been a definite decline in my strength and abilities. It hasn't stopped me from doing things, but certainly more weakness in the last ten to fifteen years. I walked until I was 12 or 13. I transitioned into a wheelchair in late middle school. I drove up until fifteen years ago and then decided that my strength was such that I didn't feel safe, so I didn't want to drive anymore. I was very ambitious. I went away to school. I traveled by myself in my early years, probably until I was in my thirties. So I've had periods of time where I've been very strong. I used to be able to do all kinds of things. Doctors would bring in residents when I would go to the clinic, and they would be amazed at what I was able to do. I didn't have a motorized wheelchair until I was 18 years old, and I went away to college. So I used a manual wheelchair and propelled myself. As we age, and I'm at a desk job, you lose things that you wish you hadn't lost. Gaining weight has been a struggle my whole life. My SMA is such that when you think about SMA, I always feel like there's two body types with SMA. There are those that are skin and bones and weigh barely eighty pounds, and then there are those of us like me that are on the very heavy side. No matter what we do, I have what I call an SMA belly. It's just very rounded. I think having the weight on me has made it easier for me, maybe because I don't know. That's kind of a weird thing to say, but it's made me more stable. I'm able to sit up. I've been able to do a lot more things because I had more muscle mass.

**Moderator:** Thank you for all of that. To what extent do you feel connected to the broader SMA community?

**Respondent:** I feel pretty connected. I belong to a number of groups on Facebook. I'm of the age where Facebook is my friend. My daughter would tell me that Facebook is lame, but for me, it's a place I go to for information. I belong to a number of SMA-specific groups. I also belong to a trach group, where I can post questions and say, hey, how are you handling this? I just posted the other day, has anybody ever gotten their employer to pitch in for caregiver support? I feel pretty connected. I went to MDA summer camp until I was in my early twenties.

**Moderator:** What kind of summer camp?

**Respondent:** The Muscular Dystrophy Association runs summer camps. I started going when I was five, and I went every summer until I was, like, 22. I have a real connection. I have very good friends that I met at summer camp, and we're still friends thirty years later.

**Moderator:** Was there an SMA subgroup in the muscular dystrophy camp?

**Respondent:** No. The camp was for anybody with any of the neuromuscular diseases. SMA is a pretty broad population that was at the camp. I would say probably a good thirty to forty percent of the kids at camp had SMA, but it was a mix of all the different diseases under that umbrella.

**Moderator:** Are you connected to any other SMA resources, like SMA Connect or an SMA magazine?

**Respondent:** I don't know if I get a magazine, but I like Cure SMA. I'm on their news group. My sister stays very well connected, and then she sends me links all the time.

**Moderator:** As your advocate, kind of?

**Respondent:** Yeah. She's great. She lives out of state. We don't live near each other, but I've seen her a lot because she has been flying in multiple times this summer. She also is really connected to different groups.

**Moderator:** Wow. On your behalf.

**Respondent:** Yeah. She's great. So I feel connected to SMA groups, and then, of course, through my neurologist.

**Moderator:** Which SMA-specific groups do you belong to on Facebook? You mentioned the trach, which may not be specific to SMA.

**Respondent:** There is a Living with MD group, where the majority of people on that list have SMA. Of course, Cure SMA. Those are the two biggest groups that I interact with.

**Moderator:** What healthcare providers do you talk to about SMA or are part of your SMA care?

**Respondent:** My primary care physician is often my starting point just because she's close by. My neurologist is an hour away. My neurologist, of course, and recently, a pulmonologist. Those are the three main doctors that I interact with regarding my SMA.

**Moderator:** How often do you see this neurologist who is an hour away?

**Respondent:** Once a year.

**Moderator:** What type of medical environment are they involved in?

**Respondent:** They're at a university-based medical environment. They're a center of excellence.

**Moderator:** Great. So you see your neurologist once a year. What's your relationship like with that neurologist?

**Respondent:** I love her. She's amazing. Very knowledgeable. She mostly deals with children. I don't know why, but the adult population of SMA, we're living a lot longer now, so there's more of us. She's amazing. She takes time. I can send her a message in the portal. She'll answer me. If I need a quick video visit, we can do that. I really trust her. She's really great.

**Moderator:** How much of your dynamic with her is her saying, "This is what I think you should do, Michelle," versus you saying, "I've read about this. What do you think about this?"

**Respondent:** I probably bring up the ideas and say, "Oh, I read about this. What do you know about it? Do you think it would help me?" Like, I recently did that because there's this new drug on the horizon, and I'm like, "Hey, I read about this. I want to know more. What can you tell me?"

**Moderator:** What role does your PCP play in terms of your SMA?

**Respondent:** She often helps if I need specific equipment because today, you have to have a script for everything, including a shower chair or whatever. She's the point person for me now, as I deal with things with my employer and this new trach. She consults with the pulmonologist because they're in the same umbrella practice. That's where I go when I'm sick. She has to know how to treat, in conjunction with, "Hey, I gotta keep in mind this SMA." For instance, the hospitalist wanted me to have a muscle relaxer, and in conjunction with the PCP, she said, "Hey, because of her SMA, we don't really like giving muscle relaxers. She already has enough muscle problems. I don't think that's a good idea." So she advocates to say, "Hey, I have to think about the SMA with all the medications we're gonna give her or any treatments we're going to have."

**Moderator:** It's striking to me that she knows enough about SMA to even say that.

**Respondent:** Well, she didn't in the beginning. I'm a very good educator. I don't know how many times when I was in the hospital, either me or my wife would say, "Have you read the chart? Maybe you should go read the chart and then come back and talk to me." I expect that if you're gonna treat me, you need to learn a little bit. I get that SMA is not something that every doctor might have heard about, but it's not their thing. But you should at least go Google it and be a little bit smart about it.

**Moderator:** Again, your attitude is impressive. I wanna transition to talking about treatment. I have written down here that you were on a brisee liquid as well as SPINRAZA, but that you haven't been treated since September 2020. Is that right?

**Respondent:** I might have filled that out wrong. I started with SPINRAZA because it was the only treatment that was out there and was finally approved for adults. I started it in March 2020. At the height of the pandemic, it was a joy to go for treatments. I did it until Evrytesse was released later that fall. I asked my neurologist. I'd had one really bad experience with SPINRAZA. One, it's very difficult to get the injections. They were painful. Plus, the two days that you would have to stay pretty much immobile after the injections. It interrupted my life too much. The second to last one that I had in July, I got a spinal headache following the injection. I have never experienced anything as horrible as that spinal headache. When my sister called me and said, "If RISD is released, you need to talk to your neurologist," I called her and said, "I wanna switch. How do I do it?" Because I could do it at home, and I wanted to switch. She said, "Okay. Let's do it."

**Moderator:** Besides all those downsides to SPINRAZA, were there any upsides for you with SPINRAZA as a treatment?

**Respondent:** I never felt any upsides. I have a lot of friends, again, through some of my communities on Facebook, that were like, "Oh my gosh. I feel so energized after it." I never felt that. I never had any of the upsides that people were talking about.

**Moderator:** What motivated you to go on Spinraza in the first place? What were you hoping to achieve?

**Respondent:** For me, anything that would stop the progression. That was my number one. Stop the progression. I would have loved to have seen some strength increases, some noticeable things change in my world, and I just didn't. But my hope going on it, I was super excited. I absolutely was gonna try it. There was no doubt in my mind in the beginning. It was like, yes. It's now approved. I had a tough time getting approved, and my insurance never approved it. I ended up going through the Spinraza Foundation or through the Powell program, and they paid for it.

**Moderator:** Do you know why the insurance denied you? Did they give a reason?

**Respondent:** A lot of it was my age. They kept saying that I didn't fall within the age bracket even though I did, and there wasn't enough evidence in the clinical trials to show that there was benefit.

**Moderator:** You obviously advocated very hard for yourself and found a way. And then your sister is like, "Michelle, your wrist is available," and you're like, "I don't have to go into the hospital. Let me call my neurologist." When did you start that in 2020?

**Respondent:** I started in September of 2020, and then I was on it until, like, February '22.

**Moderator:** So a year and a half.

**Respondent:** Yeah. I stayed on it. Then things that I was experiencing, just again, I wasn't seeing any improvements. I didn't feel any different. I was still losing strength, and I was gaining weight. Whether that was tied to the Avisd or not, it was correlation for me that it was connected. After talking with my doctor, my neurologist, I just said, "I'm frustrated. I don't know that it's really working." My upper limb evaluations that I was doing every year, it's a test that they do, they were pretty much the same. They weren't increasing. It was just pretty much stable. In my thought process, I should have had improvements because everybody was seeing improvements. I'm like, I'm not seeing any improvements. So I made the decision. It was a tough one, but I made the decision to just go off it. It was my decision to do it. I discussed it with my family. Everyone was a little leery, like, "Are you sure you're gonna go off it? We don't know if it's stopping the progression." That upper limb, it's been pretty consistent. Maybe that's the sign that it is working. But I just went off it, and so I've been off it now for a while.

**Moderator:** You spoke to my specific question because I even highlighted when you talked about a RISD, you said, "Should I be seeing improvement?" But when I asked you earlier why you went on treatment, you said, "I went on treatment to stop progression." Can we list all the factors that led you to stop Everest? The things you've mentioned so far are weight gain, the sense that you couldn't see improvement. What other reasons do you think led you to stop?

**Respondent:** The portability, like, traveling was super challenging. I don't know how we would have done the cruise because we were on a ten-hour flight and trying to keep it cool and all of that. I wasn't approved through my insurance. So that was all being paid for through the program. I had to jump through a lot of hoops, periodically, to get reapproved for that. Every time it was delivered, I was like, "Oh my god. Don't break anything. That's $38,000 in medication in there." As I was just thinking about things, traveling and the portability of it, and you have to take it at the same time every day, which I did pretty good. My wish of wanting to see more in my body, and I just wasn't. I just wasn't sure it was worth it. There was a lot of conversation in some of the groups, again, from adults saying, "I get it. In kids, they're still growing, and you can see change. In adults, it's probably slower," but there were a number of people that were doing the same thing, saying, "Hey. I'm kinda done trying."

**Moderator:** With the weight gain, how significant was that in terms of your decision to stop?

**Respondent:** I gained 30 pounds in that year and a half. That's a lot for someone with SMA. Have I lost that 30 pounds? No. I've lost some, but I haven't lost that whole 30. To me, it just correlated with, "Hey. I started gaining weight when I started taking a freezed eat." They say weight gain is not one of the side effects. I'm like, maybe it's not, but that's what I experienced.

**Moderator:** What was the physical, emotional impact of the weight gain for you?

**Respondent:** A lot. Just fitting into my clothes and feeling confident. Extra weight makes it harder to do things. The harder it was, for instance, like, eating and lifting things up to my mouth. I started noticing that I was doing things like instead of sitting at the table, I'd sit at our little bar area because I could put my elbow up on the bar. I just noticed little things or the way that I would carry my laptop. There were just little things that probably in the bigger scope aren't very big of a deal, but somebody with SMA is that little incremental loss of things.

**Moderator:** You talked about portability. Anything else I should understand about the impact of the liquid and needing to be refrigerated that you didn't mention?

**Respondent:** Just the refrigeration and then the regimen of having to remember to take something every single day.

**Moderator:** What's the downside of having to remember to take something every day?

**Respondent:** I'm terrible at that. I am awful at that. I'm a very organized person, but regimenting, like, medications that have to be I don't take a lot of medications.

**Moderator:** Really? Wow.

**Respondent:** No. My spouse is really good at it. Thank goodness. She'd be like, "Hey. It's seven. We have to take it's time to take a freeze." I'm like, "Oh, yeah. Right. Let's do that." When you think about it, it really shouldn't have been that big of a deal, but it just was.

**Moderator:** You talked about the you couldn't get insurance, and so you were getting money through the risk manufacturers. You said you had to jump through a lot of hoops to get reapproved. Can you talk about that?

**Respondent:** Both of Brindsey and the Spinraza people, super nice, really great people. They wanted to do whatever to try to get it covered, but you had to be reapproved every so often, and I think it was different for both of them. I can't quite remember. I always felt like I have really good insurance. I'm taking this drug from somebody else that doesn't have insurance. I was so mad at my insurance. We went through I don't even know how many appeal processes. I think part of it too is that self-guilt of I have really good insurance, and why aren't they paying for this? I'd see it online, but everyone's like, "Oh, yeah. I went through my insurance. No problem." I'm like, what is wrong with my insurance?

**Moderator:** What do you mean self-guilt?

**Respondent:** I was taking money to pay for my drug over somebody that probably really needed it.

**Moderator:** Did it feel like it was sort of a zero-sum game? Like, if you got money that someone else couldn't get money?

**Respondent:** That's kinda what I felt like, possibly. I didn't know if that was true or not, but I don't know. I make a good salary, and I have good insurance. I don't qualify for disability programs in general. My caregivers are paid out of my pocket because I chose to work, and I also choose promotions when I get to.

**Moderator:** Can I ask you, Michelle, though, just I'm sort of interested in what when you talk about getting it funded through Eversity, what those hoops like, what those pain points were for you in that process?

**Respondent:** Well, I think it's all that financial stuff. I just had to answer a lot of questions, and it was just recertification. It really wasn't that painful, but just having to get recertified so that I was covered under their program to pay for it.

**Moderator:** When you say appeal processes to the Avrisky manufacturer or to insurance?

**Respondent:** To the insurance. Every day, there was never an appeal process. They wanted me to be on the medication if that's what I wanted, and so they were very helpful in trying to get that done.

**Moderator:** It's also really interesting what you said about the chatter online that there was sort of a trend of people talking about how it made sense that it was making an impact on kids, but that it was harder to see the impact on adults and people were dropping it.

**Respondent:** Yeah. There was a lot of that. I don't see it as much anymore. I don't maybe because it's been out for so many years now. I don't see as much chatter about it anymore. I know of a number of people that also dropped it. Many had stomach issues. I didn't necessarily have the stomach issues that I would attribute it to, but I know a lot of adults, you know, over 30, several that I know also quit taking it.

**Moderator:** When you stopped, I'm interested. So that was 22.

**Respondent:** Yeah.

**Moderator:** So that's been three and a half years. I wanna understand how you're feeling about treatment now. But I also wanna understand how you've and we have twenty-two minutes. So I'm just mindful of time.

**Respondent:** They told me I'm extending fifteen minutes or something.

**Moderator:** Oh, okay. My bad. I wasn't a 100% sure about that. Okay. So that gives me a little more flexibility. Well, thank you for extending fifteen minutes. So I wanna get to, eventually, how you're feeling about treatment now. Obviously, you had you alluded to having stuff happen over the summer. But up until what happened over the summer, for those almost two and a half years, how are you feeling about you and treatment? Because my understanding, you were not on treatment for those two and a half years. Right?

**Respondent:** I've not been on treatment since '22. It's been back and forth in my mind. Like, should I go back? Should I not? I don't think I'll ever go back to SPINRAZA unless they change the way that it's given. It would take a lot to convince me that that would be a good drug to try again. It's possible that I would consider d again, but probably only in conjunction with some sort of muscle builder. For me, I wanna see some increase. I wanna see improvements even if they're incremental. Since my hospitalization in July, I basically cannot lift my left arm. I can do some things with it, but I can't necessarily lift it in the strength in my left arm, the grip. My right is better. I can move it just fine, but my left arm is one that's really bothering me right now. So to me, I'm like, gosh. If I could get just a little more strength back in my left arm. Again, my sister is the one that told me about the new drug that's on the horizon. Starts with the a, I think.

**Moderator:** Antimyostatin.

**Respondent:** Yeah. I think it's the myostatin. Scholar Rock, I think, is the one. She was just here a couple weeks ago for my surgery. She spends the night with me at the hospital, and we often will talk until very late. We spend a lot of time talking about, well, maybe now is the time to rethink going back on something. That I've had this trauma, and maybe my body would respond differently now. It was quite a scare this summer. From her perspective, my wife, my daughter, they want me to be here for a lot longer. Part of that is reconsidering how do we stop the progression. Because now that I'm a trach user, I have a lot more risks. I don't wanna stop living, so how do I try to improve the quality and the length of my life? It might involve going back on some sort of treatment to really slow that progression. Because it feels like the progression is accelerating the older I get.

**Moderator:** How does it make you feel when you think about going back on treatment, Michelle?

**Respondent:** I would say nervous, but I'm not really nervous about it. I'm more open to it today than I was a year ago. I think of RISD, you know, I went on it right away. I think there's been a lot, you know, number of years now that it's been out, and certainly the transition from the liquid to the pill is an improvement. So there is that. So I think there are, you know, kinda like don't buy the first model of the car. Maybe I jumped it really fast. So maybe there would be a difference now. I don't know. But I think I'm more curious now about restarting something or starting something new. I think the other thing that I've heard about is combining Solgensma with one of those treatments as well, and that that's slowly going to be approved possibly for adults at some point as well. I don't know. That could be something that I would consider. I mean, I think there are more options available today. It's not just SPINRAZA or Infrisbee. There's more knowledge about it. I think there's probably more data. I'm a data person.

**Moderator:** Have you talked to your neurologist at all about what did your neurologist say when you stopped the treatment?

**Respondent:** She was supportive. She's like, "Listen. I don't wanna talk you out of it." I think that's kinda what I like about her is that she really listens to me, and she'll support it. She said, "If you wanna go back on it, we'll discuss that whenever you're ready." She understood my concerns. I think she reported them to, like you said, to the patient advocacy or whatever. I had a call, I think, with somebody from Ebrisdee soon after I quit to tell them my experience. But she wasn't negative about me quitting. She didn't say, "Oh, I think that's a bad idea. I think you should stay on it." She supported me getting off the medication because that's what I wanted to do.

**Moderator:** Have you guys talked about going back on treatment since February '22?

**Respondent:** No. We do not.

**Moderator:** Looking back on that, are you glad she didn't bring it up again?

**Respondent:** No. I mean, I think I kinda wish that she came up with the ideas sometimes. I feel like I'm always the one saying, "Hey. What about this?" I really wish sometimes she would say, "Hey. This is gonna be really good for you, and I think you should do it." She's really smart, but I wish sometimes she would push me a little bit. Like, my pulmonologist is that way. I'll say, "I really don't like this new trach." And she's like, "Well, I'm sorry to hear that, but it's staying."

**Moderator:** Why do you wish your neurologist sometimes would be a little bit more assertive about recommending things?

**Respondent:** Because I think that she knows more than what I know, and I wish she would share more of that.

**Moderator:** It's like an interesting balance because on the one hand, you like that she respects that you're a smart adult who can make decisions. But on the other hand, you're like, "Dude, you know a lot. This is what you do full time for your job."

**Respondent:** Yeah. I know that she speaks in a lot of places, and so I know she knows a lot. I do wish she would push that envelope with me. Maybe she will now that I've had this major medical thing. Again, I was really healthy for really my whole life until this summer.

**Moderator:** It's striking to me that you said I mean, I'm hearing you a little bit, like, have some ambivalence. You're like, on the one hand, you're like, "I only wanna go on treatment if it's in conjunction with a muscle builder," but then you're also talking about, like, thinking about taking treatment just to stop progression. But then you're also saying, "I wanna see improvement."

**Respondent:** I think, really, for me, the muscle builder piece is really important to me because I think that's the see improvement piece. That ties with gaining a little bit of strength, seeing some improvements in my mobility. To me, that's what I was hoping for with Spinraza and Adversity, and I didn't see it. Part of my conversation would be I really need to see improvements. I'm a I have to see it person. So if there's a combination of things or if that muscle myostatin builder gets approved, I wanna try that.

**Moderator:** Why isn't stopping progression good enough?

**Respondent:** That's a good question. Because I was never convinced that I was actually stopping progression. It should be enough, but for me, it wasn't because I wasn't seeing it. I was still experiencing loss of strength. I was still experiencing that weight gain, and that was making it harder for me to do things. While the upper limb tests were pretty stagnant, they moved a little bit. The physical therapist will say, "Well, you went up a point in this area, but you went down a point in this area," so it sort of was a wash. I wasn't really seeing that significant indicator that there was slowing of progression because my upper limb test didn't really change from before I started the treatments to while I was on the treatments. They were really just the same, and I expected them to improve.

**Moderator:** Are you saying before you were on treatment, you also well, you're on Evrisky, but you saw us were even before you got on the Evrisky, were you stable?

**Respondent:** Yeah. I was already stable. So I think that for me and, again, I keep saying this, and I know it's redundant, but I really expected to either feel different or see something different, and I didn't have either. I didn't feel different. I didn't have any burst of energy. Like so even energy is not strength. But everyone talked about, "Oh, I have this boost of energy, you know, that's really great." I didn't have it. Maybe because I was so active, I was already pushing my body to limits. I don't know, but I just wanted more, and I just wasn't getting what I thought I should. I might have had greater expectations than I should have.

**Moderator:** But there's also no way, at least during the time of the medication, to say your body would have definitely done this if you weren't on the medication and your body did this. Right? Like, you can't do a control.

**Respondent:** The body did that. I still was experiencing some loss.

**Moderator:** With the weight gain and so you're losing strength. Right. Okay.

**Respondent:** I was still experiencing some loss of abilities or strength even though my upper limb tests were coming back stable. I'm like, how can it be stabilized? I can't. I now can't brush the back of my hair. I used to be able to do this, and now I can't do that.

**Moderator:** Your hair looks very brushed.

**Respondent:** I didn't brush it. My spouse did.

**Moderator:** The medical event you had this summer, what impact, if at all, do you think it had on this conversation we're having about your orientation towards treatment?

**Respondent:** Oh, a lot. It was very scary. I've never wanted a drink my whole life. I remember even my mom, you know, whenever I get sick, how careful we were because respiratory illness, of course, is the number one thing with people like myself. I've been sick. I get upper respiratory infections, but I've never been that sick. I've never been in ICU before. I think I said I never had a surgery. No surgery. I broke both my femurs in February, and I recovered from that. Now that was a huge loss of strength, but there was that event where I couldn't my legs were immobilized completely. Even though I used a wheelchair full time in February, I came back from that, and I gained my strength back. For me now and this event in July were I was very close to not making it. That has scared me because I always think of myself as invincible, and I was completely helpless. People will tell you if you ask any of my friends, I have this weird I don't really look at myself as disabled. It's weird because I use a wheelchair full time, and I need help doing things like getting dressed and showering. But I just don't see myself in that same light. When I describe myself to people, I don't really talk about the disability first. I talk about myself, and so it was really scary, and it still is. My future is really scary, and so I think I have a different perspective. What do I need to be focusing on to maintain my health? I've been thinking about it a lot the last couple of months. What do I need to be doing different, and should I go back on to Thursday? Do I need to try it again to help extend my life further? As my daughter, she was laying in bed with me the other night, and she's like, "Okay. So how old were you when Gigi died?" My mom. I said I was 58. She said, "Okay. So you have to live until I'm 58." I'm like, you're 22. It's like, that's a long time. She's like, "Yep. So you're gonna have to live in '96." I'm like, okay. Well, that's thirty-six more years. How do I do that? Right? Things like that when she says stuff or when I think about what I could miss if I can't go out of the house. I don't wanna be afraid to leave my house. What do I need to do? Because now SMA has really, excuse my language, fucking impacted me. With this breathing stuff, and now I wanna fight harder.

**Moderator:** I was talking to someone yesterday, and I might be this guy I'm not gonna use his name, and I might not be characterizing it a 100% correctly. But I think there was a way in which for this person that it was kind of a bummer for him to be on medication. Like, it somehow reinforced that this was a part of his identity. Right? That, like, I'm taking this medication, and it's reminding me that I have SMA, and I don't wanna think of myself that way.

**Respondent:** I share a lot of that sentiment. I lived my life before this, again, as if I didn't even have a disability. It was not the first thing I identified with myself. Now that I have this trach, that's how I feel. It's visible. Everybody sees it. I'm pretty vain. I don't like it. I try covering it with scarves. I'm afraid to go back to work because I don't want people to look at me differently. Right? SMA did that to me. This is all because of SMA and the anatomy that my throat and being able to intubate or not intubate. It has changed my future. I have to figure out how to do that and still be who I was. That means I gotta figure something out so that I'm one, that I can get beyond that this is who I am and that people aren't gonna see this. I know that that will be the case again, but, oh, man. It's a long ways from where I am right now.

**Moderator:** Thanks for sharing that. Do you think there's any kind of psychic downside to taking a daily medication for you for SMA?

**Respondent:** No. I don't think so. I was really excited when I started. I told everybody about it. Everyone knew, and everyone was so excited that there was this possible treatment. People are like, "Oh, I'm so glad there's a cure." I'm like, no. It's not a cure. It's a treatment, and it's different. I was really proud in the beginning to be on it. I really was. I was really proud to be on it, and I've always wanted to be part of some clinical trial. I always said, I'm like a perfect sample. I'm an old woman, older. We're living so much longer. Take this population of adults with us today and do something with us. But, yeah, I think it's not that I'm embarrassed of the disability. It's just it doesn't define me. I'm worried that now it does define me because of my new breathing situation.

**Moderator:** Let's imagine you decide to transition to treatment whether Ryzdalone or a Ryzdalene combination with this anti-myostatin or some other muscle builder. What would help you make that transition? I don't know if there's anything the manufacturer could do, if there's anything your doctors could do, if there's any peer talking to a peer about SMA and being on one of those medications.

**Respondent:** I think there are a couple of things because, again, I'm very data-oriented. That's what I do in my job. I would love to see more data on adults. I would love to have a conversation or some sort of group that is made up of adults talking about their experiences. I would love to see some of the marketing directed specifically for adults. There's a lot of data about people 18 and younger, and I would like to see more data related to those of us 30 and older.

**Moderator:** Do you feel like there's kind of a disregard or invisibility somehow with the people regarding?

**Respondent:** I think so. I felt sort of that we don't matter kind of experience from the Muscular Dystrophy Association. They do a lot of services, camps, and this and that for kids. They don't really have a lot in place for 21 and up. They used to provide assistance towards a new wheelchair. They only do that for kids. Even with the new drugs, the adult approval was secondary and took longer to get. It's like, okay, this drug's out, but I can't take it because I'm not 18. I think that there is a little bit of that, especially when you go to look at the data. I know that Resideo has done a specific trial with adults. Now that was early on, so they probably have a lot more data now, and I just haven't looked at it. I would like to see more data. Here's an example. This group that I'm on, Living with MD, one of the things that I had done earlier this year was a suprapubic catheter, which helps me urinate. It just makes it easier. It's not that I needed it, but it makes it easier. This group, first of all, lots of information in the group about people that have done it. But when they find out that there's a new person that's going through the procedure, they give you a buddy. So I had a buddy that was like, "Hey. Here are the supplies you might need. Here's what you might experience. Here are some questions you should ask." While that seems silly, it was great to have somebody that I could talk to. I think having that one-on-one relationship with somebody else, going through the same things that's close to my age, would be amazing. I don't know if it'd be impossible, but it would be amazing.

**Moderator:** Going through what would what in your case, going through a similar thing, what would that mean? What would that other person?

**Respondent:** For me, like, now, I would love to know how many people have trachs that are on the medication. What kinds of improvements to their respiratory function they're seeing. That's sort of changed my perspective, going through this. Also, the pros and cons. What are they feeling? I go to these groups and I'm like, well, who's experiencing this? It'd be great to have a group. I don't even know if there isn't a Frisbee group.

**Moderator:** A trach Evrisky adult group. Is that what they're doing?

**Respondent:** Like that. Or even just a plain, a RISD group.

**Moderator:** On Facebook, you mean?

**Respondent:** Yeah. I'm looking. Oh, yeah. Oh, there's only a 100 people in it.

**Moderator:** Does that seem big or small? I'm sorry. I'm not a.

**Respondent:** That's small. Oh, here's one with 6,000. RISD department, RISD information for SMA. That has six thousand members. Oh, I'm already in that group. That's hilarious. It must not show up on my feed.

**Moderator:** The settings are just such that it doesn't show up.

**Respondent:** They just don't show up. But, you know, so I'm on there now, and it's a lot of kids.

**Moderator:** You talk about data, and you just mentioned some like, what specific data would be and you've also talked about the fact that Aviso has been out longer. But so what data is of interest to you?

**Respondent:** For me, data that I want to see involve respiratory improvement function. What is the percentage of progression slowing or stopping? What do the age brackets look like that we're seeing that in? I'm in an age bracket now, which is amazing that I'm so old. What do they know about people above 50? Because I think when you lump adults, they could be all 25-year-olds or 30-year-olds. That's different than somebody that's in their fifties and now 60. I'd like more data that I can tease out that looks like me.

**Moderator:** It's so interesting. I don't know how I'm a qualitative research person, not a quantitative research person, but it's interesting, like, to what extent does data show the stopping of progression of disease. Does that only come from people who are on placebo who are not getting treated versus people who are getting treated?

**Respondent:** I don't know. I don't know what the answer is, but I know that the data that was out there again, this has been five years ago. It was very limited because they were just beginning. It might be interesting to see what their data says now that they've been out for five years, and they have a new way that you can take the medication.

**Moderator:** The tablet. Yeah. Is there anything else? It has been we have five more minutes technically, but this has been I know it's a lot a long time to talk to someone for seventy-five minutes.

**Respondent:** That's okay.

**Moderator:** It's been so enormously helpful. Just personally meaningful. So just from a selfish perspective, I've just really enjoyed our conversation.

**Respondent:** Thank you.

**Moderator:** Is there anything else you would want manufacturers of SMA treatments to know about you and how to make it easier for you to access treatment or to feel comfortable with treatment or just anything else that would be useful for you to share?

**Respondent:** Again, I think something that would be an improvement is, somewhat in their marketing materials. The way that it's marketed, I think that it's a different conversation when you're talking with a family with a 10-year-old than it is for adults. I think that kind of marketing and experience sharing would be really important. Now that they're five years old, right, like, they've been in the market for five years, really honing in on different populations. Because I think that, you know, just as anybody that's trying to sell me something or you, they're honing in on who we are and what our lives are like. I think I'm an anomaly. I work full time. I have a family. I'm super active. I'm not the only person with SMA in their upper years doing that, but there aren't a lot of us. I know that, so use me to learn as an anomaly. I've lived a very long time with this disease. I've known since I was two years old that I've had this disease. I think that really honing in on education towards people of an older age, you know, that above like, he's saying above 30, but even older and realizing, you know, that selling me on the possibilities, I think, is what they need to do. My life is, you know, not gonna be thirty-six more years, but it might be with the right treatments.

**Moderator:** Are you still like, you talked about that, like, through the pause like, the process of, like, getting at RISD to cover the cost of it, you were in touch with someone who, like, you talked to somebody from them from them from their from the manufacturer. Does that person still ever reach out to you?

**Respondent:** No. I know with SPINRAZA that it was called their program. They had a really good program. If RISD had something similar or whoever the manufacturer, I forget. But they just weren't as engaged with me. I know that I have compared it to the Spinraza program. That person contacted me all the time. Even after I've left, she called me, and we had a long conversation. Then she sent me an email even a few months after that. "How's it going on the New Drawing? I hope it's going well." I didn't get that experience with the InfraMD patient advocates or whatever they're called. That was another thing that could be improved is that advocacy through their company and that person checking in, you know, and serving as an actual advocate. I was not impressed with them. Because, really, that person should have tried to talk me into staying.

**Moderator:** Michelle, thank you so much. I feel so lucky that I got to talk to you.

**Respondent:** Well, thank you very much. I really appreciate the opportunity to share my opinions.

**Moderator:** It's so interesting. Like, for me, like, you just look like you're wearing, like, a hip necklace from here. I mean, granted, you're across the screen, but that's honestly what it looks like. It's like a little bit like punk rock or something.

**Respondent:** Well, I like hearing that because I need to know that it's really not that big of a deal. So thank you. That helps a lot.

**Moderator:** I mean, it's a transition. You know? Thank you for the work that you're doing too, in your job.

**Respondent:** Thank you.

**Moderator:** Let's go higher ed. Okay?

**Respondent:** Yeah.

**Moderator:** Alright. Thank you.

**Respondent:** Thank you.

**Moderator:** Take care so much.

**Respondent:** Okay. Bye.

**Moderator:** Bye.