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*Oct 1, 2025 | 3:00 PM | R01 Transcript*

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Time: 3:00 PM

**Moderator:** I'm seeing you're a Buckeyes fan. Do you live in Columbus, or did you go to school there or have a kid who went to school there?

**Respondent:** 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. 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 one.

**Moderator:** That's an important distinction. I actually used to work out with a woman who was the captain of her field hockey team at Ohio State. Her husband played for the baseball team. Remarkably driven human beings. Thank you so much for joining. Let me give you 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. 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 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 want to understand everything I'm going to ask you about your journey with SMA. We are recording, but it's just for research purposes. Your name doesn't get attached to that or anything like that. 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:** 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 my first interview in this study. Sometimes what happens in the first interview, like, I'm sorting out the timing just to be completely transparent. 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?

**Respondent:** I'm good with that. Just cut me off.

**Moderator:** Okay. So can you tell me a little bit about yourself? 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 years old. I'm married. We have two kids from a previous marriage, but I've been in their life. They're now 24 and 22, out of the house. We have a little dog, a little Morkey. I just quit working back in October 2024. So my days are now filled with walking the dog, fiddling on the computer, watching stuff, reading a lot. No longer the corporate hustle and bustle.

**Moderator:** What kind of work did you do until a year ago?

**Respondent:** I was a service manager for a telecom company. I was like a customer advocate liaison between the technical team and the customer, normally the director VP level.

**Moderator:** That sounds like a high-pressure job because 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 text to speech, and I just couldn't keep up. I'm a perfectionist at heart. I started to fall back into a b-minus c player, and that wasn't good for me.

**Moderator:** Are you on disability at this point?

**Respondent:** Yeah. I went on my employer's long-term disability, and then I just got approved for Social Security disability, like, a month ago.

**Moderator:** Congratulations. I know it's maybe a mixed bag on the congratulations, but...

**Respondent:** It could be worse. There could be nothing, so I'm grateful.

**Moderator:** I have written down that you're type three SMA. Is that correct?

**Respondent:** That's correct.

**Moderator:** When were you first diagnosed with SMA? How old were you?

**Respondent:** I was around 10, 11 years old. There was a little bit of trying to figure out what was going on with me. I kept on falling down when running. It was just my right leg that would give out. That started doctor's appointments. About a year into that journey, I saw a doctor who said you're showing all the signs of muscular dystrophy, which then led me to the Ohio State University and a doctor who was a leading pioneer in that, who then diagnosed me officially.

**Moderator:** How would you describe the role of SMA in your life now?

**Respondent:** It's affecting 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 recently got a caregiver that comes in and bathes me and helps me with little things around the house. As a man, I'm a fix-it guy, and I look around and I can't fix anything. In fact, when I try, I end up causing more issues. So quality of life is diminished a lot.

**Moderator:** When did you start to lose more of your abilities?

**Respondent:** It's been slow. I've never been super strong in my upper body, but I've been stronger than most. Over the last six, seven years, it's just started to get worse. It's hard to track it day to day. It's like you look back on it, like, a month or a couple of months, and it's like, oh, I'm doing it different, or I'm not doing it at all, or I'm avoiding it.

**Moderator:** How would you describe your current health?

**Respondent:** Everything but SMA, I'm as healthy as any. I have my regular physicals, and I'm in great health, but I have spinal muscular atrophy. My doctor does that. My primary care physician doesn't really know the intricacies of the different diseases, so they just sort of lump it in as muscular dystrophy. I have to remind them, well, it is, but it isn't.

**Moderator:** How does that make you feel, the doctor not really understanding the intricacies of SMA?

**Respondent:** It used to bother me until I really understood what health care is. A doctor can't know everything, especially a primary care physician that's more treating your immediate needs. I know I'm very special. I have a very unique situation. It doesn't bother me as much. But when I was younger, looking at doctors as they're supposed to be the experts, they're supposed to know. Then you get a little bit older and wiser and you realize, no. Nobody can know everything. So I cut them some slack nowadays.

**Moderator:** Who are you seeing for SMA-related care?

**Respondent:** I have a neurologist who is in the world of SMA. 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. I don't see her on a regular basis. I was doing a case study, which I had stopped doing, and she was involved in that. I haven't officially gone to her office in probably three years.

**Moderator:** When you say case study, do you mean clinical trial?

**Respondent:** A little trial case study.

**Moderator:** You said you weren't seeing anyone for a while. When were you last seeing a neurologist regularly?

**Respondent:** To be honest, I was never seeing a neurologist on a regular basis. When I got diagnosed at a young age, I went into denial. I was still playing sports. I did not want to hear what they were telling me. I avoided doctors like the plague. I went back to a neurologist when I graduated college at 24 to see if there were any changes. When I was young, I had asked what should I be doing? Should I be on a diet? Should I be exercising? The answer was we don't know. Try it and tell us. When I went back at 24, I got the same response. They did a genetic test to confirm and verify because before I had a muscle biopsy that confirmed what I had. At 24, they did the genetic test and confirmed spinal muscular atrophy type three. But since they couldn't give me any hope of what to do, I didn't want to wrap myself up into doctor appointments. I was young and ambitious and wanted to make money, so I worked. I didn't go back to see a neurologist until 2017, 2018.

**Moderator:** What motivated you to go back?

**Respondent:** I was aware of a particular treatment, but I did not like the way it was administered. There was a drug called Spinraza, and you had to get a lumbar puncture for it. I didn't know if I wanted to do that. But I had started to hear about an oral medication called Evrizdi. I was like, if I'm going to be able to get this prescribed, I need to get aligned with a neurologist because my primary physician was not going to be able to help.

**Moderator:** How were you learning about these developments in SMA treatment?

**Respondent:** Through Facebook. That was the only thing I was getting my information from, these Facebook support groups for spinal muscular atrophy.

**Moderator:** When do you think you joined those groups?

**Respondent:** I was a late bloomer to the whole Facebook thing. I avoided it. I think I didn't join until either my very late thirties or early forties. I've only been on it for probably ten years, maybe eleven at the most.

**Moderator:** What prompted you to visit those Facebook groups?

**Respondent:** I've tried to not associate myself with the disabled community. But my reality was becoming that I am part of the disabled community. It was me tiptoeing into that without fully being in it. I went to a face-to-face support group. This was a way to read about it and see what other people are doing about it. If I wanted to click to see what these people look like, I could. It was me getting used to the idea that this is where I'm headed.

**Moderator:** Why were you trying to keep all of this at arm's length?

**Respondent:** Prejudice around it, of myself as well. I had an early experience as a kid, hearing how people talked about disabled people. I knew that was part of my denial. Doctors told me I was going to be like that as well, and I didn't want to be perceived that way and be ridiculed. I had it myself, which was ugly of me.

**Moderator:** Was there a specific event that you remember?

**Respondent:** My adopted father made an off-colored joke about a man in a motorized wheelchair. It really hit me, knowing I have this disease. I don't even know if he was aware of it. It just came out. It wasn't an apology. It was just what it was. I don't blame him. He was raised in a certain way, and it was common to make fun of people less fortunate because it's funny, but it's not funny.

**Moderator:** When you were diagnosed at ten or eleven, how was it described to you what SMA is?

**Respondent:** Before I was told I had SMA, my primary care physician in Ohio sent me to a doctor at a local university. That doctor said, you're showing all the signs of muscular dystrophy. You're going to 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 accept or get ready for what's to come. It was hard. There was no bedside manner. I think he was trying to say, I'm not going to beat around the bush. It's time to swallow the hard truth. But as a kid, that was hard.

**Moderator:** It's just so intense. You've described these lows that you're receiving, but then you're also describing yourself as this remarkable young person. It's striking.

**Respondent:** I truly believe the reason why I was able to do what I've been able to do is because of my denial and stubbornness. I was not going to fall victim to what they were classifying me as. If I had listened to what they said and internalized that, I think I would have been worse off quicker and wouldn't have achieved what I have.

**Moderator:** For you, a lot of your health, you attribute to keeping the SMA at bay in some ways.

**Respondent:** Yeah.

**Moderator:** Do you remember what led you to go to these Facebook groups?

**Respondent:** One of the changes that occurred when we moved to Georgia was going to an abilities expo. There was a gentleman interested in my wheelchair because it was good for power wheelchair soccer. He wanted to buy my chair and get me to play. Through that, I started getting on Facebook. I'm thinking that's what led me to join or look at the support groups.

**Moderator:** When did you start using a wheelchair?

**Respondent:** I got one right before I turned 21 when I transferred to a major university. But I wasn't using it full time. I would ride the wheelchair to class, park it outside, stand up, grab my cane, and walk in. I wanted to show everybody that I'm not the chair. I didn't start using a chair constantly until I had a leg break when I was 34, 35. I've been confined to the wheelchair for about eight, nine years now.

**Moderator:** How did you feel when you were on these Facebook groups?

**Respondent:** A little bit of a lurker. I still might be a lurker. Sometimes I'll post, but most of the time, I'm just looking, observing, reading.

**Moderator:** How did you feel reading about SPINRAZA and treatment for SMA on those Facebook groups?

**Respondent:** It made me feel hopeful. For so long, I heard there was nothing. Then to hear that there's something that stops the progression made me feel hopeful. If I can get comfortable with where I'm at today, if we could just stay like this, I can function the rest of my life like this. But with the administration of SPINRAZA, I did not like it. I want something non-intrusive, easy. I don't like spending a lot of time at doctors. When I started hearing about Evrizdi, I thought, I can get on board with that.

**Moderator:** I had written down that at some point, you have taken both Evrizdi and SPINRAZA. Is that accurate?

**Respondent:** That was part of the clinical trial I was a part of for the past year and a half.

**Moderator:** When did you take Evrizdi?

**Respondent:** I've been on it on two separate occasions. The first time, I started in April 2021 and took it until April 2022.

**Moderator:** And then?

**Respondent:** I stayed off all medicine until April or May 2023. Then I stopped it in December 2023 and started the clinical trial with SPINRAZA in December 2023.

**Moderator:** Was it a certain kind of SPINRAZA with the clinical trial?

**Respondent:** It was the double dose of SPINRAZA. The reason I started Evrizdi again was that the clinical trial was based on having been on Evrizdi. They wanted to see the transition from Evrizdi to the higher dose.

**Moderator:** You were on Evrizdi from '21 to '22 for a year. You had a year of no treatment, and then you started the clinical trial in December '23 for the higher dose of SPINRAZA?

**Respondent:** Yes.

**Moderator:** How long were you on that clinical trial with the higher dose of SPINRAZA?

**Respondent:** Up until April. My last dose was April.

**Moderator:** And you're not on anything right now?

**Respondent:** No. I decided not to continue with the trial.

**Moderator:** Why did you exit the clinical trial?

**Respondent:** I was a non-responder to that medication. It did not keep me stable. I continued to digress the entire time. There was no change. I actually got worse.

**Moderator:** You were initially averse to the lumbar puncture. Any thoughts about why it was different later?

**Respondent:** I was a workaholic and didn't want to take the time off, travel, and possibly have adverse effects. I didn't like the idea of putting needles in my back. My tune changed when I saw people having more stability on SPINRAZA. I had a different experience with Evrizdi, and my interest was piqued because of the double dose.

**Moderator:** Your perception of the costs and benefits shifted. You heard the benefits more from your peers.

**Respondent:** Yeah. I reached back out to the neurologist to see if I could get back on Evrizdi after that year hiatus because I wanted to see if I could get the same boost. But we were running up against insurance denying it. The day I looked into the clinical trial, my advocate for Evrizdi called and said I got accepted. The trial needed me to be on it to get accepted. I look at that as a coincidence that maybe this is something I need to do.

**Moderator:** Did you go back on Evrizdi to get in the trial?

**Respondent:** Yeah. In April or May '23, I started taking Evrizdi again. I had to be on it for six months before I could start the SPINRAZA trial.

**Moderator:** How did it go for you the second time you were on it?

**Respondent:** The second time, I didn't get the boost I did the first time. The first time, I had stopped driving my accessible vehicle several months prior. I started taking it and within a month and a half, I was strong enough to start driving again. But after six months, it was like the bottom fell out, and I got worse quickly. It was devastating and never rebounded.

**Moderator:** What prompted you to stop Evrizdi in '22?

**Respondent:** Insurance wouldn't pay for it. They wouldn't allow me to renew. There was part of me going, wait a minute, I got worse. And then the other part was like, well, insurance has denied it anyways. That's the writing on the wall.

**Moderator:** You think insurance denied it because they did an annual baseline test and didn't see any progress?

**Respondent:** That's my understanding. That's what the letter they sent back to me and the neurologist said.

**Moderator:** It's striking that you talk about things being like signs. You had an acceptance of it being meant to be.

**Respondent:** Yeah. It all happened on the same day. It's almost like hitting me upside the head going here, dummy. Laying it out for you.

**Moderator:** The second time you were on Evrizdi for six months, what was your experience?

**Respondent:** It wasn't as dramatic, but I did feel over time that I got stronger. By the time I was getting ready to switch to the clinical trial, a friend said, you seem to be doing really good. If this new medicine could just keep you where you're at, you'd probably be good. I was in a good spot with my functionality. But I thought, let's go for the gusto and do the clinical trial. My expectations were shattered.

**Moderator:** Your experience with the double dose was you described yourself as a non-responder and that you got worse.

**Respondent:** Yeah. By January, I felt like I was worse than where I'd started even with the Evrizdi.

**Moderator:** You were on the high dose of SPINRAZA from December 2023 to April 2025?

**Respondent:** Yes.

**Moderator:** Why did you exit the clinical trial?

**Respondent:** It was my decision. I was not responding to it. The manner in which the testing was being done bothered me. They would do PT testing before the dose, and I knew I was getting worse. The researcher would give me the same score even if I couldn't do it the way I used to. I didn't like that. I realized if I was a kid, I would want to get the better score, but that's not giving a true representation. Then when I brought it up, the lady no longer did my physical test. There was a lot of turnover there. I wasn't getting anything from this. I started to read more about these clinical trials. People are dying for a solution, and they're trusting all this, not realizing they might be a non-responder. I didn't want to participate.

**Moderator:** How are you feeling about treatment now moving forward?

**Respondent:** I don't want to put my foot back into the arena, but I'm not going to close the door on it completely. I feel as if the only two options I have are Evrizdi or SPINRAZA. I know there's some other things on the horizon, but they've not been approved yet. I don't know if I want to be in the arena, but I have a feeling I wouldn't be eligible anyways. There's always in the back of my mind, do I just want to try Evrizdi again? Because now it's in pill form.

**Moderator:** Talk to me about your thoughts about you and Evrizdi.

**Respondent:** I wish I would know what would have happened if I stayed on it more than six months. Would I have had the same experience I had the first time? There's part of me that wants to give it another try. If it happens the same way again, then that's a definite. I never stayed on it longer to see if it rebounds after being on it longer than a year. I've heard some people say the research shows after a two-year period there's more stability. Being a tablet now, since I'm weaker, would be an easier way for me to take it.

**Moderator:** What do you think has stopped you from going back on Evrizdi?

**Respondent:** Probably my own laziness to make an appointment with a neurologist and have to get someone to take me down to fight the Atlanta traffic. I'm not a very good passenger because I drove all my life. Having to go through the whole process of will it get denied and just the process. I just don't like the process.

**Moderator:** It's interesting that you don't see a neurologist regularly. Can you tell me more about that?

**Respondent:** What does the neurologist do for me when I see them? They don't do anything for me. I don't get any benefit from seeing a neurologist. Unless I need something from them that is outside of the scope of what a primary physician can provide, there's nothing I get from that. It's a I'm going to get, not to give. I went to see her in the beginning because I needed her ability to write a prescription that my primary physician could have written, but wouldn't have been able to back up to fight the insurance to get it approved.

**Moderator:** What do you wish you would get from a neurologist?

**Respondent:** Here's the latest and greatest for me. I've been looking into this knowing your particular situation. I know somebody that can get you into this trial or I know what we should do. I want to get you on this. And then in turn with that, we're going to 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. I had to go to her. I've heard this. How can I get that? 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.

**Moderator:** How do you feel when you're sitting in the waiting room in this hospital setting?

**Respondent:** I feel as if I'm checking a box. I've checked in with my neurologist. I can say they have on record I've had an appointment. It just feels like I'm sick. I'm in a hospital setting where you're sick. Even with the primary care physician, I'm going into a facility and there's other things going on there. But for whatever reason, it's not a I don't know. It's almost like in my mind, there's a difference. That is more of an acute illness to where neurologist is a chronic illness. This is bad where the other one is, oh, this is temporary.

**Moderator:** You talked about some awareness of there being some treatments in the pipeline. Do you know anything about what's out there?

**Respondent:** I don't know them off the top of my head. I know there's one that's more associated with muscle therapy, to actually strengthen the muscle. I think it was going to be approved, but then they saw some issues with manufacturing. I know there's a couple of other things, but I haven't really followed them because I know they're still in the clinical trial phase. I get the SMNA news updates. I see that. I don't really pay any attention to them until they start getting a little bit further on.

**Moderator:** What are your thoughts about that one?

**Respondent:** I don't know. It seemed like it was to do in combination with the therapy. I would still have to pick SPINRAZA or Evrizdi and then this on top of it. My concern is what's insurance going to pay? The other ones are pretty expensive and then you're adding that one as well. Is my life really worth that price tag? I don't know.

**Moderator:** How do you answer that question?

**Respondent:** It's hard. You see insurance companies having these profits, and then it's a million dollars or more just to possibly try something that may or may not benefit. I don't know. Could we use that money for other things? There are people in much worse scenarios than I'm in. I live a lavish life.

**Moderator:** 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 treatment route?

**Respondent:** I don't see any change in how I have to go about it. 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 an advocate. It's interesting you described how the Evrizdi advocate called you and said the insurance is approved.

**Respondent:** She still keeps in touch with us, which is cool. She reached out to me just a month ago.

**Moderator:** How did that go? What's that like for you when you get those reach outs?

**Respondent:** I felt a connection with her. We went to one of the little luncheon things and got to meet her. We would have conversations. That was really helpful through the whole process of getting on Evrizdi, having somebody in my corner. I don't think I would have gone down the rabbit hole if it wasn't for that. It's nice that I wasn't just a, oh, I got him on it and let's move on. She just continues to check in.

**Moderator:** Did you ever talk to her about the possibility of restarting the treatment?

**Respondent:** Yeah. I mentioned to her that I had stopped doing the clinical trial and that I was hesitant about the Evrizdi based on the past scenarios. She said, well, there's also another drug on the horizon of getting approved. You might want to look into it. I've heard of it, and that was it.

**Moderator:** It's so nice that you feel like she's in your corner. 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. I really enjoyed talking to you. Thank you so much for sharing everything. 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.