**2025 SMA Adult Activation Qual Transcript**

*Oct 3, 2025 | 12:00 PM*

**Moderator:** So my name is Moderator. This is what I do for a living. My nose is itching. I apologize. I don't know what it's let me just try to get that over with so I don't do that the whole time. It's Friday afternoon. It's officially, like, time that I get punchy, so I apologize. Okay. So my job today is to ask you a lot of questions about SMA,

**Respondent:** Mhmm.

**Moderator:** 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. Yeah, none of us know who you are, how to reach you, or anything like that, so blah blah blah. Also, we are recording. It's just for research purposes. Your name doesn't get attached to that just, you know, in case we have a call, if you wanted to hear what you have to say. To I guess one last thing. 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 sounds like you've probably done this before. I have to write up a report called an adverse event report, and then I would ask you some questions about it. Blah blah blah. I'm just gonna say blah blah blah after my whole intro. Are you good with all that?

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

**Moderator:** Awesome. Can you just tell me a little bit about I don't know anything about you, like, how old you are, if you live with anybody, how you.Spend your days. Yeah.

**Respondent:** So I am 60 years old, and I live with my spouse of thirty four years. And 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.

**Moderator:** Oh.

**Respondent:** I oversee a team of researchers in the fundraising arm of the university and I have been doing that for I've been at this university for almost thirteen years and I was at a university, 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 and bing bang bang, 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 I see it as purple. Am I seeing that correctly?

**Respondent:** The the speaking valve is purple.

**Respondent:** In order to speak, you have to have either a cap, which I have not graduated to using cap, or a speaking valve. So my speaking valve is purple. I have another one that's green. But,

**Moderator:** Okay.

**Respondent:** Yes, it is purple. We're just favorite color, so that was a good thing.

**Moderator:** Okay. Well, we that is a a remarkable story that you just told. Rest you used the word respiratory failure, which is a dramatic were you intubated?

**Respondent:** They could not intubate. They tried three times in.

**Moderator:** Wow.

**Respondent:** The ICU, and then they had to perform an emergency cric, which is a trach, but not permanently placed and very I was sedated for several days. It was very scary for my family, for me when I came out of the sedation. And then a few days after that emergency trike, they went I went into surgery and they put in the permanent trach.

**Moderator:** Wow.

**Respondent:** And I was in the hospital for three and a half weeks.

**Moderator:** Wow.

**Respondent:** I've been home since early August, and two weeks ago, I was back in the hospital, to have my gallbladder removed.

**Moderator:** Wow.

**Respondent:** 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, I mean, other than my spinal muscular atrophy, but so I consider that a win and so do the pulmonologist that I see. So.

**Moderator:** I'm just I'm just letting it all absorb everything. You just .. You're set present as an incredibly joyous human being. Like.

**Respondent:** Thank you. I really am trying, and, I'm hope I'm on short term disability from my job right now,

**Moderator:** Okay.

**Respondent:** 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:** And boy, it's a time where universities need you to be doing some fundraising.

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

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

**Respondent:** Okay. That's accurate. When I was diagnosed, there were no types. And, actually, they, at the time in 1967 when I was diagnosed at.

**Respondent:** Three years old,

**Respondent:** 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:** How this could be a very, very long question, and I have given that I have so many questions. Before this the crisis in July, the medical emergency, how was how would you describe your SMA, like, had it been and the arc of the SMA up until that point?

**Respondent:** Sure. So 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. Hasn't stopped me from doing things, but certainly more weakness in the last ten to fifteen years. I think going back, I walked, until I was 12 or 13. I transitioned into a wheelchair in late middle school.

**Moderator:** Wow.

**Respondent:** I drove, really up until fifteen years ago and then I 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 all kinds of residents when I would go to to clinic, you know, and they would say You know, it it I felt like a monkey sometimes because they were 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.

**Moderator:** Wow.

**Respondent:** So I used a manual wheelchair propelled myself and then, you know, as as it does for all of us as we age, and I'm at a desk job, you lose things that you wish you had lost.

**Moderator:** Yeah.

**Respondent:** And, youKnow, gaining weight, that's been a struggle, my whole life. I my SMA is that when you think about SMA, I always feel like there's, like, two body types with SMA. There are those that are skin and bones and weigh, you know, barely eighty pounds, and then there are those of us like me that are on the very heavy side. And no matter what we do. I I have what I call an SMA belly. It's just very rounded. So I think having the weight, though, on me has made it easier for me maybe because I don't I don't know. That's kind of a weird thing to say, but It's made me more stable. You know? I'm able to Sit up. I've been able to do a lot more things because I had more muscle mass.

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

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

**Moderator:** Wait.

**Respondent:** So I.

**Moderator:** And what what kind of summer camp?

**Respondent:** So the Muscular Dystrophy Association runs summer camps.

**Moderator:** Okay.

**Respondent:** I started going when I was five, and I went every summer until I was, like, 22.

**Moderator:** Wow.

**Respondent:** And so I have a real connection. Like, I have very good friends that I met at summer camp, then we're still friends, you know, thirty years later.

**Moderator:** And are they was there, like, an SMA subgroup in the muscular dystrophy?

**Respondent:** No. I mean, the camp was for anybody with any of the neuromuscular diseases. SMA, you know, is a pretty broad population that was at the camp. I would say, probably a good thirty, forty percent of the kids at camp had SMA,

**Moderator:** Wow. Wow.

**Respondent:** But it was a mix of all the different diseases of that umbrella.

**Moderator:** Fascinating. Are you, like do you get any emails from, like, I don't know, people I've heard of, like, SMA Connect, and some people get, like, an SMA magazine, and some people.

**Respondent:** Oh,

**Moderator:** I don't know.

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

**Moderator:** As, like, your advocate kinda.

**Respondent:** Yeah.

**Respondent:** She's great. She lives in well, she lives out of state. We don't live near each other but ive seen her a lot because she has been flying in multiple times this summer.

**Moderator:** Oh, that’s so sweet.

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

**Moderator:** Okay. And I will get toThat. Yeah. Let me just put that side, because that's part of my endless list of questions For you. But Just just because I'm curious, which groups do which SMA specific groups do you belong to on Facebook? You mentioned the trach, which mayNot be specific to SMA.

**Respondent:** So there is a living with MD group, or the majority of people on that list is SMA. Of course, Cure SMA. What's the other ones?

**Moderator:** So I can it's I can never remember what I had for breakfast, so it's like.

**Respondent:** I know.

**Moderator:** Yeah.

**Respondent:** There's a number of them. Hold on. Can I look on my Facebook page?

**Moderator:** Yeah. Sure. Sure. Sure.

**Respondent:** Yeah. There's a number of them. The Living with MD and the Cure SMA are the two biggest groups that I interact with.

**Moderator:** Okay. That's that's what's most helpful.

**Respondent:** Is that good? Yeah. I I.

**Moderator:** So, you mentioned your neurologist. Who.

**Respondent:** Up.

**Moderator:** What health care providers do you talk to about SMA or or at all any way a part of your SMA care?

**Respondent:** Mhmm.

**Respondent:** Primary care physician, is often my starting point just because, she's close by. My neurologist is an hour away.

**Moderator:** Wow.

**Respondent:** And, my neurologist, of course, and recently, pulmonologist.

**Moderator:** Okay.

**Respondent:** 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:** Okay. What's how would you first of all, what type of medical environment are they involved? Like, are.

**Respondent:** They're,

**Moderator:** They yeah.

**Respondent:** They're at a without telling you, they're at a university based medical environment.

**Respondent:** Environment. Does that help? Yeah.

**Moderator:** Very much. Are they at, like, any kind of SMA center of excellence or anything.

**Respondent:** Yes.

**Moderator:** Like that?

**Respondent:** They're a center of excellence.

**Moderator:** Okay. Great. These are all great. So you see your neurologist once a year.

**Respondent:** Yes.

**Moderator:** What's your relationship like with that neurologist?

**Respondent:** I love her. She's amazing. Very knowledgeable. She she mostly I think a lot of the physicians deal, 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. But 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, but she's I really trust her. She's really great.

**Moderator:** How much when you think about the dynamic with her, how much of it is and there this may not be a fair dichotomy. But how much of it is, like, her saying, this is what I think you should do, Respondent. This is what's happening versus you saying, I've read about this. What do you think about this? I really I'm advocating myself to get that. Yeah.

**Respondent:** I probably more Bring up the ideas and say, oh, I read about this. What do you know about it? Do you think it would help me?

**Moderator:** Okay.

**Respondent:** And so, you know, 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:** Okay. Brilliant. And.

**Moderator:** Okay. And what role does your PCP play in terms of your SMA, just out of curiosity?

**Respondent:** It's really mostly, she often will help if I need specific equipment, You know, because today, you have to have a script for everything,

**Moderator:** Yeah. It's interesting.

**Respondent:** Including, you know, a shower chair or whatever. She also is, you know, like, she's the point person for me now, as I as I deal with, like, things with my employer and this new trach and things like that. She consults with the pulmonologist because they're in the same umbrella practice. But, you know, that's where I go to when I'm sick. So she has to know how to treat, you know, in conjunction with, hey. I gotta keep in mind this. I may so for instance, the hospitalist, when I was in the hospital, 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 you know kind of 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. So she's.

**Moderator:** Wow.

**Respondent:** She's been good too.

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

**Respondent:** Well, she didn't in the beginning. So I'm I'm a very good educator, and then I mean, 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.

**Moderator:** Wow. Wow. Okay.

**Respondent:** 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 they might have heard about it, but it's not their thing. But you should at least go Google it and be a little bit smart about it.

**Moderator:** Yeah.

**Moderator:** Again, your your attitude is impressive. Okay. I wanna talk transition to talking about treatment.

**Respondent:** K.

**Moderator:** 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:** No.

**Respondent:** So I mightHave filled that out wrong. I start I started with SPINRAZA because it was the only treatment that was out there and was finally approved for adults. And I started it in March 2020. At the height of the pandemic, it was a joy to go for treatments. And I did it until Evrytesse was released later that fall. And I asked my neurologist. I'm like, 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. I I just it interrupted my life too much. And then 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. And so I when when my sister called me and said, if EVRYSDI is released, you need to talk to your neurologist. I call I called her and I said, I wanna switch. How do I do it? Because I'm like, I can do it at home, and I wanna I wanna switch. And she said, okay. Let's do it. So.

**Moderator:** Okay. Can I pause you can I pause you for a second? Because you've already said so many interesting things. So, how did the besides all those downsides to SPINRAZA, right, like Painful injection, schlepping to the hospital during the joyous time of COVID, immobilized this hideous spinal headache. 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:** That sounds fun. You only had downsides. Great.

**Respondent:** Yeah. And I kept you know, I was like, should I stay on it longer? Because so many people were like, I can tell when it's time for my injection because.

**Moderator:** Yeah.

**Respondent:** I'm feeling weaker. I feel stronger after I never felt stronger. I didn't feel anything different in between the injections.

**Moderator:** So interesting. What this may seem like an obvious question, but what motivated you to go on Spinraza in the first place? What were you hoping to achieve?

**Respondent:** So I think for me, anything that would stop the progression, that's that was my number one. Stop the progression. I would have loved to have seen some strength increases, like Some noticeable things change in my world, and I just didn't.

**Moderator:** Yeah. Yes.

**Respondent:** But my hope going on it I mean, I was super excited. I absolutely was gonna try it. There was there was no doubt in my mind in the beginning. It was like, yes. It's now approved. And I had a tough time getting approved, and my Insurance never approved it. And I ended up going through the Spinraza Foundation or through the Powell program, and they paid for it. Spinraza's who's the main company?

**Moderator:** I don't remember.

**Respondent:** Don't remember either, but that company offered, you know, programs to help financially. I I mean, I had good insurance, and the frustration, it took several months to even get to the start of it. Like, I probably should have started Spinraza in December of of nineteen or at least January.

**Moderator:** Yeah. Because I think it had been out for a yeah.

**Respondent:** Had been there for a number of years, but it wasn't approved for adults. IWas waiting for it to get approved for adults.

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

**Respondent:** Yeah. A lot of it was at 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 a benefit

**Moderator:** Okay. But you really it's but it's noteworthy. You obviously advocated very hard for yourself and found a way….

**Moderator:** And you're like, I don't have to go into the hospital [for Evrysdi] so let me Call my neurologist. Okay. And so when. Did you started that in the 2020?

**Respondent:** I started in September.

**Moderator:** Okay.

**Respondent:** Of twenty twenty, and then I was on it until, like, February '22.

**Moderator:** Oh, wow. So a year and a half.

**Respondent:** Yeah. I stayed on it. And 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. And so whether that was tied to the Evrysdi or not, I it was correlation for me that it was.

**Moderator:** Yeah.

**Respondent:** Connected. And after talking with my doctor, my neurologist, I just said, you know, 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 they do. They were pretty much the same. They weren't increasing. I I would it was just pretty much stable. I'm like, so in in my thought process, I should have had improvements because everybody was seeing improvements. And 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. And it was my decision to do it. And, I mean, mine and, you know, I discussed with my family. And.

**Moderator:** Yeah.

**Respondent:** Everyone was a little leery, like, are you sure you're gonna go off it? You know, we don't know if it's it's stopping the progression. You know? I mean, that upper limb, you know, 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.

**Respondent:** Yeah. And I'm losing strength. Of course, this summer, being in the hospital, I've lost a lot of strength that I'm trying to regain.

**Moderator:** You spoke to my specific question because I I even, like, highlighted when you talked about a EVRYSDI, you said, should I be seeing improvement? And this is not meant to catch you in an thing. That's exactly what you were talking about. And but when I asked you earlier why you went on treatment, you said I went on treatment to stop progression. You said,

**Respondent:** Yeah.

**Moderator:** You know, I would love to see strength improvements. Can we list all the factors that led you to stop Evrysdi? So the things you've mentioned so far are weight gain,

**Respondent:** Mhmm.

**Moderator:** The sense that you couldn't, like, see improvement. Right?

**Respondent:** Yeah. Right.

**Moderator:** What other reasons do you think led you to stop?

**Respondent:** You know, I think the portability, like, so traveling was super challenging.

**Moderator:** And you just talked about a cruise. Right? Yeah.

**Respondent:** Right.

**Moderator:** Yeah.

**Respondent:** 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, you know, all of that. So that was something. I think, again, I wasn't approved through my insurance.

**Moderator:** Oh, you weren't?

**Respondent:** So no. And so that was all being paid for through the program.

**Moderator:** Wow.

**Respondent:** I had to jump through a lot of hoops, you know, periodically to get reapproved for that.

**Moderator:** I'm gonna come back and ask you about that.

**Respondent:** Yeah.

**Moderator:** Okay?

**Respondent:** And I think every time it was delivered, I was like, oh my god. Don't break anything. That's $38,000 in medication in there. So I just I think, you know, 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 pretty good. And then just, you know, my wish of wanting to see more in my body, and I just wasn't.

**Moderator:** Yeah.

**Respondent:** And so I just wasn't sure it was worth it.

**Respondent:** I think 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. And 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:** I really I really, really appreciate everything you just laid out and because, right, sometimes it's not just one factor. There are lots of different factors, all Those things. I I sometimes I just ask stupid questions. So with the weight gain, how significant was that in terms of your decision to stop? Like, it's not like we're, like, I'm asking you to quantify percentage weight wise, but, I gained 30 pounds

**Respondent:** That's a lot for someone with SMA. And so have I lost that 30 pounds? No. I've lost some, but I haven't lost that whole 30. But it to me, it just correlated with, hey. I started gaining weight when I started taking a freezed eat.

**Moderator:** Yeah.

**Respondent:** Now they say weight I think I did another study and they were like, well, weight gain is not one of the side effects. I'm like, maybe it's not, but that's what I experienced.

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

**Respondent:** Oh, a lot. I mean, just fitting into my clothes and feeling confident and, extra weight makes it harder to do things. So 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. Like, okay. This I shouldn't have to do this. And so 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 that big of a deal, but somebody with SMA is that little incremental loss of things.

**Moderator:** Yeah. You talked about portability. Anything else I should understand about the impact of the, you know, the liquid and needing to be refrigerated that you didn't mention? Just. I don't think so. Just the refrigeration and then, you know, just the regimen of having to remember to take something every single day.

**Moderator:** Well okay. Here. More of my dumb question. What's what's the downside of having to remember to take something every.

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

**Moderator:** Really? Wow.

**Respondent:** No. And so it's like, oh, I don't know. My spouse is really good at it. Thank goodness. She'd be like, hey. You know, it's seven. We have to take it's time to take a freeze. I'm like, oh, yeah. Right. Let's do that. So I I just I don't know. You know, when you think about, it's really shouldn't have been that big of a deal, but it just it was.

**Moderator:** This obviously, you know that I'm .. It's not a judgment issue for me.

**Respondent:** Oh,

**Moderator:** This is interesting.

**Moderator:** You talk yeah. No. I mean, I understand. And then 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?

**Moderator:** That's fine. You no. It's no problem because it it was, like, funny how it happened for both of us the exact same time. It's really not supposed to happen for me. We were talking about the hoops that you had to go through to get a EVRYSD approved.

**Respondent:** Both of Evrysdi and the Spinraza people, super nice, really great people. They wanted to do whatever, you know, to try to get it covered, but, you know, you had to be reapproved every so often, and I think it was different for both of them. I can't quite remember. And I always felt like I have really good insurance. Like, I'm taking this drug from somebody else that doesn't have insurance. And I was so mad at my insurance. I mean, like, we went through I don't even know how many appeal processes. And then finally, you know, I so I think part of it too is that self guilt of I have really good insurance, and why aren't they I think, paying for this? And then.

**Respondent:** I'd see it online, but everyone's like, oh, yeah. I went through my insurance. No problem. I went through my insurance. No problem. Like, what is wrong with my insurance?

**Moderator:** What do you mean self guilt? What is that?

**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.

**Respondent:** I mean, I make a good salary, and I have good insurance. And, you know, like, I don't qualify for disability programs in general. Like, my caregivers paid out of my pocket because I chose to work, and I also choose promotions when I get to.

**Moderator:** Yeah.

**Respondent:** So,

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

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

**Moderator:** Yeah. Like, I wonder.

**Respondent:** So it involved a phone call a Couple of times a year probably. I can't remember if I had to provide financials. I probably did.

**Moderator:** Okay. Okay.

**Respondent:** You know, I just remember that it took a while and, again, multiple appeal processes. And, you know, I accepted it because that's the only way I could get the drug. I certainly couldn't afford the drug out of pocket.

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

**Respondent:** No.

**Moderator:** To insurance?

**Respondent:** To the insurance.

**Moderator:** I see. Okay.

**Respondent:** No. Every day, there was never an appeal process. Again, they. They wanted me to be on the medication if that's what I wanted, and so they were very helpful in trying trying to get that done.

**Moderator:** It's also really interesting what you said about the the chatter online that there was sort of a trend of people talking about.

**Respondent:** Yeah. Their insurance covered it.

**Moderator:** Oh, not that. That that is also interesting. But also you mentioned that, like, in some of the Facebook groups, you were like that people were talking about how it made sense that it was making an impactOn 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. It's just it's I don't see as much chatter about it anymore. I know Of a number of people that also dropped it. Many that 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 that quit taking it.

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

**Respondent:** 22.

**Moderator:** Okay. So that's been three and a half years. I I wanna ask you I wanna understand how you're feeling about treatment now.

**Respondent:** Mhmm.

**Moderator:** Because I but I also wanna understand how you've and we have twenty two minutes. So I'm just mindful of time.

**Moderator:** Obviously, you had you alluded to having stuff happen over the summer and that, like and I'm right. And we'll talk about that and but up until what happened over the summer,

**Respondent:** Mhmm.

**Moderator:** 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.

**Moderator:** Okay.

**Respondent:** I it's been back and forth in my mind. Like, should I go back? Should I not? You know, I've I've 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. You know, for me I mean, this sounds terrible. I wanna se some increase

**Respondent:** I wanna see improvements even if they're incremental, you know, little. So for instance, since my hospitalization in July, I basically cannot lift my left arm. I can 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. And so, again, my 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.

**Moderator:** Yeah.

**Respondent:** I think it's the myostatin. Yeah. And Scholar Rock, I think, is the one. But, you know, she she was just here, a couple weeks ago for my surgery. And, you know, she spends the night with me at the hospital, and we often will talk until very late. And we spend a lot of time talking about, well, maybe now is the time to rethink going back on something. You know, that I've had this trauma, and maybe my body would respond differently now. You know, it was quite a scare this summer. And I think, you know, from her perspective, my wife, my daughter, they want me to be here for a lot longer. And so 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.

**Moderator:** Really?

**Respondent:** And so I don't wanna stop living, so how do I, like, how do I try to improve the quality and the length of my life? And it might involve going back on some sort of treatment to really slow that progression. Because it feels like the progression is accelerating the the older I get.

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

**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 EVRYSDI, 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, you know, 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 I'm I'm more curious now about restarting something or starting something new. I think the other, thing that's 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 or Infrisbee. You know, that there's just and there's more knowledge about I think there's probably more data. I'm a data person.

**Moderator:** Yeah. Mhmm.

**Respondent:** So there may be more data to say, hey. Look. This is what we've seen in adults,

**Moderator:** Mhmm.

**Respondent:** And that might help convince me too.

**Moderator:** Okay. So it's so many interesting things. I know I'm a breeding a dead horse. So have you talked to your neurologist at all about what did your neurologist say? I know you talked about this a little bit. What did your neurologist say when you stopped the treatment? Did she say it was up to you?

**Respondent:** She was supportive

**Respondent:** Yeah. She was supportive. She's like, listen. You know, 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.

**Moderator:** Mhmm.

**Respondent:** 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 EbEvrysdiee soon after I quit to, like, 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:** Keep going.

**Respondent:** Am I haven't had any discussions with her this year. I had an appointment with her, but I was in the hospital. So she and I are scheduled to meet in November. And I think in the when we do have that, in person meeting again, that that's where we would I think part of my agenda is to discuss that. Like, what's Available? What do you think? What should I do?

**Moderator:** Yeah. Had you and your doctor talked about going back on treatment since February '22? No.

**Respondent:** No.

**Moderator:** Never she never brought it up again.

**Respondent:** No.

**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. Like, you Know, I feel like I'm always the one saying, hey. What about this? And 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, like, I wish sometimes she would push me a little bit. Like, my Pulmonologist is that way. Like, 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 recommend recommending.

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

**Moderator:** So interesting. Yeah. 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 you job

**Respondent:** Yeah.

**Respondent:** And I know that she speaks in a lot of places, and so I know she knows a lot. And I do wish she would push that envelope with me. And 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:** Yeah.

**Moderator:** It's striking to me that you said I mean, I'm hearing you a little bit, like, have some ambivalence. Right?

**Respondent:** Yeah.

**Moderator:** Like, you're like, on the one hand, you're like, I only wanna go on treatment if it's a 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.

**Moderator:** Yeah.

**Respondent:** I think, really, for me, the muscle builder piece is really important to me because I think.

**Moderator:** Yeah. Why?

**Respondent:** That's the see improvement piece. You know, that ties with gaining a little bit 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. And so part of my conversation would be I really need to see improvements. I'm a I have to see it person. And so if there's a combination of things or if that muscle myastatin builder gets approved, I wanna try that

**Moderator:** My I am tempted to ask why isn't and this is not a judgment question, but why isn't stopping progression good enough?

**Respondent:** That's a good question.

**Moderator:** I hope that doesn't that I hope that doesn't feel like a judgy question. This.

**Respondent:** Because I was never convinced that I was actually stopping progression. Yeah. It should be enough, but I and for me, it wasn't because I couldn’t see it.

**Moderator:** Yeah. Can you say a little bit more about that, how you can't see stop and progression? I think it makes intuitive sense, but I just wanna make sure I understand.

**Respondent:** Yeah. For me, I wasn't 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.

**Moderator:** Oh, yeah.

**Respondent:** And while the upper limb tests were pretty stagnant, they moved a little bit. Like, the physical therapist will say, well, you you went up a point in this area, but you went down a point in this area, so it sort of was a wash. And so 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:** Because 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. And maybe Because I was So active, I was already pushing my body to limits. And I don't know, but I just Yeah, it was really I wanted more, and I just wasn't getting what I thought I should. And I might have Greater expectations than I should have.

**Moderator:** Yeah.

**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.

**Respondent:** Yeah. In general the body did that .. I was experiencing some loss .. 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, or **:** I used to be able to do this, and now I can't do that.

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

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

**Moderator:** Sorry. The medical event you had this summer,

**Respondent:** Mhmm.

**Moderator:** 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.

**Moderator:** Sorry.

**Respondent:** 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 with people like myself. And while I I mean, I've been sick. You know, 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 you know, my legs were immobilized completely. And even though I used a wheelchair full time in February, I came back from that, and I gained my strength back. So for me now and this event in July were you know, I was very close to not making it. And I think that has scared me because I always think of myself as invincible, and I was completely helpless. And 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, you know, full time, and I need help doing things like getting dressed and showering. But I just don't see myself in that same light. Like, I just don't like, 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. You know, my future is really scary, and so I think I have a different perspective. And what do I need to be focusing on to maintain my health? And so I've been thinking about 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. And she's like, yep. So you're gonna have to live in '96. And I'm like, okay. Well, that's thirty six more years. How do I do that? Right? So, you know, 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. And so what do I need to do? Because now SMA has really, excuse my language, fucking impacted me.

**Moderator:** Yeah. Yeah.

**Respondent:** With this breathing stuff, and now I wanna fight harder.

**Moderator:** Yeah. I was talking to someone yesterday, and I might be this guy I'm not 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.

**Respondent:** Yeah. I share a lot of that sentiment. I I lived my life before this,

**Moderator:** Way.

**Respondent:** Again, as if I didn't even have a disability. It was not the first thing I identified with myself. And 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. You know? Like, I'm afraid to go back to work because I don't want people to look at me differently. Right? Like and 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. And it's it has changed my future. And I have to figure out how to do that and still be who I was. Yeah. And so 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. You know? Like, 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.

**Respondent:** Yeah.

**Moderator:** Do you think, and please tell me if there isn't because I don't wanna presume anything about how you feel. Do you think there's any kind of, like, down psychic downside to taking a daily medication for you for SMA? Like, you know.

**Respondent:** No. I don't think.

**Moderator:** Okay.

**Respondent:** So.

**Moderator:** Okay.

**Respondent:** No. I was really excited when I started. mean,

**Moderator:** Yeah.

**Respondent:** I told everybody about it. Oh, that's how much my medication is. Everyone knew, and everyone was so excited that there was, you know, this possible treatment. People are like, oh, I'm so glad there's a cure. I'm like, no. It's not a cure. You know? It's a treatment, and it's it's different. You know? So I I was really proud in the beginning to be on it.

**Moderator:** Okay. That's helpful

**Respondent:** 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. You know? We're living so much longer. Like, 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. And I'm worried that now it does define me because of my new breathing

**Moderator:** Let's decide you 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 any of your doctors could do, if there's any like, a peer talking to a peer about SMA and being on one of those

**Respondent:** There are a couple of things because, again, I'm a very data oriented. I mean, that's what I do in my job.

**Moderator:** You've mentioned that. Yeah.

**Respondent:** But I think, for me, I would I would love to see more data on adults.

**Moderator:** Mhmm.

**Respondent:** I would love to have a conversation or some sort of, you know, 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.

**Moderator:** Yeah.

**Respondent:** 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 a disregard or, like, an invisibility somehow with the people.

**Respondent:** Think so. I think, you know, here's the thing. Like, I felt sort of that we don't matter kind of ex you know, experience from the Muscular Dystrophy Association. You know, they do a lot of services, camps and this and that for kids. And they don't really have a lot in place for 21 and up. You know, they used to provide assistance towards a new wheelchair. They only do that for kids. And then even with.

**Moderator:** Yeah.

**Respondent:** The new drugs, you know, the the adult approval was secondary and took longer To get. And it's like, okay. This Drug's out, but I can't take it because I'm not 18

**Moderator:** Yeah.

**Respondent:** I think that there is a little bit of that, especially when you go to look at the data. And I know that Resideo has done a a specific trial, oh, I forgot the name of it, with adults. Now that was early on, so they probably have a lot more data now, and I just haven't looked at it. But I would like to see more data, and I would like so here's an example. So 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, you know, 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. And so while that seems silly, it was great To have somebody.

**Moderator:** It's awesome. Doesn’t seem silly.

**Respondent:** And so 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:** And that going through what would what in your case, going through a similar thing, what would that mean? What would 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.

**Moderator:** Oh, interesting.

**Respondent:** And.

**Moderator:** Uh-huh.

**Respondent:** What kinds of improvements to their respiratory function they're seeing.

**Moderator:** Okay.

**Respondent:** Right? So that's sort of changed my perspective, going through this. Also, you know, the the pros and cons. What are they feeling? Do they, again, I I go to these groups and I'm like, well, who's experiencing this? So it'd be great to have a group. Like, I don't even know if there isn't a Frisbee group.

**Moderator:** A trach Evrysdi adult group.

**Respondent:** Yeah.

**Moderator:** Is that what they are doing?

**Respondent:** Something.

**Moderator:** What they're doing?

**Respondent:** Like that. Or even just a plain, a EVRYSDI 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 mean does that seem big or small? I'm sorry.

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

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

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

**Moderator:** It's Interesting you talk about data, and you just mentioned some like, what specific data would be and you've also talked about the fact that Evrysdi has been out longer.

**Moderator:** But so what data is of interest to you?

**Respondent:** So I think for me, you know, data that I want to see involve, like, respiratory improvement function. What is the percentage of progression slowing or stopping? What is what is the age brackets look like that we're seeing that in? So I'm in an age bracket now, which is amazing that I'm so old. What do they know about people above 50? You know?

**Moderator:** Yeah.

**Respondent:** 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. So I think I'd like more data that I can tease out that looks like me.

**Moderator:** Yeah. It's so interesting. I don't know how I'm 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.

**Respondent:** Yeah.

**Moderator:** Does that only come from people who are on placebo who are not getting treated Versus People who are getting treated? Yeah.

**Respondent:** Yeah. Well, because I think I don't know because I 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. There it was very limited because they were just beginning. So 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.

**Respondent:** Mhmm.

**Moderator:** 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.

**Moderator:** Yeah. And 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:** I've again, I think, something that would be an improvement is, somewhat in their marketing materials. You know, 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. And so I think that kind of marketing and experience sharing would be really important. Now that they're five years old, right, like, they've got they've been in the market for five years, really honing in on different populations,

**Moderator:** Yep. Yep.

**Respondent:** Because I think that, you know, just as anybody that's trying to sell me something or you,

**Moderator:** Yeah.

**Respondent:** You know, they're they're honing in on who we are.

**Moderator:** Yeah.

**Respondent:** And what our lives are like. And 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.

**Moderator:** Yeah.

**Respondent:** And 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.

**Moderator:** Yeah.

**Respondent:** I was two years old that I've had this disease. And I just 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.

**Moderator:** Yeah.

**Respondent:** Realizing, you know, that selling me on the possibilities, I think, is what they need to do. I know.

**Moderator:** Are.

**Respondent:** My.

**Moderator:** You.

**Respondent:** Life is, you know, not gonna be thirty six four years, but it might be with the right treatments.

**Moderator:** Yeah. Are you still like, you talked about that, like, through the pause like, the process of, like, getting at EVRYSDI to cover the 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, or is that.

**Respondent:** No. I know with SPINRAZA that it was called their program. They had a really good program. If EVRYSDI 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. And 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. And so that was another thing that could be improved is is that advocacy through their company and that person checking in, you know, and serving as an actual advocate.

**Moderator:** Yep.

**Respondent:** I because I was not impressed with with them. Because, really, that person should have tried to talk me into steak.

**Moderator:** Respondent, 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:** Yeah. And it's so interesting. Like, for me, like, you just look like you're wearing, like, a hip necklace from here.

**Respondent:** Thank you.

**Respondent:** Okay. Bye.

**Moderator:** Bye.