**qual test**

*Oct 7, 2025 | 3:00 PM | R01 Transcript*

Date: October 7, 2025

Time: 3:00 PM

**Moderator:** How's it going?

**Respondent:** It's going okay.

**Moderator:** You look so cozy in your blanket.

**Respondent:** It's been getting colder.

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

**Respondent:** Colorado.

**Moderator:** It's hot here today. I have to give you the same intro I gave you last time. Everything you say is confidential. I know your first name is James, but I don't know your last name. We're recording for research purposes. There are no right or wrong answers. I have more questions about treatment. If you mention a less than ideal experience on a medication my client makes, I may ask you a few questions about that and write up an adverse event report. Are you good with all that?

**Respondent:** Yep.

**Moderator:** My brain is a little wobbly, so remind me of some basics. How old are you again, James?

**Respondent:** 42.

**Moderator:** Do you live alone, or do you live with your parents?

**Respondent:** I have a roommate at the moment. That's going to change in February.

**Moderator:** What's happening in February?

**Respondent:** The roommate's moving out. My mom's moving back in.

**Moderator:** Is this originally their house?

**Respondent:** This is where we moved when we came to Colorado. It was my family's house. I had a fiance, and they moved out so we could live here. That didn't work out. Then I have a roommate that turns me at night.

**Moderator:** Why is the roommate moving out and your mom coming back in?

**Respondent:** Because my needs have elevated since then.

**Moderator:** Would you mind telling me a little bit about how your needs have elevated?

**Respondent:** Just the natural progression of the disease. I got a cold over a month ago, and it affected me more than it should have. It decreased my swallowing capability a little bit. Even before that, my movement capabilities have been slowly declining.

**Moderator:** Sorry to hear that.

**Respondent:** It is what it is.

**Moderator:** So you've been seeing a natural progression. Did it start a month ago?

**Respondent:** It's probably been going on for about a year or so.

**Moderator:** What other declines have you seen in that year?

**Respondent:** Mostly just arm mobility and muscle loss, which is expected with SMA, the atrophy of the muscles.

**Moderator:** Remind me when you were diagnosed with SMA, how old you were?

**Respondent:** A year and a half.

**Moderator:** What have you been told about that? Do you know anything about how that went when you were diagnosed?

**Respondent:** I don't remember much, but my mom told me they did a spinal tap and genetic testing, and that's how they knew what it was. They didn't tell her not to move me for a half an hour because the spinal fluid was low. So she picked me up, and I screamed my head off.

**Moderator:** Poor kid. Something she remembers maybe more than you do.

**Respondent:** Yeah. I don't remember any of it.

**Moderator:** What are your memories about the care you had from a medical professional in your childhood and younger adulthood?

**Respondent:** Probably the earliest I have is around five. We would go to Saint Louis Children's Hospital, where the MDA clinic was. I would see the same five or six doctors, and they would poke and prod and test reflexes and muscle strength. It was just a yearly checkup kind of thing. I became friends with a few of them.

**Moderator:** You became friends with a few of the doctors?

**Respondent:** Yeah. They also ran the MDA camp that I would go to every year.

**Moderator:** So you went to an MDA camp? How many years did you go to that?

**Respondent:** Like eight, maybe nine.

**Moderator:** How was that camp experience for you?

**Respondent:** It was a blast. It was a week away from the parents, so that was fun. I made some bonds with counselors. I was a huge flirt back then, so I would let the girls paint my fingernails to spend time with them.

**Moderator:** How old were you during those years?

**Respondent:** I think I started going when I was 11, maybe 12, up to when I was 19.

**Moderator:** What impact do you think that had on your life?

**Respondent:** It was a good opportunity to see what it was like in the outside world. I grew up in a very country area, so there wasn't a lot around. It was fun to be away from that. I was relatively social, so being around people who liked the same things I liked was great. My favorite part was going swimming every day. There was a midweek dance and a graduation dance at the end of the week. It was always too short but a lot of fun.

**Moderator:** Coming to the present, to what extent do you feel connected to any kind of SMA community now?

**Respondent:** There's not really one here where I am. There was an MDA call center here for several years. I would visit them during the summer and hang out. I sort of became their mascot. Whenever they had an event in the area, I would get invited.

**Moderator:** How did that make you feel being the mascot?

**Respondent:** It was fun. I got to be around people.

**Moderator:** When was that you were involved with the folks at the call center?

**Respondent:** Probably ten plus years ago.

**Moderator:** Do you have any connections to the SMA community, like online or in person?

**Respondent:** Not particularly. I'm still friends with my counselor from camp on Facebook. We don't talk, but I see his life events.

**Moderator:** Does he have SMA?

**Respondent:** No. He was a counselor, so he was able-bodied.

**Moderator:** Have you ever looked into or involved yourself in any of the online SMA stuff?

**Respondent:** No. Not really. I find it more depressing than anything.

**Moderator:** Tell me about that. I'm really interested in that.

**Respondent:** I've never particularly seen myself as disabled. It's just something that I live with. I find that mindset unique among the disabled community. Most of the time, they're like, "I'm so crippled. I can't do this and I can't do that." Talking and whining about it isn't going to change anything. I've never found anything worth my time to be a part of as far as Facebook groups or anything.

**Moderator:** That's really helpful for me to understand. Do you feel like people almost adopt a victim identity?

**Respondent:** Yeah. Basically. It's just not how I see it.

**Moderator:** How does it make you feel when you see some of the posts that people are posting on Facebook around SMA?

**Respondent:** The posts I see aren't usually around us. I follow a lot of content creators with ADHD because they also do things like Dungeons and Dragons or comedy skits. The couple of posts I do see on SMA are from a charity called Wheels Up, looking at making air travel more accessible for wheelchairs.

**Moderator:** How do you feel about that?

**Respondent:** It would be great. I've heard horror stories of disabled people getting on planes and watching their chairs get thrown in the luggage, arriving at their destination destroyed.

**Moderator:** It's upsetting, actually. Are you interested in finding out information about developments in SMA?

**Respondent:** I don't really have a drive to. If I come across new information by happenstance, that's fine, but I don't go looking for it. It's not part of my routine.

**Moderator:** If new information about treatment happened to come across your screen, would that be something you'd actually want to learn about?

**Respondent:** If it's something I came across and it wasn't one of the four I already know, then I would be interested to see what the differences were. Unless it's a miracle cure on the horizon, it's not something I would consider taking.

**Moderator:** What are the other treatments that you know about?

**Respondent:** Spinraza, Evrysdi, Zolgensma, and I've recently heard of another one, but I don't have it written down. Maybe it's only the three I know.

**Moderator:** When did you first hear about Evrysdi?

**Respondent:** That was through a rare patient survey. It was one of the options, and I hadn't heard of it yet.

**Moderator:** What was your reaction to learning about it in the research?

**Respondent:** It was good to see they weren't just stopping with Spinraza. It was more accessible. As I took more surveys, I learned more about it. It's nearly as effective as Spinraza, just in a different form. I was glad to see they were continuing to find different ways of combating the disease.

**Moderator:** What did you think about the possibility of you using Evrysdi?

**Respondent:** It wasn't a reversal. It's just a plug in the hole kind of situation. With my swallowing capability decreased, taking a pill is more difficult than it used to be.

**Moderator:** Do you know what forms Evrysdi comes in?

**Respondent:** I think it comes in a tablet and a liquid.

**Moderator:** The tablet might be difficult for you. Any thoughts about the liquid?

**Respondent:** In a hypothetical situation where I was interested in taking the drug, that's the way I would choose to take it.

**Moderator:** What do you perceive as the potential benefits of taking Evrysdi for yourself?

**Respondent:** In a hypothetical situation, I would need to know there wouldn't be any side effects. Currently, as I am, not particularly.

**Moderator:** Why isn't stability good enough given that you're having some progression?

**Respondent:** Because the chance of losing quality of life is an unknown, and I like to know the variables before I make that decision.

**Moderator:** When you say losing quality of life, how would taking a medication like Evrysdi specifically impact your quality of life?

**Respondent:** It depends on what the side effects were. The liquid form needs to be refrigerated, which would be another thing to consider if traveling. If there were any interactions with alcohol, that would be a concern since I enjoy my drink every night.

**Moderator:** What is it about the potential side effects that makes you concerned?

**Respondent:** I like routine. If I take something that messes up my routine, it messes up my entire day. Some side effects I've seen are pretty unpleasant, like diarrhea, nausea, internal bleeding, migraines, and headaches.

**Moderator:** Are you aware of any other treatments besides Zolgensma that are in the pipeline?

**Respondent:** I've gotten a couple of surveys asking about product X kind of stuff, but I don't know any names. I assume there's at least one more in the pipeline.

**Moderator:** I guess they're doing research on a higher dose of Spinraza. Any thoughts about that?

**Respondent:** If it works for the people it works for, that's great. I think it also decreases the amount of times they need the injection.

**Moderator:** Would you ever consider anything like a higher dose of Spinraza?

**Respondent:** It's nearly always going to be no.

**Moderator:** There's another medication being tested under the category of an anti-myostatin that you have to take in combination with either Evrysdi, Spinraza, or Zolgensma. It targets the muscles specifically. What do you think about that?

**Respondent:** It's a step in the right direction. Assuming there were no side effects and they could show mild to moderate improvement in motor function on someone my age, it would be something I want to pursue.

**Moderator:** If it were something you would try to pursue, what would you want to take that anti-myostatin in combination with?

**Respondent:** Probably Evrysdi because it would be easier, and I wouldn't have to leave town to get it.

**Moderator:** If it did show mild to moderate improvement, what would you want or need to feel good about pursuing that treatment option for yourself?

**Respondent:** Insurance would be one of them for sure because I couldn't afford something like that. I would probably want to talk to a peer who is or has taken it and get honest answers.

**Moderator:** Do you feel like if you spoke to the manufacturer, you would just be getting fluff answers?

**Respondent:** It depends. If I was talking to a representative, they might glaze over the downsides. They have a way of silver-tonguing their way through things.

**Moderator:** Do you ever talk to your primary care physician about SMA at all?

**Respondent:** Not really. I have a yearly checkup, but it's not something I go in-depth with him about.

**Moderator:** If the primary care physician was a route to medication or treatment, would that be of any interest to you?

**Respondent:** Not entirely sure it would make much of a difference. I use doctors as I need them, and they tend to be bought out by pharmaceutical companies. If the treatment was covered by insurance and I could talk to a peer, I would pursue it.

**Moderator:** So for you, the most trusted sources of information about treatment are peers. Do you trust any other sources of information about SMA treatment?

**Respondent:** The raw data, but that can be cryptic sometimes. I would look at what the manufacturer had to say, but I'd be skeptical of what they were saying.

**Moderator:** Was there anything else you wanted to share about SMA treatment?

**Respondent:** Not that I can think of.

**Moderator:** I learned so much. I was excited to talk to you again. I love your sense of humor. It has been so helpful to talk to you, James. Thank you so much. I wish you the best with everything.

**Respondent:** You're a pleasure to talk to. A couple more of these, and we'll be close friends.

**Moderator:** Exactly. I'll be sharing with you all my recent stamp purchases. Have a great rest of your day.

**Respondent:** You too.