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*Oct 6, 2025 | 3:00 PM | R01 Transcript*

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Interview Date: October 6, 2025

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**Moderator:** Good. Before we begin, I just want to give you a quick introduction. I really appreciate you joining today, and we're going to be talking all about SMA and SMA treatment in particular. Everything you say today is completely confidential. I don't even know your first name, and I don't know your last name, so let's just keep it that way. Please try not to tell me the name of any doctors involved in the SMA treatment. I do have a few colleagues listening in. They don't know who you are either, and we're recording just for research purposes. If by any chance you mention that you've had a less than ideal experience on a medication my client makes, I have to write up a little report called an adverse event report, and I might have a few questions to ask you about that. Also, I think you gave seventy-five minutes of your time. Is that correct?

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

**Moderator:** So even though that seems like a really long time, every once in a while, we're like, can we really talk to each other for an hour and fifteen minutes? Every once in a while, I still need to interrupt folks just to hustle things along because I do have a ton of questions. Are you okay with all that, everything I said?

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

**Moderator:** Any questions? Okay. Will you tell me, are you the person who has SMA, or are you a caregiver of someone who has SMA?

**Respondent:** I have SMA.

**Moderator:** You have SMA? Okay. Can you tell me a little bit about yourself, if you live with anybody else, how you spend your days, just so I have a sense of who you are?

**Respondent:** Sure. I live with my children and my husband, and we have two dogs. I work part-time for a local school, and I am enrolled in school to earn my bachelor's.

**Moderator:** Wow.

**Respondent:** And I do all the other daily things in between there.

**Moderator:** That's a lot going on there. How old are your kids?

**Respondent:** I've got 19, 17, and 15.

**Moderator:** Wow. Alright. You're full on. I have one 16-year-old and two 14-year-olds, so just behind you.

**Respondent:** Yep.

**Moderator:** And what do you do when you work part-time for the local school district?

**Respondent:** I work for the front desk. When the kids come in late or need to check out, I ensure that they're safely checked in or out and then input some data into the system, whether it be attendance or other various things that are assigned.

**Moderator:** That's great. And amazing you're getting your bachelor's. What are you majoring in? Sorry, these are just nosy questions. They're not crucial, but it helps me get to know you a little bit.

**Respondent:** You're good. I've got my associates in medical coding and billing.

**Moderator:** Interesting.

**Respondent:** In medical office admin, so I thought it'd be fun to get my bachelor's in human resource management. Nothing together.

**Moderator:** I mean, there's a lot of detail in both.

**Respondent:** Yeah.

**Moderator:** Right? A lot of systems stuff. Even like, I think about HR as people, people, people, but it's a lot of systems too. Right?

**Respondent:** Yeah.

**Moderator:** So when your type, your type three SMA. Is that right?

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

**Moderator:** Okay. And when were you diagnosed with SMA?

**Respondent:** October 2012.

**Moderator:** Wow. Wait. I never asked you how old you are. How old are you?

**Respondent:** I'm 43.

**Moderator:** So interesting. You were diagnosed at, like, 30 years old?

**Respondent:** Yeah. Right around, I think.

**Moderator:** Wow. So interesting. How did the diagnosis come about?

**Respondent:** Our pastor's wife is a physical therapist. She noticed my gait and told me I needed to see a neurologist. They tested me for a couple of things, and one of our friend's daughter actually has SMA type zero or one, and something told me to get tested for it. I had never really heard about it, didn't know anything about it. But when the testing came back negative for MS and a couple of other things, I just said, hey, test me for this.

**Moderator:** But the upsetting part of that story is that it wasn't part of the panel of original things that they tested you for too. Like, if you hadn't asked.

**Respondent:** Yes.

**Moderator:** Sorry. That's just a little heartbreaking there.

**Respondent:** Yeah.

**Moderator:** What a remarkable physical therapist to know that you should see a neurologist.

**Respondent:** Mhmm.

**Moderator:** Wow.

**Respondent:** Yeah. Just watching me walk on Sundays and the little bit of interaction we had, she just said, there's something going on with your gait. You need to get in, and so I did.

**Moderator:** Wow. So all the testing came back negative, and then you said to the neurologist, did the neurologist even know when you said what was the reaction when you said, actually, I want to get tested for?

**Respondent:** He was pretty open to it, and he wasn't very well-versed on it. He had to do some research. I was sent to a muscular dystrophy clinic in another town once I got diagnosed to help me learn some more about it. He was very limited in his research. Basically, what he found on the Internet is what he knew.

**Moderator:** Sorry. I don't know why I just giggled, but it's just intense. It's a bit like, gosh. Had you been seeing a physical therapist or have your own physical therapist before the interaction with the pastor's wife?

**Respondent:** So I had seen a doctor. I had some lower back pain, and they called it a limp at the time. They were attributing it to maybe childbirth. I'd had my third child by then, and they wanted to do an epidural to block some of the nerves and the pain. I didn't have it done. My husband was in the military, so we moved and things like that. When we got to our new duty station and got settled in, I was going to try to continue care there, but she intervened before then. I just chalked it up to maybe I had gained some more weight because I was always really thin before kids. I lost my ability to run. I was slowly noticing a few other symptoms, but not being knowledgeable, I just chalked them up to, hey, I'm just fifty pounds heavier than I had ever been.

**Moderator:** Wow. 50, but it was a big change.

**Respondent:** Mhmm.

**Moderator:** Wow.

**Respondent:** Yeah. So I just thought my weight was causing some of my issues. Maybe I was too heavy for my body frame or something like that.

**Moderator:** Yeah. Wow. It's so interesting. It's so funny. Somebody attributed it to the birth. Yeah. You're like, oh, let's just chalk it all up to childbirth.

**Respondent:** Yeah. Yep.

**Moderator:** It's just a female problem. Yeah.

**Respondent:** Right.

**Moderator:** Wow. Okay. So how did you feel when you were diagnosed? What was that like?

**Respondent:** It was a little bit scary. I didn't know much about SMA as a whole. I only knew what I had seen in our friend's daughter. I'll be honest. I didn't know much about it. So I had to dive in and try to research. Obviously, I was scared. I have three young kids. I'm married. What is my life going to look like? All those things crossed my mind. My biggest fear was, are my kids affected?

**Moderator:** Oh, what do you mean? Can you say more about that?

**Respondent:** I was worried if my kids would have SMA, just part of processing it all.

**Moderator:** Like, was there a genetic thing that you passed that was passed down to them? I see.

**Respondent:** Yep.

**Moderator:** And your friend's daughter, you said she had type zero, type one. Is she pretty severely impacted if she's type one?

**Respondent:** Yes.

**Moderator:** Yeah. And that was your one example of SMA?

**Respondent:** That's all I knew.

**Moderator:** Yeah. So you were referred to a muscular dystrophy clinic. Is that what you said?

**Respondent:** Yes. So they referred me to a muscular dystrophy clinic in a city close to us, just to get their insight on it and see if they could give me some more information, maybe learn a little bit more. I took it into my own hands and pushed to actually see an SMA doctor.

**Moderator:** Oh, at the same place?

**Respondent:** No. I pushed to go to Utah to see a specialist out there. That was the closest SMA doctor I knew.

**Moderator:** Without telling me your actual address, what state do you live in?

**Respondent:** Oh, currently, I'm in Alabama. We were in Kansas when all this was going on.

**Moderator:** Oh, okay. How did you know about the doctor in Utah?

**Respondent:** I joined a few SMA pages and heard that this clinic was very well known. So I pushed my insurance to go out there because I did see the neurologist in Kansas. Well, two of them actually. They didn't really know much. Couldn't really give me much guidance. So I decided it was my right to see somebody who could maybe inform me a little bit more.

**Moderator:** So when you say they didn't give you much guidance, can you say a little bit more about that?

**Respondent:** I'm being told things like live your life the best you can. I was told at one point I may, you know, hey, your life is going to be in a wheelchair. Like, there was no if, ands, or buts about it. And while it's not horrible, but to be newly diagnosed, not know what's going on. I've got three young kids. All these things are just spiraling. Like, what do you mean? Like, is there's nothing to help me. Basically, go home and figure out the next wheelchair that you would like. That's what it's being presented to me as. And it was a lot of, well, you're walking and you're moving. We'll see you in six months. We'll see you in a year. There was not a lot of, like, hey, try these therapies or do these things. It's we'll just keep watching.

**Moderator:** And you said you were on some SMA pages. Tell me about that. Did you go on Facebook?

**Respondent:** On Facebook. The one lady, whose daughter we knew, she did a lot of research, found some pages. So I reached out to her. My husband and her husband were in the army together. So I just found some pages on Facebook. I don't recall exactly what the names were, and just got some information from there. At the time, I was told there were really no doctors that knew a lot about adult SMA. There was one doctor in Ohio that kind of knew a little bit about adult SMA, but I had heard there was she's more of a pediatric doctor there in Utah, and that's where they sent me. I went to a children's doctor actually in Utah.

**Moderator:** And this is a neurologist?

**Respondent:** I think she is a neurologist. Yeah.

**Moderator:** Okay. And how did that go when you met with her?

**Respondent:** Very well. She was very insightful. I spoke to a dietitian there. They did some testing. I had by then lost my ability to climb stairs without a railing. But as they were testing me to climb the stairs, I could do it in her office. I'm like, this is not something I normally do. But it was probably one of the most informational doctors I had seen. They broke down SMA for me, how they think I was affected, gave me more information about it, better outlook. Here's some diet. Here are some things to maybe eat and not eat. Watch your calorie intake because you obviously are not as active as what a normal human being is.

**Moderator:** Okay. So I'm just going to pause there, and I want to understand where you're at now, and then maybe we'll get to fill in between now and then because I just have a little it's a fascinating beginning story that you've told me. In terms of now, do you…

**Respondent:** I have a neurologist that I see twice a year. I'm actually in the process of swapping, but I have a neurologist I'll see basically twice a year, every six months. And that's more so because I asked to go every six months versus every year. And then just my primary care doctor.

**Moderator:** Why did you ask to go twice a year?

**Respondent:** Just to maybe track my strength and things like that. Just to keep tabs on myself.

**Moderator:** It's interesting you're switching. What's your relationship like with the current neurologist? And do you have a new neurologist picked out?

**Respondent:** I do. I just have to wait a few months to get in. I'm just swapping more so because I've got a lot of knee issues, and I just feel like he doesn't give me the best care. I feel like maybe he could do more for me or try to help me more in certain areas, and I don't get that. I don't feel like I get that from him.

**Moderator:** I'm really interested in that. Can you tell me a little bit more about what you wish you would get from him and what he doesn't give you? Just help me understand what's lacking for you.

**Respondent:** Sure. I've had surgery on both my knees due to the way I walk, I hyperextend. I went into him and said, I've seen orthopedic doctors for my knees and stuff. I'm in a lot of pain. I live in pain daily. My primary care doctor, she's giving me muscle relaxers and anti-inflammatories and things of that nature, but I don't feel like I should have to live in chronic pain daily, especially with my knees the way they are. I asked him about a few medications, like, can we try this or this?

**Moderator:** What kind of medications did you say can we try?

**Respondent:** Sure. I asked him to maybe try Lyrica. I had heard it's good for inflammation and that type of thing. He doesn't want me to do gabapentin, just due to the fear of falling and things of that nature. He basically just told me I had to go back to my primary care doctor, and she would need to manage all of that. Basically, anything I was asking for was outside of his scope. I just felt like maybe as a neurologist, there should be a few other things he could do for me. I get it if he can't prescribe the Lyrica, but he should be able to say, hey, maybe we could try this medication with you or do this.

**Moderator:** How much do you actually talk to your primary care doctor about the SMA, and how much do they even understand about SMA? How much do you talk about it? How often does it come up?

**Respondent:** It only comes up as often as I bring it up.

**Moderator:** They don't bring up the SMA, the primary care person?

**Respondent:** No. I mean, no. I get the, well, your knee pain is probably due to your SMA. I'm like, okay. I get that to a certain point, but there's got to be underlying. I do a lot of advocating for myself.

**Moderator:** That is clear. It's really impressive, actually.

**Respondent:** You know?

**Moderator:** Yeah.

**Respondent:** I've gotten to a point with my primary care doctor. I tell her I respect her as a doctor, but as somebody who lives in my body, when I ask you to go to orthopedics or wherever, I'm asking you to send me because I obviously need to go. I don't go in and chase medications by any means.

**Moderator:** Do you feel like she somehow prevents you from accessing care for your SMA?

**Respondent:** I don't think she does. I do think sometimes, we're on Tricare. I do think sometimes they try to make it a little bit harder for the doctors. She'll give me a referral where I ask to go. I just don't feel like there's ever the initiative of maybe let's try orthopedics or rheumatology or you know what I mean?

**Moderator:** Yeah.

**Respondent:** To combat some of this.

**Moderator:** Yeah. That sounds like it's with both of those doctors. Let's dig in together and try to help you. Okay. What I want to understand now is treatment for SMA specifically. I have written down here that at some point, you were on Evrysdi liquid. Is that correct?

**Respondent:** Mhmm. It is.

**Moderator:** Okay. So tell me, when did you start the Evrysdi? Do you remember?

**Respondent:** I don't remember exactly. I think it might have been in, like, November '21 August or November in that time frame of '21.

**Moderator:** When did you take it until?

**Respondent:** I stopped it about a year later. I would say probably well, November '22, I believe I stopped it.

**Moderator:** Okay. So, I have so many questions. First, I need to ask what led you to the Evrysdi, and I would need to ask what led you to stop Evrysdi. But, why don't we lead me up to when was the first time you even ever considered taking any treatment for your SMA specifically?

**Respondent:** So when the SPINRAZA came out, it did cross my mind. And, honestly, one of the reasons I don't take it is I don't want the intrathecal injections in my spine. That is a huge reason why I stay away from SPINRAZA. And I personally like some of the Facebook groups I'm in and stuff, yes, I've seen individuals benefit, but nothing enough for me to say I'm going to do this every four months. So that's why I don't do the Spinraza.

**Moderator:** Can I pause you for a second there?

**Respondent:** Absolutely.

**Moderator:** What is it about the intrathecal injection that is such a downside for you? Why is that a huge, like, stay away from that one?

**Respondent:** Well, I just don't want to go every four months and have them injected into my spine, and I know there's sorry. I know there's ports that they can put in.

**Respondent:** I just don't want to have a port placed into my back or my neck or whatever. Just a personal choice more than anything.

**Moderator:** Is it the inconvenience of getting there? Is it a fear of feeling pain? Or is it a fear of something going wrong or just too much invasive? Help me break it down for me a little bit.

**Respondent:** I think my biggest fear is something going wrong. Just as when you do anything, any surgery, any injection, there's always that risk. And then part of it too is just getting there and possibly some of the side effects. I've seen a lot of people go down with migraines and things. So that does play a part in it for me, but I would say my biggest fear is something going wrong.

**Moderator:** Okay. It's interesting you said in your Facebook pages, you also didn't see a huge upside from your peers online. You didn't see people benefiting that much. Is that what you're saying?

**Respondent:** From what I've seen now, I'm not in every Facebook page. I'm in a limited amount. But just from some of them that I've seen, it's not enough to make me say this is worth it.

**Moderator:** Okay. How did you even find out about Spinraza? Do you remember how that first even crossed your mind?

**Respondent:** Probably one of the Facebook groups I was in.

**Moderator:** Did you have the neurologist then who you still have now, who you're trying who you're leaving?

**Respondent:** No.

**Moderator:** You probably were living somewhere else.

**Respondent:** Yes.

**Moderator:** Right? Because you move pretty regularly. You don't have a consistent neurologist.

**Respondent:** Yeah.

**Moderator:** Do your neurologist ever bring up the SPINRAZA with you?

**Respondent:** Yep.

**Moderator:** You had at the yeah.

**Respondent:** Yep. So the neurologist that I am leaving, he was actually pushing for Spinraza. He actually when I first started seeing him back in 2019, he was pushing for it, I would say. And I just said I wasn't comfortable at that time. And then, so I just didn't do anything at that point in 2019 when I started seeing them.

**Moderator:** How was your SMA at that point?

**Respondent:** I mean, I didn't have an ability to run. I lost that probably back in 2006 after I had my first daughter. I would say I was pretty stable, pretty well off.

**Moderator:** Okay.

**Respondent:** So back in 2019, I would say I was still pretty good. I had some knee issues with my right knee, but otherwise, I was pretty.

**Moderator:** And were you able to, like, by that point, you were not walking upstairs. Is that what you're saying?

**Respondent:** Good. I would use railing. I would pull myself.

**Moderator:** Okay. Really?

**Respondent:** Yeah. So anytime I do any type of stairs even today, I have to essentially have a rail and pull myself up. It was easier back in 2019 than it is today.

**Moderator:** But I forgot to ask you. How does your SMA affect you today?

**Respondent:** I cannot run. I cannot squat. Well, I can squat down. I can't get back up without using my arms. I use my arms to get up from any seated position. Obviously, my gait's affected since it's been affected for a long time, like curbs and things of that nature. I have to have somebody pull me up, or I have to hold on to somebody to get a curb.

**Moderator:** Do you use any kind of mobility assistant, like a cane?

**Respondent:** Nope.

**Moderator:** You don't use a wheelchair? I know you mentioned the wheelchair before. No.

**Respondent:** Mhmm.

**Moderator:** Okay.

**Respondent:** Nope. I don't use any type of assistance.

**Moderator:** Okay. And you're able to lift things? I mean, I see your arms moving, but does it affect your upper body at all?

**Respondent:** A little bit. My upper body, I'm actually fairly well off. I did notice, like, we replaced a gate, our fencing in between our houses. Trying to lift a 50-pound bag of concrete, it's out of the question. Things like that. I do have limitations on what I could lift, but yeah.

**Moderator:** But you're remarkably, though, you have a lot of physical capabilities, though, still.

**Respondent:** Yes.

**Moderator:** Yeah.

**Respondent:** Yeah.

**Moderator:** Yes. That is remarkable. Okay. Sorry to interrupt. Do you feel like today your SMA is stable or slowly progressing or more rapidly progressing?

**Respondent:** I'd say probably slowly progressing. I see little things. I feel like I maybe fall a little bit more often, and I go in spurts almost. I might fall two or three times and be good for a couple of months. Noticing that some things I really can't lift that I used to be able to.

**Moderator:** 50 pounds of concrete.

**Respondent:** Yeah. Yeah.

**Moderator:** Okay. So let's go back to your timeline. So 2019, your neurologist was pushing the SPINRAZA, and you're like, look, I'm just not comfortable. It sounds like you're describing the potential what you understood to be the upsides, not outweighing what you felt were the downsides, if that seems okay.

**Respondent:** Yes.

**Moderator:** Okay. And then what led to going on the Evrysdi? Talk to me about all the little details of how that ended up happening.

**Respondent:** I probably heard about it through another SMA page or the current one I was on. The convenience is what attracted me to the Evrysdi. Let's hold out hope that this would be a great medication that would potentially either keep me where I was at or maybe show some improvements. So that's why I chose Evrysdi.

**Moderator:** Did you bring it up to your neurologist or did the neurologist bring it up to you?

**Respondent:** I brought it up to him. I came in with my papers printed so he could sign them.

**Moderator:** Isn't that fascinating?

**Respondent:** Yep.

**Respondent:** He was on board.

**Respondent:** Oh, yeah. He was always on board for me to be on some type of treatment.

**Moderator:** Mhmm.

**Respondent:** And when I would go in for my every six months checks, he'd be like, do you want to go on the Spinraza now? Nope. Nope. It was me. A 100% me that stopped that. It was not him at all.

**Moderator:** But it's interesting. I'm not good at remembering when Evrysdi came on the market. But my sense is that it was on for at least a year by the time you got on it, that it was available for at least a year, I think.

**Respondent:** I think so. Yeah. And maybe I went on it a little bit earlier, but this is kind of like what I'm trying to remember.

**Moderator:** Right. It doesn't matter. It's all but yeah.

**Respondent:** Take a little while in order to get it processed. I had some denials through insurance, ended up going on a foundation. It wasn't a quick in and out process for me.

**Moderator:** What do you mean a foundation? You got denied a couple of times with insurance. Is that what you're saying?

**Respondent:** Yeah. So our insurance denied me. We appealed it. I feel like it was a generic denial, something to do with my age, which at that time made no sense to me. So we submitted all my background so they could see why I needed it, and I was still denied. So at that point, I think it was the Genentech Foundation, they picked me up or provided the medication to me through their foundation at no cost to me.

**Moderator:** Wow. How did you find out about that? Or how did that come about?

**Respondent:** So when I applied for Evrysdi, I was assigned a representative from Genentech. They reach out to you like, hey, we're here. Walk through the steps with you. Any questions? Basically, just your friend from Genentech to help you through, which is sweet. I like it.

**Moderator:** How was having that person?

**Respondent:** I enjoyed it, actually. She checked in on me quite often and just tried to encourage me to work through it. Obviously, couldn't tell me a lot of things, but was baffled that I was denied because she had several others on my type of insurance who were getting approved. Still to this day, kind of baffled why I was denied. But anyway, she told me about the foundation. You had to apply, and there were some income limitations and guidelines and stuff like that. So yeah, that's how I got on it.

**Moderator:** Okay. And so you stayed on it for a year. How did that tell me all the ups and downs, ins and outs, or what was it like for you to be on it?

**Respondent:** I started out on it. I can't remember. I think it was five point five mls was the full dose. Did okay for a little while, and then I started noticing a few side effects. I was having a left foot drop. I had noticed a little bit where my left foot would almost slam more when I walk.

**Moderator:** What does that mean exactly?

**Respondent:** Like, I was having more trouble controlling my left foot. I drive independently, and I was getting in my car one day and noticed I was picking up my left leg and slinging it in my car. I had almost lost some function of my left leg or weakness. I was becoming weaker. So I came home and thought about it and took myself off of it for a little while. Just to see, like, hey, did this improve? Did this not improve?

**Moderator:** Just decided on your own to do, like, kind of an experiment?

**Respondent:** I did. So I was like, I think I went off of it for about two weeks just to see what I was feeling. Was it in my head? Was it not in my head? And I felt like I was feeling better. Didn't really notice the foot drop or the leg weakness. And then I was like, well, maybe it was a fluke.

**Moderator:** Okay.

**Respondent:** So then I went back on it, and I was starting to notice some weakness again. So then I reduced my amount I was taking a little bit because it's just what I do. Before going to my neurologist and saying, hey, I want to take half a dose, I wanted to try it myself just to see. So I reduced it. I think I started taking about three, three and a half ml just to see. And I did notice that the foot drop or the leg weakness really wasn't there, but I started to notice some personal side effects, like, I guess, intimacy side effects that I wasn't liking.

**Moderator:** And do you think they were would you feel comfortable sharing them with me? I know we're being recorded, but.

**Respondent:** You're fine. And I don't know if it was exactly the medication, because I have had a hysterectomy in life, but I just started losing any desire for sexual activity, which really was not me. So I decided to go back off the medication again. I felt like they came back. And I don't know. It could have been coincidence, could have been whatever. I just have mentally tied it to that, and I don't find the weakness. I didn't have the left leg weakness and the foot weakness while I was off the medication. So I decided to go off of it. Well, back up a little bit. Sorry. I ended up having a procedure done. I had some liposuction done in November '22, I think. And I had to go off of the medication just because they weren't quite sure if there were any side effects. So I went off of it, and I just kind of made the decision. And even still today, I don't have some of those side effects I was having while on the medication, so I do feel like it was really the medication.

**Moderator:** Yeah. It's interesting. The libido thing is really interesting. That plus the foot drag and that you seem to feel better seem to be negatively correlated for you with being on medication. Like, when you're on the medication, you felt worse. Did you ever talk to your doctor about getting off the medication? It sounds like it was a very self-managed I mean, on some level, your SMA whole experience sounds self-managed.

**Respondent:** So I did talk to my neurologist about going off of it. He did not support me. He highly encouraged me to stay on a form of medication. He's like, there are studies out there. There are statistics behind these medications showing they're beneficial. And I said, well, with all due respect, I'm staying off of it. We can talk again in six months. We had talked a little bit here and there, and I ultimately was going to go back on Evrysdi at about a half dose just to give it another try. I thought I'm older, maybe life changed, maybe my body's changed. I don't know. I'll give it another go. Again, I did run into the roadblock of insurance. Insurance did deny me again.

**Moderator:** Wait. Wait. Hold on. When did you decide to go back on half dose? When were you going to do that?

**Respondent:** So we're in October. I would say it was probably in June of '25. I was going to go back on it.

**Moderator:** Okay. Let me pause you for a second. Tell me about that because that's a pretty that's like, you had made this decision to not go back on after the liposuction of twenty-two. And then two and a half years later, you're like, I think I'm going to try it again. Talk to me about what was up with that.

**Respondent:** Mainly just I've seen some decline in my mobility and my doctor keeps asking me to go back on, keeps telling me all the reasons to go back on, and I thought, okay. Maybe I'll actually listen.

**Moderator:** Can I pause you there for a second?

**Respondent:** Yeah.

**Moderator:** What do you think I'd love to get some detail there on, like, what did he say that actually motivated you to consider going back on? What were the pros and cons going through your mind in June '25?

**Respondent:** He just really tells me and he's told me pretty much from the beginning, there are studies and statistics that show it is beneficial. Patients are seeing either an improvement or a steady hold, not declining or improving. And he keeps telling me, there's a reason for this medication. There's a reason why we're pushing. Of course, he always threw SPINRAZA back out there to me, but I kind of said, that's off the table. If I go back on anything, I'll go back on Evrysdi. And so that's just kind of where I was.

**Moderator:** How did you feel about his sort of pushing, like, continually bringing it up and saying there's a reason for this medication or these studies?

**Respondent:** I can't say I was opposed to it. I'm not shy. So I've had a couple of conversations with him that sorry. I respect him. I know he's a very well-trained doctor. He's excellent in his field, but at some point, you have to respect me and my choices.

**Moderator:** Yeah.

**Respondent:** I'm not mad that he asked me, but I've stood firm a couple of times. Like, I've told you this isn't what I want to do. I'll see you again in six months. That's just where I'm at.

**Moderator:** Ultimately tipped the balance for you from holding off to, like, okay. I think I'm going to try. What was it?

**Respondent:** I think just to see, maybe because I was declining a little bit, I felt like that.

**Moderator:** What were the specific things that you felt like were declining?

**Respondent:** I think my stamina has gone down a little bit in some areas. And, obviously, my hyperextension bothers me with my knees a lot, and I thought, oh, maybe if I can at least sit idle where I'm at, I'd rather sit idle. If I can't improve, let's not get any worse. I'm 43. I still have a lot of life left. And I know I can always come back off of it if I get the same side effects. I know that this isn't permanent, I guess. I know that I do have the option of coming back off.

**Moderator:** Did you talk to anybody about the decision to go on the medication? Besides your neurologist, anyone else you would talk to about this decision?

**Respondent:** I mean, I've chatted with my best friend about it. Just in casual conversation, but no other medical professionals.

**Moderator:** What about anybody else? Is it something you talk to people about on your SMA pages or your primary care physician or your husband?

**Respondent:** I mean, I've thrown it out to my husband, and he supports whatever choice I make. Ultimately, it's my body. I'm the one that lives with it. I do read on some of the SMA pages when there's people having side effects just like I was. There are people coming off of it just like I chose to. There are people having great you know, either staying steady or gaining some abilities. So I feel like it's very individualized, maybe.

**Moderator:** Yeah.

**Respondent:** It wasn't something I went into blindly. I didn't wake up on a Monday and be like, well, I'm just going to go off of this. It's something I felt like I had to do to decide if this is really what it was. Is this the medication, or is this the SMA?

**Moderator:** You mean, in past June, you were like, look. I need to really make sure that.

**Respondent:** Well, yeah. And I just my whole experience through. Let's go back on the medication this past June, and let's just see if I can hold steady. Let's see if the foot drop comes back.

**Moderator:** Okay.

**Respondent:** I look at it as a big picture. I've talked to my best friend. And, of course, she's like, you should go on it if it's a medication that'll help you. But I've told her, here's where my holdup is. And that's kind of where I've come to the conclusion of why not try it again? It's not permanent forever. That's the kind of the way I look at it. If I'm seeing a hold steady or an improvement, okay, I can stay on it. If I start declining or seeing that maybe I'm having a foot drop, can we reduce the amount I take? Do I just go back off of it?

**Moderator:** Okay. Alright. So June 2025, you decide to go back on. What happens?

**Respondent:** So my neurologist submits the paperwork to my insurance, and I'm denied. And we did the appeal and just denied again, more so just age. And so I do have the option to go back through the foundation, and I just never really applied to go back through them. I guess by that time, I knew that I was potentially going to leave his office. So why start going through the foundation if I know I'm probably going to leave and go to another neurologist? So I just sit idle, and I'm waiting. I go in December to my new neurologist and just want to have that conversation with him and see where he's at, kind of see where his knowledge is and his take on.

**Moderator:** When did you decide to switch neurologists? When did that happen?

**Respondent:** I think probably in July, August, I was having a lot of issues with my knees. And I think probably what ultimately sealed the deal for me is when I went in to see him in the June time frame, and he basically told me my primary care is the one who basically has to control the pain and stuff like that. I thought, I've been coming to you for so long. No. I need somebody who's actually going to help a little bit more.

**Moderator:** Yeah. It's interesting about I'm just interested in staying here for a second about these this you that you had made a decision after these two and a half years or whatever, if I'm getting the time right, to finally go back on Evrysdi. You have these two roadblocks of being denied twice. It's hard for me not to express my frustration about that, but whatever. How did that make you feel? I must just say my feelings. How did that make you feel when you got that second denial because of your so-called age?

**Respondent:** I mean, it's irritating, but I'm almost not surprised. It's like I feel like everything's kind of been a fight. I had to fight to go to see the SMA doctor out in Utah. I had to fight insurance for that. I have to fight to see doctors who I want to see. I've seen local orthopedic doctors here for some of my knee issues. Wasn't getting what I thought was quality care, so I had to fight to go to Vanderbilt. It's just like a constant fight. And I guess so it's par for the course. I knew it was going to happen.

**Moderator:** When you thought about the foundation as a potential next step, how did that make you feel? What would it have entailed for you to have worked with the Genentech Foundation to get the Evrysdi through them? I'm asking two questions. Literally, what did you have to do, and how did that make you feel, the prospect of doing that?

**Respondent:** So I just had to apply through them, and I had to tell them what our yearly income is. They do a checkup every so often. Like, hey, is your income still here? I'm grateful that they offer it. I wouldn't say you've got to jump through any hoops or anything. Of course, there's a little bit of paperwork, obviously. And they do have it set. Like, if you make over a certain amount, then it's like $30 a month or whatever. Don't quote my prices, but it's not just a no. Okay. So your income bracket is from here to here. Well, then you paid this portion of it or whatever. So I'm grateful they do have foundations like that that can help people.

**Moderator:** How did the prospect how did you feel when you were because it sounds like you were at this fork. Right? Like, that you could have either reached out to the foundation or decide to just put it on hold for my next doc to talk about with my next doctor. I guess I'm trying to understand why you decide to just to understand a little bit more about why you didn't just continue on. Was there some sort of sense of, like, I don't know, like, didn't want to deal with it or, like, this is a pain in my butt or, it wasn't worth it or I don't know. I'm just trying to understand a little bit more.

**Respondent:** I didn't apply for the foundation this time just because I knew I was going to switch neurologists, and I didn't want to go on it, you know, if Evrysdi or apply through the foundation. And then here comes December. I'm leaving that doctor to have to do it all over again. I've been off of it for this long. What's a couple more months? Like, that's honestly what my mindset was when I was denied. Why apply to the foundation if I know I'm not staying there? I knew if my insurance picked it up, I could stay consistently. It's just a change of doctor.

**Moderator:** About the program. Is there some way in which the foundation coverage is tied to a specific doctor? So if you had switched doctors, you would have to reinstate the coverage or something?

**Respondent:** Honestly, I don't know. I guess in my mind, that was my thought process. Like, I didn’t do the research. The first time I was on it, I knew I was staying with my neurologist. This time, I was like, why am I even going to apply if there's going to be more paperwork in three months? Like, let's just do this.

**Moderator:** Right. Right. Right. Right. Yeah. So how do you thank you for putting up with my thousands of questions too.

**Respondent:** Oh, no. You're good.

**Moderator:** How are you feeling about the pro like, how are you feeling about you and treatment moving forward? Just for in general about you and SMA treatment broadly.

**Respondent:** I'm hopeful. I'm hoping that there's more advances. I do see the younger crowd, the ones that do the Zolgensma, how well they've done on it. I'm hoping us old folks, we get some of that too. I'm open. I'm open to treatment, but for intrathecal and stuff like that, it would have to be I'd have to see a proven hardcore record for me to say I'm jumping on that treatment. And I know there's no clear cut for every you know, just because this person on SMA did well doesn't mean I'm going to. I get it. It's very individualized just like SMA is. But I guess I just don't see enough people saying, I've gained for me to say this is the path I'm going to take. So that's probably why right now, why I'm looking at the Evrysdi.

**Moderator:** Are you aware of any besides Zolgensma, are you aware of any other SMA treatments coming down the pipeline that might be?

**Respondent:** I feel like I am, but I can't remember right now. Like, I feel like I've seen one that they opened up, and maybe it was the Zolgensma that they've opened up into, like, an older not under two. They're, like, up to 10 or something like that. I think that's what I'm thinking of.

**Moderator:** Yeah. Zolgensma is being tested on two to eight two to less than 18 years old, so. Have you heard of anything else coming down?

**Respondent:** There's a guy in one of my groups. I know he was on a trial. I don't remember what it was called, but that's all I really know right now.

**Moderator:** Yeah. I guess there's a higher dose of SPINRAZA that's being tested.

**Respondent:** Oh, yes.

**Respondent:** I heard that one. I know the tablets are out for Evrysdi.

**Moderator:** Right. Yeah. There's a higher dose of SPINRAZA, the tablets, and then there's a medication you would take in combination with either Evrysdi or Spinraza called an anti-myostatin either through that's being tested in Scholar Rock or Roche. It targets muscle growth specifically. You'd have to take it in combination with the. Get rid of that one.

**Respondent:** I think I have is that the peptide one? I want to say someone posted where it's like a peptide. I was trying to see if I could tell you really quick.

**Moderator:** Someone will tell me. Isn't it great that I'm looking it up as we someone's writing it while I'm googling it.

**Moderator:** Yeah. It's an anti-myostatin SMU peptide.

**Respondent:** Okay. So I've heard a little bit. One of the ladies posted in the group. Something to do with the peptide. It had been tested, if I recall correctly, in a I think it was a gentleman who was paralyzed, and he has the ability to walk now due to this therapy. It's the article she had posted.

**Moderator:** Yeah.

**Respondent:** Yeah.

**Moderator:** I don't know about that specific ability, but there is this Scholar Rock study and they're testing these anti-myostatin. Ugh. I'm so bad with these chemical names.

**Respondent:** Oh, you're good.

**Moderator:** Oh, no. You don't want to hear me try to say this. Pitagramab. That's totally wrong. But I don't know where the peptide situation comes. I'm not a chemist or whatever. But,

**Respondent:** Yeah.

**Moderator:** What are your thoughts about those I mean, well, first, let's start with a higher dose of Spinraza. Any thoughts about that?

**Respondent:** If it works for others, I'm all for it. Right now, it's not on my radar.

**Moderator:** Any thoughts about Evrysdi in a tablet versus the liquid?

**Respondent:** I think it's awesome. It sounds like you can travel a little bit easier with it and maybe ease of it didn't taste the best in the liquid form, so maybe the tablet's good. I don't know.

**Moderator:** Does that make any difference to you, the fact that it's available in a tablet in your interest in taking Evrysdi, the fact that it's in tablet now and not just liquid? Did that matter at all to you?

**Respondent:** No. I'm okay either way. I mean,

**Moderator:** Yeah.

**Respondent:** I guess, if I traveled a lot or something like that, maybe the tablet would be more convenient, but I just took my little cooler.

**Moderator:** Okay. So are you I mean, just broadly, to what extent do you feel connected to the SMA community, would you say?

**Respondent:** I wouldn't say I'm I'm probably about a 30. I don't really get super involved in the little community walkathons or rollathons and stuff like that. I read on some of the SMA groups and stuff. I don't have any close friends that have it. Honestly, that's where I get most of my information. I either read on the estimate groups on Facebook, might pick up an article here and there, that type of thing.

**Moderator:** What do you say article? Like, where are you getting those articles?

**Respondent:** Sometimes people share them on the SMA groups I'm in, or I can was it SMA? Trying to think of the page now. There's an SMA page. It's probably just more for the yearly conventions and stuff they do. I just read articles that people.

**Moderator:** You don't get any, like, website or, like, there's a Cure SMA and.

**Respondent:** Cure SMA. That's the one. Yes.

**Respondent:** On there sometimes. Yeah.

**Moderator:** Would you say there's any you know, some people I meet are really involved in the SMA community and some people are not, and then there are people in between. Anything you can tell me to help me understand why you feel like you're only a 30? I'm assuming that's out of a 100. But, like, why.

**Respondent:** Yeah.

**Moderator:** Yeah. Like, do you have any ambivalence about being further part of the SMA community, or do you not really identify with the SMA community, or does it make you feel a certain way?

**Respondent:** I guess it's just a choice. I don't exclude myself by any means. I'm part of this community with them. Most of it's, like, two hours south of me, where.

**Moderator:** People are meeting in person, you mean?

**Respondent:** Yeah. In my local community, I don't really know of anybody. In my state, there's a couple of ladies I talk to via Facebook in my state, but I've never met them.

**Moderator:** Do you wish you were more involved with the SMA community, or are you sort of just good with the amount that you have now?

**Respondent:** Yeah. I mean, if time allowed, I would be more involved, and maybe if there are things closer. I just can't drive two hours one way.

**Moderator:** Right. Totally. Yeah. Yeah. If you were to think about well, first, you get to meet with this how did you find your new neurologist?

**Respondent:** I googled just local neurologists in the area. There were two that I was interested in, and I'm on a mom-to-mom group. And this neurologist that I'm going to has a lot of patients who respect him. Now they're not SMA patients. They're just neurologist patients. They go there for whatever needs they have, but I figured it's worth a try. There's nobody in my area that specifically is SMA specific or whatever.

**Moderator:** What kind of, do you live in a rural area, suburban area, urban area?

**Respondent:** I guess it's kind of rural. We're not far from a big city. We're kind of outskirts, I guess.

**Moderator:** Okay.

**Respondent:** About thirty minutes.

**Moderator:** Okay. Imagine that you at your next doctor's appointment at the neurologist, what do you think you might say to the doctor about treatment and possible treatment? How do you think you might do you think you'll be the one my sense is do you think you'll be the one to bring it up? Almost I'm, like, ridiculous asking that question, like, duh.

**Respondent:** Yeah. No. You're good. Yeah. And that's part of what I want to talk to is, tell me what you know about both of them and tell me your reasonings why you're going to sway towards one or the other. Maybe he's got the magic word to say, go on, Spinraza. This is where I believe it's going to take you, or, hey, stay with Evrysdi. Here's my reasonings why. That type of thing. I want to know how involved he can help me get also. If a clinical trial comes around, how do I look into that? I know I can apply to some of those on certain sites, but what do you know? What other avenues do you have? Who else do you think I need to see? I just want to see what his outtake is.

**Moderator:** Ultimately, what do you think is going to be most influential in your decision to start or not start treatment? What do you think will be the biggest factor for you?

**Respondent:** I mean, if he's got great information and maybe some success stories for either one, sharing those with me may sway me. Right now, I do know that I'm going to go in, and I'm probably going to ask to go back on Evrysdi. I want to go in at half strength to try it to even if I have to taper up. That's my goal right now. If I was sitting in his office right now, I would say, I'd like to go back on to Evrysdi. This is where I want to start, and this is why I want to start here.

**Moderator:** If the doctor says I don't want you to start on a half a dose, would you just do it anyways without?

**Respondent:** Probably.

**Moderator:** Yeah.

**Respondent:** I know that.

**Moderator:** No.

**Respondent:** Shocks you, doesn't it?

**Moderator:** It doesn't. It just.

**Respondent:** We know.

**Moderator:** Cracks me up. It's not shocking based on what you said before. It's hysterical.

**Respondent:** No.

**Moderator:** No.

**Respondent:** I know.

**Moderator:** I think it's funny. Is there anything that you could wish for, like, to help make the path to treatment better for you? What would make you feel more confident or make it just feel better about potentially restarting a treatment? What would help you right now? Imagine I think you're and that isn't till December. Right? That,

**Respondent:** Yeah.

**Moderator:** If you could wave a magic wand, what would you say, gosh. I wish the doctor would do this or the manufacturer would do this or the world would be like this or the blah blah blah,

**Respondent:** For the current treatment that's out or just in general?

**Moderator:** Whatever. For the current say you were going to go back on Evrysdi, what would make that easier or make you feel more comfortable?

**Respondent:** Just not finding any decline. I did have it before. And maybe this is where I'm pivoting in my life to say, giving it another try. If I do see the decline, maybe I do try the Spinraza at that time. Maybe that's what I need. I'm not there yet, but maybe this chance helps me decide. Hey. You know what? I'm staying where I'm at. I'm not seeing any decline. This is where I need to be. Hey. You know what? No. I am declining. This is not where you need to be. Maybe you do need to try a different therapy.

**Moderator:** Is there anything that SMA medication manufacturers could do to support you in the possible transition back to treatment?

**Respondent:** I don't know. I think maybe doing a little bit more for the older crowd. I get it. The young kids are young and we want to save them all, but I almost feel like some of us older type three, type fours, we're just kind of left out there. Throw some more type threes, type fours into the mix.

**Moderator:** It's so interesting because you're not the first person to say that to me because, what would that actually look like? What would it be more specific? Tell me about this more.

**Respondent:** I think it is amazing, these two-year-olds, eight-year-olds, 10-year-olds, or whatever, how well they're doing, how wonderful the therapies are for them. Show me what 43-year-old out there has had the same outcome, or 63-year-old or whatever. Maybe try some of the older crowd to actually see what it does. I get it. I get you have to build up. You have to try things. I get it's all learning, but there's a lot of us out there that would be willing to be part of it to open up to just more than the younger crowd. I call them the younger crowd, but you know what I mean? Those under 18 or 18 and under. Show some of us older folks who are type threes and fours. Yes. We're very blessed to be where we are in life, but we still matter.

**Moderator:** Do you feel sometimes that it's not being made clear that the older SMA patients still matter to these manufacturers?

**Respondent:** Mhmm. Yeah. And I get it. I understand the zeros, ones, twos are a little bit more critical than we are. I do understand that concept, but I also feel like we don't matter as much.

**Moderator:** Yeah. I've definitely heard that. Other people saying that too. Other adults, obviously.

**Respondent:** No.

**Moderator:** I'm sorry. That must be a crappy feeling. One last question I have. I asked you about sort of feeling connected to the SMA community and you're linked to these Facebook pages and you're clearly getting information from these sources. Would you ever want are there any other ways you wish you were connected to the SMA community? Like, do you ever wish that you were talking to a fellow peer about SMA? You've talked about not having friends for SMA. Would you ever wish you were connecting with a peer, online or over video or on the phone or on email? I don't even know exactly how the connection would happen.

**Respondent:** I think that opportunity is there. I've private messaged on Facebook a couple of the ladies on the groups and stuff. Just a couple of questions and stuff like that.

**Moderator:** Yeah.

**Respondent:** So I do think that avenue is there. I just I guess I don't necessarily pursue it. I know.

**Moderator:** Do you wish someone would proactively reach out to you?

**Respondent:** No. Not really. I think I'm content in that area. I have enough support through my husband, through friends who are not SMA.

**Moderator:** That's nice.

**Respondent:** They may not exactly understand 100% what I'm going through, but I know that they are there to support me. I do know that there's a couple of SMA women's groups. Some of us all go down there and say, hey. This or that, and we all kind of chime in. So there is a little bit of a sisterhood there. So I don't feel like I'm outcasted by any means. And I do know if I wanted more, I could have more.

**Moderator:** Great. Thank you so, so much for participating, for putting up with my endless nosy questions and just being willing to share so much. It really was enormously helpful. And I know this is a long interview, so thank you for persisting through all of it. And hats off to you being an amazing health advocate. I oftentimes wonder what are the factors that lead people to be able to be amazing advocates because a lot of people aren't. And it's really moving to hear that you are.

**Respondent:** Well, thank you.

**Moderator:** Yeah. I wish you all the best.

**Respondent:** Goodbye.