**qual test**

*Oct 21, 2025 | 1:30 PM | Transcript*

Date: October 21, 2025

Time: 1:30 PM

**Moderator:** Hi. Nice to meet you.

**Respondent:** I see we are joined by your furry baby there. Oh my goodness.

**Moderator:** This is Daisy.

**Respondent:** Daisy is beautiful.

**Moderator:** Thank you.

**Respondent:** What kind of cat is she?

**Moderator:** Besides trouble, she's a Cornish mix. They have curls like waves, ripples, and they don't have the top guard hairs, which a lot of people are allergic to. So they just feel like soft, soft kind of bunny almost. And as you can see, humongous ears.

**Respondent:** Yeah. I have two cats as well. They're regular garden variety chunk of monks. But my dog, a little chihuahua with these giant ears, so I'm a sucker for big ears.

**Moderator:** Well, you've got them.

**Respondent:** Oh my god. Yes. She does. That's awesome. Well, she's very welcome to be here. So pleased to meet her.

**Moderator:** I can't get rid of her. So let me give you a little background so you know what we're up to. First of all, have you done this kind of thing before?

**Respondent:** Yes.

**Moderator:** Okay. Alright. So this will probably all sound familiar. Obviously, our topic is SMA. I'll ask you some things about you and your life, and then the majority of our time is going to be spent reviewing some materials. I'll tell you a little bit more about them when we get there, but really just hoping for your candor. I am an unbiased third-party researcher, so hoping you'll share your very candid comments with me.

**Respondent:** Absolutely.

**Moderator:** We will be recording. We also have some listeners from the research team in the background, and we also want to make you aware that we have the requirement to report side effects, adverse events, or side effects if they pertain to the study sponsor. So to the best of our ability, let's avoid discussing any particular issues that you've had with any medication that you've taken, but not to worry if it does come up.

**Respondent:** Okay.

**Moderator:** And, gosh, I think that covers everything. Any questions?

**Respondent:** No.

**Moderator:** Okay. Let's dive in. Can you start by just telling me a little bit about you?

**Respondent:** Sure. I am 56 years old. I've been married for thirty-five years to my English husband. Lived in England for fifteen years and have lived back in the States now for about twenty. We have two kids, a 20-year-old girl and a 16-year-old boy. My husband works. I'm just a homemaker. That sounded terrible. I just meant I stay home. I don't go out to work. But I have an Etsy shop. I sell vintage clothing on Etsy.

**Moderator:** Good. Okay. Thank you. Tell me about SMA, that part of your life. How would you talk about your health currently?

**Respondent:** I mean, it's not great. I don't think anybody with SMA can say their health is great. It affects every aspect of my life from waking to bed at night. It's awful. But I think you learn to deal with it and you make the best of it and you focus on the good stuff. It's debilitating and exhausting and a tough disease. But I'm lucky that I have type three because it could be a lot worse.

**Moderator:** Thank you for your candor with that.

**Respondent:** Anyone that says they don't mind having SMA or that SMA has made them who they are, in my opinion, is not being truthful. I would get rid of this in a heartbeat if I could. I don't think who I am would change. I am who I am with me, not because of the disease. I don't buy that for a second.

**Moderator:** I appreciate you, Laurie. We're gonna get along just fine here. Tell me about treatment and what you're currently being treated with and if you've ever switched your treatment.

**Respondent:** Right. I'm currently being treated with SPINRAZA. I started SPINRAZA when it was approved in December 2016. It took eleven months to get approved, so I started in November 2017, and I stayed on it until June 2020. I felt like I had plateaued, and I wasn't sure if I was getting everything I wanted out of it. EVRISDI had come on the market, so my doctor and I made the decision to take a break from SPINRAZA and try EVRISDI. Maybe it'll work better. It's worth a try.

**Moderator:** Mhmm.

**Respondent:** I started EVRISDI in September 2020, and I took it until August 2021. I stopped it and then restarted SPINRAZA in June 2022.

**Moderator:** Got it. So you really did start EVRISDI very quickly after it was made available.

**Respondent:** I've always said, whatever medicine comes on the market, I will try it. I'm not gonna know, and I'm gonna wonder if I don't at least give it a shot.

**Moderator:** Well, can you say just a little bit more about was it the idea of giving it a shot as well as feeling that you had plateaued?

**Respondent:** Yeah. And I think because I wasn't sure that I was getting as much as I wanted out of SPINRAZA, as soon as the new drug came out, also, you know, a syringe of five mil or whatever it was a day as opposed to an intrathecal injection in my spinal cord three times a year, I would take every day of the week because that's a pain. The administration process is a pain with SPINRAZA. And I would kick myself because I would always think, well, what if EVRISDI worked better? I don't know if I don't try.

**Moderator:** Just under a year, you switched back to SPINRAZA. Tell me about making that decision.

**Respondent:** EVRISDI was not working for me at all. It was probably having the opposite effect. I noticed my upper body losing strength while I was on EVRISDI. That was enough for me. I was done. I'm off.

**Moderator:** Got it. And so when you said not working for you, that's what you meant that your upper body was losing strength.

**Respondent:** Yes. It wasn't a side effect like stomachaches. It just wasn't effective for me. I know there are people it is very effective for, but it just wasn't for me.

**Moderator:** Got it. Alright. And so you went back to SPINRAZA.

**Respondent:** Yeah. Eventually.

**Moderator:** Eventually okay. Not right away.

**Respondent:** It took like nine or ten months, part of which was getting insurance preapproval and part of which was because I upset my doctor by stopping EVRISDI without telling him. He was rightfully annoyed with me because I shouldn't have done it without speaking to him. But I know my body best, and I knew it wasn't working, and I knew it was the right decision to make for me. But he had a little bit of a fit and made me wait a few months.

**Moderator:** Got it. So meaning before he would prescribe, and then you had to wait on top of that for insurance preapproval?

**Respondent:** Yeah.

**Moderator:** Okay. Tell me about the insurance reapproval challenge that you experienced.

**Respondent:** To be honest, I don't remember the actual details. I think the first time we applied for it, it got denied, and then we just had to go through the appeal process, and then it was approved after that. Luckily, we get our insurance through my husband's job, and they have a really good advocate in HR that deals with the company and speaks up for the employees and the families. So that was a big help.

**Moderator:** Okay. I was gonna ask if you had gotten any help with that.

**Respondent:** Yeah.

**Moderator:** Okay. Now I know that the manufacturers, most of them offer support programs. Did you have any help from the manufacturer with that reapproval process or just from the HR?

**Respondent:** I don't know that I had help with the reapproval process from the manufacturer. We have a family access manager with Biogen, but they're a huge help with all the other stuff, like making sure the drug's at the facility and just helping with any issues. I'm in a co-pay assist program, so they help with making sure the bills get taken care of.

**Moderator:** Got it. Alright.

**Respondent:** I would hate to do this without them, honestly. They're phenomenal. They really are a shining example of the company. They make the company look good.

**Moderator:** That's really helpful to understand.

**Respondent:** Wonderful. I love them to death.

**Moderator:** What kind of experience did you have with EVRISDI's equivalent support program?

**Respondent:** I don't feel like I had as much of an interaction, and maybe that's because it was just a matter of getting medicine delivered. I can't remember how often we used to get it, if it was monthly or not. But they were very good. We never missed a dose. They always had the medicine there on time, never had any issues. But it was a simpler procedure, whereas with SPINRAZA, it's booking the radiologist, booking the hospital appointment. There's more to it.

**Moderator:** Got it. But as far as you recall, the insurance reapproval process, it was more the advocate in your husband's HR department that helped?

**Respondent:** It may have been the family access manager with Biogen helped out as well, but I don't honestly recall.

**Moderator:** Okay. No problem. And since then, any insurance issues since getting preapproved for SPINRAZA?

**Respondent:** No. Not since then.

**Moderator:** Got it. Now just to go backwards, when you went from SPINRAZA to EVRISDI, did you have any access issues at that time?

**Respondent:** I don't recall whether EVRISDI was approved the first time or not. I don't think I had issues getting EVRISDI approved, but I don't recall exactly. It was during COVID, so mostly blacked all of that out, haven't we?

**Moderator:** Yeah. For sure. Alright. Then let's take a minute more on the reapproval challenges. What do you recall about what was required? Like, what did you or the doctor have to provide?

**Respondent:** For SPINRAZA, I think, basically, what they had said was that it was not medically necessary. I think all it required was a letter of medical necessity from the doctor, which I believe I found a form online. There's a good resource on Facebook with a lady who made up lots of letters of medical necessity for different things, for durable medical equipment, for cough assists, for wheelchairs, for all that kind of stuff. There was a good one just for the treatment for SPINRAZA, and I might have used that. I can't remember.

**Moderator:** Are you involved with the SMA community in any way?

**Respondent:** Yeah. I'm part of Cure SMA. We have our walk coming up in about three weeks. We do a walk and roll every year.

**Moderator:** Got it. Oh, I like that walk and roll.

**Respondent:** Yeah. It's fun.

**Moderator:** Any of the Facebook groups or any other?

**Respondent:** Just like the Cure SMA page, and there's Adult SMA and Women of SMA. I'm not overly active on them, but I see what goes in, and I'll comment.

**Moderator:** And I'm curious if you just happen to be aware of any treatments in development.

**Respondent:** Oh, yeah.

**Moderator:** What are you aware of?

**Respondent:** I'm aware of the EVRISDI tablet form now. SPINRAZA was supposed to have a larger dose for adults. I'm not exactly sure what's going on, but I'm pretty sure it has something to do with the federal government at the moment. I know ZOLGENSMA is going to be approved for up to the age of 17. And then there's another one. I think Scholar Rock has a drug coming out called apitegromab, which is supposed to be taken with other treatments. It's like an extra treatment on the muscles as opposed to the genetic component.

**Moderator:** Where do you get your information from about new treatments?

**Respondent:** Cure SMA. They just had a Summit of Strength all over the country, and they just had the Orlando one recently. We attended that at Disney World.

**Moderator:** Thank you for all that background. Really helpful. I'm gonna shift gears because I want to start to share the materials with you. I have a series of messages that have been developed for individuals and families, adults and also parents of children with SMA. The messages are broken into four categories, so we'll just go one category at a time. These are draft messages. They have not yet been polished or perfected. That's kind of the point of this research is to get some feedback on these draft ideas. In fact, you will notice even in some cases that very similar ideas may be written a different way sometimes. Again, just trying out different ways of communicating these things.

**Respondent:** When you say messages, these are things that would be like advertisements, like for magazines or online?

**Moderator:** These are very raw messages that could be used in a lot of different ways. On a website, in a brochure, maybe not necessarily destined for an advertising scenario, but certainly more perhaps in an awareness or educational idea. I think they'll make sense when you start to see them. For each category, I'm gonna bring it up on the screen, but also just want to give you a little sentence or two to have in mind before you read them. Let me just share my screen. Alright. You should be seeing just it says welcome at this point.

**Respondent:** Yep.

**Moderator:** Okay. So here's our first category. Just to read this little blurb first. This first set of messages are related to this company's legacy and experience in SMA. So just wanted you to have that in mind. Please go ahead and read this first set.

**Respondent:** That's okay.

**Moderator:** What are your thoughts? I'm curious just your reaction to this first collection of messages. What thoughts or impressions were?

**Respondent:** It seems like a good amount of information given out in a small amount of typeface. Seems like there's a lot of good information in there.

**Moderator:** Got it. And when you describe it as good information, how so? Like, what makes it good information, or what's good about it?

**Respondent:** It's hitting on the important things. Like, in the first message, it's talking about how they collaborate with the SMA community. So it's showing that they get input from patients and doctors and that kind of thing, and that they're listening because it says reflect your feedback. So that tells me that they're talking to the patients and asking what works for them.

**Moderator:** And what does that say to you? Like, the fact that they're collaborating, the fact that they're listening, like, what feeling or impression does that give you?

**Respondent:** I think it would give me more trust in the company and maybe a little more assurance that they're interested in what's important to the patients.

**Moderator:** Got it. Laurie, do you lack trust in the company currently?

**Respondent:** Not in Genentech specifically, but I think most people lack trust in big pharma in some way or the other. I mean, and I don't know if it's lack of trust, but they're a for-profit business. Their main goal is to make money for their shareholders. That's what companies do. But this would make me think that they're actually thinking about the needs of the patient as well. And I don't hate big pharma. I love these treatments for SMA. Don't get me wrong. But, obviously, they're there to make a profit. That's their job. So you just have to balance the two in your brain kind of.

**Moderator:** Got it. So based on reading these three messages, does it change your perspective on Genentech at all seeing this information?

**Respondent:** I mean, I don't have a bad view of Genentech. They produced a drug that just didn't work for me. I love that my sister takes it. She's doing great. It works for her and not me when we're siblings? I don't understand. But she's doing great on it, so I don't have any feelings towards Genentech. I'm glad it's helping my sister.

**Moderator:** Got it. I'd like to have you rank these messages from one to three just based on your preference.

**Respondent:** The first one is number one. Probably in the order they're in. One, two, three.

**Moderator:** Let's talk a little bit more about the first one. Can you tell me more about why you're preferring that statement overall?

**Respondent:** I think, like I said before, because it talks about collaborating with the SMA community, listening to the patient's needs, taking their feedback. So, obviously, they're actively getting information back from patients. That would make me feel more secure in a drug company that the patient's being listened to and that they can base a drug on patient input or a formulation of the drug.

**Moderator:** Got it. Alright. Thank you. And why? Tell me your thoughts about that one.

**Respondent:** I like that it says that they have over five years of experience within the community. It just shows that they're not brand new. Like, they've obviously, if they're still this drug's still being made, they're still helping people. It's working for people. I think that's a good thing to lead with for someone who's trying to decide on which drug to take, what therapy.

**Moderator:** So you made me just think of something. I want to be sure that as we talk about these, that you're coming just from your thoughts and feelings. In other words, don't worry how anyone else would react to these.

**Respondent:** Oh, absolutely.

**Moderator:** Okay. And so tell me more.

**Respondent:** If I weren't on a treatment and I was trying to decide which one, it would make me feel good that they'd had experience.

**Moderator:** Okay. Got it. I just don't want you to pretend. I want you to be who you are in the situation you're in. You are on treatment. In fact, you've been on this drug before. So hearing that, you know, has five years of experience in, you know, people with SMA, does that yeah. I mean, is that meaningful to you at this point in time?

**Respondent:** Yeah. I mean, it's meaningful. It's not gonna get me to switch back, but it's a good thing to say. I mean, it's a good thing to say.

**Moderator:** And as you said, it tells you that it's not brand new. Like, why is that a good thing to say? What do you like about hearing that?

**Respondent:** I think it shows that it's a trusted and proven therapy, that it's not I don't think I would worry so much about taking it because I know it's proven, and it's been used, and it's safe. It was scary taking SPINRAZA right off the bat when it first got approved. That was scary. You know? But I would've taken it without the approval. I would've tried anything.

**Moderator:** Okay. And then in statement y, it is saying that it is the most chosen medicine. I'm gonna highlight that for SMA.

**Respondent:** That makes sense to me. Yeah.

**Moderator:** Does it? Tell me.

**Respondent:** Because of the administration. I would choose that over a spinal injection if I could. Yeah. I think anybody would.

**Moderator:** Right. Okay. I'm curious. Right now, it says most chosen. It could say most prescribed. I'm curious what is the more meaningful idea for you.

**Respondent:** I think chosen because it relates more to the patient than the doctor. It means you know, I'm sure you sit down with your doctor and discuss things, but chosen means, like, I chose that.

**Moderator:** Okay. Got it. You know, I have a landline still. And, yeah, unlike a phone, you can't silence it ahead of time.

**Respondent:** No. You can't. No.

**Moderator:** And then k statement k took your third spot. Tell me why that actually, no. Forgive me. Before I don't want to go so quickly. Seeing the twenty-one thousand patients worldwide, curious just thoughts about that data point.

**Respondent:** I mean, it seems like a lot of patients. I don't know how many patients there are worldwide, like, on treatment. So but that seems like a lot of people on EVRISDI.

**Moderator:** Yep. Got it. Okay. And then statement k, that got your third ranking.

**Respondent:** I think the thing that put me off right at the start was the largest biotech in the world. Like, I don't care how big they are. Like, that doesn't mean anything to me. I don't know. It's like saying some companies, like, we're the largest manufacturer of soda or whatever. Like, I don't care. Just be the best.

**Moderator:** Got it. Okay.

**Respondent:** Doesn't cut it for me. It might for some people, but I just feel like that's like, oh, look at us. You know? Like, a bragging kind of, like,

**Moderator:** Yep. Yep. Okay.

**Respondent:** And I'm not impressed. But maybe I'm fussy.

**Moderator:** What else? Anything else in k that put it at the bottom?

**Respondent:** I mean, it's good that they say they're committed to helping people access the medicines they need, which makes me think that, you know, what it goes on to say about support programs. Because I was on the one when I started. I don't I think I paid, like, 5 to $10 a shipment, so it was I couldn't have afforded it otherwise.

**Moderator:** Right. Okay.

**Respondent:** That's not a bad. That third one's not awful. It's just that largest biotech in the world. I'm not sure that's, like, the brag they think it is.

**Moderator:** Got it.

**Respondent:** I don't know. I think that's a better way to say something like that.

**Moderator:** Well, and that's exactly why we're testing these, so I really value that input. What about the whole leading the way with groundbreaking therapies and pioneering innovation? Like, what was your impression of that part?

**Respondent:** Yeah. I mean, they're the only ones with an oral therapy, so that's very true. I mean, but they're the only ones doing an oral therapy, so they're not really comparing to anyone but themselves.

**Moderator:** Got it. Okay. Let's do our next category. So as you well know, new treatment options are on the horizon, including the expanded indication for the gene therapy. It's actually anticipated to be up to 18 years old. So the introduction of new therapies may prompt patients to start a conversation with their doctor about switching. However, managing SMA is a lifelong journey, so it's important to consider how switching therapies might impact your insurance coverage and future coverage. So this set of messages is really looking to the future state, the future scenario where there will be additional options, including gene therapy for older children. So I'll go I'll let you have a look at this.

**Respondent:** Okay.

**Moderator:** What's your impression of this collection of messages?

**Respondent:** I think it's good information. And I would hope that most doctors would be telling their patients all of this. Because gene therapy is extremely expensive. And I know that I know people who have had that and then had a really hard time getting something like afterwards.

**Moderator:** Got it.

**Respondent:** I think last time I looked, gene therapy was, like, over 2,000,000. So it's a big ask.

**Moderator:** Yep. Okay.

**Respondent:** Yeah.

**Moderator:** So this rings true then from based on conversations and things that you know about what's happening.

**Respondent:** Absolutely. I do know people who have managed both, who have had the gene therapy for babies and or, you know, under twos, basically, when they were little and then still got approved for either SPINRAZA or EVRISDI after.

**Moderator:** Do you know how they manage that?

**Respondent:** No. I'm guessing good insurance.

**Moderator:** How would you rank these statements in terms of your preference?

**Respondent:** I feel like they tell a story kind of. I wasn't really thinking of them as one as better than the other. I think important wise, the second one is to the point like, it's it doesn't mince words. Like, it's saying be careful.

**Moderator:** Okay.

**Respondent:** If you get this gene therapy, you might have trouble getting anything afterwards.

**Moderator:** That's okay that it's to the point and doesn't mince words?

**Respondent:** Absolutely. Yeah. I think it's it's make it as help navigating health insurance and this kind of stuff is so ridiculously and unnecessarily complicated as it is. I think most people would be genuinely pleased just to have something straight and to the point.

**Moderator:** Alright.

**Respondent:** 100%. And maybe the last one would be number two because I think maybe people don't realize that such a large percentage of patients who get gene therapy still go on to need further treatment. A lot of people think, you know, gene therapy is a one and done, and it is, but that doesn't necessarily mean you won't need something different going forward.

**Moderator:** So what are you thinking? Like, seeing this average of forty-four percent of patients still need a DMT, like, what impression or impact is it having for you to see that?

**Respondent:** I'm a little surprised. I didn't realize it was that high. I don't know. I'm surprised. You know? I didn't realize that that higher percentage of patients still had another disease-modifying treatment after gene therapy. It would make me wonder, is that because patients were closer to two when they started it? Because I believe it's more effective the earlier the treatment is received. I'd be curious to know, but that would be thought. But I would wonder if it was the older kids that were still having to go on other treatments.

**Moderator:** Okay. Got it.

**Respondent:** And that makes me think that if it's gonna be approved for kids up to 18, that number will probably go up.

**Moderator:** Lori, I'm gonna pause for one second and bear with me. I have an important note that's coming from the team that I just need to take a look at, so just bear with me for a moment. Alright. So let's we've had some version changes, and so let's see. What would be the best way to go here? Bear with me for a second, Laurie. Luke, how about if I just briefly show the next two sections for you to take a quick look at, and you can tell me if this looks to be the right, okay. I don't I don't think I have another version to go to. Okay. And then here's the last section. Okay. Good. Alright. Okay. Great. So, Luke, I'm gonna keep going then. Is that okay? Okay. Perfect. Alright. We're gonna keep going. Thank you, Laurie. I appreciate that.

**Respondent:** That's why I gave you the message check the email that came in.

**Moderator:** Oh, good. Okay. Good. So let's see. There's a question. You know, I'm gonna there's a again, I'm just gonna be transparent. There's a question that we added relating to this. I don't see it in this guide, but I'm gonna go ahead and ask it anyway.

**Respondent:** Okay.

**Moderator:** Just in case, they'll let me know if I shouldn't have asked it, but it won't hurt anything. It says an average of forty-four percent of patients still need a disease-modifying therapy. We could have stated it as anywhere from twelve to seventy percent. So instead of giving an average, we could have given a range from twelve to seventy percent.

**Respondent:** I'd give the average.

**Moderator:** Okay. Tell me why the average is.

**Respondent:** I don't wanna hear seventy percent.

**Moderator:** Got it.

**Respondent:** To lower the number I mean, I know you say twelve to 70, but my brain is focused on the 70.

**Moderator:** Okay. Got it.

**Respondent:** Forty-four sounds better.

**Moderator:** Yes. Okay. Alright. Understood. Thank you. Alright. Let's finish ranking these. What would you put third? What would you put fourth?

**Respondent:** The s to l. Because I'll go ahead. Okay. Alright.

**Moderator:** So.

**Respondent:** I think it's basically it's reassuring you that the I forgot they were called pals. That they have the pals that are there to help you if you have any issues with getting back on EVRISDI afterwards. So that's reassuring.

**Moderator:** Got it. Okay. And then w came in fourth. Tell me why that is the least preferable of these.

**Respondent:** I don't think it gave a lot of information. It seems very, I'm trying to think what the word is. It's a little confusing. It's really not telling you much.

**Moderator:** Got it. Okay. Thank you. Let's Okay. Yeah. I will ask one more question before I shift gears. So based on this you know, seeing these messages, would this kind of information in any way impact your willingness, your desire to want to switch therapies?

**Respondent:** I don't know that it would sorry. Can I ask that again one more time?

**Moderator:** Yes. Would exposure to this information, learning this information on this page, would it impact your willingness and your desire to switch therapies in the future?

**Respondent:** I don't know that it would impact my desire to switch therapies. I think it would give me a little more assurance that if I want obviously, I'm too old. But if I wanted to try the gene therapy, that there are things out there to help me get back on if I find that I need to in future. And by the looks of it, almost half the people do. So, yeah, it would reassure me that there's help if I needed it.

**Moderator:** Alright. So this section pertains to the moment you and your doctor have made the decision to start treatment. Think about that moment and the processes that you've had to go through as you consider these.

**Respondent:** Okay. Okay.

**Moderator:** What is your reaction to this collection?

**Respondent:** Again, a good amount of information. The bottom one is I like the best because just the fact that it starts out with living with SMA can be a lot to manage. Like, it makes me feel heard. You know? Like, they understand that this is not an easy road, but it just feels kind maybe.

**Moderator:** Okay.

**Respondent:** Like, they understand how hard this is. I was surprised to see it there, but in a good way.

**Moderator:** Okay. And can you say more about being surprised to see it there?

**Respondent:** I guess I don't maybe wouldn't normally it felt personal, kind of. I felt like it related to me. Like, they understand kind of.

**Moderator:** Got it.

**Respondent:** Maybe sympathize.

**Moderator:** And does that give you any particular thought or impression about Genentech or first rate?

**Respondent:** I mean, it would my again, not that I have a bad impression, but it's just reassuring, and it feels like something they didn't have to do, but it feels nice that they did it kind of.

**Moderator:** Understood.

**Respondent:** Unnecessary, but nice to see.

**Moderator:** Got it.

**Respondent:** I think a lot of times, most people with SMA don't people who don't have SMA, like, you cannot understand how much it is. People laugh at me about how I overthink everything, but I have to. I have to plan for every possible thing that can go wrong because stuff goes wrong all the time. I just got we're on step three of getting my wheelchair fixed. So to not have my wheelchair and to be in my crappy eight-year-old one that isn't as easy to transfer from, the battery doesn't last as long. It's super uncomfortable. So it's just there's always something you've always got a plan for every possible outcome.

**Moderator:** Got it.

**Respondent:** Absolutely exhausting.

**Moderator:** I knew at the beginning of our conversation that we had barely tipped the iceberg not even tipped the iceberg of what we could have talked about. So thank you for giving me some additional insight there.

**Respondent:** Sure.

**Moderator:** Yeah. We could fill the hour and plus there, but I appreciate the insight that you're giving me. Anything else about q that had you rank it first?

**Respondent:** Just the fact that they talk about how the PALS can assist with all aspects of the EVRISDI experience. So it makes you feel like you're not alone. Like, there are people that help you on this journey. And I like that it says all aspects, like, all aspects of your EVRISDI experience. I wouldn't have access in there, but, you know, I don't direct you for a living.

**Moderator:** Yeah. No. Understood. Yeah. The focus of this particular communication is access, but I get where you're coming from with that.

**Respondent:** Yeah.

**Moderator:** What would you rank number two?

**Respondent:** And the fact that it's personalized support also.

**Moderator:** Got it. Okay. Thank you.

**Respondent:** That would make me think, you know, there's one person I'm gonna get to speak to all the time who's gonna be my liaison, who's gonna help me, which is nice. It's nice to have, like, a continuity to have the same person all the time. I think that's helpful.

**Moderator:** Okay.

**Respondent:** I'm trying to think best the second one. Probably the second one would be number two.

**Moderator:** Mhmm.

**Respondent:** V. No.

**Moderator:** Oh, forgive me. Yes. You said it, and I processed it incorrectly. Thank you. Okay.

**Respondent:** I think just the fact that it talks about understanding insurance coverage because insurance is so hard to understand. I still I mean, I lived in England for fifteen years, and, you know, you didn't pay for anything over there because it was National Health Service. So had I known what it was like here, I don't think I would have moved back to the States. I think I would have stayed in England. Because the amount of money we pay every month for health insurance should be criminal. So I definitely would have stayed there and raised my family in Europe, not in America.

**Moderator:** Got it. Okay.

**Respondent:** But I'm glad I didn't know in a way because my parents are here and my sister. And so.

**Moderator:** Right. And then tell me about x, about that being your third ranked.

**Respondent:** I think probably I mean, it's just being honest, but I think the fact that it talks about denials are common, it just has such a negative connotation. That probably put me off, and that's probably why I put it as number three. I'm sure that's information that's important to get to people, but you know? And then it says they can always be appealed. So that's the positive side.

**Moderator:** Yeah. Okay. What did you think about the four out of five appeals are successful?

**Respondent:** I thought it would have been higher. So that makes me a little sad that twenty percent of people are being denied a medicine that their doctors say they need.

**Moderator:** Okay. Okay.

**Respondent:** But that's the American way, unfortunately.

**Moderator:** Yeah. Alright. Let's take a look at our final category here.

**Respondent:** Okay.

**Moderator:** What do you think about this collection overall? How are they landing for you?

**Respondent:** The last one, I think, would be my favorite because I like that it says it's even including people who are uninsured and letting you know that there's a patient foundation to help even if you don't have insurance.

**Moderator:** Got it.

**Respondent:** Because there's a lot of uninsured people in this country.

**Moderator:** Had you heard of the Genentech Patient Foundation previously?

**Respondent:** I don't know that I may have, but we've had insurance, so it wouldn't have applied to me. So I don't recall specifically. But that's a nice thing. And that's what the drug company should do. I mean, that's what they should do. Whether they can continue to keep doing it at the levels they're doing it at in the next year or two, I'll be curious to see.

**Moderator:** How so? Tell me more what you're thinking.

**Respondent:** If the subsidies aren't extended for the people on the ACA, then there's gonna be millions and millions of people who are losing their health insurance, which is gonna be a real issue. We're lucky. You know, my husband has a good job, touch wood. We get, I'm told, really good insurance, but it's really expensive through his company. So it's not gonna affect me, hopefully, but we could see our premiums go up. And they're all crazy high. So. But I, you know, I worry about the people who don't who can't afford insurance, who don't have insurance.

**Moderator:** What would you rank second and third?

**Respondent:** I would say going up again. The middle one's number two. Because I like that it talks about improving access and options for patient assistance, which kind of reminds me of the bottom one. It's a similar message just written differently.

**Moderator:** What about that phrasing? We've worked to minimize insurance bottlenecks and support patient and provider choice. Just curious what you think about that.

**Respondent:** I mean, I think the insurance bottlenecks resonates, I'm sure. Because people who either parents with children with SMA or adults, I mean, certainly understand the minefield that can be, you know, health insurance companies and getting things approved. So I think that would be reassuring to people to see, or that would be reassuring to me to see.

**Moderator:** Okay. Tell me about r.

**Respondent:** I mean, it kinda says what the other ones say. It's just maybe with a little less information and finesse.

**Moderator:** Tell me about it being less finesse.

**Respondent:** And this is just picky on my part. I don't like sentences that start with numbers. I don't know why.

**Moderator:** No. I hear you on that.

**Respondent:** It could say up to 90%, or there could be a. It just I don't know. I'm sure it's English, but I just don't like sentences that start with a number.

**Moderator:** Yeah. And, yeah, I'm sure the proper way would be to do it otherwise. Oh, let me ask you this. Given that these are meant these are such, like, rough drafts, is there anything about the content that had you place it third? Because of, you know, like, smoothing out the sentences is an easy fix. So I'm curious.

**Respondent:** I think yeah. I think in the first one, the bottom ones talk about, like, improving access and support for uninsured and our commitment extends to everyone. And the first one kinda just says we have resources to help ensure that you have access to the treatment. But that kind of to me, it feels like it's laying it on me. Like, we're gonna give you the resources to sort it out. Whereas the other one, more like, we're going to help you.

**Moderator:** Got it. Okay. Got it. What do you think about the 90%?

**Respondent:** I don't know. Like, I don't know whether I would expect that to be higher or lower. Or I mean, I would have thought anybody with SMA should be covered for any drug their doctor says that they need. That's not the way it goes. So maybe I honestly don't know. I don't know what I think about that.

**Moderator:** Alright. Scale of one to seven, how strong or not strong is that statistic? One would be that is not at all strong, 90%, and seven would be,

**Respondent:** Then I would probably say six.

**Moderator:** Mhmm. Okay.

**Respondent:** I mean, it's a high number. It's a high percentage.

**Moderator:** Yeah. Okay.

**Respondent:** So.

**Moderator:** What if it was 80% or 70 or 60? Like, at what point would you say, okay. That's not a strong number anymore.

**Respondent:** Probably 70.

**Moderator:** Mhmm. Okay. Why 70?

**Respondent:** Because it should be higher than that. Like, that's not good that if 30% of people are being you know, don't even have it covered, that's high a number of people not getting what they need.

**Moderator:** Okay. Right now, says insured people. What if it said commercially insured people? What does that phrase commercially insured mean to you?

**Respondent:** People like me. I assume commercially to me means, people who get and I might be wrong, but people who get their insurance through their employer or who purchase insurance through the ACA, that would be I don't know if the ACA includes commercially insured. I would think it would, but I don't know for certain.

**Moderator:** Okay. Thank you. Here is the full list of messages that we just looked at. Let me go a little bit larger.

**Respondent:** Please. My reading glasses can only do so much.

**Moderator:** Alright. So could you select the statements, the messages that, in your opinion, create just the most compelling story regarding access to EVRISDI?

**Respondent:** How many am I looking?

**Moderator:** You are not limited. You can choose as few or as many as you'd like. You can include one or more from every category, or you can omit one or more categories altogether. You have free rein.

**Respondent:** Okay. Well, definitely d. First one. Yep. N l y. SL. HQ. CP and CF.

**Moderator:** Okay. What order would you feel would be the most compelling?

**Respondent:** I like LD a lot.

**Moderator:** So start out. That would be the first like, if you were telling the story, you would start with LD. Oh, I forget. Not this. So not ranking them per se, but just, like, the order like, if, like, if we were to weave those statements together into the story, which one should they start with? And, like, you know, like, how should be the progression of combining these ideas?

**Respondent:** I would say if you want to appeal to people straight away, then you would start with something like HQ.

**Moderator:** Okay. And that would so.

**Respondent:** Yes. To me. Yep. Because that's the one that really pulls me in. And says, we understand. You know, we're here to help. We know you're going through a lot. So that one to me would instantly get my attention and get me to read all the rest of it.

**Moderator:** Okay.

**Respondent:** So, I'd probably go to LD at that point.

**Moderator:** K.

**Respondent:** It talks about the collaboration within the SMA community, so that also gives me reassurance that there's a dialogue between the company and people like me.

**Moderator:** And then wherein.

**Respondent:** You don't need to justify your order per se at this stage,

**Moderator:** But would love to just kinda capture your order.

**Respondent:** Okay. So after that, probably the one below it, l y, and then HV, CP, CF, SL.

**Moderator:** Okay. Got it.

**Respondent:** That's the order I would do.

**Moderator:** If I forced you to identify the top two most important messages out of the seven that we have here.

**Respondent:** HQLD.

**Moderator:** Okay. Boy, that did not that did not require a thought.

**Respondent:** I'm a girl who knows her mind.

**Moderator:** I know why HQ, I feel like, because you've spoken about that. And why LD? Why is that the other really important one here?

**Respondent:** Again, it makes me feel like my voice is heard that they're listening to people within the SMA community. They're not just doing this on their own. They're actively collaborating with the SMA community.

**Moderator:** Okay. Got it. What just, like, what just really stood out for you today? We saw all of these different things. Was there anything that, like, okay. Wow. Like, that that that stuck with me or that had an impact on me, whether it's a message or a word or a phrase. Like, yeah, just where, if at all, was there impact in all of this?

**Respondent:** You know what I'm gonna say? Living with SMA can be a lot to manage. That was the really that was the one thing that I read today that I thought, yeah, it can.

**Moderator:** Yeah. Yeah.

**Respondent:** Like that hits. You know? That was the one thing, and I'll remember that.

**Moderator:** Okay. Alright. Got it. I wanna go to h x for just a moment.

**Respondent:** Okay.

**Moderator:** I'm curious just if there was anything new or helpful in Ajax.

**Respondent:** I don't think so.

**Moderator:** Okay.

**Respondent:** I mean, I know about the pals, so I know that's what they did.

**Moderator:** Laurie, I'm curious, whether you had already had a sense for the fact that patients can appeal. In other words, an appeal doesn't have to just come from the doctor. The patient themselves can also appeal.

**Respondent:** Yes.

**Moderator:** Okay. So that's something you were already aware of.

**Respondent:** Yep.

**Moderator:** Got it. So I'm just giving my team just a second to see if there's anything else that they might want to and I'm doing a quick check here, see if there's anything else. I guess, while I'm waiting on them, the I feel like you answered this very early in our conversation, but now that we've been through all of this, based on what you've seen today, is there anything that would increase your likelihood to consider EVRISDI in the future? Or let me rephrase it. Did your likelihood to consider in the future stay the same, increase, or decrease based on this?

**Respondent:** Stay the same.

**Moderator:** Okay. You had indicated that earlier, but I wanted to be sure.

**Respondent:** Yep.

**Moderator:** Got it. Okay. Alright. I am not seeing any other questions come in, so I think we are good. Laurie, thank you. Truly a pleasure to talk with you today.

**Respondent:** Really nice to speak as well.

**Moderator:** Thank you.

**Respondent:** Alright. Have a good day.

**Moderator:** Take care. You too.

**Respondent:** Bye.

**Moderator:** Bye.