**2025 SMA Adult Activation Qual test**

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**Moderator:** Anyways, thank you so much for joining, Shelby. I really appreciate it. Let me just give you a quick intro before we begin. So my name is Nancy. This is what I do for a living. I feel actually so lucky that I get to do this. I get to meet people and ask you lots of nosy questions, and then you never have to see me ever again. So that's the good news for you. Today, I have been asked to ask you lots of questions about your SMA journey and about treatment. I am not an expert in SMA. I'm just a person who asks people about SMA, so you will know much more about it than I will. Part of my job is to protect your confidentiality, Shelby. I did not know your first name. Hold on. I'm just having a technical difficulty. Sorry. I'm having a person who can't see the screen. Mohammed, I think anyways. So what I'm also I meant to tell you so protect your confidentiality. So but I also do have a couple colleagues who are listening in. They one of them can't see you right now. That's what the message is I got. They have no idea who you are, and none of us know how to contact you or anything like that. We are recording. It's just for research purposes. And if by any chance you mentioned that you've had a less than ideal experience on a medication my client makes, then I may ask to ask you a few additional questions about that because they ask us we're required to write up a report called an adverse event report if ever, yes, to the manufacturer and the government is aware of that. Finally, I know they asked you to spend an extra fifteen minutes with me, which was nice. And I know it's you're like, wow. That's a wicked long interview. We may not need it. And despite that, sometimes I still do need to interrupt folks just to keep us moving because I do have so many questions. Are you good with all that, Shelby?

**Respondent:** Sounds great.

**Moderator:** Speaking of your background, I would love to know how old you are, if you live with anybody, how you spend your days if you're working now or used to work, or just so I have a sense of who you are.

**Respondent:** So I am 33. I grew up in Seattle. And then after I graduated high school, I moved to Utah to go to college. And so I am and then I wasn't going to live in Utah, and here I am fifteen years later, still in Utah. I graduated in public relations and then worked at the local news station here, the NBC affiliate here, for a few years as a producer. And then I moved over to doing social media for a health provider here. I just recently got laid off, which is a bummer. So I'm job hunting right now. And then so if you know anything, feel free to you can contact me with that. But I do work on the side doing a lot of freelance stuff for political candidates here. So that's kinda keeping me afloat at the moment.

**Moderator:** Awesome. What kind of work do you do for political?

**Respondent:** So, like, social media and comms stuff.

**Moderator:** That's awesome. Well, it's all that's what it's all about these days. I live in New York City, and the mayoral candidate who's in front has definitely mastered social media.

**Respondent:** Oh my gosh. She's doing so good.

**Moderator:** So do I see there's a furry person in your furry animal. Yeah. But are do is it just you and that furry friend in your house, or you live with anyone?

**Respondent:** So I have a roommate, and that is actually her dog. But, yeah, I bought my house a couple years ago. I had a house here in Salt Lake, and it's my favorite. I love it. It's a very cute little 1955 ranch house, and I'm obsessed with it. And then, yeah. And I have roommates, and my family still lives in Seattle, but I feel a pretty good community here. So.

**Moderator:** That's awesome. So how tell me about your SMA in your life right now. How is your health? How has it been yeah. How is it have there been any changes over the last couple years?

**Respondent:** Yeah. Definitely, I can tell that it's progressing a bit. I'm definitely having some more weakness in my arms. But it's so gradual that it's usually just kind of like a you just adapt as it's happening. But I've been really healthy. I wear a BiPAP at night, and then I have a cough assist machine that I use if I'm sick, but I don't really need it. And then I was on Avirizdi when it was first released, and it just felt like more of a hassle than it was worth trying to get it and order it every month. And my insurance didn't cover it, so I was through the foundation. And then I wasn't gonna be covered through the foundation anymore. So it was just kind of one of those, like, it's not worth it. But I am interested in getting back on it now that they have the tablet form that will also just make it easier to take. But, yeah, that's the only thing I've done.

**Moderator:** Okay. You said so many important things that I really want to ask you tons and tons of questions about. Just before I go there, talk to me a little bit about to what extent do you feel connected to any sort of SMA community, whether in person or online?

**Respondent:** So I have a few friends that have SMA that I met as a kid at MDA camps. And then I'm in some Facebook groups. If we're being perfectly honest, it's mostly for entertainment.

**Moderator:** What do you mean?

**Respondent:** I don't often get a lot of great advice out of them. Just sometimes people have stupid questions because they do exist. There is such a thing. And there's just, like, sometimes, like, drama and stuff, and it's just kind of fun. It's like your small town or your, like, town Facebook group, then you're just like, wow. So I enjoy just, like, lurking on this. Then I do, like, contributing, like, my advice and my experience, especially when it's a parent asking questions about their kid. So it's because I think that listening to adults with SMA is their best source of information.

**Moderator:** Mhmm.

**Respondent:** So, yeah, I'm on there. I get information from them, but I don't necessarily feel like, oh, this is a community. This is a new it's a newsletter.

**Moderator:** Yeah. That's really interesting. Where do you speaking of information, where do you tend to learn about what's happening around?

**Respondent:** It would probably be those Facebook groups. I think that's yeah. But I get, like, a Quest magazine from NDA, and I usually, like, give that a cursory glance. But, yeah, it would probably be those Facebook groups.

**Moderator:** And in terms of your health care providers, who do you talk to at all about SMA?

**Respondent:** I have a neurologist at the University of Utah who's in their MDA clinic. And I just see her yearly. If not, I'd probably, it's been a little bit more than a year at this point, because their clinic is not very conducive to people who have lives. But now I don't have a life, so maybe I can get in sooner.

**Moderator:** What do you mean by that? I mean, I can imagine, but I'm.

**Respondent:** Yeah. They only have clinics on Fridays. My neurologist was gone the entire summer. Like, she just wasn't seeing any patients all summer. And, like, half the time, it's like, the appointment is, like, 8AM. And it's like, okay. Well, you know, I have other people that I have to rely on to help me, and I can't change their schedule as much. I need my health care to work with my schedule and not the other way around.

**Moderator:** Yeah.

**Respondent:** Yeah. So they yeah. I feel like every time I try to make an appointment there, they're like, k. We have an 8AM on a Friday in six months.

**Moderator:** Wow.

**Respondent:** I understand why they do the clinics on Friday because then they just have, like, everybody there in one place, and you can do everything you need to in one day, which is really a great option. But it's like, also, sometimes I don't need to see everybody. I just need to see my doctor, and I need it to fit with my life.

**Moderator:** Yeah. When you say I don't need to see everybody, does it.

**Respondent:** So there's usually a pulmonologist. There's usually, like, an equipment person. There's usually a social worker. I don't know. Those are the people I see. I'm sure there are other specialists as well that can just kind of pop in.

**Moderator:** Wow.

**Respondent:** So I think that's a great setup, but it shouldn't be the only option.

**Moderator:** So there are a lot of barriers for you just accessing your neurologist. Is that fair to say?

**Respondent:** Yeah.

**Moderator:** Like, just literally getting in there at it, relying on other people, the availability of the appointments.

**Respondent:** And I don't really like her that much either. And she's the only one I can see. Because I've tried to see somebody else, and they're like, no. You need to see her because she's the expert. And I'm like, okay. Well, I don't feel like that. But okay. I'd rather have somebody who knows I feel like a lot of times in the clinical setting, especially as an adult, they do this really well in pediatrics, at least in my experience. But as an adult, it's just your life is entirely your disease, and that's all they really care about. And it's not like, hey. Let's talk about how are you doing financially and how are you doing you have that holistic wraparound services, all of that, that I think can handle a lot better when you're a pediatric patient.

**Moderator:** Yeah.

**Respondent:** And then you become an adult, it's just like, okay. Well, how's your disease? You know, what's your physical you know, how's your body? Which is important. But, also, it's like, yeah, but also, like, for me, you know, finding caregivers and finding transportation and applying for Medicaid and staying on Medicaid and all of those things are much more difficult, than just, you know, getting a treatment or, you know, knowing what the latest treatment is. And, also, I mean, like, this is a really great example. I went to the doctor, and I was talking to her, and I said well, actually, there's two examples from the same appointment. I said, well, first of all, I need to see a gynecologist.

**Moderator:** Mhmm.

**Respondent:** But most gynecologist offices are not accessible or just difficult. I mean, it's difficult for everybody. And then you add this other layer on top. I said, you know, who do you recommend your patients see? And she was like, oh, I don't know. And she's like, let me think about that. And then I never heard from her again about it. And then in that same appointment, I said, you know, I need to talk to a therapist, a mental health therapist, because I'm getting really burnt out on, just, like, accessibility and just the day to day kind of micro things that you just deal with every single day. I'm getting really burnt out on it. And she said, okay. Let me talk to the social worker. And the social worker sent me a very short list of some people that they recommend. Because I also said, I wanna see somebody that understands disability, and I don't have to, like, explain all of the, like, day to day things and educate somebody on top of.

**Moderator:** Yep.

**Respondent:** Getting the treatment that I need. And so I made an appointment with one of the ladies on the list, and it took a long time to get in. She didn't have appointments. And then I just had the thought that I should look at her office on Google Maps. And I pulled it up, and it wasn't accessible.

**Moderator:** Sorry.

**Respondent:** Yeah.

**Moderator:** It's sorry to laugh. It's just yeah.

**Respondent:** No. That's the appropriate answer. And so I called her, and she's like, yeah. I don't know why they recommended me to you because my office is not accessible. So it's all of those kinds of things.

**Moderator:** Yeah.

**Respondent:** That just make it like it makes it feel very much like going to the clinic is going to be more of a hassle than it's actually worth. I'm not gonna get what I actually need, and I'm just gonna leave frustrated.

**Moderator:** Thanks for sharing all that. It really paints a very clear picture. It's not a happy picture.

**Respondent:** Yeah.

**Moderator:** Sorry. I'm just absorbing it. Sometimes it takes me a little while when I hear a sad story. I have to just be with it for a second. Do you have a primary care physician who you talked to about?

**Respondent:** My primary care doctor is great. He's awesome. But, yeah, it's very I mean, he's I think what's great about him is he knows he's not an expert. So he, you know, he, like, wants to find stuff out and wants to be helpful and all those things, but he's also not like, oh, yeah. I know what what you're talking about. He's a very like, yeah. I that's outside of my scope. Let's get you you know? And he's been very, like he's just yeah. He's great.

**Moderator:** What yeah. So what role does he play in terms of your SMA specifically? Like, what would you say?

**Respondent:** I wouldn't say much. He knows like, I will go to him when I'm like, if I have a if I'm sick, if I have a viral thing or whatever. And so he's really proactive on, like, okay. If you have a cough, let's get you on antibiotics just to try to keep it from turning into pneumonia.

**Moderator:** Okay.

**Respondent:** Know? And so he's very, like, proactive about that. And, I mean, the SMA does kind of impact has a ripple effect on pretty much everything.

**Moderator:** Yep.

**Respondent:** But I wouldn't say he does much, like, directly related to my SMA. It's just, you know, taking that into account with everything.

**Moderator:** But at least he knows enough to take it into account in terms of the rest of your care and be proactive. Yeah. Have you ever talked to your primary care physician at all about SMA treatment about, like.

**Respondent:** I haven't.

**Moderator:** No.

**Respondent:** No.

**Moderator:** What would that even I mean, I know it's a rare disease, and a lot of neurologists don't even know much about it. So yeah.

**Respondent:** I'm sure if I went to him and said, hey. Will you write me a prescription for this? He'd say, yes. I trust that you know what you're doing.

**Moderator:** Wow.

**Respondent:** I mean, yeah, I'd probably, you know, wanna look it up a little bit, but he I think we have a good enough relationship that he's like, yes. I know that you are smart enough to figure out this is what you need, and I am here to facilitate that.

**Moderator:** Have you ever thought about that? Is that something that's ever.

**Respondent:** Not really. I need to. Because, also, there was that thought too of just, like, he you know, there he may say, why don't you talk to your neurologist about that?

**Moderator:** Above my pay grade kinda thing.

**Respondent:** Right. Right.

**Moderator:** Yeah. Yeah.

**Respondent:** But I'm sure if I said, hey. Look. She sucks. And I know this is what I need. If you need to take a little bit of time to, like, do some research, that's fine. He'd probably be like, okay. Yeah. Let's do it.

**Moderator:** So I wanna go back well, just quickly, how old were you when you were diagnosed, Shelby?

**Respondent:** Like, a year.

**Moderator:** Wow. Okay. And did you see a neurologist pretty regularly growing up?

**Respondent:** Yeah.

**Moderator:** Yeah. What was that ex what was your, like, pediatric experience with a neurologist?

**Respondent:** Pediatric experience was great. So I saw a provider at well, so.

**Moderator:** Don't tell me the names of any of the doctors. Just FYI.

**Respondent:** I saw a provider actually here in Utah very first, when I was really, really, really little. I don't remember it. And then we got connected with a provider in Seattle at the Children's Hospital there. And so just I had a great experience with that. We would basically see them once a year. We'd do a clinic day. I'd go in. They would just be like, oh my gosh. You're so great. And so I loved that. And then we'd go out to eat.

**Moderator:** Oh, that was smart of your parents. Like, they bookended it in a positive way too. It's like a whole adventure. Yeah. Yeah.

**Respondent:** I think I mean, my pediatric experience was great because I also just wasn't really difficult, and I didn't have like, I had a really good support system. My disease wasn't really, like, super progressing. I mean, I've always used a wheelchair. I never walked or anything.

**Moderator:** You're type two. Right? Is that right?

**Respondent:** Yep. As far as they know, I feel like there's a bunch of us that I'm like, okay. We're all supposed to have the same thing here, but we all have very different bodies.

**Moderator:** But you were pretty you weren't you were stable.

**Respondent:** It was pretty stable. And, I mean, as a kid too, I think I'm sure my parents have a different experience of it being, you know, stressful and scary and, you know, all those things. And to me, I was just like, yeah. Go to the doctor, and they say, wow. You're doing great. And they think I'm funny and cute, and then we go to lunch for dinner. And I get a day off of school.

**Moderator:** Yeah. Right. So when did you first notice that you because you talked about now that you're slowly progressing, and you're 33. When did you when do you think you noticed that that stability wasn't holding as much?

**Respondent:** So I think there's a bit of a decline. It's been over the last, like, five years.

**Moderator:** Okay.

**Respondent:** Definitely, like, more recently, I've like, it was kind of one of those, like, this is interesting. This just feels like a coincidence. And then, you know, I get a little bit I, you know, have, like, kind of a bad day, and then it'd be fine for a while. And then, but now it's kind of like, no. I think it is getting a little bit worse. Mostly just in my arms, and just, like, more of, like, the gross motor strength in my arms. I did have I'm pretty sure I had COVID in 2019, in, like, December 2019.

**Moderator:** Wow.

**Respondent:** And if it wasn't COVID, it was something very, very similar, and I was very sick. And then I got it again in February 2020, whatever it was. So I think that kind of kinda got that.

**Moderator:** Oh.

**Respondent:** Those sicknesses. Because I was pretty, I was really sick and then had a pretty long recovery from both of them. And then the world shut down.

**Moderator:** Yeah. Right. Exactly. So I wanna talk about treatment. When before so just in terms of timing, when did you get on the Everest D, and when did you get off the Everest D?

**Respondent:** Oh, it was right when it was released. So I am not totally sure. It was so it would have been, like, 2019, I think.

**Moderator:** Okay. And do you know how long you were on it for?

**Respondent:** Then it was, like, three years.

**Moderator:** Oh, you were on for three years.

**Respondent:** Yeah.

**Moderator:** I didn't realize it was that long. Okay. You think until about 2022?

**Respondent:** Yeah.

**Moderator:** Okay. Interesting. What was your I wanna even ask you about treatment before that, but since I'm here, I'm gonna ask now. But what was your I know that you faced a lot of administrative hassles, like, burdens, challenges, barriers. Your insurance didn't cover it, then it got up to the foundation. The foundation wasn't gonna cover it. But what was it like for you? How was the experience for you on it?

**Respondent:** I really didn't notice that big of a difference.

**Moderator:** Okay.

**Respondent:** And if I had noticed a difference, I probably would have dealt with the administrative stuff more.

**Moderator:** Yeah. Yeah.

**Respondent:** And, I mean, the biggest it's so hard with SMA as an adult, especially, because it's like, okay. Is this helping in that it's just keeping me at a baseline, or is this just gonna be my baseline? And just you have no there's nothing to benchmark your progress with really, as an adult because everybody's just so different.

**Moderator:** Yeah.

**Respondent:** And so it just kind of so now I'm like, okay. I'll get back on it, just to try to maintain at least. And the fact that there's so little research in adults with any of the treatments, really. And, yeah, and everybody's experience is just so different. And it's just like, oh, I don't it's hard to know if it's if it's actually doing anything.

**Moderator:** Yeah. Okay. You were raising so many I keep whenever you do this, I'm like, oh my god. There's so many important things I need to ask you here. Let me just back up because I wanna talk to you about what your expectations were about what is helping actually mean, what you're hoping it was gonna do, blah blah blah blah. And it's interesting what you said about this little research. But so back me up a little bit. How did you even end up getting on Epristy? Had you ever considered getting on SPINRAZA?

**Respondent:** I did, but I was just like, yeah. I'm not doing all of that for so little of a guarantee of it actually either maintaining or making me stronger. The cost benefit analysis to me was just like, this is not worth the stress of, you know, finding someone to take me. And, you know, and then it's also like and it's gotta be somebody that can help me transfer, and I feel comfortable with, you know, getting positioned and all of those things. That pool of those people is already small. And then, you know, is it somebody that could come during the middle of the day? You know, are they not at work or their kids or, you know, all of those things. And then a spinal puncture, no. Thank you. That does not sound super fun. And then I had a couple friends that had, like, not great that had had a few not great experiences. One ended up in the hospital with, like, a cerebral fluid headache. Or I was just like, I just don't have the bandwidth to deal. If it was, like, one of those things where there was, like, research and we knew that it was going to help for sure in adults and, you know, we could point to that and say, yes. It's gonna be worth it. I might have considered it more. But the fact that it was just so, like I mean, it might help and it might not. And my thing too is I was like, well, I really would only wanna do that if it was gonna make help me improve and have a higher Hammerstein score or whatever it's called.

**Moderator:** Hammersmith. Yeah.

**Respondent:** Yes. I'm Jewish.

**Moderator:** I was gonna say that. As a Jewish person, I figured I could.

**Respondent:** And I know that, you know, anecdotally and there are and I feel like a lot of the research now that I've seen at least I mean, I'm not, like, diving into the papers and stuff. I'm reading the abstract and, yeah, kinda moving on. But just for people my age, there's not as much research.

**Moderator:** What yeah. That's a whole other thing. The research piece I need to come back to, but and it's I hear you talk about the lack of research and specifically with adults and people your age. And where when you say you read the abstracts, how does when does that happen, or what leads you to do that?

**Respondent:** I usually I'll see stuff on those Facebook groups. People will post. I do follow, like, Cure SMA on Facebook too. And so I'll see, you know, like, their papers and stuff, or the press release. I guess it's probably more often the press release than the actual paper. So I'll just see him there, and I'll, like, take a look at it.

**Moderator:** It's interesting. Okay. So talk to me about how you ended up on EmbrisD then.

**Respondent:** I think I had seen gosh. I don't even remember. I feel like I had seen just some chatter about it on one of the Facebook groups or something. And then I just asked my doctor about it, and she's like, yeah.

**Moderator:** She didn't bring it up? You brought it up?

**Respondent:** She might have been on her way to bringing it up, but I brought it up first, because I do only see her, like, once a year. So I honestly don't totally remember, though.

**Moderator:** What were you if I know this, again, like, five years ago, maybe even six. What were you hoping at risk you was going to do for you?

**Respondent:** I think at that point, I was hoping just to kinda maintain a baseline. And I mean, obviously, it would be awesome if it helped improve some of my arm strength. Yep. My expectations are very low of what a treatment would do, for an adult. I see how great it is for kids as an adult. I'm like, I just want to, like, kinda maintain where I'm at or get, you know, just a little bit more energy or a little bit more arm strength. I don't anticipate that I'm gonna, like, be walking one day. You know? Like, that's not gonna happen.

**Moderator:** Yeah. Like okay. And what happened with insurance? What did they tell you why you were denied?

**Respondent:** It was just too new and expensive. And, yeah, and it was I think it yeah. I think it was. Because the doctor did say too he's like, you know, if we can get more people on it through the foundation so we can prove that it does actually work or, you know, have some effect, then more insurances will start covering it.

**Moderator:** Interesting.

**Respondent:** So, yeah, it was just and, I mean, I'm sure I could have, like, gone through a fight with them, but also I'm like, yeah, I get why you don't want to cover this thing that is so expensive. And especially at the time, not a whole lot of evidence as for an adult of what it's gonna do.

**Moderator:** What was your experience working with the foundation to get the medication?

**Respondent:** I think it was fine. It was just another bureaucratic administrative thing on my plate. But, yeah, I mean, I don't have any, like, horror stories that I can think of. So.

**Moderator:** And you said I didn't notice that much of a difference.

**Respondent:** Mhmm.

**Moderator:** Can you talk a little bit more about that?

**Respondent:** Yeah. I didn't notice that I was, like, getting I was improving at all in strength or, oh my gosh, what's endurance or any of those kinds of things. I mean, again, like I said earlier, it's really hard to know if you're not declining because of the medication or because you're just at a natural plateau, that you would have just plateaued anyway. So it was just a lot of that, like, okay. Cost benefit analysis of is the administrative burden of this worth something that I can't see results? And I guess I should say too, I didn't correlate this until later, but I do think it gave me some gastrointestinal, just kinda upset stomach and that kind of thing, which I would have just dealt with also if I had and I started seeing after I got off of it people being like, yeah. It was causing me lots of tummy issues. And I was like, oh, you know? Yeah. I was having some stomach issues at the time too, but I just didn't correlate it with.

**Moderator:** But you didn't but that wasn't part of the reason to go off of it. It sounds like you just noticed it later. Okay. Can I just pause here for a second? It's interesting. It's just you're not the only person I've heard talk about this sort of tension around knowing if it works or not. Right?

**Respondent:** Right.

**Moderator:** And so I don't even have a specific question, but I'm just gonna talk stay here for another minute. It's interesting because, like, on the one hand, you talk about your expectations being like, I wanna maintain this baseline.

**Respondent:** Mhmm.

**Moderator:** But on the other hand, you're also saying, I didn't notice any improvement. Right? And I can't see the results. Sort of like I wonder if you could just talk a little bit more about your how you were gauging if you could see results or not. Yeah.

**Respondent:** Yeah. Let's see. I'm trying to think if there's anything. I mean, it really is just that I think it's hard as kind of the, like, second generation of people with SMA to live into adulthood. One, I don't really I don't know what to expect.

**Moderator:** Yeah. Yeah.

**Respondent:** And because there are also so many different experiences.

**Moderator:** Yeah. Yeah.

**Respondent:** That I think I think it was a just a psychological burden as well as trying to think how to because, yeah, when you say that, it's like, yeah, it doesn't make sense.

**Moderator:** No. No. No. But we don't necessarily need to make sense as human beings, but I'm interested in what you just said about first, a psychological burden. What does that mean exactly? And don't have to have it all fully formed, just whatever comes to mind.

**Respondent:** Yeah. Yeah. I just I think it was really just this idea of this is just one more thing I've gotta deal with. And I don't know if it's going I just don't know if it's worth having to deal with.

**Moderator:** When you would go to the neurologist, maybe every year, maybe not every year. I don't know how often you were going over those three years. Did you talk to your neurologist about the Epstein when you would go?

**Respondent:** A little bit. And we did do it okay. This is another thing that I think also complicates things is, you know, we did another Hammersmith.

**Moderator:** Let's just call it the Hammerstein for the rest of the call.

**Respondent:** Made him a wasp. He's a we yeah. I told her, like, I don't know if this is really doing anything, and we did another test. And she's like, well, your results are better, you know, than the first time, but there was also like, yeah. But that could this test would be different every single day for me.

**Moderator:** Yeah. Yeah.

**Respondent:** So I doing it once a year or once every couple months even is I would need to see do, like, a daily or weekly having a little bit more data to kinda average out.

**Moderator:** But it's interesting. So I wondered about the Hammersmith if you have been doing it. So when you would go annually, the data, the annual data would show that the results were better to some extent,

**Respondent:** Yeah. And I really only think I did it once.

**Moderator:** Okay. You only did it once. That's interesting. But for you, you definitely experienced both an administrative burden as well as some sort of psychological burden.

**Respondent:** Yeah.

**Moderator:** Yeah. And can you and I also wanna talk about why I wanna make sure I leave room for how you're considering going back on the medication now. I'm just acknowledging. You're a smart person. There's just a lot of things to talk about. So just if we're gonna detail the administrative, what were the burdens you experienced with being on at Frisbee?

**Respondent:** I think the biggest thing was just, like I think I'm remembering this correctly. I felt like I had to, like, reorder it every month, or there was some there was something that I it wasn't just I think I had like, authorize it every month or something. And dealing with the specialized pharmacy was just kind of a pain. And then, yeah, and then it got delivered. And, I mean, it would come in a refrigerated pack, but there was that stress of like, okay. It's gotta get in. Is somebody gonna be here to bring it in and put it away in, you know, the timeline. And, this is expensive, and I don't want something to happen to it because I'm not getting you know, I'm getting what I get for the month, and that's it. Yeah. And I just feel like I can't remember specifically, but I just remember being like, every time I would see them calling, I'd be like, oh my gosh. Okay. Another.

**Moderator:** Oh, you had to sort of steal yourself to the yeah. That's so interesting.

**Respondent:** Just like, okay. It's another thing. I don't really remember exactly specifics of what the problems were, but there was just that, like, okay. It's another thing.

**Moderator:** Yeah. And then you talked about the manufacturer no longer or the foundation wasn't gonna cover it. What happened with that?

**Respondent:** I honestly don't remember. It was at the point where I was just like, I think I might have I think what it was was I got a new job, and I was gonna be making a lot more money. And so I was already going through having to lose Medicaid. Because I figured out how I was gonna pay for everything out of pocket. And so it was just like, I even I mean, I have no idea. It could have been super easy, and they wouldn't I'm like, yeah. It doesn't matter that you're making a $100,000 now. You're never gonna be able to pay for this, so we'll just keep paying for it. But it was just another thing that I was like, I just do not have the bandwidth to deal with this. And I think this is an important thing to talk about. It's like, my parents are amazing. I love them. They are great. They are not involved in all of this stuff. I don't want them to be. I don't think they need to be. But they and they, but that's just kinda I'm doing it by myself. I don't have a partner that's helping, you know, take some of that load. I mean, my parents, it was kinda to the point where they were like, wait. What's that medication called that you're taking? Are you still taking that? You know? And then I get on, like, the Facebook group, and it's like, I need help for my 27 year old son who is, you know, doing this. And I'm like, I don't want I don't want that. But there's a lot of people that have. And not to say that I wouldn't get that support if I asked my parents, like, hey. I need your help with this. But there is also a limit to what they can do when you're an adult and, you know, because then it's like, okay. Well, I could give them my power of attorney, but then that's just another thing to have to deal with, and figure out. So they can help with these things. And so I think that's another part of it too. It's, you know, as an adult, you just have less. I mean, in theory, you have less support.

**Moderator:** Yeah. Yeah. Is there and is there anything well, I I'm just looking at the time. I guess but also what you just said about losing Medicaid and, like, somehow being, like, almost punished because you were starting to make.

**Respondent:** Right.

**Moderator:** Yeah. Right. Exactly. So I wanna save time, but I'm I guess I have one last quick question. I wonder if there's anything that they could have done at the foundation during that time to have been more to have taken some of that burden off of you. But it just seemed like a lot yeah. Also, just was a lot going on for you right then.

**Respondent:** Yeah. I mean, I really think that when it comes down to it, a lot of it would have been more on my neurologist. I think because I think it was I think and this is what happens a lot with her too. And I don't know that there's a solution to this, but it is just kind of like, oh, here are the resources. Go forth and take care of it. And so if there had just been I mean, if it was just somebody that I could have just said, yes. I'd say that this person can speak on my behalf from their clinic or something along those lines that I could just kind of pass off to somebody who was gonna get paid to do that as opposed to losing pay to do that.

**Moderator:** Yeah. Yeah. of advocate to help you. Yeah.

**Respondent:** Or, you know, something I could talk to once a month who I can give updates to, and then she can go handle the administrative bureaucratic stuff. And, you know, once you understand how to deal with it, it's a lot smoother. But when.

**Moderator:** How much the what were the conversations like with the people at the foundation? Like, what was that?

**Respondent:** I don't really remember much. I know they've tried to get me to come to some events, and I'm like, guys, I am already really busy. And it kinda felt a little bit like, you know, come to these events so that we can, like, convince other people that you should be on this med they should be on this medication. And I'm like, I am not have spent enough of my life being a poster child. I'm not doing it anymore. If you wanna pay me, I would. But I'm not gonna come.

**Moderator:** Yeah. Not just doing it for free. Okay. Just, again, looking at the clock. Alright. So it's interesting because that was very, very helpful, and I learned so much. And that was maybe two or three years ago you stopped the treatment.

**Respondent:** Yeah.

**Moderator:** And you mentioned now that you're at a place of considering going back on the treatment, and you mentioned the tablet. So help me understand how you got to this place now.

**Respondent:** Well and I think a good hallmark of it is I've been thinking about it for a while, but haven't done it. So, I mean, that's a big red flag right there.

**Moderator:** But it's interesting. Yeah. How long do you think how long do you think you've been thinking about possibly going back on treatment?

**Respondent:** Well, I learned about the tablet, through a survey, doing a survey like this. And I think it may have been before it was that may have even been before it was released. It was one of those, like, you know, this is a possibility. You know? What do you think? And so I knew I was kind of on the lookout for it. And I think there's a couple like, a combination of things. So one, I mean, a tablet is just gonna be way easier. Just I don't have to try to refrigerate it. I can I imagine I can probably get more of it at a time? Much easier to just store in a cupboard as opposed to at the fridge. Just easier to take. I don't have to, like, remind somebody every day, okay. It's this much fluid, you know, and taking it twice a day and all of those things.

**Moderator:** Who would be the person who had typically helped you get?

**Respondent:** I have, like, a small army of personal assistants. It's basically, like, one person a night, and it's a different person every night.

**Moderator:** Okay. So, like, even just the dosing is easier. They just give you.

**Respondent:** It's just like one pill or whatever. You know? It's not like, okay. One point two five milligrams. And then, yeah, that it's that third line, you know, all of those kinds of things. So that makes it easier. I do think, you know, seeing a bit of a decline and noticing a decline, I'm like, okay. I should probably just get back on this in hopes of either maintaining or maybe improving just a little bit. I feel like I've seen anecdotally people, like, on the Facebook groups and stuff talking about seeing the difference. I don't know how much of that is just placebo. But, also, if you notice a difference, it doesn't matter if it's a placebo or not. I don't really care what the if it's a psychological or physiological thing. And yeah. So I think just the, like, noticing a decline and seems like it's gonna be simpler. And then also from what I anecdotally, people have said the tablet doesn't upset their stomach as much too. Now I'm kind of like, ugh. I already have, like I'm a 33 year old woman. Like, we all have stomach issues at this point too. I don't wanna do something that's gonna make it worse. So hearing that people say they are having a better experience in that regard, also, I'm like, okay. Well, that's not gonna add another burden to what I'm dealing with. But, yeah, just haven't. And I think also the thought of trying to deal with insurance and the foundation and all of that. Again, it's just one of those, like, okay. I just need to, like, steal myself to do that. And it may be totally easy. It may be, like, not a big deal at all, but there's just that underlying trauma of dealing with this for so long that it's just like, oh gosh. Okay. This I may be pleasantly surprised, and it may be totally fine, but it also may confirm all of my previous experiences.

**Moderator:** Yeah. Yep. The underlying trauma of dealing with it for so long, can you say a little bit more on how that is sort of connected to the what what anticipating the administrative burden of navigating?

**Respondent:** I see it yeah. It really I mean, you said it there just that dealing with when you when it's been when you've been doing thirty years and I would say too, even as a kid, I mean, my parents were amazing. But part of what made them amazing was they were like, you need to advocate for yourself and do some of this. And I had the personality to do that too, which was helpful. But so I learned a lot of that, and I am good at it, but it's just it's just burnt out. It's just been so long of dealing with that. And then also kind of be I think this is another interesting part is a lot of times and this kinda goes with the Medicaid thing too. Like, I'll talk to people, you know, who this is their job to be doing these things. And I'll say, yeah. I'm gonna use Medicaid as an example, but it kind of filters into a lot of other things. I said, yeah. I don't qualify for Medicaid anymore because I make too much money. And these people who it's their job to know how to do to deal with the system said, well, that can't be right. I'm like, well, it is. And they're like, but have you tried this and this and this? And I'm like, yeah. I've tried all of those things because and I just there's not a lot of people that are in the a similar situation as I am. I'm kind of in this weird in between of really independent but needing a lot of support. And so and there's gonna be a lot more people like me as just general medical treatment gets better.

**Moderator:** Can I interrupt you for a second? Sorry, Shelby. What kind of given the realities of the administrative lift and, frankly, the psychological lift or, like, the psychological dread and then the burden that gets created from the administrative burden. And also just feeling like like just the fatigue of having to be one's own relentless self advocate. I hear that. What could let's say I wanna come to your neurologist in a second. But, like, what could your man, what could the manufacturer of a treatment, what could let's start with that, maybe we'll talk about the PCP. What could a manufacturer of a treatment do to help make it feel less burdensome?

**Respondent:** Yeah. I think one that would be helpful is just having information about who qualifies for, you know, assistance more readily available and having you know, saying like, hey. These are our kind of general guidelines. Obviously, we take individual accounts into you know, or whatever. You know, you can really couch that in this isn't the end all be all, but this can kinda give you an idea. Because there is a part of me too that's like, okay. Well, if I'm am I gonna start this whole process? And then they're gonna be like, oh, you know, you need to, like, go ask your insurance to cover it, and you need to go through because I think a lot of things too is it's like for me, it's like, you've gotta go through all of these things that you know aren't gonna work first before we will do the thing that we know will work.

**Moderator:** I think that that yeah. They because they're always like, just make sure they won't pay.

**Respondent:** Right.

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

**Respondent:** And I get it. I totally understand because, yeah, if the insurance is gonna pay for it, they.

**Moderator:** Yeah. Yeah.

**Respondent:** Yeah. But so maybe there's some, yeah, some information that's just, like, a little bit more accessible of, okay. Do I even possibly meet the criteria?

**Moderator:** Is this even worth some transparency around, is this worth my while?

**Respondent:** This is even worth exploring, because I just don't know.

**Moderator:** If and if you were going to get if you had decided, okay. I'm just I'm gonna try to get this insurance. What would you actually would do you feel like you'd have to go through your neurologist? Would you have to actually find a way to get into the office to get to the neurologist? Do you think you would ask your primary care physician? Had it occurred to you to ask your sorry. I have so many questions.

**Respondent:** Yeah. No. I mean, it really hadn't like, for me, I would have just gone to my neurologist. Because I think my primary care doctor event if I, like, explains the whole thing of, like, you know, this is what I need. This is really you're much easier to work with. You know, you can go do some research if you want. I think eventually he'd be like, okay. Yeah. I'll just write the script for you.

**Moderator:** But just to be clear, that hadn't occurred to you, though, before this call. It wasn't like something.

**Respondent:** Not really. I mean, I thought about it once, but it was just very much like, no. I need to ask my neurologist about that. Because I do think a lot of PCPs would just say, talk to your neurologist. That is so outside of my scope, which I think is the appropriate response.

**Moderator:** Yeah.

**Respondent:** But that's the appropriate response in best case scenario.

**Moderator:** Can I ask you another question? Would there ever be any value for you in talking directly to someone else who has SMA about their route to treatment? Like, would there be.

**Respondent:** For me, I feel like my situation is just really different than most people's. So I think it would be helpful for a lot of people, but I don't know that it would be super helpful for me. And, I mean, I have a friend who does SPINRAZA, and that's kind of why I decided I didn't want to do it from hearing her experiences. So, yeah, I mean, I think I don't think it would be helpful in a like it's helpful in, like, an organic way, if, you know, you already have this relationship and this friendship. And that we already trust. It wouldn't be if it was just like, hey. We have a patient advocate for you to talk to.

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

**Respondent:** I know I feel like everything I've seen has been more of, like, changes to already available treatment. So, like, increasing the dosage on SPINRAZA, doing, like, the port on SPINRAZA, as opposed to the which I would not be opposed to doing a port if I would wanna do some more research about, like, infection and all of that kind of stuff. But I wouldn't be opposed to doing something that sucks once and then is, you know, not a huge deal as opposed to sucks every.

**Moderator:** However many weeks.

**Respondent:** Do like, every four weeks for the first few ones and then every three months forever. And then yeah. And I just I feel like there's just a lot okay. You'll probably appreciate this as a marketing person and research and stuff. I feel like there's a lot I mean, there's tons of research about how it works in kids and how it does in kids. And because it's so successful in kids, it's like, let's just focus on them and curing them, and we are kind of the, like, unfortunate.

**Moderator:** Yeah.

**Respondent:** We were born too late.

**Moderator:** And when you say it, a lot of research on it working in kids. What's it?

**Respondent:** Oh, just I mean, any of the treatments. The Zolagen or whatever it's called, the and Spinraza. And, I mean, just in I mean, even just from a, like, clinical standpoint, like, getting SPINRAZA as a kid is just easier because they'll sedate you, and the Children's Hospital experience is just much nicer.

**Moderator:** Than. I can I interrupt you for a second? Have you heard of a treatment coming down the pipeline called an antimyostatin, something that you would take in combination with either SPINRAZA or a FRISD?

**Respondent:** I think I've heard of it in the same kind of context. And in, like, a hypothetical, like, would you do this? And, yeah, that felt and I feel like what I saw of it was it helped a lot if you were doing SPINRAZA and helped a little bit if you were doing a VRISTI. But I haven't, like, explored a ton of it. That's one that yeah. Haven't done a ton of research into that one, but I have hypothetically heard of it.

**Moderator:** Okay. Interesting. But I hear what you're saying coming back to the research. You talked about research at the beginning. You know, I know that, like, SPINRAZA and Embrisdi have been around both at least for five years. And so theoretically have more research about the impact on adults.

**Respondent:** Uh-huh.

**Moderator:** And I know that's true. And there's also, like, true that adults every adult I talk to sort of feels like all the focus is on children.

**Respondent:** Uh-huh.

**Moderator:** Right? That the fact that there is more clinical data from being around for five years plus SPINRAZA. And EBRIDG, is that how much of an impact would seeing that actually, like, honestly make on your feelings about treatment?

**Respondent:** Yeah. It would have to be pretty and I think I've said this a few times, but I think it really is. It is just so hard to know if that is just your natural plateau where you're just gonna because I think I think what I was kind of told about these treatments for adults is they're really just gonna, like, maintain where you're at and just, you know, have that be your expectation. And it's like, okay. That's great. But it's so hard to know if that is because of all of this extra work I'm doing or this is just what it's gonna be. With or without this. Then I think and then there's also, you know, a level of, well, I didn't start this five years ago. So is this worth starting now? Or, you know, that kind of thing too. We have more data on what it's like for adults. But when you're in that exact same cohort as opposed to, you know, this was a cohort from a couple years ago, and, you know, we're seeing the data. And so I'm at the point where they started, so I'm gonna start now. It's like, well, no. We're at the same point. Is it actually going to there's not a lot of data for somebody older than me. And, you know, what does that look like for somebody who is further down the road than I am? It's kind of where I'm at or younger. And, I mean, there's not I don't think there's an answer to that. It's just, it's just the unfortunate reality of it. And I don't like that there's a lot to there's not there's not really a scientific and logical way to fix that. But I think, you know, seeing seeing more research and hearing more from people that are on the older end, yes, does make me feel like, okay. I do feel like this will at least not hurt at this point.

**Moderator:** Shelby, you have been so helpful. Like and just it's always such a treat when I get, like, a really smart person, a really thoughtful person who's able to articulate themselves so clearly.

**Respondent:** I well, I was telling my friend this morning that was helping me. I was like, yeah. I'm, you know, doing this thing. And I was like, I just love to tell my opinions and then to get paid on top of it. Because I would tell you I mean, I'm not gonna say I would tell you my opinions for free or any ideas, but I have lots of thoughts.

**Moderator:** Yeah. No. It was great. I learned so much talking to you. I wanna wish you all the best, and I just wish I sort of have this instinct to sort of validate that how strong you have been all these years and how exhausting it is to keep going, but also to say you're lucky that you have yourself. I know that's such a weird thing to say, but, you know, like, you sound like an amazing self advocate even though it's so hard sometimes.

**Respondent:** Well, thank you. I appreciate that.

**Moderator:** Yeah. Thank you so much for sharing everything.

**Respondent:** Yeah. Tell tell Zoran good luck.

**Moderator:** Okay. Next time I have a one on one chat with him. Although I missed one at the at the he came to my temple, and I could I was away. I really wanted to people had a lot of tough questions for him. I was curious to be there, but missed that one. Anyway, thank you so much. Okay.

**Respondent:** Thank you. Bye.