**qual test**

*Oct 28, 2025 | 5:00 PM | R01 Transcript*

Interview Date: October 28, 2025

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**Moderator:** Let me dive right in with the disclosures. We will be talking about SMA, so hopefully that matches your expectation for our topic. We'll be recording the conversation, and we've got a few members of the research team in the background who will be listening and taking notes along with me. I am an independent researcher, so I have no skin in the game, as they say. Just hoping for your candor as we work our way through our conversation. I want to make you aware that we are required to report adverse events as they pertain to the study sponsor. So to the best of our ability, let's avoid discussing any issues that you've had with any of the treatments that you use for SMA, if possible. In terms of our road map, I have some opening questions for you, and then we will spend time reviewing some materials that I have. I'll tell you more about that when we shift gears, but wanted to let you know what to expect. Any questions?

**Respondent:** No. Sounds good.

**Moderator:** So let's dive in. Doctor, can you start by telling me a little bit about you and your practice?

**Respondent:** Yeah. I'm a professor in pediatric neurology, and I work in an academic medical center. I've been working this job for over twenty years. We have a dedicated MDA clinic and see these patients in this dedicated clinic. In my life, I've probably seen about 30 patients. Right now, I have 13 patients I see on a regular basis.

**Moderator:** Got it. 13, you said? One three?

**Respondent:** Yes, one three.

**Moderator:** Thank you. Can you tell me a little bit more about the patients? Are they all under 18? Do you have any young adults?

**Respondent:** We have eighty percent under 18 and twenty percent are 18 or older. The oldest is 22. We typically continue to see them as they age.

**Moderator:** Thank you. At a high level, can you talk to me about your approach to treating SMA?

**Respondent:** Treating SMA, we know it's a genetic mutation causing death. The best treatment is still gene therapy if patients are younger than two years old before they are able to receive SPINRAZA. Gene therapy has an age limit, so we definitely put younger patients on it when they are diagnosed. For other patients, we have two options: intrathecal injections like SPINRAZA, and I have five patients on that. The rest are on the pill. I have not had any patients on double therapy. One patient after gene therapy started having muscle weakness, so we are considering adding the PO medication, but it hasn't started yet.

**Moderator:** Got it. And you're saying it's muscle weakness that has you considering the combo therapy?

**Respondent:** Yes, correct.

**Moderator:** Thank you for that overview. What that tells me is that other patients who have been treated with Zolgensma have not gone on to receive SPINRAZA or Evrysdi. Correct?

**Respondent:** Correct. After the first treatment, if they show rapid progression of muscle weakness or deteriorating motor function, that's when they require additional treatment. But it's not required if you're not seeing a decline.

**Moderator:** Thank you. Just to wrap up our initial conversation, doctor, I'm curious what kind of experience have you had with insurance access to these different therapies? Are there any challenges?

**Respondent:** Insurance is much better now. It used to take us six months at least, but now it's much quicker. Combinations are still challenging because they have to be justified. We have to document the patient's condition after gene therapy. With monotherapy, it's justified, and the insurance company usually approves it.

**Moderator:** Have you already gone to appeal in that one patient?

**Respondent:** Yes, we are currently appealing. We have to document their motor scale and symptoms.

**Moderator:** What are the kinds of things they're asking you to document or provide?

**Respondent:** Motor function, fine motor skills, breathing, and the speed of regression. We have to use some motor scale to document the loss of motor function.

**Moderator:** Is the manufacturer helping with any of the paperwork or the appeal process?

**Respondent:** Yes, they are helping.

**Moderator:** For time's sake, I'm going to move on. If the team has any follow-up questions, they'll let me know. I have a series of messages broken down into four categories. These are draft statements, and the point of this research is to get feedback on these draft ideas. With each category, I have an opening statement that I'll read to you before we dive in. I'm going to bring it up on the screen, but bear with me. Don't start reading until I have a chance to give you my opening statement.

**Respondent:** Okay.

**Moderator:** Our first category pertains to the moment you and your patient or the patient's family have made the decision to start treatment. I want you to think about that moment and the processes you and your patients go through as you consider these messages. You want me to rank these three statements by preference?

**Respondent:** Yes. I think WHC is one. The first one. G is second, and HR is third.

**Moderator:** Let's start with statement G. What is your reaction to that statement?

**Respondent:** Manufacturers often provide information about appeals and have experience with different patients and physicians. They can guide the process and help with successful appeals. The support can help ease the burden for authorization. The PA checklist is helpful to organize appeals.

**Moderator:** Have you used that PA checklist?

**Respondent:** Yes, they provide a lot of information, including the checklist and templates. It's very helpful.

**Moderator:** How willing are you to pursue getting through all of this for your SMA patients?

**Respondent:** When we see the patient declining, we definitely want to get treatment. It's important for any patient whose symptoms are getting worse with SMA.

**Moderator:** Doctor, I know you said the patient is rapidly progressing. Have you ever heard of the Evrysdi Bridge program from Genentech?

**Respondent:** Yes, I've heard about it. It's for short-term support during the appeal process. It's helpful, but I have not used it.

**Moderator:** Is it something you plan to consider for this patient?

**Respondent:** Yes, it will be considered.

**Moderator:** Thank you. Let's touch on N and R quickly. What was your reaction to statement N?

**Respondent:** It's a generous statement, but not very concrete.

**Moderator:** And R, what is your reaction to statement R?

**Respondent:** It's true that you shouldn't give up after a denial. It's encouraging to know that it's a typical process.

**Moderator:** Why did you rank it in third place?

**Respondent:** I think it should be second, and N should be third. Sorry for the mistake.

**Moderator:** No problem. Let's move on to our next category. These messages pertain to the ease of getting Evrysdi from an insurance coverage standpoint.

**Respondent:** I think statement K is strong. Ninety percent of patients with commercial or Medicaid coverage is higher than I expected. It's a strong statistic.

**Moderator:** On a scale of one to seven, how strong do you feel 90% is as a statistic?

**Respondent:** Seven. It's strong.

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

**Respondent:** Zero co-pay. It doesn't cost the patient anything when approved. Third would be M.

**Moderator:** Why rank J fourth?

**Respondent:** It's comprehensive, but I would put it as two. It's a very comprehensive statement.

**Moderator:** When you hear "underinsured," what do you think they're referring to?

**Respondent:** Patients worried about their coverage. Different types of insurance may not authorize this treatment. The statement reassures them.

**Moderator:** Let's take a moment on statement M. What are your thoughts there?

**Respondent:** It's a generic statement about coverage and authorization. The company is committed to ensuring pricing.

**Moderator:** Let's circle back to L. I know we initially had it ranked high, but then moved it down.

**Respondent:** Initially, I thought about zero co-pay, but the main thing is coverage. Zero co-pay is a benefit, but coverage is more important.

**Moderator:** Thank you. These messages are related to Genentech's legacy and experience in SMA.

**Respondent:** The first statement, E, is number one. Over twenty-one thousand patients globally is a strong number. It shows the market share and the efficacy of the treatment.

**Moderator:** What impression does that number give you?

**Respondent:** It suggests a large market share and that many patients have been using this medication. It indicates efficacy and tolerability.

**Moderator:** The statement says Evrysdi is the most chosen disease-modifying therapy. Is "most chosen" or "most prescribed" stronger for you?

**Respondent:** "Most chosen" is more meaningful.

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

**Respondent:** Second is S. It emphasizes the company's commitment to SMA treatment with innovation. Third is W. It's about collaboration with the community, but it's more general.

**Moderator:** This category addresses a future state. New therapies may prompt conversations about switching. It's important to consider how switching could impact access to other options long term.

**Respondent:** It's important to be prepared for additional treatment if gene therapy doesn't meet expectations. Even with gene therapy, secondary treatment is important.

**Moderator:** How would you rank these statements?

**Respondent:** Y is number one. It gives the situation you will most likely face. Second is A. It clearly states that forty-four percent of patients require additional therapy.

**Moderator:** What is your reaction to the data that forty-four percent on average require additional therapy?

**Respondent:** It's a high percentage, suggesting you need to be ready for additional treatment. It's higher than expected.

**Moderator:** We could state it as a range from twelve to seventy percent. Is it stronger as an average or a range?

**Respondent:** The average of forty-four percent is stronger.

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

**Respondent:** Third is B, and fourth is O. Switching back to Evrysdi is not common.

**Moderator:** Here are the messages we have looked at together. I'd like you to create the most compelling access story for Evrysdi. You can choose as many or as few of these messages as you want.

**Respondent:** I would choose the statement about twenty-one thousand patients, the forty-four percent needing secondary therapy, and the manufacturer's support. Also, the innovation and commitment statement.

**Moderator:** Which two are the most important?

**Respondent:** The twenty-one thousand patients and the manufacturer's support for financial challenges.

**Moderator:** One last section. This section dives deeper into Genentech's free drug programs. There is concern that misuse of the program could jeopardize its future as it is a limited resource.

**Respondent:** L is strong. It guarantees treatment for those with limited financial means. Q and L are similar. They provide bridging support. B is last.

**Moderator:** What is your reaction to the concern about misuse of the program?

**Respondent:** It's important to use the program in good faith. The majority of patients need it, and the company's support is essential.

**Moderator:** Is it okay for the company to remind doctors to use the program as a last resort?

**Respondent:** Yes, it's important to be cautious and ensure the program is used appropriately.

**Moderator:** Doctor, thank you. We got through it. Thank you so much for your time today. I really appreciate it.

**Respondent:** Thank you. Have a good rest of your night. Bye.