Respondent: Yes. I am.
Moderator: I'm seeing you're a Buckeyes fan. Do you live in Columbus, or is it like you used to you went to school there or have a kid who went to school there?
Respondent: Well, so I we used to live in Ohio. We moved to Georgia about sixteen years ago. I went to Ohio University, not the Ohio State.
Moderator: A liberal arts school?
Respondent: Yeah. Yep. So they didn't have a football team, so you grow up with the one that has a football team. I mean, they had a football team, but just not a very good football team. Right?
Moderator: Yeah. That's an important distinction. I actually used to work out with, a woman like, I am not an athlete. This is we'll we'll get to what we're supposed to start with. I booked a woman in my gym who I became very dear friends with was the captain of her field hockey team at Ohio State, and it was, like, my first entrance into, like, at superior athlete land. Like, I just never grew up where I was just and her husband played for the baseball team. It's it's, like, remarkably driven human beings. Anyhoo, thank you so much for joining. Let me give you just my intro, and then we can just get to talking. It's really nice to meet you. I'm an independent market researcher. This is what I do for a living. I ask people typically somewhat nosy questions, and then you usually don't ever have to see me again. So, part of what makes the nosiness a little bit more tolerable is that I have no idea who you are, and we'll just keep it that way. So don't tell me your last name or the name of your doctor. I don't I don't know what your phone number is or how to contact you or who you are beyond Michael who went to Ohio University. I do have a few colleagues listening in who just really wanna understand everything I'm gonna ask you about your journey with SMA. And we are recording, but it's just for research purposes. Your name doesn't get attached to that or anything like that. Just here everyone's here just to learn and to understand your experience.
Respondent: I'm an open mic. I'm not really too concerned about any of that, but I appreciate it.
Moderator: Okay. Good. Two last things I'm required to say. One of them is my most formal thing I will say is that if by any chance you mentioned that you've had a less than ideal experience on a medication that my client makes, I have to write up what's called an adverse event report, and I might ask you some additional questions about that. Finally, you are you are my first interview in this study. So sometimes what happens in the first interview, like, I'm sorting out the timing just to be completely transparent. And so what'll happen is that I'll sometimes be going down, I really wanna understand. Also, I'll look at the time and say, oh, no. I need us to keep moving, and then I might interrupt us and move us along. So this is my upfront blanket apology for any of my interruptions. Are you good with everything you said?
Respondent: I'm good with that. Just cut me off.
Moderator: Thank you. Okay. I got I have permission to cut you off. Okay. So can you tell me a little bit about yourself? Like, how old you are, if you live with anybody, how you spend your days, just so I have a sense of who you are.
Respondent: Yeah. So I'm 50 year old 50 years old. I'm married. We have two kids, but kids are from a previous marriage, but I've been in their life. Corona was three, Jaden's eight months. They're now 24 and 22, out of the house. We have a little yeah. Thanks. We have a little dog, a little Morkey. I just quit working back in October 2024. So my days are now filled with, I don't know, a lot I walk the dog every day, sort of fiddle fart on the computer, might watch stuff, I read a lot. Not no longer the corporate hustle and bustle anymore.
Moderator: Wow. What kind of work did you do until a year ago?
Respondent: I was a service manager for a telecom company. So I sort of was like a customer advocate liaison between the technical team and the customer, normally, the director VP level.
Moderator: Wow. Okay. That sounds like a high pressure job. Because people if there's anything wrong with anyone's digital or telecommunications in a company, that's very stressful. What prompted you to stop working?
Respondent: My upper body is now being affected, so I was struggling to type. I tried doing, you know, text to speech, and I just could not reinvent the wheel. I just couldn't keep up. I'm a perfectionist at heart. Used to be an a player. I started to fall back into, like, a b minus c player, and that that wasn't good for me.
Moderator: Yeah. Are you on disability at this point?
Respondent: Yeah. So I just got, I went on my employer's long term disability, and then I just got approved from Social Security disability, like, a month ago.
Moderator: Congratulations. I know it's, like, maybe a mixed bag on the congratulations,
Respondent: Yeah. It could be worse. Right? There could be nothing, so I'm grateful.
Moderator: Yeah. So I have written down that you're type three SMA. Is that correct?
Respondent: That's correct.
Moderator: Okay. Okay. So when were you first diagnosed with SMA? How old were you?
Respondent: I was around 10, 11 years old. I don't remember the exact date and time. There was a little bit of, trying to figure out what was going on with me. I kept on falling down when running. Not all the time. So they and it was just my right leg that would give out. So then that started doctor's appointments. And about a year about a year into that journey, I saw a doctor that said you're showing all the signs of muscular dystrophy, which then led me to the Ohio State University and a doctor Mendel who was, a leading pioneer in that that then diagnosed me officially.
Moderator: That's so interesting. I'm I think I meant to ask you not to say any doctors, which is totally fine. Who knows if that doctor's even practicing. Right? It was forty years ago. So it may be irrelevant. So I know that's not really crass, but that's fascinating. Right? Because it seems so long ago, forty years in terms of SMA and development, stuff like that. So interesting. How is your I mean, you just talked about sort of things evolving for you. But how would you describe sort of the role of SMA in your life now? And I don't know. Just talk to me a little bit about SMA's impact in your life.
Respondent: Where it's affecting my life now is just my day to day abilities to do meaningful activities. My wife gets me in and out of bed. She dresses me. Up until recently, she was bathing me. We we recently just got a caregiver just about a month ago that comes in and bathes me and then helps me with little things around the house. You know, as a man, I'm a fix it guy, and I look around the and I can't fix anything. In fact, when I try, I end up causing more issues. So that's it's frustrating. So quality of life is diminished a lot.
Moderator: Thank you for sharing that. When did this when did you start to lose more of your I mean, you talked about your upper body struggling with your upper body to get stuff done at work. When did that evolution I don't know if it was a slow process over time, or was there a certain moment?
Respondent: No. It's been slow. I've I've I've never been super strong in my upper body, but, I mean, I've been stronger than most. But I would say probably over the last six, seven years, it's just started to get worse and worse and worse. It's hard to you know, I don't track it day to day. It's like you look back on it, like, a month or a couple months, and it's like, oh, I'm doing it different, or I'm not doing it at all, or I'm avoiding it. Right?
Moderator: Wow. So you don't track it, number one, but and it's not necessarily something you see changes day to day, but over a couple months, you can see differences. You can see how it evolves. Okay. Broadly, how would you describe your current health, Michael?
Respondent: Everything but SMA, I'm as healthy as any, but I mean, I have my regular physicals and all that. And I mean, I'm just she was like, you are, I mean, in great health, but you have muscular dystrophy. Yep.
Moderator: Wait. Muscular dystrophy?
Respondent: Or spinal muscular atrophy. My doctor does that. My primary care physician, it's sort of funny that they don't really know the intricacies of the different diseases, so they just sort of lump it in as muscular dystrophy. And sometimes I have to remind them, well, it is, but it isn't. Right?
Moderator: That's so interesting. How does that make you feel? How is that for you, the doctor? Your primary sort of not really understanding the intricacies of SMA?
Respondent: I think it used to bother me until I really understood the the what health care is. A doctor can't know everything, especially a primary care physician that's more treating your sort of immediate needs of, you know, a rash or this, that, and the other. So having a very I know I'm very, special. Right? I have a very unique, situation. So it doesn't bother me as much. But when I was younger, looking at the medical, I guess, looking at doctors as they're supposed to be the experts. They're supposed to know. And then, you know, you get a little bit older and wiser and you realize, no. Nobody can know everything. So I I cut them some slack nowadays.
Moderator: That's so interesting. Speaking of doctors, who are you seeing, if anyone, for specifically for health care providers for, SMA related care. Who makes up your do you have an SMA team or is it?
Respondent: Well, no. I have a neurologist that she is, in the world of SMA. So she know and I sought her out several years ago. I wasn't seeing anybody for a long time specifically. But when I started to get wind of possible, treatments, that's when I was like, I need to associate and get connected. And so but I don't see her on a regular basis. I was doing a case study, which I had stopped doing, and she was she knew the doctor that was leading some of that case study. So she would come over and visit and do some of the testing when they needed a neurologist or somebody to do that testing. So then I would have, I wouldn't have a appointment with her, but we had a appointment. Right? It was we would catch up on but I haven't officially went to her office in probably three years at this point.
Moderator: Okay. When you say case study, I'm familiar with the language clinical trial. Is that what you mean?
Respondent: A little trial case study.
Moderator: Okay. This is everything you just said was so interesting to me, and I wanna dig into all of that. So I'm just making note out loud. Okay. So you you said maybe we could just do a little bit of a timeline. You said you weren't seeing anyone for a while. When were you last seeing a neurologist regularly? What when was that? I mean, I know you don't know the exact date, but ish.
Respondent: So to be honest, I was never seeing a neurologist, on a regular basis. So when I got diagnosed at a young age, right, 11, 12 years old when my official diagnosis came in, I went into denial. I was still playing sports. I was still active. I did not want to hear what they were telling me what was going to happen, and so I sorta avoided doctors like the plague. I then went back to a neurologist when I graduated college. I was 24 years old to see if there was any changes or, you know and when I was young, I had asked what should I be doing? Should I be on a diet? Should I be exercising? This, that, and the other. And the answer that I got when I was young was we don't know. Try it and tell us. And so right. And so when I went back at 24, here's almost, like, twelve years later. Right? I went back and I got the same response. The only thing that they did different when I was 24 was they did a genetic test to then confirm and verify because the before I had a muscle biopsy that confirmed what I had. At 24, they did the genetic test was, you know, yes, you have spinal muscular atrophy type three. They were able to zero it in. But since they couldn't give me any hope of what to do, why I didn't want to wrap myself up into doctor appointments. So and I was young and ambitious and I wanted to make money and so I worked. Then I did not then go back to see a neurologist until, that would have been 2017, 2018.
Moderator: And let's pause there for a second because you I think you alluded to this, but I wanna make sure I understand what motivated you to go back. First of all, it's very compelling what you just said earlier. I wish I had, like, four hours to talk to you because you're such first of all, you're very articulate, and you have a really interesting story. So I'm so thank you so much for taking part in this. So helpful. It's been so helpful already. But it's it's striking what you just said. Like, the combination of denial and not getting any hope from the medical that didn't feel like there's anything they could do for you.
Respondent: Right.
Moderator: Yeah. So that okay. And yeah. And maybe there was even sort of negative association with going okay. Alright. So what happened in 2017 and 2018 that prompted you to go back to the doctor?
Respondent: So I was aware of a particular, treatment, but I was not I did not like the way that it was, administered. I I that was not something that I wanted to go down. But
Moderator: Can you tell me more can you pause there and tell me a little bit more about that? I think I know what you're saying, but just wanna make sure.
Respondent: There was a drug called Spinraza and that you had to get a lumbar puncture for it. And that was I wasn't, the way that it was being administered, I just I didn't know if I wanted to do that. So but I had started to hear that there was something on the horizon about an oral medication. So and that's called Everest. And so I I was like, well, if I'm going to be able to get this prescribed, I need to get aligned with a neurologist because my primary physician who just lumps everything together as muscular dystrophy, he was not going to be able to help.
Moderator: Let me pause. Okay. I'm sorry I keep pausing you, but everything you're saying is interesting. It's a problem. You're too interesting. So it's striking that you said in 2017 that you were aware of treatment and also that you're saying that oral was on the horizon. So you weren't going to a doctor. How are you learning about these developments in the SMA treatment?
Respondent: Through Facebook. That was the only thing that I was getting my information from was these Facebook support groups that I was a part of for spinal muscular atrophy. That was where I was getting my information.
Moderator: So interesting. When do you think you joined those groups? Do you have any memory?
Respondent: That I can't yeah. I don't know.
Moderator: Do you think you were in your twenties, your third like,
Respondent: No. I would relate Bloomer to the whole Facebook thing. I avoided it like the plague.
Moderator: Yeah. There wasn't I don't think that Facebook was around when you were in your twenties anyway,
Respondent: Yeah. No. So I think I didn't join until either my very late thirties or it would have been my early forties. I've only been on it for probably ten years, maybe eleven at the most.
Moderator: Okay. So mid two thousand like, 2014 or '15.
Respondent: Yeah. No. Yeah. Ish.
Moderator: What prompted you to take part or, you know, to visit those Facebook groups? And what was your how did you feel about those groups? Yeah. That's something.
Respondent: That's a difficult one as well. Just on the basis of I've tried to not associate myself with the disability disabled community. But, obviously, my reality was becoming I am part of the disabled community. So it was sort of me sort of tiptoeing into that without full fledged being in it. Like, I went to a face to face support group. This was I can read about it and sort of see what other people are doing about it. And if I wanna click to see what these people look like, unfortunately, you know, I'd that's how I was sort of getting used to the idea that, wait a minute, this is where I'm headed.
Moderator: Yeah. Can you tell me I I can sort of guess from some of the things that you said, but if you don't mind in telling me a little bit about why you were trying to keep all of this at arm's length. What do you think what did it feel like? What were you trying to avoid?
Respondent: Prejudice around it that of myself I had and as well. Right? I had a early experience as a kid, prior to all this going down of hearing how people talked about disabled people. And then here I knew that was part of my denial that doctors told me I was going to be that like that as well, and then not wanting to have that pers being perceived that way and being ridiculed and looked down upon. And then in turn, I I I had it myself, which was sorta ugly of me. Right?
Moderator: I just so appreciate your honesty. Was there a specific event or a specific set of events that you remember in terms of the people?
Respondent: Do I remember my my, I have a weird family dynamic, but my I'm not right. Yeah. Adopted father, he wasn't my biological dad, but adopted dad. Married my mom. But anyway, so we were driving down the road. There was a gentleman, and a motorized wheelchair coming down the sidewalk. I would've, I was early teenage years, and he was bumping on the sidewalk. Right? I mean, the little cracks in the and my dad made a an off colored joke. And it that really just knowing that this is I have this disease and that and I don't even know if he was even aware of it. Just based it just came out. Right? And it wasn't I'm sorry or it would just was what it was. I don't blame him. He was, you know, raised in a certain way as well, then then that was sort of common. Make fun of people less fortunate because it's funny, but it's not funny. So
Moderator: When you were diagnosed at eleven ten, eleven twelve, were you told you have a how was it described to you what SMA is? Do you remember? Yeah.
Respondent: Well, so before I was told I had SMA, the way that I got up to the doctors that were more familiar with this, my primary care physician in Ohio knew somebody. He was asking around trying to find out what was going on with me. He couldn't figure it out. This So he sent me to an, a doctor at a local university where we lived. And that doctor described that he he said this. Basically, he had no bedside manner whatsoever. He said, you're showing all the signs of muscular dystrophy. You're gonna be confined, to a wheelchair with no movement from the neck down before you graduate high school. I'm sorry to be blunt, but I just want you to to basically accept or get get ready for what's to come. This is now time to start planning and making the decisions your family needs to make. And then so I had my adopted dad and my stepmom sitting there, both uneducated, and it it was like, wait a minute. We just I just got done with baseball practice. It was hard. It was very hard. There was no bedside manner whatsoever. I think he was trying to, in one sense, look, I'm not gonna beat around the bush. I'm not gonna give you hope. It's time to just swallow the hard truth. Now let's move forward. But as a kid, shoot. That was hard.
Moderator: Sorry. Sometimes when things are just so hard, I have to just pause for a second. It's just so intense. Yeah. Because you've described these, like, lows that you're receiving. Right?
Respondent: Mhmm.
Moderator: But then you're also describing yourself as this incredibly remarkable young person. Right? Like, going to this great university and and being very ambitious and going even going to football game. I mean, like, you're I'm hearing these two things at the same time, and it's striking. Yeah.
Respondent: Yeah. I I truly believe the reason why I was, able to, I think I mean, to do what I've been able to do because of my denial and stubbornness that I was not going to fall victim to what they're classifying and say I was going to be. I think if I would have listened to what they said and internalized that, I think I would have became it would have been worse off quicker, and I don't think I would have, achieved anything that I've achieved.
Moderator: So for you, a lot of your health, you attribute to sort of keeping the the SMA at at bay in some ways. Like, keeping some separation between you and sort of involving yourself in this disease and and, yeah, identifying with with it. So it's also striking so you start you start going to these face do you remember so do you remember even what led you to go to these Facebook groups? Because I'm very I wanna get to the point that it was knowing that there's treatment available that was clearly significant for you. But there's a step before that,
Respondent: So
Moderator: Where you just Facebook groups.
Respondent: Yeah. So I don't know if I joined them before or after, but one of the the, changes that occurred when we moved to Georgia, We went to an abilities expo type conference thing, trade show that had, you know, different things. And the type of wheelchair that I was in, there was a gentleman that was in the same type of chair that was very interested because of the wheelchair I was in. And so he was trying to promote power wheelchair soccer. And and because of my wheelchair, my wheelchair was the type of ones that are very good for that sport. And so really what he wanted was to buy my chair from me. Right? And and then then he was trying to get me to sort of play power wheelchair soccer. And through that is when I was starting to get on Facebook, and I'm thinking that's when either through connections with them that then maybe that popped up that you might be interested in the support groups because he and some of the other players were in these groups that then I'm thinking that's what led me into going, oh, well, let me just go ahead and join or look or whatever. I'm I'm pretty sure that's sort of what happened.
Moderator: And when did you start using a wheelchair? How old were you when you were?
Respondent: So I started I got one right I went I went to a community college right out of high school, but then I went away to a major university right before I turned 21. I transferred. And that's when I got the motorized wheelchair. Because the college I went to was basically built on a hill. But I wasn't using it full time. I would literally ride the wheelchair to class, park it outside the classroom, stand up out of the chair, grab my cane, walk in. Wherever I went, it never went in with me. It stayed out. I wanted to show everybody that I'm not the chair. But then I didn't start using a chair constantly. I had a leg break when I was 34, 35. And then another one, and then another one, and then it sorta led me. So I've been sorta confined to the wheelchair for about eight, nine years now.
Moderator: How did you feel when you're on these Facebook groups? It sounds like you were initially kind of a lurker. Is that a fair word?
Respondent: A little yeah. Lurker. I still might be a lurker. Right? I mean, sometimes I'll post, but I mean, most of the time, I'm just looking, observing, reading.
Moderator: Me too. Most of us. And then there are those people who, like, over post and be like, dude, don't you have any sense that you're sharing this face? Okay. How did you feel reading about SPINRAZA and I guess you probably first start learning about tell me about when you first just started hearing about treatment for SMA on those Facebook groups. Like, that's very important to me to understand how that made you feel.
Respondent: It made me feel hopeful that wait a minute. Because for so long, I heard that there's nothing. There is nothing they didn't even know what was causing it. And then when they were able to identify what the cause was, then it was like, well, it took them this long. There's never going to be anything. And then they hear that, wait a minute, we've I've we found something that stops the progression, then it started to make me feel hopeful. If I can get comfortable with where I'm at today, if we could just stay like this, okay, I can function the rest of my life like this. This is I'm I'm doing it now. This is good. I don't you know, I've gotten pretty bad, but at least I've, at that point, I was still driving. Right? There was I was still working. There was a lot of stuff that I was still bathing on my own. There was a lot. So it was a a lot of hope. But with the administrating of it with Spremerasa, I just I did not like I don't know why I was so against it. I think because I just want something that's non intrusive, that's easy. I don't like spending a lot of time at doctors, this, that, and the other. I wanna live my life. And so when I started hearing about, oh, there's something you could just take. It was like, oh, I I take medicine at night for, you know, pain and to help me sleep, and I take vitamins. I'll swallow a pill all day long. Right? Or I'll take cough I'll take a liquid. That was so when I started hearing about Everesti, that was okay. This is I can get on board with that.
Moderator: Okay. I had written down here that at some point in your history, you have taken both at RISD, but also I've written down that you actually did have SPINRAZA at some point. Is that accurate?
Respondent: That was part of the clinical trial that I just was a part of for the past, year and a half?
Moderator: Oh, alright. Okay. So when did you let me just get these dates down. So when did you take Evrisky liquid from one to one? Do you happen to remember?
Respondent: So I've been on it on two separate occasions. The yeah. So the first time, I started in April 2021, and I took it until April 2022.
Moderator: Wow. Okay. Twelve months.
Respondent: Yep.
Moderator: Mhmm.
Respondent: And then I stayed off of all medicine from that time until, April or no. I think it was more like May 2023.
Moderator: Okay.
Respondent: And then I stopped it in December 2023 and started the clinical trial with SPINRAZA in December 2023.
Moderator: Was it any particular I mean, given that Spinraza was already out, was it a certain kind of Spinraza with a clinical trial? Or,
Respondent: It was the double dose of spend it was the higher dosage. Yeah. That was what the and the reason I started Evrisky again because the clinical trial was based on having been on Evrisky. They wanted to see what the transition would be from Evrisky into to the higher dose.
Moderator: Just to be clear, you have we're on a Frisbee from '21 to twenty two April for a year. You had a year of no no treatment, and then you started the clinical trial in December '23 for the higher dose of SPINRAZA? Okay. And how long were you on that clinical trial with the higher dose of SPINRAZA?
Respondent: Up until April, my last dose of that that I participated in was April.
Moderator: April 20 everything happens for you in April.
Respondent: Yeah. I don't know what it is about April. But it makes it easy to keep track of it all. Yeah. is it is odd.
Moderator: And you're not on anything right now?
Respondent: No. I'm not on anything. I decided to not continue with the trial.
Moderator: Oh, there was a did you opt out?
Respondent: I coulda kept on doing it. I coulda kept doing it. I'm I'm a non responder to that medication. Did not it did not keep me low. I continued to digress the entire time. There was no change. It actually I got worse.
Moderator: Okay. Alright. So, again, 02/2017, 02/2018, you're aware. It's interesting initially that, right, you were so averse to the lumbar puncture. Obviously, you then did that for a year. Any thoughts about back then, what was what was the major, like, for for you and maybe why it was different later?
Respondent: I think the big part was, I'm I was just a workaholic and I was more I didn't wanna take the time off, the travel, to, you know, have to transfer to then possibly have any adverse effects, which I did have, you know, a little bit of that once I did start taking in. I didn't realize what the adverse effects. But I just didn't like the fact of putting needles in my back or just, now my my tune changed when with these support groups seeing that a lot of people were being having more stability on SPINRAZA. I had a different experience with Everest, and it was even more, I don't know. My I my interest was peaked because, oh, here's double the dose of this stuff that other people are saying they're having more stability and actually maybe more gains on SPINRAZA than Everesti.
Moderator: So, like, your perception of the costs and the benefits shifted. Number one, you heard the benefits were being brought were being more in your face. You were sort of seeing them with your from your peers. And you saw that maybe even I could get double that or something like that. But
Respondent: Yeah.
Moderator: Okay. Alright. So
Respondent: And to be and to be honest, I had reached back out to the neurologist to see if I could get back on Everest after that year hiatus anyways, because I wanted to see if I could get the same boost because I got one hell of a boost from it the first time I was on it. I wanted to see if I would get that boost again, but I kept, we we were running up against insurance denying it. And then what's funny, or not funny, or coincidental, is the day that I looked into the clinical trial, is the day that my my advocate for Evriski called and said, hey, I know you didn't want to pursue this anymore because you got denied twice, but we kept pushing and you got accepted. And the trial, like, needed me to be on it in order to get accepted for the trial. So I look at that as a coincidence that, okay, maybe this is something and I need to do it.
Moderator: What what do you mean maybe this is something?
Respondent: Well, whatever your beliefs are. Right? Okay. God, is this a sign that you're giving me something that, okay, this I need realize I'm going downhill and all the stars just aligned. Okay. I'm just gonna go forward with it.
Moderator: So just to be clear, did you go back on Avisdi in order to get in the trial or
Respondent: Yeah. So in April '20 or well, April, May '23, I started taking EverestD again. I had to be on it for six months before I could start the spironza trial.
Moderator: Oh,
Respondent: Yeah. That's why I said I've had a Vrisky twice.
Moderator: Okay. Got it. Got it. Okay. And how did it go for you the second time you were on it?
Respondent: The second time, I didn't get the boost that I did the first time. The first time when I took it, I literally had stopped driving my, accessible vehicle several months prior. I started taking it that first time and within a month and a half, I was strong enough to start driving again. I was able to wash my hair again. I was it was like, oh my gosh. This is amazing. But then what happened is after six months, it's like the bottom fell out and it's like I got worse quickly. And it was devastating and I couldn't and it never rebounded. So
Moderator: Wait. Wait. Was when when was that?
Respondent: That was the first time I've taken Everesti in '20 April 2021.
Moderator: So at the six month point, the first time after you've taken Efrizi for six months, you sort of bottomed out. You had peaked and bottomed out still while you were on Efrizi. Is there okay.
Respondent: Right. And then so since I wasn't there was no testing of my physical ability during that time. They just gave it to me. And then so, if I think that if they would have tested and saw the gains that I was having at two months, four months, even six months, and then tested to see that, oh, wow, he's really gotten weaker and dramatically weaker. But then when we tried to get it renewed, insurance because then they test you once a year, all insurance call was, well, he's gotten worse. This hasn't kept him stable. This didn't do anything. So I don't know if I would have stayed on it longer if it would have came back around. I have no idea.
Moderator: So just so just so I'm clear, I haven't even a 100% got to how you got on the Avisi, but we'll get there. So you got on the Avisi in May in April 21. You had six months of, like, remarkable progress, and then six months of it sort of bringing you back down to where you were before, kinda.
Respondent: I'm not even back down where I was before. Worse than where I when I started it. That's what was so, like, scary.
Moderator: So what prompt that yeah. So what prompted you to stop the Aviso in '22 in April?
Respondent: Because insurance wouldn't wouldn't pay for it. They wouldn't allow me to renew. So there was there was part of me going, wait a minute. This is I got worse. And then the other part was like, well, insurance has denied it anyways. That's the writing on the wall. Again, the stars aligned. Okay. You're telling me I need to stay off of it. I'll stay off of it.
Moderator: Interesting. And I know you just said you think insurance denied it because they did a annual baseline test and didn't see any progress. Is that why your understanding of why they denied you in that year point?
Respondent: That's my understanding. Yes. That's what they've had the letter they sent back to me and the neurologist.
Moderator: It's striking to me that a couple points in your journey that I understand so far, you talk about, I don't really know how to talk about this, like, that sometimes things are kind of like signs. Right? Like, given that you sort of declined on the Abrasdi and the insurance denied you, you sort of had that almost like an acceptance of, like, it was meant to be. Am I under am I getting that right?
Respondent: Yeah. You get that right?
Moderator: Please tell me if I'm misunderstanding because you also said something similar about another time that I can't even I think it was when the insurance when if RISD called you back and said, actually, we the advocate called you back. Actually, we accepted you. We got it for you in the clinical trial, and so you're like, okay. So maybe this was also meant to be.
Respondent: Right. Because it all happened on the, like, the same day. Like, what's the coincident? What's that? I mean, okay. I've if you I can it's almost like hitting me up as upside the head going here, dummy. Laying it out for you. Okay.
Moderator: And the second time you're on Everest for six and the the second time you're on it for six months, May 23 for six months, Tell me again what your experience was with the upris date at that point.
Respondent: It wasn't as dramatic with the, you know, all of a sudden just feeling super strong and and having this increased stability, but I did feel over time that I got stronger. It wasn't as quick as what that first time was, but by the, it was sort of odd. By the time I was getting ready to switch and get on to the clinical trial, a friend of mine said, you know what? You seem to be doing really good here lately. If this new medicine could just keep you where you're at, you probably would be good, wouldn't you? And it was like, yeah. I hadn't thought about it, but, yeah, I'm in a good spot with what my functionality is. It it got until a point to where I could manage life within that the parameters I was at that second time on Everesti. But hence, here I thought, well, let's go for the Gusto and do the clinical trial and get double the dose of some medicine and yeah. My expectations were shattered.
Moderator: Yeah. And so your experience with the double dose was you you were you described yourself as a nonresponder and that you got worse.
Respondent: Yeah. So I they did the loading doses, in December, by the January. Yeah. January, almost the January. It was I I felt like I was worse than where I'd started even with the Everesti that I started six months prior.
Moderator: And so then you were on just to be a 100% clear on timing, you were on the high dose of SPINRAZA.
Respondent: So December 2023 was the loading doses, and my last dose was April 2025.
Moderator: Wow. Okay. So, like, fifteen months.
Respondent: Yeah.
Moderator: Okay. And when did you exit the clinical trial because you like, why did you exit the clinical trial? Was that your decision, or was it just when the
Respondent: My decision, and it was, it was twofold. One was I I was not responding to it. The other was the manner in which the testing was being done. So before I would get the dose, they would do PT testing. And the course of that, I knew I was getting worse. And so they would go through these things and here, do this, do that. And if I couldn't do it, the lady would be like, well, the directions say if you could do it this other way, you still get the same score. And I was like, but doing it the other way discounts the fact that I can't do it the way that I used to be able to do it, but yet the researcher is gonna see you're giving me the same score. How is that showing anything? And it and I just I did not like that. And and I and what bothered me was I'd I realized because this, clinic does a lot of trials for kids. And I realized that if I was a kid, I would wanna get the better score, and I would do that in order to get the better score, but that's not giving a true representation.
Moderator: Yeah.
Respondent: Of what's going on.
Moderator: Yeah. That's just concern. Yeah.
Respondent: And then what was funny was then when I brought it up to somebody else at that place, that lady no longer did my physical test going forward. And I'm just like and and then they were having a lot of turnover there. And then the one girl that I really liked, she didn't leave because of the job that her husband got a opportunity in London. And who doesn't go to London. Right? So they moved to London. And so it was just a lot of different things that it was like, I'm not getting anything from this. I'm not really for sure they're testing in a in a way that is really showing the truth about what's going on. I started to read more about these clinical trials that, you know, as long as thirty percent of the participants are showing either staying the same or sudden improvement, that's all the FDA needs to And then here are the people and that's where I go back to these Facebook support groups. These people are dying for a solution, and they're trusting all this, not realizing that they're possibly going to be a non responder just like the rest. But here, they're putting all their faith and hope, and no one's explaining to them what these trials really are. And that's when I was like, I don't wanna participate.
Moderator: Okay. No. That was but it's really helpful to understand your general orientation towards all that because that is part of who you are in your journey. Okay. So I'm writing down all the things, if we have time, that I wanna ask you. But, how are you feeling so what so tell me about you got off the clinical trial. And in April year, what has your thinking been about you and treatment now moving forward? What is your thinking been for the last few months? What are you thinking about the next few months, next year?
Respondent: I think for right now, I I just don't want to put my foot back into the arena. But I'm not going to close the door on it completely. I don't know which way I wanna go. I feel as if the only two options I have are Evrisky or Spinraza. They I know there's some other things on the horizon, but they've not been approved yet. And so I don't know if I want to be I I don't know what the truth is because I haven't really re looked into it of how long you have to be off of a trial before you're eligible to then join another trial. So there's part of me that, you know, I need to look into that. I heard something that you need to be free and clear for a year. Right? Or the medicine that you're a trial of had to be FDA approved. Once it's FDA approved, then you're free and clear to try whenever. But if it hasn't been FDA approved, you have to wait a year before you can get engaged in another one. So part of me is I really don't wanna be in the arena, but also I've I have a feeling I wouldn't be eligible anyways. And then there's always in the back of my mind, do I just wanna try the Evraz again? Right? Because now it's in pill form. Let's just take a pill.
Moderator: So I was curious about this. This you you brought up my next exact question. So talk to me about your thoughts about you and Evrisky. You and Evrisky and your future, I feel like a Hallmark card. But
Respondent: Right.
Moderator: Yeah.
Respondent: Yeah.
Moderator: Yeah.
Respondent: I wish I would, you know, like, not knowing what woulda happened this last time when I was on Everest, if I would have stayed on it more than six months. Would I have had the same experience I had the first time I was on it? Because that's what's odd. Right? Here I was on something for six months, I felt good, but then I changed the something. Was it that I changed the something? Or was it that here after six months, my body just starts rejecting it or it stops responding? I I don't know. Right? So there's part of me that, well, let's give it another try. And then if third time's a charm, if it happens the same way again, then that's a definite. This is how my body reacts to this medicine. The other is I never stayed on it longer. The c, does it ever rebound after being on it longer than a year? Because I've heard some people say the research shows after a two year period that there's more stability, this, that, and the other. Does it sorta ebb and flow and come back? I don't know. The third being a tablet, right, is now, since I'm weaker, would be a easier way for me to take it because I don't think I can draw it up and take it in the syringe like I was prior because of the weak the more weakness that has occurred over the years.
Moderator: What so that was so helpful. If you're like like, what do you think really is has stopped you at this point from going back on that first date.
Respondent: Probably my own laziness to go make an appointment with a neurologist and have to get somebody to take me down to and fight the Atlanta traffic, and I am not a very good, passenger because I drove all my life and being on the highway with eight lanes of cars and it freaks me out. And then having to then go through the whole process of will it get denied and just the I mean, if it was as simple as calling the neurologist, prescribe it, get it shipped to me, I'd probably just take it. It's the the process. Right? I just don't like the process.
Moderator: Why this I wanna figure I wish I could ask this in a less loaded way. I'm interested in the fact that you don't have you don't see a neurologist regularly. That is striking to me. Can you tell me more about that?
Respondent: Because what does the neurologist do for me when I see the neurologist?
Moderator: K. I don't know what you would answer that. Yeah. Yeah.
Respondent: They don't do anything for me. I I don't get any benefit from seeing a neurologist. I am able to update them on where I'm at, and unless I need something from them that is outside of the scope of what a primary physician who is closer to me can provide, there's no the I there's nothing that I get from that. Unfortunately, it's a I'm going to get, not to give. Right? I mean, I'm there for my my own medical reasons. And that's why when I went to see her in the beginning was because I needed to get her ability to write a prescription that my primary physician could have wrote, but wouldn't have been able to back up in the manner to then fight the insurance to get it approved.
Moderator: Yeah. What does it
Respondent: That sounds sick. Right? I'm just
Moderator: No. It actually sounds like you're in charge.
Respondent: Okay.
Moderator: Right? Like, you like, you were like you know, I am in charge of my health care and, you know, what does it entail for you to go to the like, can you compare going to the neurologist, the logistical challenges of that versus or the realities of that versus, going to your primary care physician? Like, what does that look like in terms of
Respondent: Yeah. Neurologist is probably a forty five minute drive, down a super busy, highly trafficked, scary drive down I 85, traffic. Then it involves, you know, a hospital setting, and then a very quick almost you feel like you're a bothersome because there's so much you there's so many people in the waiting room to see somebody and they're in and out and it's quick and it's and it's just not very personable. The we're going to my primary care physician, it's a ten minute drive back roads to a place to where we joke around, there's familiarity. It's like I'm seeing my friend and we, you know, we just chat it up and she laughs at the way that I talk about, you know, being disabled, and and and it's all good. Right? There's the difference. There there it's the there's a in my neurologist, she's a nice lady, but most of the time I'm not seeing her. I'm seeing a nurse practitioner. And so then the nurse practitioner that I was seeing on a regular basis, she's no longer there. So I would have to get reestablished with somebody new because I I doubt I would see the actual neurologist. And then, there's just no and again, what am I getting from that appointment?
Moderator: I'm gonna what do you wish what that you would and if if you could create, like, the perfect neurologist relationship, what would you wish you could get from an a neurologist? Like, what, in an ideal world, what could they do for you?
Respondent: Shane, here's the latest and greatest, you know, for Michael. Should I go by middle name? Never mind. Anyway so I go by I've been re I'm looking into this knowing your particular situation. Here's the latest and greatest. I know somebody that can get you into this trial or I know what we should do. I wanna get you on this. And then in turn with that, we're gonna get you set up to do these certain activities to help benefit not only the medicine, but your physical abilities. Not here what you know, you tell me. Right? I had to go to her. I've heard this. How can I get that? And then none of it was, well, if you're on that, you should also be doing these things as well. See, I was reading through Facebook groups of people that were, well, here, I take this type of protein and I'm doing this. I don't get anything like that. And then I would rather have a doctor tell me medically, this is what we think will help versus, try it and tell me if it works. Try what? Right?
Moderator: How do you when you're sitting when you're sitting in the waiting room in this hospital setting, I'm just sort of curious. How do you feel? When I'm seeing my waiting to when I'm heading and seeing my neurologist, I feel x. What would you say you feel? Going to my neurologist makes me feel
Respondent: I feel as if I'm checking a box. I feel as if I'm checking a box going, okay. I've checked in with my neurologist. I can say they have on record I've had a appointment in x amount of time.
Moderator: Okay. It's just was noteworthy to me that you said it's a hospital setting. What does it feel like that it's a what's what does it feel like to be in a hospital setting?
Respondent: I mean, I just there's a lot of other, I don't know. It it just feels what's the feeling that you get in the hospital? Almost like, you know how I avoided the whole time with trying to not be sick? I'm in a hospital setting where you're I'm sick. I'm sick. Where even with the the primary care physician, I'm going into a facility and there's other things going on there, But for whatever reason, it's a not a I don't know. It's almost like in my mind, there's a difference. That is more of a acute illness to where neurologist is a chronic illness. And this is a almost like this is bad where the other one is, oh, this is temporary. I don't know why my mind has that, but that's in my mind.
Moderator: That makes total sense to me. Total, total sense. Yeah. You talked about some awareness of there being some treatments in the pipeline. Any any do you know anything about what's out there?
Respondent: I don't know them off the top of my I know there's one that's more associated with a muscle therapy, to actual strengthen the actual and I forget what it's called, and that one's real close. I think it was going to be approved, but then they saw some issues with manufacturing just like the double dose of the, Vimraza. And I got the right? So, and then I know there's a couple other things, but I haven't really followed them because I know they're just still in the clinical trial thing. But, you know, you I get the the SMNA news type of updates. I see that. So I sorta get, you know, when they send out the magazine or letter or whatever, I sorta see what medicines and where they're at in the pipeline. And I don't really pay any attention to them until they start getting a little bit further on.
Moderator: Yep. Okay. Yeah. I that muscle one, I think, I in my mind called an anti myostatin.
Respondent: Yeah.
Moderator: I don't know if that's familiar.
Respondent: That's not. No. Yeah.
Moderator: What are your thoughts about that one?
Respondent: I don't know. That one, I sorta it the way that I initially read it, it sorta seemed like it was to do in combination with the therapy. So then that's where it's like, okay. I would still have to pick Spinrazer or Everesti and then this on top of it. And then my concern is is what's insurance going to pay If the I mean, the other ones are pretty expensive and then you're adding that one as well. I mean and this is gonna sound sort of bad, but is my life really worth that price tag? And I don't know.
Moderator: How do you answer that question?
Respondent: Know. Well, it's hard. Right? You see insurance companies having these profits out the yin yang, and then here, you know but then, I mean, it what? A million dollars or more just to possibly try something that may or may not benefit? I don't know. What could we use that money for other things? Right? I mean, there's people on much worse scenarios than I'm in. Right? I mean, people that don't I'd I live a lavish life. Lavish. I
Moderator: Yeah. Yeah. If you think about the future and the possibility of these treatments for you, what or whom do you think will have the most influence on whether or not you actually go down the because it sounds like if you're gonna go down the treatment route, it's gonna require work for you because it's not already, like, set up. You have a doctor who thinks you have muscular dystrophy who's in the groove. But But if you're gonna go get act like, SMA specific treatment, you're gonna have to, like, find a doc. You don't even have a nurse practitioner. You're gonna have to set it all up. You're gonna have to go to a place that feels really uncomfortable. You might have to fight the insurance. Like, what do you think maybe let me frame that a different way. What do you think would be the biggest help for you in accessing treatment? Say say you decide on some level you would like to get treatment, but you wish it could be sort of emotionally and logistically easier. What would make what what would make it easier for you in those in both of those ways? Sorry. That was a convoluted question.
Respondent: Yeah. I don't I mean, I don't see any change in how I have to go about it. So then what would be the perfect scenario? I don't know. Have an educated primary physician that could just take the place of the neurologist. That would probably be the easiest route.
Moderator: I wonder about, like, an advocate. Right? Like, some sort of it's interesting you described how the Avrisky advocate called you and said the insurance is approved.
Respondent: Yeah. And she still keeps in touch with us, which is cool. She still keeps reached out to me just a month ago.
Moderator: How did that go? What was that what's that like for you when you get those reach outs?
Respondent: I don't know. I sort of felt a connection with her, and we went to one of the little luncheon things and got to meet her or whatever. But, I mean, we would have conversations and, you know, it just I mean, that was really helpful through the whole process of getting on on Everesty of having somebody that was in my corner. I don't think I would have went down the down the rabbit hole if it wasn't for that. Right? So yeah. I mean, it's just nice that I wasn't just a, oh, I got him on it and let's move on. She just continues just to check-in.
Moderator: That's do you ever talk to her about the possibility of restarting the team?
Respondent: Yeah. I had mentioned to her that I had stopped doing the clinical trial and I that I was hesitant about the Everest just based on the past scenarios that I've gone through. And that's when she had said, well, there's also another drug that's on the horizon of getting, you know, approved. You might wanna look into it. And then it was like, yeah. I've heard of it. And then that was it.
Moderator: It's so nice that you feel like she's in your corner.
Respondent: Yeah.
Moderator: Yeah. I this is not me just blowing smoke, whatever. I could not have asked for a better first respondent. I feel so lucky that I got to talk to you. You are so self aware. It's striking. And so it's just really, really helpful. And, also, just I really enjoyed talking to you. So
Respondent: Yeah.
Moderator: Thank you so much for sharing everything. I have no idea. I am actually married to a gator, a Florida gator who may
Respondent: You gotta tell them that us buckeyes aren't that bad. We're not that bad.
Moderator: I feel like I'm always listening to, like, Michigan, Buckeyes, Gator. Even my, like, 14 year old son, when he uses the the, pronoun is it the pronoun? He says, well, we. And I was like, Harry, who's the we? He's like, the Gators, mom. I was like, oh, brainwash.
Respondent: That's funny.
Moderator: Listen. I wish you all the best. You've been so great, and, thanks so much for your time.
Respondent: Alright. You too.
Moderator: Okay. Bye.
Respondent: Bye.