SMA Adult Activation Qual Transcript

Oct 7, 2025 | 3:00pm

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

**Respondent:** And it's going okay.

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

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

**Moderator:** Where oh, can without telling me your actual address, what state do you live in? Oh, okay. I'm in, Brooklyn, New York,

**Respondent:** Colorado.

**Moderator:** And it's, like, hot here today. I was schmoozing. So I have to give you the exact same intro, I'm sorry, that I gave you last time. So I apologize, and it's.

**Respondent:** Yeah.

**Moderator:** Like I it's almost like a thing I just say by memory. So, as you know, everything I say is I'm sorry. Everything you say is completely confidential. I know your first name is Respondent, but I don't know your last name still. I don't know how to reach you. I didn't even know what state you live in. So, we're recording. It's just for research purposes. Just really no right or wrong answers. I just have a ton more questions to ask you about treatment, which I know we talked about last time, but I wanna talk to you about in a little bit more depth. We our recording is just for research purposes. Your name doesn't get attached to that. I have some colleagues listening in listening in. They don't know who you are. And if by any chance you mentioned that you've had a less than ideal experience on a medication my client makes, I just have to maybe ask you a few questions about that and write up a report called an adverse event report. Are you good with all that?

**Respondent:** Yep.

**Moderator:** Alright. Okay. So my brain is a little wobbly, so you're gonna have to indulge me and remind me, like, of some of the basics. So, how old are you again, Respondent? I'm sorry. I forget.

**Respondent:** 42.

**Moderator:** 42. And do you I remember your dad was there last time. Do you live alone, or do you live with your parents? Or.

**Respondent:** I have a roommate.

**Moderator:** A roommate. Okay.

**Respondent:** At the moment. That's about it. That's gonna change in February, but,

**Moderator:** Okay. Oh, really? What's, what's happening in February?

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

**Moderator:** Oh, is it, you say back in, is this was this originally their house.

**Respondent:** This is where we moved when we moved here to Colorado.

**Moderator:** I see.

**Respondent:** So thisWas my family's house. I had a fiance, and then they moved out so we could live here. And then that didn't work out.

**Moderator:** Sorry.

**Respondent:** And, then I have a roommate that, turns me at night, basically.

**Moderator:** That turns a roommate what what about the roommate?

**Respondent:** That turns me at night.

**Moderator:** It turn turns you.

**Respondent:** Yeah.

**Moderator:** I see. And why is the roommate why is the roommate moving out and your mom coming back in?

**Respondent:** Because my needs is elevated some since then.

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

**Respondent:** Just the progression natural progression of the disease. I got a cold, just a cold, little over a month ago, and it kind of affected me more than it should have. It decreased.

**Moderator:** Oh.

**Respondent:** My swallowing capability a little bit. And even before that, my, movement capabilities have just been slowly on the decline.

**Moderator:** Sorry to hear that.

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

**Moderator:** Can you make that sound again? Yeah. That's a that's a New York sound.

**Respondent:** Well, I come from Illinois. So.

**Moderator:** Maybe there's a Chicago sound in there too somewhere.

**Respondent:** Yeah. Maybe.

**Moderator:** So you've been seeing a natural progression. What has this progression did it start a month ago?

**Moderator:** Or do you no problem. Or do you feel like the progression started?

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

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

**Respondent:** Mostly just arm mobility.

**Moderator:** Yeah.

**Respondent:** And muscle loss, which is expected with SMA, the Atrophy of the muscles. So.

**Moderator:** Yeah.

**Moderator:** Now I know I asked you some of these questions last time we spoke, so I apologize. But remind me when you were diagnosed, Respondent, with SMA, how old you were?

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

**Moderator:** You've only been diagnosed for a year and a half?

**Respondent:** No. I was diagnosed at a year and a half.

**Moderator:** A year and a half. What have you been told about to about that? Do you know anything about how that went when you were diagnosed? Or.

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

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

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

**Moderator:** She must have been upset.

**Respondent:** Yeah.

**Moderator:** What are you what do you recall so that was almost forty years ago. What do you what are your memories about to what extent you talked to doctors about or any sort of care that you had from a medical professional in your childhood and your younger adulthood. Yeah. Like, did you ever yeah.

**Respondent:** Probably the earliest I have is maybe around five. And, we would go to Saint Louis Children's Hospital, which is where the MDA clinic was.

**Moderator:** Oh.

**Respondent:** And I would see the same five or six different doctors, and they would poke and prod and test reflexes and muscle strength and, you know, kinda just a yearly checkup kinda thing, Became friends with a few of them.

**Moderator:** Say again?

**Respondent:** Became friends with a few of them.

**Moderator:** Of the doctors?

**Respondent:** Yeah. Because they would also run the MBA camp that I would go to every year.

**Moderator:** Oh, wow. So you went to an MDA camp?

**Respondent:** Yeah.

**Moderator:** I heard just heard about that the other day for the first time. How many years did you go to that?

**Respondent:** Let's see. Like, eight maybe, nine, something like that.

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

**Respondent:** Oh, it was a blast.

**Moderator:** Really?

**Respondent:** Yeah. I mean, it was a week away from the parents, so that was.

**Moderator:** As as the parents, we're like, oh, we get a week away from the kids. Yeah.

**Respondent:** Right. It was, yeah, it was a lot of fun. I, made some bonds with counselors. I was a huge flirt back then, so I, I would always let the girls, paint my fingernails so I could spend time with them. And.

**Moderator:** What how old were you the year those eight or nine years that you went?

**Respondent:** I think I started going when I was 11, maybe,

**Moderator:** Oh,

**Respondent:** 12, something like that.

**Moderator:** Oh,

**Respondent:** And then I went up until I was 19.

**Moderator:** That's great. Wow.

**Respondent:** Yeah.

**Moderator:** What impact do you think that had on your life so much of your teenage years going to that camp every summer and being with other people with I don't even know what category to put everybody in. But.

**Respondent:** Muscular Dystrophy Is a decent Category.

**Moderator:** Okay.

**Respondent:** It was it was a good opportunity for me to see what it was like in the outside world for a lack of better words, I guess. I grew up in a very country area, so there wasn't a lot around. So, you know, it was fun just to be away from that, and I am was still kinda am, relatively social. So, you know, being around people that like the same things I liked, we a couple of the counselors would bring a TV and a console of some kind, and we'd play video games. And, they always had activities and stuff to do. And my favorite parts was the going getting to go swimming every day. And then there was, it was only a week long, so that was, like, a midweek sort of well, it was a dance, a midweek dance.

**Moderator:** Oh.

**Respondent:** And then, like, a graduation and a dance after at the end of the week. And so, you know, it was it was always too short, but it was always a lot of fun.

**Moderator:** That's awesome. I coming to the present, to what extent do you feel connected to sort of any kind of SMA community now?

**Respondent:** There's not really one here where I am. To my knowledge, there well, there was an MDA call center, I guess. I'm not sure what it was called here, for several years. And I would actually they were on the Second Floor of a bank of all places. But, during the summer, I would go down and visit them and hang out and get to know them and stuff. And, I sort of became their mascot. Whenever they had an event in the area, I would get invited and, like, they had golf tournaments and stuff. So I would go to that and kinda show the people what they were donating to.

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

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

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

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

**Moderator:** Oh, okay. Otherwise, have you do you have any connections to the SMA community, like, whether online or in person?

**Respondent:** Not particularly. I'm still friends with my counselor. I had the same counselor for four or five years in a row. So I had a good bond with him. Still friends with him on Facebook. I mean, we don't, like, talk, but I see, you know, his life events evolving.

**Moderator:** Does he have SMA?

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

**Moderator:** Okay. Okay. Speaking of Facebook, like, have you someone I've been some some of the people I've spoken to this week, are involved in different Facebook groups related to SMA. Is that something have you ever looked into that or involved yourself in any of the online SMA stuff?

**Respondent:** No. Not really. If I'm being honest, I find it more depressing than anything.

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

**Respondent:** I've never particularly seen myself as disabled. I mean, I'm obviously disabled, but it just it's not who I am. It's just something that I live with. So I find that that is a unique mindset among the disabled community. Most of the time, they're like, you know, I'm so crippled. I can't do this and I can't do that. I'm like, yeah. And you've been that way your whole life. And talking and whining and complaining about it isn't gonna change anything. So I've never really found anything that would, be worth my time to be a part of as far as Facebook groups or anything.

**Moderator:** Yeah. That's really, really helpful for me to understand. Like, I feel like what you're describing is people kind of let the SMA fully define who they are. And do you feel like it almost and please tell me if this is not what you're saying. But it people almost, like, adopt, like, a victim identity almost or something that.

**Respondent:** Yeah.

**Moderator:** It.

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

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

**Respondent:** Well, the post that I see here, not usually around us. I mean, it's I follow a lot of, like, content creators that, have ADHD of all things. But that's mostly because they also do, like, Dungeons and Dragons or comedy skits or something. So I.

**Moderator:** No.

**Respondent:** I follow stuff like that. The couple of posts that I do see on SMA are is from, like, a charity called Wheels Up, and They're looking at, making air travel more accessible for a wheelchair.

**Moderator:** Interesting.

**Moderator:** And is that what how do you feel about about that?

**Respondent:** I mean, it would be great.

**Moderator:** Mhmm. Mhmm.

**Respondent:** At the moment. I mean, I've heard dozens of horror stories of disabled people getting on planes and watching their terror get just thrown in the luggage, and they get to their destination. And it's just destroyed, basically.

**Moderator:** It's upsetting, actually.

**Respondent:** Yeah.

**Moderator:** Do you are you interested or I don't know exactly how to ask this. Some people I talk to have different ways of finding out information about things that are happening in the SMA world, like, developments in SMA. My I have two questions. Number one, is that of any interest to you, if you could find out that information in a non depressing way? And number two, do you have any way of doing that now? So maybe start with the first question.

**Respondent:** I don't really have a drive to to I mean, if I come across new information by happenchance, that's fine. But I don't, like, go looking for it. It's not part of my daily routine or weekly or monthly. It's just if I happen to see that there's been a development and what it is, then that's fine, but I don't actively seek it out.

**Moderator:** If, like, new information about treatment happened to come across your screen, would that be and this is when it's so important if I could get your honest answer. Would that be something you'd actually wanna learn about or, like, you know what? I don't even really wanna learn about new developments in treatment.

**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. At this point, I'm unless it's like, you know, miracle cure on the horizon. It's not something that I would consider, taking but if It was, you know, information on a new drug and, again, something one of the four that I didn't already know of, then I wouldn't, yeah, look at the differences and see what the the application method was and what they claim it will do.

**Moderator:** Okay.

**Moderator:** Claim? What's that about claim?

**Respondent:** Oh, you know, drug drug companies always claim grandiose things and very rarely deliver.

**Moderator:** Are you skeptical sometimes when you, read about what the what different SMA medication could do.

**Respondent:** I mean, I'm skeptical of everything. So yeah. But that's everyone's different. So.

**Moderator:** Yeah.

**Respondent:** Especially when it comes to genes. So even if research trial has been done on 10,000 people, is it it's like a drop of water in the ocean of genetics as far as compatibility with one person to another.

**Moderator:** It's striking to me what you just said, Respondent, about, like, if I were to paraphrase, like, it would need to be a miracle cure for you to really consider taking it. Is that fair?

**Respondent:** Yeah.

**Moderator:** So I would would love to understand that more because that's really is exactly what I'm trying to understand. So as much as you feel and I'm not here to judge. I just, like, wanna know.

**Respondent:** You're a very curious person.

**Moderator:** Everything. I know my my sixth grade English teacher said to my parents, Moderator asked too many questions, so I just stopped calling on her. That's a really high quality, school district I grew up in. I'll show you. I'll be a researcher when I grow up. Okay. Sorry. Thanks for laughing.

**Respondent:** That's great.

**Moderator:** It's very kind of me.

**Respondent:** No. I mean, questions is fine. So when I say miracle cure, I mean, like, a complete one eighty on the disease. So completely, it would the way I understand it, it would essentially be completely rewriting the SMN gene to be active and to work properly. And I know that's what a lot of the drugs are attempting to do, but I also don't know enough about genetics to know exactly what a miracle miracle cure would involve, but, basically, it's just a complete reversal of the disease.

**Moderator:** Okay. Okay. Well, let me I'll come back to that question because it's, like, sort of a fundamental question that I would like to have more insight into by the time we hang up hang up the phone. But let's go back. So okay. So growing up, you went to this MGA clinic in Saint Louis. Is that right?

**Respondent:** Yeah.

**Moderator:** Okay. And you also, you went to this camp. And then once you became an adult, how often were you continuing to see any kind of health care providers for your SMA?

**Respondent:** Well, we moved here when I was 20, and.

**Moderator:** Colorado. Uh-huh.

**Respondent:** That was my also my last year of camp. We moved here in November of when I was 20, and the camp was in summer of right before I turned 21, which was the cutoff age for camp. So, I think I saw them that year. And then after that, it was just PCP, yearly checkups. I there wasn't a a SMX or a MDA clinic in the area. You think the closest one is, like, four hours away.

**Moderator:** From One way trip, four hours from where you Live now. Do you Live in a a rural area of Colorado?

**Respondent:** One way.

**Respondent:** Yeah.

**Respondent:** I forget what. I think it's become big enough to become urban, But, at the time, yes.

**Moderator:** Okay.

**Moderator:** So back then, it was four hours. Is it still four hours now to see?

**Respondent:** Yeah. I think we're getting big enough that there might eventually be something MDA related in the area, but to my knowledge, we haven't gotten that big yet.

**Moderator:** Have you ever seen since moving to Colorado, have you seen a neurologist at all even if it wasn't someone associated with an MGA clinic?

**Respondent:** I did once at the behest of my girlfriend at the time.

**Moderator:** Who was later your fiance?

**Respondent:** Yeah.

**Moderator:** When was that when was that? I love the word behest.

**Respondent:** Let's see. That was maybe seven years ago.

**Moderator:** Yeah.

**Moderator:** Seven years ago, so.

**Moderator:** 02/2018, maybe.

**Respondent:** Yeah. Something like that. It was yeah. Sounds about right.

**Moderator:** How did that go, seeing that neurologist?

**Respondent:** About how I thought it would go. He didn't really know anything about SMA except for the research that he had done the days before. And, I essentially knew about it as much of it as he did. So, some of it was we were trying to figure out if there was a way to, practically without too much trauma, if there was anything to do with my jaw slipping back some.

**Moderator:** Your jaw?

**Respondent:** Yeah. I have a severe overbite underbite. I don't know. My my bottom jaw is really far back, and that's because the muscles have atrophied and it just has slowly slipped back. So and it Turns out that there's not really a lot they can do aside from breaking the jaw and resetting it and then, you know, months of having wire on my face.

**Moderator:** Yeah. Why why did your girlfriend at the time want you to see a neurologist?

**Respondent:** Partially for that and partially to talk about any drugs that were available and, if he knew anything about them, which he didn't because he had done, you know, three days worth of.

**Moderator:** Did you or your girlfriend Ask the doctor about drugs?

**Respondent:** Yeah. I think she did.

**Moderator:** And and what did the doctor say? Do you remember it? That's a long time ago. Yeah.

**Respondent:** Yeah. I'm relatively sure I knew more of it than he did, so I don't exactly remember.

**Moderator:** Okay.

**Respondent:** And at the time, I think Spinraza was the only one out.

**Moderator:** Yeah. How how had you heard about Spinraza?

**Respondent:** That one, I heard it was a grapevine, actually. I had I ended up becoming Facebook friends with a mom that had a younger child with SMA. I don't actually remember how that came about. I think this kind of sound very shallow, but,

**Moderator:** Alright. Now wait.

**Respondent:** It started, I think, because she was incredibly hot. And she posted something about SMA, and I was like, hey. I know about that. I have it. And we talked for a couple of months, I think. And, became friends on Facebook, and I wanna say her son's birthday was in July also, but I'm not positive on that. But, she actually did send me a present one year. It's a shirt that says, Jedi masters are born in July.

**Moderator:** Oh, so she told you about the Spinraza? She had learned about that.

**Respondent:** Yeah.

**Respondent:** I think that's where I heard of it first was through her.

**Moderator:** How did you how did you Feel about SPINRAZA when you when she told you about it, what was your reaction? Did you do any research to learn about it, or what what did you think?

**Respondent:** I did a little bit of research. Again, I was skeptical. New drug. You know? It's chances of it working or not having excruciating or terrible side effects or flow. But I did some research. I think my girlfriend probably did a lot more than I did.

**Moderator:** K.

**Respondent:** I think she went into the she had ADHD, so she hyper focused very easily.

**Moderator:** Mhmm.

**Respondent:** And went down rabbit holes.

**Moderator:** Mhmm. That's classic. Uh-huh.

**Respondent:** Ended up I think she ended up deep diving in on it and finding out that it, it was if I remember right, she said it was attempting to activate the backup SMN protein because everyone has two.

**Moderator:** Okay. HowDid you how did you feel about it when she told you when you learned about it and your girlfriend did the research on it? Like, what were your reactions to it? I know you said you were skeptical and you worried about the terrible side effects, but, like, that was the first medication for SMA. Did it yeah.

**Respondent:** There there was a little hope there. When I found out it was more of a preventative than it was a reversal, It I kinda rolled it out as a viable option.

**Moderator:** That's yeah. Tell me about that. Why is that?

**Respondent:** At the time, I hadn't declined for ten, fifteen years. I was relatively stable, and it wasn't worth giving up my quality of life, especially with the, application method and travel time and because I wouldn't have been able to get in here. I would have had to go somewhere else. So it was just not a viable option at the time

**Moderator:** It's interesting.

**Moderator:** I hear what you're saying too, that you were stable and so the appeal of stability was limited.

**Respondent:** Right.

**Moderator:** Yeah. Okay. So have you been back to to a neurologist since that one in two.

**Respondent:** No. I thought about it a couple of times, but, I mean, they're just gonna give me all the information that this is what your body is doing. No shit. I'm in my body. I know what it's doing.

**Moderator:** Yeah. Yeah. Yeah. Yeah. So you mentioned you knew about four different treatments.

**Respondent:** Yeah.

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

**Respondent:** I have them written down because I always forget the names.

**Moderator:** That's okay. I can remind you.

**Respondent:** Spinraza, Evrysdi, xyloglasam, 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:** Okay. So when did you first hear about Evrysdi?

**Respondent:** That was through rare patient. I took a survey, and it was one of the options. I was like, oh, I haven't heard Of that one yet.

**Moderator:** So you're doing mark this kind of survey, you mean, you learned About it? Oh, how long ago was that survey?

**Respondent:** Yeah.

**Respondent:** Oh, that's probably been five five ish years.

**Moderator:** Wow. Okay. Fascinating, the impact of market research. What was your reaction to learning about it in the research?

**Respondent:** I mean, it was good to see that they weren't just stopping with Evrysdi. It was, more.

**Moderator:** Mhmm.

**Respondent:** Accessible application. And, as I took more surveys, I learned more about it. And, essentially, it's nearly as effective, I think, as Evrysdi. It's just in a different form. But mostly, I was just glad to see that they were continuing with trying to find different ways of combating the disease.

**Moderator:** What did you think about the possibility of you and Evrysdi? Did that ever cross your mind?

**Respondent:** No. It wasn't either one of those, slower, halt progress kind of thing, and they never talk about side effects in the especially in the surveys. So the benefits didn't outweigh the potential cost in my mind.

**Moderator:** Can we just make a list? So from yours, I wanna make a list of what you perceive to be the benefits versus the cost of a treatment like Evrysdi specifically.

**Respondent:** K.

**Moderator:** So in your mind, what were the costs or the downsides or the challenges of Evrysdi?

**Respondent:** Well, I didn't know what any of the side effects were. I still don't really know. Well, I I do kind of know to a point, And it's not a reversal. It's just a whole, plug in the hole kind of Situation. And with my swallowing capability decrease, taking a pill is a bit more difficult than it used to be.

**Moderator:** Mhmm.

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

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

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

**Respondent:** Yeah. I mean in a hypothetical situation where I was actually interested in taking the drug,

**Moderator:** Yeah.

**Respondent:** That's the way I would choose to take it.

**Moderator:** Okay. What do you perceive as the potential benefits of taking Evrysdi for you yourself, if you see any.

**Respondent:** In a hypothetical situation, It would I would need to know that there wouldn't be any side effects. And.

**Moderator:** Do you see any potential upside for you in taking a medication like adversity? Does there seem like there would be any benefit for you?

**Respondent:** Currently, as I am, not particularly. No.

**Moderator:** And just imagine I'm actually a nice person gonna ask this question. Why not? Yeah. Why don't you see why is, like, like, what about if at Evrysdi offered stability? Right? Like, that you would you would you know, you talked about that you've been seeing natural decline. And what about the is there any appeal to you for a medication that I don't know if it stops decline or slows the decline. I don't really know. I'm not an expert on it. But does that I'm curious to know if that has and I don't care what your answer is. I just care that I understand your.

**Respondent:** Yeah. Yeah. For me, what was the base question? Sorry.

**Moderator:** Because, you know, some people are excited to take Evrysdi because it provides some stability that it's gonna stop what you stop or stall or slow that natural progression that you talked about. Right? And you've been talking about having more progression lately. Lately.

**Respondent:** Right.

**Moderator:** So someone could theoretically ask you, well, then why aren't you interested in something that could address that progression? I I honestly I don't care. I I just wanna understand. I Mean, I care why you think that, not I don't have a a dog in the show, pony in the show. I can't remember what that says. What's that called? I don't remember what that saying is. Dog in the a dog in the fight. Yes. Okay. 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, all of the variables, orAt least as many as I can.

**Moderator:** When you say what yeah. Keep going. Keep going.

**Respondent:** And, this is probably sound morbid, but I'm not really afraid of death. So I kinda would rather live my life the way I want to live it and not be tied to and or have my quality of life affected by something that would drag out the inevitable.

**Moderator:** Mhmm. Okay. I'm really so grateful to you for bringing that up because this is profound stuff that we're talking about. And I just it really means a lot to me that you're sharing that, so thank you. When you say losing quality of life, what would it like, what how would taking a medication like Evrysdi specifically in your mind potentially impact your quality of life?

**Respondent:** It depends on what the side effects were, what side effects presented while taking it. And I think the liquid form needs to be refrigerated.

**Moderator:** Yeah. Yeah.

**Respondent:** So, that would be a another thing to add to the list of if we were gonna take a trip somewhere, you know, finding a way of keeping it refrigerated and if there was any interactions with alcohol or something, I'd I'm a wouldn't say an alcoholic, but I enjoy my drink every night.

**Moderator:** Mhmm. Mhmm. Mhmm.

**Respondent:** So.

**Moderator:** Yep. It's a good question. I don't know about the impact of alcohol. I guess you know I know you talked about the lick the oh, yeah. You're right. Right. Right. Right. I my bad about what the tablet can be dispersed into liquid, I guess. I I don’t know if that is interesting to you.

**Respondent:** It's new information.

**Moderator:** What do you think about that? So that way the tablet doesn't have to be refrigerated. It like, you have to disperse it in bottled water, but and it can't go through a g tube. I think I'm getting this right.

**Respondent:** That would make sense. That would take away a hurdle, but there's still the the unknown side effect aspect of it.

**Moderator:** What, are you afraid, of any like, when you you've talked a lot about side effects being a big major concern ..

**Respondent:** Yeah.

**Moderator:** Thanks for putting up with this. I wonder if you could just tell me a little bit more about what it is about the potential side effects that makes you concerned.

**Respondent:** Essentially, I like routine. So it comes down to if I take something that's gonna mess up my routine, it's going to mess up my entire day. And some of the side effects that I've seen that are possible are.

**Moderator:** Yeah.

**Respondent:** Pretty unpleasant.

**Moderator:** Like like, can you give me an example?

**Respondent:** I think one of them was diarrhea,

**Moderator:** The joy of it all. Yeah.

**Respondent:** Nausea.

**Moderator:** Yeah.

**Respondent:** I think one of them was internal bleeding, but I don't know for sure. Migraines, I think, headaches, something like that.

**Moderator:** Yeah.

**Respondent:** And, you know, the diarrhea alone is just, like, not it's not something that I can handle very easily.

**Moderator:** Yeah.

**Respondent:** Or quickly. So.

**Moderator:** Yeah. Could imagine that would be bad.

**Respondent:** Yeah.

**Moderator:** I really appreciate you just elaborating all of that.

**Respondent:** Mhmm.

**Moderator:** Are you aware of any other treatment that's did I ask you this already? My brain. Are Are you aware of any other treatments beside besides the Zolgensma, like, that are in the pipeline that are potentially coming out?

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

**Moderator:** Okay.

**Moderator:** I guess I guess they're, doing research on a higher dose of SPINRAZA.

**Respondent:** Yeah. That one I've heard about.

**Moderator:** Yeah. Just any thoughts about that quickly?

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

**Moderator:** The loading doses change. I can't remember exactly. I don't know if you fewer loading doses until you get to your regular dose. But then I think once the regular dose starts, it's the same cadence. It's the same number of time.

**Respondent:** Oh, I see. So that wouldn't that would still be, like, a drawback for me. If you I mean, once a month is once a month. If it would be the, the regular doses that if, if that could be stretched out, I think would be more beneficial personally.

**Moderator:** Is that some would you ever consider anything like a higher dose of SPINRAZA?

**Respondent:** It it nearly always gonna be no.

**Moderator:** Okay. Yeah. That's fine. That's fine. I just have to ask. I assume I was even shaking my head no, like, oh, I'm not even asking this question, but I didn't I'm not supposed to presume anything. And I think another I'm not gonna do this one justice. There's another medication, that's being tested under the cat the category of what's called an anti myostatin that you have to take in combination with either Evrysdi, or Spinraza or Zolgensma if you're under if you're younger. And it it targets the muscles specifically.

**Respondent:** Yeah. I've I've heard of that. It's like a sphaspia muscle growth or muscle enhancer kind of thing.

**Moderator:** Exactly. Yeah. What do what do you think about that?

**Respondent:** I mean, it's a step in the right direction.

**Moderator:** In terms of you, though?

**Respondent:** Yeah. In terms of me, if assuming for a second there were no side effects, and they could show me a mild to moderate improvement in motor function on someone around my age, then that would be a it would put it as a top of the list for me. It would it would at least put it on a list of this is probably something that I want to try to pursue.

**Moderator:** If it were something you would try to pursue, what would you wanna take that antimalostatin in combination with? Evrysdi or SPINRAZA?

**Respondent:** Probably ever see just because it would be easier, and I know I wouldn't have to leave the town to get it at least I hope it wouldn’t.

**Moderator:** I don't think there's any medication that can ever say there are never ever ever any side effects.

**Respondent:** Yeah. I know.

**Moderator:** Right. Yeah.

**Respondent:** I know. I was just I was giving it the best chance in my head.

**Moderator:** If it did show, though, in combination with those two treatments that people your age tended to have mild and moderate mild to moderate improvement, Is that truly and even though there would be the side effects that any could have come with any because it is a treatment.

**Respondent:** With side effects. I would depending on how the secondary treatment is applied.

**Moderator:** Right. I think there are two different way two different one being tested. I think one is an infusion and one is an injection. I could be wrong. Like, I think there's, like, Roche is one of those, and Scholar Rock is the other one. One's infusion, one's an injection. Again, people will correct me if I'm wrong.

**Respondent:** What's an infusion?

**Moderator:** It's like you sit there and they it just like a liquid gets put into your body. Like, it's like a needle put in there and you oh, yeah. Infusion Scholar Rock, like, you they I don't I don't know if they connect you to a vein or something. And they put a little needle in you and the you just sit there. Have you ever gotten an IV.

**Respondent:** Yeah.

**Moderator:** It's like that.

**Respondent:** Oh, okay.

**Moderator:** And the other one's sub q, so I'm assuming that's just an injection. I can't remember if that's a spinal injection. I'm really testing the people listening to see if they remember. This is where it would help. Be so yeah. Anyway, no subcutaneous, just under the skin. Woo woo. Woo.

**Respondent:** Okay.

**Moderator:** Woo. So neither one is a spinal blah blah. Nothing nothing like Spinraza. You you still have to get to some sort of medical office, I think. Although, actually, I'm spacing out some sorry. This is so embarrassing to admit. Some I don't know if they're testing at all the idea of possible home administration. I don't think so.

**Respondent:** I think that was one of the questions I remember seeing.

**Moderator:** Just so, oh, Scholar Rock is pushing home infusions. Brain to the rescue. Yes. So let's imagine this becomes an option. You can get a RISD tablets mailed to you. You can dissolve them, or you can do the liquid, and you could do home infusions with this. And you and I I can't remember the exact data, but let's say the data does show some chance for mild to moderate improvement. What would you want or need to be able to actually feel good about pursuing that treatment option for yourself? Like, what, you know, like, would you want someone from the manufacturer to call you? Would you want a peer to talk to you? Would you want information mailed to you? Would you wanna see the data? Would you wanna see I I don't even know. Like, would you want someone to help you with insurance? And we haven't even talked about insurance and all that that.

**Respondent:** Yeah.

**Moderator:** Fun stuff.

**Respondent:** Insurance would be one of them for sure because there's no no way that I could afford something like that. It would absolutely have to be covered by insurance.

**Moderator:** Yeah. Are you on Medicare?

**Respondent:** I am.

**Moderator:** Yeah.

**Respondent:** No. Medicaid.

**Moderator:** Medicaid. It's probably joint. It's probably a dual program.

**Respondent:** Yeah.

**Moderator:** Right? Because.

**Respondent:** Probably.

**Moderator:** Yeah.

**Respondent:** I think it is changing at the end of the year. I don't know what's going on with insurance.

**Moderator:** Okay.

**Respondent:** I got a letter from, insurance agency saying that, Rocky Mountain Health Plan's going away, and they're taking over the insurance. And so I have no idea what my insurance is gonna do.

**Moderator:** Yeah.

**Respondent:** So, yeah, EAA Trump. Anyways, the, I would probably want to talk to a peer, the someone that's is or has or was taking it, the two, and talk about their experiences. And get honest answers, not, you know, fluff answers. And.

**Moderator:** Do you feel like if you talk if do you feel like if you spoke to the manufacturer, you would just be getting fluff answers, or do you feel like potentially you could get real answers?

**Respondent:** It depends. I feel like if I was talking to a representative of the manufacturer that they would.

**Moderator:** You.

**Respondent:** Glaze over the downsides. They they have a way of silver tonguing their way through things.

**Moderator:** Such a great phrase. I think I asked you this the last time I met with met you, but do you ever talk to your primary care primary care physician about SMA at all?

**Respondent:** No. Not really. I mean, I I have a yearly checkup, coming, but it's I mean, it is what it is. There's not really anything. I think he might ask, how is it like, has there been any changes or anything? But, It's not, like, something I go in-depth with him about.

**Moderator:** Yeah.

**Respondent:** Because he wouldn’t know anything.

**Moderator:** Yeah. Yeah. Never mind. The neurologist, you saw that didn't know anything either.

**Respondent:** Yeah.

**Moderator:** If for some reason there was the primary care physician was actually someone who was a route to medication or a route to treatment, Like, I don't even know. Maybe they somehow met with a like, they had access to a specialist who could help them talk to you, or they had access to a manufacturer who could provide inform I mean, would that be of any interest to you? Like, if instead of having to schlep the four hours to the the MDA clinic or whatever it's called,

**Respondent:** Yeah.

**Moderator:** Like, having that conversation it's hard because I know, like, never as we just said, neurologists general neurologists don't even know about SMA, never mind a primary care. But if somehow the primary care physician was empowered somehow to provide information about the treatment. I'm I'm a little making stuff up as I go here, but I've heard people talk about this question. So I just wanted I just wonder how you would feel about that, like, being able to talk to your primary care physician about SMA treatment.

**Respondent:** Not entirely sure it would make that much of a difference. I use doctors as I need them, and they, in my opinion, tend to be bought out by the pharmaceutical companies anyways. So they push whatever they wanna push. So if I was going to if this treatment was covered by insurance and I was able to talk to a pair and get honest answers, I would go to somebody and pursue it kind of thing. I wouldn't, ask them for a recommendation. I would I would figure out who I need to to talk to.

**Moderator:** Yeah. Yep.

**Respondent:** To get the ball rolling, basically.

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

**Respondent:** I mean, the raw data, but that can be cryptic sometimes.

**Moderator:** Totally. Yeah.

**Respondent:** And yeah. I think as far as information on the drugs, I mean, I would probably look at what the manufacturer had to say about it. But if I was, like, talking to a representative, I'd be very skeptical of what they were saying.

**Moderator:** Yeah. Yeah.

**Respondent:** I very good example. I have been looking at getting a new seat cushion. It's called the Ease cushion. And the website makes it sound like it's, you know, the best thing since sliced bread. And I actually did some research and found a Reddit post of several people who had tried it and used it and gave their actual response, you know, like, results. Essentially, at the time, it wasn't as good as the manufacturer claimed. Go figure.

**Moderator:** Yeah.

**Respondent:** And, they had gone through, like, six cushions and had $600 a fall. You know?

**Moderator:** Holy cow.

**Respondent:** And it's not covered by insurance.

**Moderator:** Oh my goodness. That's a Good thing to research. Reddit is amazing that way, isn't it?

**Respondent:** I eventually did reach out to him and talked to somebody that seemed to be honest, and they said that they were working on a second version that was better and was working on the problems that a lot of the people had talked about.

**Moderator:** That was nice. Was there anything else that you wanted to share? I know I as you said, I'm very curious, so I asked you a lot of questions. Was there anything about SMA treatment that you didn't get to share that you think was relevant to this conversation?

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

**Moderator:** Okay.

**Moderator:** I hope you have a great rest of your day.

**Respondent:** You too.

**Moderator:** Bye.

**Respondent:** See you.