SMA Demand Qual

(Sep 5, 2024 - 12:30pm)

(00:00:03 - 00:00:04)

**Doctor:** No problem.



(00:00:04 - 00:00:06)

**Stacey:** I'm assuming you've done this before,



(00:00:07 - 00:00:07)

**Doctor:** Yeah.



(00:00:07 - 00:00:15)

**Stacey:** But, good. Then, I'm gonna run through the disclosures. I'm sure they will sound familiar to you, but please do let me know if you have any questions.



(00:00:15 - 00:00:16)

**Doctor:** Okay.



(00:00:16 - 00:00:38)

**Stacey:** So our topic today is SMA, spinal muscular atrophy, and we have an hour together and want you to know first and foremost there are no right or wrong answers. I work for an independent research agency. We have no vested interest in the opinions and experiences that you shared today, so hoping you'll just be one hundred percent candid.



(00:00:39 - 00:00:39)

**Doctor:** Okay.



(00:00:40 - 00:00:50)

**Stacey:** We want to protect your anonymity, so I will ask you not to mention your name or practice name or even the state that you practice in. We'll just keep those specifics out of our.



(00:00:50 - 00:00:51)

**Doctor:** Okay.



(00:00:50 - 00:01:27)

**Stacey:** Conversation. We will have a few listeners in the background from the research team. We will be recording so that we have a backup to our note taking. And, those are the basics. Also wanted to touch on adverse events. You are probably aware that, we are required to report adverse events when we hear them in market research or product complaints. So to the best of our ability, we'll try to avoid that. If it does happen, we'll cross that bridge when we get there, but know that we will ask you whether we may include your contact details with that adverse event report or you may opt to remain anonymous.



(00:01:28 - 00:01:28)

**Doctor:** Okay.



(00:01:28 - 00:01:32)

**Stacey:** Are you comfortable proceeding with all of these parameters in mind?



(00:01:33 - 00:01:33)

**Doctor:** Yes.



(00:01:33 - 00:01:41)

**Stacey:** Okay. Great. So let's dive in. Again, without any identifying details, can you tell me a little bit about you and your practice?



(00:01:42 - 00:01:54)

**Doctor:** Sure. I work in a large academic institution, and, I'm a primary I'm a pediatric neurologist, and I work both inpatient and outpatient settings.



(00:01:54 - 00:01:59)

**Stacey:** Got it. And, doctor, I understand that you're at a an SMA center of excellence. Is that.



(00:01:59 - 00:02:00)

**Doctor:** Mhmm.



(00:02:00 - 00:02:00)

**Stacey:** Correct?



(00:02:01 - 00:02:01)

**Doctor:** Yes.



(00:02:01 - 00:02:05)

**Stacey:** Got it. Alright. And, how many years have you been in practice?



(00:02:06 - 00:02:06)

**Doctor:** About five.



(00:02:07 - 00:02:18)

**Stacey:** Okay. Alright. Thank you. Alright. So can you, talk to me about your SMA population? How many patients do you have? Can you tell me a little bit about your again, that that group of patients?



(00:02:18 - 00:02:45)

**Doctor:** Yeah. Probably about twenty, patients in the pediatric SMA population. They're mostly diagnosed, from a pretty young age. Now that we have, newborn screening, they're becoming becoming more readily detected, at birth. So that's great. What else?



(00:02:45 - 00:02:52)

**Stacey:** What is the age range? You know, how how, how old will you go in in your pediatric practice?



(00:02:52 - 00:03:05)

**Doctor:** Yeah. Yeah. I mostly see younger, in the baby to toddler range, but, you know, they still I still see some in, like, the up to, you know, around age ten.



(00:03:07 - 00:03:13)

**Stacey:** Mhmm. Got it. And anything unique about your SMA population, would you say?



(00:03:14 - 00:03:15)

**Doctor:** No. Not really.



(00:03:15 - 00:03:28)

**Stacey:** Okay. Alright. So talk to me about treating SMA. You know, what do you have preferred treatments that you're more typically reaching for for these patients? Tell me about that.



(00:03:28 - 00:04:10)

**Doctor:** Yeah. If they're diagnosed, at birth and newborn screening, then knowing that, most families opt for a one time therapy. So they would like to they prefer the one time infusion therapy versus, some some patients have already had access to SPINRAZA sooner, so they might, have been doing SPINRAZA. And, and depending on how they're tolerating it, they it might not be something that the families want to continue to do. They might prefer to do an oral therapy, so they may switch to an oral, they may switch to Averis d.



(00:04:10 - 00:04:21)

**Stacey:** Mhmm. Got it. And what what tends to be those factors? Like, can you say more about why a family might prefer an oral option to look at that?



(00:04:22 - 00:04:26)

**Doctor:** They just might not like the coming in for repeat, spinal.



(00:04:27 - 00:04:28)

**Stacey:** Mhmm.



(00:04:27 - 00:04:29)

**Doctor:** Spinal tap kind of.



(00:04:31 - 00:04:34)

**Stacey:** Is it a convenience issue with.



(00:04:33 - 00:04:34)

**Doctor:** Therapies.



(00:04:34 - 00:04:35)

**Stacey:** Coming back in or.



(00:04:35 - 00:04:35)

**Doctor:** It.



(00:04:35 - 00:04:36)

**Stacey:** Or.



(00:04:35 - 00:04:36)

**Doctor:** May be.



(00:04:36 - 00:04:36)

**Stacey:** Something.



(00:04:36 - 00:04:36)

**Doctor:** Sometimes.



(00:04:36 - 00:04:36)

**Stacey:** Else?



(00:04:36 - 00:04:40)

**Doctor:** They live far away and they don't wanna have to do that.



(00:04:40 - 00:04:40)

**Stacey:** Mhmm.



(00:04:41 - 00:04:47)

**Doctor:** So frequently. And then sometimes, they just don't feel like they wanna put their child through that.



(00:04:49 - 00:05:01)

**Stacey:** Got it. K. Thank you. So I'm gonna share my screen, and it'll just take me a few clicks to get there. Bear with me. Alright. There we go. Alright. Can you.



Screen Shared @ 00:05:01 by Stacey



(00:05:01 - 00:05:04)

**Stacey:** See that? You should see welcome on the screen.



(00:05:04 - 00:05:05)

**Doctor:** Mhmm.



(00:05:05 - 00:05:29)

**Stacey:** Okay. Good. Alright. So let me get a sense for, you know, really the the frequency that, with which you're, you know, using these different treatments with your pediatric population. So we can do this in percentage, or you can break down twenty patients into the various, rows here, whichever is an easier way for you to think about that.



(00:05:29 - 00:05:40)

**Doctor:** Okay. Let's see. I'll start with, I probably have about twelve patients doing Zolgensma.



(00:05:41 - 00:05:42)

**Stacey:** Okay.



(00:05:45 - 00:05:56)

**Doctor:** I have maybe five Spinraza and the rest Everesti.



(00:05:55 - 00:05:56)

**Stacey:** Mhmm.



(00:05:56 - 00:06:03)

**Doctor:** I don't have any combination, but I but I think I would like to start trying combination.



(00:06:05 - 00:06:07)

**Stacey:** What appeals to you about the combination?



(00:06:08 - 00:06:10)

**Doctor:** Just to see if there's any added benefit.



(00:06:10 - 00:06:11)

**Stacey:** Mhmm.



(00:06:11 - 00:06:13)

**Doctor:** Or in terms of, like, motor.



(00:06:13 - 00:06:13)

**Stacey:** Mhmm.



(00:06:13 - 00:06:14)

**Doctor:** Motor development.



(00:06:15 - 00:06:43)

**Stacey:** Okay. Got it. Alright. So okay. Thank you. So tell me, in terms of the Everesti and the Spinraza patients, looking at Everesti first, those three, how many of those started on Efris d and have remained versus maybe switched from, SPINRAZA?



(00:06:45 - 00:06:50)

**Doctor:** They probably have switched from Spinraza. Those are probably they've already tried Spinraza.



(00:06:50 - 00:06:50)

**Stacey:** Got it.



(00:06:50 - 00:06:51)

**Doctor:** Oh,



(00:06:50 - 00:06:51)

**Stacey:** So all.



(00:06:51 - 00:06:51)

**Doctor:** Yeah.



(00:06:51 - 00:06:52)

**Stacey:** Has. Okay.



(00:06:52 - 00:06:53)

**Doctor:** Yeah.



(00:06:53 - 00:07:06)

**Stacey:** Got it. Alright. And what, you know, what tends to be the the prompt, for I know we touched on this a little bit. Anything else you wanna add there in terms of what prompts, a, you know, a.



(00:07:06 - 00:07:07)

**Doctor:** No. It's mainly,



(00:07:07 - 00:07:07)

**Stacey:** Family.



(00:07:07 - 00:07:08)

**Doctor:** Like, they don't wanna travel.



(00:07:08 - 00:07:08)

**Stacey:** To.



(00:07:08 - 00:07:09)

**Doctor:** Frequently or.



(00:07:09 - 00:07:09)

**Stacey:** Make.



(00:07:09 - 00:07:10)

**Doctor:** They don't,



(00:07:10 - 00:07:10)

**Stacey:** That.



(00:07:10 - 00:07:11)

**Doctor:** Wanna put their.



(00:07:11 - 00:07:11)

**Stacey:** Switch?



(00:07:11 - 00:07:12)

**Doctor:** Child through.



(00:07:12 - 00:07:12)

**Stacey:** Okay.



(00:07:12 - 00:07:13)

**Doctor:** The mode of administration.



(00:07:14 - 00:07:24)

**Stacey:** Got it. Alright. And the five on SPINRAZA, how many of those began and remained on SPINRAZA versus, perhaps switched from the first e?



(00:07:26 - 00:07:30)

**Doctor:** Those have probably all began remained on Spinraza.



(00:07:35 - 00:07:57)

**Stacey:** Spelling mistakes don't count, so bear with me. Okay. Got it. That is helpful. Thank you. So when you are, you know, thinking about the the treatments that are available for your SMA patients, you know, what is what is most important to you as the the physician?



(00:08:00 - 00:08:15)

**Doctor:** The, what the family finds to be beneficial is the most probably important. And I would say making sure that they stop losing milestones. That.



(00:08:15 - 00:08:15)

**Stacey:** Mhmm.



(00:08:15 - 00:08:22)

**Doctor:** Development continues to, continues to improve and not decline.



(00:08:23 - 00:08:30)

**Stacey:** Mhmm. Got it. And when you say what the family plan is beneficial, meaning like, what kinds of things are you thinking about.



(00:08:30 - 00:08:30)

**Doctor:** Meaning,



(00:08:30 - 00:08:31)

**Stacey:** There?



(00:08:30 - 00:08:33)

**Doctor:** Mainly their motor gains. They wanna.



(00:08:32 - 00:08:33)

**Stacey:** Okay.



(00:08:33 - 00:08:36)

**Doctor:** Make sure yeah. Feeling like the the child is developing appropriately.



(00:08:37 - 00:08:49)

**Stacey:** Okay. Doctor, what's your wish list? When you think about the treatment options that you have available to to you today, you know, what are some of the the things that you'd most like to see in new, treatments that may come along?



(00:08:51 - 00:09:05)

**Doctor:** I would like to see, onetime treatments that are just, like, very effective. Like, I think Zolgensma is a good onetime treatment, but I think it could be better. It could just be more effective.



(00:09:06 - 00:09:12)

**Stacey:** And more effective in sense of what? Like, what if you could make Zolgensma more ideal, what would you.



(00:09:12 - 00:09:12)

**Doctor:** Like,



(00:09:12 - 00:09:13)

**Stacey:** Improve.



(00:09:12 - 00:09:13)

**Doctor:** Making the.



(00:09:13 - 00:09:13)

**Stacey:** About.



(00:09:13 - 00:09:13)

**Doctor:** Patient.



(00:09:13 - 00:09:13)

**Stacey:** It?



(00:09:13 - 00:09:19)

**Doctor:** Develop completely normally, whereas I think that they still aren't they're not developing completely normally.



(00:09:19 - 00:09:20)

**Stacey:** Got it. Okay.



(00:09:20 - 00:09:23)

**Doctor:** And I think some patients would want additional therapy.



(00:09:23 - 00:09:39)

**Stacey:** Okay. Got it. What else? So, you know, short of a cure or cure or, like, just, you know, like, getting everything, are there other incremental items on your your wish list for new therapy options or advancements.



(00:09:42 - 00:09:43)

**Doctor:** Like, minimal side effects.



(00:09:44 - 00:11:08)

**Stacey:** K. Got it. Okay. Thank you. Alright. So I wanna share a few more things with you. I have four pages of materials that we will look at, for this next section. What you're going to see is a, a compilation of different product features and attributes that relate to, you know, current and and potential SMA treatment options. So the team has attempted to be as comprehensive as possible, but I'll be interested as we go through to know if anything is missing, just so that you know. And so just to give you context, this list of attributes will be used in future research where there won't be someone like me moderating the conversation, where a physician would be maybe answering questions online. And so we're very interested to know about clarity, you know, do the things you receive make sense? If you were, you know, working with these things on your own, would you get stuck on anything? You know, those kinds of things. So I think it will become clear once I bring up the first page, which I will do now. So you can see it's broken down by sections. So take a, you know, take a a look through, and if anything immediately catches your eye positively or negatively, let me know.



(00:11:21 - 00:11:27)

**Doctor:** Okay. I mean, these are all, like yeah. I mean, this all sounds reasonable.



(00:11:28 - 00:11:41)

**Stacey:** Okay. So are the are these written in the the clinical language that resonates with you? Is there anything that was clunky, or you're like, oh, I wouldn't say it that way. I wouldn't expect to read it that way.



(00:11:44 - 00:11:45)

**Doctor:** No.



(00:11:45 - 00:11:58)

**Stacey:** Okay. Alright. Anything that left you with a question, like, oh, I'm not maybe I'm not sure what that means, or I wish I had I wish a follow-up or want more detail.



(00:11:58 - 00:11:58)

**Doctor:** No.



(00:11:58 - 00:12:19)

**Stacey:** On any of these points. Okay. Alright. So wanna have you look a little bit more at the route of administration. You'll see a couple of items there. How important is the route of administration to you when you're, you know, deciding what you wanna recommend or prescribe to an SMA family?



(00:12:20 - 00:12:36)

**Doctor:** I choose whatever is most effective. So the route of administration, I mean, isn't going to affect it too much. But, I mean, if it's an infant, they're not gonna take a non chewable tablet by mouth. So that's just yeah. That wouldn't be a a feasible thing to do.



(00:12:36 - 00:12:46)

**Stacey:** Got it. So forgive perhaps the obvious question, but can you say more about that effectiveness trumps, you know, route of administration? Like,



(00:12:46 - 00:12:53)

**Doctor:** Yeah. Like, it doesn't matter if it is intrathecal or IV. It's all able to be it's all will to be done,



(00:12:53 - 00:12:54)

**Stacey:** Okay.



(00:12:53 - 00:13:05)

**Doctor:** At our institution. So it doesn't make much of a difference. Families do prefer probably, like, a a one time IV versus an intrathecal injection, and that's easier for providers as well-to-do IV.



(00:13:05 - 00:13:06)

**Stacey:** Yeah.



(00:13:05 - 00:13:11)

**Doctor:** But, if intrathecal is more effective, then, I would be more inclined to do that.



(00:13:11 - 00:13:21)

**Stacey:** Mhmm. Okay. Alright. Got it. Do you feel like the route of administration has any impact on the efficacy of the treatment? Like, do you presume.



(00:13:21 - 00:13:22)

**Doctor:** I mean,



(00:13:21 - 00:13:22)

**Stacey:** Effort?



(00:13:22 - 00:13:27)

**Doctor:** Maybe the CNS, you know, drugs that go to the CNS have some have better penetration because they're already in CNS,



(00:13:28 - 00:13:28)

**Stacey:** Mhmm.



(00:13:28 - 00:13:34)

**Doctor:** Than an IV. But I think we have plenty of CNS penetrating medications that work just fine.



(00:13:34 - 00:13:53)

**Stacey:** Mhmm. And, mechanism of action at the top. First of all, you know, fully familiar with all? Like, is there anything you look at there and you go, well, you know, I see words, but maybe I don't know the details on them. Again, it's just helpful to know.



(00:13:54 - 00:13:56)

**Doctor:** It's my field, so I understand what.



(00:13:56 - 00:13:57)

**Stacey:** Okay.



(00:13:56 - 00:13:58)

**Doctor:** The mechanism action.



(00:13:58 - 00:13:58)

**Stacey:** Okay.



(00:13:58 - 00:14:12)

**Doctor:** Is. Maybe a pediatrician wouldn't, but, they're not prescribing it. But I would go for a gene therapy. Something that would go right to the the cure of the disease or the the problem. So I think a gene therapy is favorable.



(00:14:12 - 00:14:30)

**Stacey:** Got it. Okay. Alright. Thank you. Alright. Let's take a look at the next page. So, again, take a look through anything that's not clear, not in clinical language that makes sense to you or is appropriate in your mind. Let me know.



(00:14:48 - 00:14:50)

**Doctor:** Okay. It all makes sense.



(00:14:50 - 00:15:05)

**Stacey:** Okay. Nothing stopped you. Nothing gave you pause. Okay. Looking at the, efficacy section here, there's a couple of different things listed. You know, which like, what's most important to you in your decision making?



(00:15:07 - 00:15:10)

**Doctor:** Preserving and improving bulbar function.



(00:15:10 - 00:15:11)

**Stacey:** Okay.



(00:15:12 - 00:15:16)

**Doctor:** The limited bulbar function data is not really helpful. I mean, that's.



(00:15:16 - 00:15:16)

**Stacey:** Understood.



(00:15:16 - 00:15:18)

**Doctor:** Not Yeah.



(00:15:17 - 00:15:18)

**Stacey:** Yes.



(00:15:18 - 00:15:26)

**Doctor:** But preserving or improving would both be great. If they haven't had symptoms, then preserving is great.



(00:15:25 - 00:15:39)

**Stacey:** Okay. So that row nine where it talks about preserving function in infants with presymptomatic SMA, do me a favor. Just tell me how you're interpreting presymptomatic SMA.



(00:15:39 - 00:15:45)

**Doctor:** That they were probably detected on newborn screen, and they don't have any weakness yet.



(00:15:45 - 00:15:56)

**Stacey:** Okay. Alright. Got it. Anything missing? You know, when you think about, efficacy endpoints that are important to you, is there anything missing from that list?



(00:15:56 - 00:15:58)

**Doctor:** Not for a bulbar function. No.



(00:15:58 - 00:16:06)

**Stacey:** Okay. Alright. Here's our third page.



(00:16:24 - 00:16:30)

**Doctor:** Yeah. I'd want there to be an statistically significant improvement in upper limb function.



(00:16:33 - 00:16:34)

**Stacey:** And tell me more about that.



(00:16:35 - 00:16:48)

**Doctor:** I would want to see that there actually have been studies showing that upper limb function has improved or parent and also saying that parents say that there's an improvement.



(00:16:49 - 00:16:50)

**Stacey:** Okay.



(00:16:54 - 00:16:56)

**Doctor:** Otherwise, yeah, everything else seems good.



(00:16:56 - 00:16:57)

**Stacey:** Okay. Language is clear,



(00:16:58 - 00:16:58)

**Doctor:** Yeah.



(00:16:59 - 00:17:01)

**Stacey:** Set in a way that you would think or say it yourself.



(00:17:02 - 00:17:02)

**Doctor:** Mhmm.



(00:17:02 - 00:17:25)

**Stacey:** Okay. In terms of the efficacy section at the top, I mean, is upper limb function most important well, forgive me. Let me think how best to phrase that. So in terms of the efficacy, how important is upper limb motor function? Like, where where does that fall in the the priorities in terms of efficacy?



(00:17:26 - 00:17:36)

**Doctor:** I think it is equally important to, like, other motor functions. So and lower motor. It's very important.



(00:17:36 - 00:17:52)

**Stacey:** Okay. In terms of the safety factors, there's a couple of things, mentioned here on this page. What, you know, what is more important to you, than, you know, in terms of these different safety aspects?



(00:17:58 - 00:18:00)

**Doctor:** Like, renal toxicity versus liver damage?



(00:18:01 - 00:18:06)

**Stacey:** Yeah. And we've got the warning and precautions there and the effect on male male fertility.



(00:18:06 - 00:18:07)

**Doctor:** Yeah.



(00:18:06 - 00:18:07)

**Stacey:** You know?



(00:18:07 - 00:18:12)

**Doctor:** I mean, it's good to know about male fertility, but I think that's going to be kind of minimally important at this.



(00:18:12 - 00:18:13)

**Stacey:** Okay.



(00:18:12 - 00:18:13)

**Doctor:** Point in.



(00:18:14 - 00:18:25)

**Stacey:** Okay. So then with those next three sections, what stands out as being more important? Like, what what would be the things that would really get your attention if you were evaluating an SMAA treatment?



(00:18:27 - 00:18:39)

**Doctor:** Long term problems, like or, like, the coagulation thrombocytopenia, I think that's very important to know. And then knowing renal and liver toxicity problems. Yeah. That's important to.



(00:18:39 - 00:18:40)

**Stacey:** Okay.



(00:18:39 - 00:18:40)

**Doctor:** Know.



(00:18:40 - 00:19:00)

**Stacey:** Okay. Got it. Alright. Let's take a look at our next page. And, again, we'll just start with clarity you know, language and clarity and go from there.



(00:19:08 - 00:19:09)

**Doctor:** Okay.



(00:19:10 - 00:19:12)

**Stacey:** Anything standing out that's an issue?



(00:19:11 - 00:19:12)

**Doctor:** No.



(00:19:12 - 00:19:13)

**Stacey:** Okay.



(00:19:12 - 00:19:13)

**Doctor:** No.



(00:19:13 - 00:19:31)

**Stacey:** Alright. So in terms of the persistence section there, you know, what what is your impression of those different levels of persistence, shall we say, and and what where's that threshold where it starts to be meaningful to you?



(00:19:33 - 00:19:43)

**Doctor:** I think it's I think this is all meaningful data. Yeah. I would definitely wanna know if there's less than fifty percent staying on a medication over twelve months. And,



(00:19:43 - 00:19:44)

**Stacey:** Okay.



(00:19:43 - 00:19:47)

**Doctor:** Like, how does this factor in with the one time therapy?



(00:19:47 - 00:19:51)

**Stacey:** Okay. What would you consider really good in terms of persistence?



(00:19:53 - 00:19:55)

**Doctor:** Eighty percent greater than eighty percent.



(00:19:56 - 00:20:09)

**Stacey:** Okay. Alright. And, you know, when you think of your SMA families, you know, what percent of them do you expect or experience to be fully compliant with their treatments?



(00:20:10 - 00:20:13)

**Doctor:** Yeah. Most are almost all all are completely compliant.



(00:20:13 - 00:20:24)

**Stacey:** Okay. Again, forgive. I I have lots of questions that may seem really obvious, but what what you know, that's not the case in every disease. What drives that high compliance in your mind?



(00:20:25 - 00:20:31)

**Doctor:** The severity of the disease and how, how devastating it can be to motor development.



(00:20:31 - 00:21:00)

**Stacey:** Mhmm. Okay. Got it. So alright. You just saw four pages of attributes that, again, pertain to current, and possibly future SMA treatment options. What's what's missing? And I can quickly run through the pages again. I'll just kind of flip through them. Is there something missing that would be important to you? It's a factor or an attribute that we didn't include and we really should have.



(00:21:02 - 00:21:04)

**Doctor:** No, I don't. Not that I think of.



(00:21:04 - 00:21:10)

**Stacey:** Okay. Alright. Just clicking through. Let me know if anything comes.



(00:21:09 - 00:21:10)

**Doctor:** What.



(00:21:10 - 00:21:10)

**Stacey:** To.



(00:21:10 - 00:21:10)

**Doctor:** Did.



(00:21:10 - 00:21:10)

**Stacey:** Mind.



(00:21:10 - 00:21:17)

**Doctor:** You do lower motor function? Maybe not. Maybe you didn't do lower limb function, just an upper limb. So that might be important.



(00:21:17 - 00:21:30)

**Stacey:** Okay. Yep. We have the bulbar function and, upper limb. Okay. So that's helpful. Tell me, tell me more about, what, you know, the importance of lower limb, why why that might be an important addition here.



(00:21:30 - 00:21:31)

**Doctor:** Are they going.



(00:21:31 - 00:21:31)

**Stacey:** Mhmm.



(00:21:31 - 00:21:33)

**Doctor:** Are they walking or are they not able.



(00:21:35 - 00:21:35)

**Stacey:** K.



(00:21:36 - 00:21:40)

**Doctor:** Can they sit up or can they not are they not able to sit up?



(00:21:40 - 00:21:46)

**Stacey:** Mhmm. Got it. Okay. Thank you. Anything else that comes to mind?



(00:21:48 - 00:21:48)

**Doctor:** No.



(00:21:49 - 00:22:10)

**Stacey:** Okay. And, I you know, we talked a little bit about what you you know, what's on your wish list for future SMA treatments. Was there anything on these four pages that seemed to address any of your wish list items in any way?



(00:22:13 - 00:22:20)

**Doctor:** I guess just the primarily, I want there to be something that's very efficacious. So I I'm not sure.



(00:22:21 - 00:22:47)

**Stacey:** Mhmm. Okay. Got it. Alright. So thank you for going through that. And, why don't you now just get a sense for, you know, whether you know, what what new formulations or dosing options, you know, what what kinds of things have you heard of, if any? You know, what's hitting your radar in terms of what may be coming?



(00:22:47 - 00:22:49)

**Doctor:** I am not aware of what is happening.



(00:22:50 - 00:22:51)

**Stacey:** Mhmm.



(00:22:50 - 00:22:53)

**Doctor:** If if there's any new drugs in the pipeline right now.



(00:22:54 - 00:23:07)

**Stacey:** Got it. Anything, anything regarding any of the existing options? Any advancements regarding any of the existing SMA and SMA You know?



(00:23:08 - 00:23:09)

**Doctor:** Not that I'm aware of.



(00:23:09 - 00:23:22)

**Stacey:** Alright. Thank you. I appreciate that. So I wanna bring up a page here that, will give you some information. So first, you know, take a read down the, the left hand side.



(00:23:38 - 00:23:41)

**Doctor:** Okay. Interesting. I didn't know about these, but this is good to know.



(00:23:41 - 00:23:55)

**Stacey:** No. I mean, that's that's why we do this, so I I appreciate that. Alright. So let's we'll just go down. So, tell me where you would be on that scale. Oh, you know what? That's a little typo. I see that that should be seven.



(00:23:56 - 00:23:59)

**Doctor:** I'd say one for all of them because I just didn't know about these. Are these trials?



(00:24:01 - 00:24:02)

**Stacey:** Yes. Yes.



(00:24:01 - 00:24:02)

**Doctor:** Okay.



(00:24:02 - 00:24:06)

**Stacey:** These, rainbow fish, dual fish to vote, they were the names of trials. Yes.



(00:24:05 - 00:24:06)

**Doctor:** Okay.



(00:24:06 - 00:24:21)

**Stacey:** Exactly. Got it. Okay. Alright. Thank you. Very, very helpful. Alright. So, you know, what is standing out to you? You know, what what what if anything is catching your interest, here on this list?



(00:24:21 - 00:24:35)

**Doctor:** I think the combination treatment is good. I would like to start doing that. And, I have not tried yet. Just but I think it would be helpful for the patients to start doing that.



(00:24:36 - 00:24:44)

**Stacey:** Tell me more about that. Tell me what what you feel is good about combination treatment, what, you know, what what is really interesting you about that.



(00:24:45 - 00:25:07)

**Doctor:** Just to give extra benefit to the family, especially in patients who didn't have a great response, you know, to Zolgensma or didn't have a great response to Spinraza and might want something extra. So I think they might need more like, they're worried that motor function isn't doing as well as they should be for their age. So adding additional treatment could be great.



(00:25:07 - 00:25:21)

**Stacey:** Got it. What kind of expectations do you have, you know, for accommodation? Like, what yeah. Like, how much but, you know, how much benefit? Just what yeah. What, like,



(00:25:21 - 00:25:21)

**Doctor:** I.



(00:25:21 - 00:25:21)

**Stacey:** What.



(00:25:21 - 00:25:21)

**Doctor:** Don't.



(00:25:21 - 00:25:22)

**Stacey:** Expectations?



(00:25:21 - 00:25:22)

**Doctor:** Know.



(00:25:22 - 00:25:22)

**Stacey:** Yeah.



(00:25:23 - 00:25:27)

**Doctor:** I would have to see. I mean, I hope it would be helpful, but I'm not sure if it would make them completely normal.



(00:25:28 - 00:25:46)

**Stacey:** Mhmm. Got it. What kind of incremental, you know, benefit is helping you know, like, when you say extra benefit, you know, how how much benefit does there need to be for combination therapy to be useful?



(00:25:48 - 00:25:57)

**Doctor:** Parents have to find that it's helping them at at home. Parents have to tell me that they're noticing that it's helping and that they would want to continue trying this.



(00:25:57 - 00:26:20)

**Stacey:** Mhmm. Got it. Got it. Okay. So in terms of, you know, parents saying it's helping at home helping at home, give me some texture there. Like, what kinds of things would, you know, parents be interested in to say, okay, yes, it's helping me at this way in home at home. What, you know,



(00:26:20 - 00:26:20)

**Doctor:** Maybe.



(00:26:20 - 00:26:20)

**Stacey:** What are.



(00:26:20 - 00:26:20)

**Doctor:** They're.



(00:26:20 - 00:26:20)

**Stacey:** The things.



(00:26:20 - 00:26:21)

**Doctor:** Feeding.



(00:26:20 - 00:26:21)

**Stacey:** That matter?



(00:26:21 - 00:26:36)

**Doctor:** Themselves. Maybe they're feeding themselves. Maybe their head wasn't holding up very high, and now they're able to keep their head up or, do becoming more independent and meeting less help with everyday.



(00:26:36 - 00:26:36)

**Stacey:** Mhmm.



(00:26:36 - 00:26:36)

**Doctor:** Activities.



(00:26:39 - 00:26:45)

**Stacey:** Got it. And less help in what sense? Again, any examples that you can give of.



(00:26:46 - 00:26:48)

**Doctor:** Eating, going to the bathroom.



(00:26:48 - 00:26:48)

**Stacey:** Mhmm.



(00:26:52 - 00:26:57)

**Doctor:** What else? Maybe walking. Either walking is better. Running.



(00:27:00 - 00:27:33)

**Stacey:** Got it. K. Okay. So if I, if we just, run down, the list, I'm gonna I'm gonna do I'm gonna add a little something on here. So we'll use that same scale of one to seven, but tell me just how for each of these, just scale of one to seven, how interesting or meaningful, is this, you know, this this potential change?



(00:27:36 - 00:27:40)

**Doctor:** I think that's very it's very, can be very impactful.



(00:27:41 - 00:27:43)

**Stacey:** Well, we we'll we'll run right down the list. So starting with.



(00:27:43 - 00:27:44)

**Doctor:** Oh,



(00:27:43 - 00:27:50)

**Stacey:** The AVRIZTE tablet formate formulation, just how how intriguing are you finding that to be, scale of one to seven?



(00:27:50 - 00:27:53)

**Doctor:** Somewhat. One is oh, so one is seven. Like a four.



(00:27:53 - 00:27:57)

**Stacey:** Okay. Got it. And rainbow fish?



(00:28:06 - 00:28:07)

**Doctor:** A four.



(00:28:07 - 00:28:09)

**Stacey:** Okay. Jofish.



(00:28:14 - 00:28:14)

**Doctor:** Five,



(00:28:16 - 00:28:17)

**Stacey:** Vote.



(00:28:20 - 00:28:20)

**Doctor:** Five,



(00:28:21 - 00:28:24)

**Stacey:** K. Respond.



(00:28:25 - 00:28:26)

**Doctor:** Seven.



(00:28:30 - 00:28:32)

**Stacey:** Okay. Resilient.



(00:28:38 - 00:28:39)

**Doctor:** A five.



(00:28:41 - 00:28:41)

**Stacey:** Steer.



(00:28:46 - 00:28:47)

**Doctor:** Probably a seven.



(00:28:49 - 00:29:09)

**Stacey:** And smart. Oops. Got it. Okay. So that's helpful. Thank you. So respond and steer. So, you know, tell me in fact, let's focus on steer. So Zolgensma having an indication.



(00:29:08 - 00:29:09)

**Doctor:** Five.



(00:29:09 - 00:29:19)

**Stacey:** Beyond two years and all the way all the way up to seventeen years of age. What what makes that one, you know, really more intriguing than most of what's here?



(00:29:20 - 00:29:29)

**Doctor:** Because it's not approved right now at that at the older age. Patients might get diagnosed older. They might not have been caught on newborn.



(00:29:29 - 00:29:29)

**Stacey:** Mhmm.



(00:29:29 - 00:29:32)

**Doctor:** Screen, and they might want that one time treatment.



(00:29:33 - 00:29:47)

**Stacey:** Okay. Got it. And the Evriski tablet formulation is, lowest, you know, has has your lowest kind of moderate entry score. Tell me about that.



(00:29:47 - 00:29:49)

**Doctor:** Yeah. I just don't use Everesti as much.



(00:29:49 - 00:29:50)

**Stacey:** Yeah.



(00:29:50 - 00:29:56)

**Doctor:** I go for the other medications, but, yeah. That's fine. That's good.



(00:29:56 - 00:30:48)

**Stacey:** Mhmm. Mhmm. Okay. Alright. So I wanna get a sense for, how how things might change, if at all. So I'm gonna copy our initial conversation here. And why are you not pasting? There we go. Alright. So this is where we started in terms of your twenty patients. So, you know, with that little bit of information about those different, new options, you know, what what, if anything, would you see potentially changing in terms of your use, you know, once the Avristi tablet and the high dose, Spinraza becomes available?



(00:30:50 - 00:31:19)

**Doctor:** I could see the, I could see Evrycie being used more in combination with Zolgensma and Spinraza in combination with Zolgensma. So I would probably divide that up equally. It would really kind of depend on family preference but maybe like actually maybe ten patients with likes I don't know. Maybe go fifty fifty. I bet everyone would like to try something additional.



(00:31:20 - 00:31:44)

**Stacey:** Well, if we, since we started with twenty patients, let's, you know, reflect twenty patients in the new column. You know, if if these new, you know, options were, you know, in your toolbox, you know, how how many patients would you allocate to, to each of these different options out of the the twenty to reflect.



(00:31:46 - 00:31:46)

**Doctor:** Okay.



(00:31:46 - 00:31:48)

**Stacey:** How you would be thinking about that?



(00:31:51 - 00:31:56)

**Doctor:** I would probably say zero new zero forever SD.



(00:31:56 - 00:31:57)

**Stacey:** Okay.



(00:31:58 - 00:32:09)

**Doctor:** Spinraza. I would probably do it all Spinraza in combination with Gentzma. So none just Spinraza only.



(00:32:08 - 00:32:10)

**Stacey:** Okay. Got it.



(00:32:12 - 00:32:22)

**Doctor:** And maybe maybe like two Zolgensma,



(00:32:23 - 00:32:23)

**Stacey:** K.



(00:32:24 - 00:32:27)

**Doctor:** Eight of the next, ten of the next.



(00:32:28 - 00:32:41)

**Stacey:** K. Got it. Okay. Okay. Thank you. And, so by default, this tells me zero for Evrisky tablet.



(00:32:42 - 00:32:42)

**Doctor:** Yeah.



(00:32:42 - 00:32:59)

**Stacey:** And, zero down here for high dose. Okay. So let's just talk about your, you know, you just your your thinking with these different options. So I'm actually gonna start at the bottom. Tell me why the Everest e tablet really doesn't make it into the game plan here. Can you say more about that?



(00:33:00 - 00:33:13)

**Doctor:** It's more it's fine that that's available. I just don't find that Evarise de itself is as effective as, like, Zolgensma or Spinraza. But in combination, I could see that that being an extra helpful thing.



(00:33:13 - 00:33:19)

**Stacey:** Mhmm. Okay. Got it. And the Spinraza high dose, tell me your thoughts there.



(00:33:26 - 00:33:30)

**Doctor:** I don't know. I would rather use the combination therapy and see if that would work.



(00:33:31 - 00:33:36)

**Stacey:** Okay. Yeah. Keep keep going. Tell me more about that. That's helpful to understand. What's your thinking there?



(00:33:36 - 00:33:38)

**Doctor:** I think two different.



(00:33:38 - 00:33:38)

**Stacey:** What.



(00:33:38 - 00:33:40)

**Doctor:** Mechanisms of action might be more.



(00:33:39 - 00:33:40)

**Stacey:** I'm.



(00:33:40 - 00:33:43)

**Doctor:** Beneficial than just increasing one dose and one mechanism of act.



(00:33:43 - 00:34:11)

**Stacey:** Mhmm. Got it. Okay. Alright. And, okay. So we're seeing Spinraza in combination with sultansma, Avrista in combination with sultansma. Tell me about eight and ten there. So Avrista is coming into play in the combination, still a little bit more towards Spinraza. Tell me about those two, you know, these these two rows.



(00:34:12 - 00:34:29)

**Doctor:** Yeah. The patients I could see might like to do just an oral, everyday oral therapy versus everyday. But, based on what I've already seen is that SPINRAZA seems to be a bit more effective. So I would combine that prefer to combine that with Zolgensma. So I think.



(00:34:28 - 00:34:29)

**Stacey:** Mhmm.



(00:34:29 - 00:34:31)

**Doctor:** People would also like to do that too.



(00:34:31 - 00:34:44)

**Stacey:** Got it. Okay. Alright. And then just to have you expand as, you have found SPINRAZA to be a a little bit more effective. How so? In in what way have you found SPINRAZA to be more effective?



(00:34:44 - 00:34:47)

**Doctor:** Just preserving motor function, improving motor function.



(00:34:51 - 00:35:11)

**Stacey:** Good. Okay. Alright. And then we'd love to hear more about your thoughts about Zolgensma, you know, possibly being indicated up to the age of seventeen. Yeah. Just tell me a little bit more about your impressions of that possibility and where you would see that fitting in.



(00:35:11 - 00:35:29)

**Doctor:** Could definitely be used for people who didn't get newborn screening or who found out late that they had SMA, and that could be beneficial for older patient populations. I think that it's a preferred mode of treatment with IV and a one time dose. So, yes, that could be helpful.



(00:35:30 - 00:35:57)

**Stacey:** Okay. So thank you. So alright. So if, if we assume hypothetically for a moment that all of these different options are available, you know, become available, you've shared your thoughts about Everest d and the Everest d tablet. Would you would you see yourself informing your SMA families about the Everest d tablet becoming available, or would you not have that? Okay.



(00:35:57 - 00:35:57)

**Doctor:** Yeah.



(00:35:57 - 00:35:57)

**Stacey:** Tell.



(00:35:57 - 00:35:58)

**Doctor:** Yeah.



(00:35:57 - 00:35:58)

**Stacey:** Me.



(00:35:58 - 00:35:58)

**Doctor:** I would.



(00:35:58 - 00:35:59)

**Stacey:** So go.



(00:35:59 - 00:35:59)

**Doctor:** I.



(00:35:59 - 00:35:59)

**Stacey:** Ahead.



(00:35:59 - 00:35:59)

**Doctor:** Would tell.



(00:35:59 - 00:36:00)

**Stacey:** Tell me.



(00:35:59 - 00:36:02)

**Doctor:** Them available. I always let them choose.



(00:36:02 - 00:36:02)

**Stacey:** Mhmm.



(00:36:02 - 00:36:04)

**Doctor:** So I think yeah.



(00:36:04 - 00:36:05)

**Stacey:** Mhmm.



(00:36:05 - 00:36:06)

**Doctor:** I I would absolutely let.



(00:36:06 - 00:36:07)

**Stacey:** Mhmm.



(00:36:06 - 00:36:07)

**Doctor:** Them let them know.



(00:36:08 - 00:36:22)

**Stacey:** K. Good. Good. But based on what I'm seeing here, there's no one that you would kind of proactively say, hey. Like, I really think you should switch to this. No one would quite fit that category.



(00:36:24 - 00:36:24)

**Doctor:** No. I.



(00:36:24 - 00:36:25)

**Stacey:** Yeah.



(00:36:24 - 00:36:29)

**Doctor:** Don't think I mean, versus what we're already doing right now, I don't think it makes a huge difference that it's a tablet versus a liquid.



(00:36:29 - 00:36:49)

**Stacey:** Okay. Alright. Got it. Alright. In terms of the high dose SPINRAZA, is there any circumstance where you would see someone being a good candidate for that versus, as you said, the combination which you would prefer? Just curious.



(00:36:49 - 00:36:51)

**Doctor:** Sure. I don't think it would.



(00:36:51 - 00:36:51)

**Stacey:** If.



(00:36:51 - 00:36:53)

**Doctor:** Hurt to try a high dose. Like,



(00:36:52 - 00:36:53)

**Stacey:** There's.



(00:36:53 - 00:36:54)

**Doctor:** Try a higher dose.



(00:36:54 - 00:36:54)

**Stacey:** Anyone.



(00:36:54 - 00:36:59)

**Doctor:** Rather than, like, adding on a second therapy. Probably just here to do a higher dose first and see if.



(00:36:59 - 00:37:00)

**Stacey:** Okay.



(00:36:59 - 00:37:10)

**Doctor:** It had any motor benefits. But then, after giving that a one time try, I would if it didn't work, then I would add on a second.



(00:37:10 - 00:37:19)

**Stacey:** Got it. Okay. Is there anyone, any type of patient that you would say is just not a candidate for the high dose SPINRAZA for whatever.



(00:37:21 - 00:37:23)

**Doctor:** If they just if they had had trouble.



(00:37:23 - 00:37:24)

**Stacey:** Reason?



(00:37:23 - 00:37:25)

**Doctor:** Tolerating the lower dose.



(00:37:25 - 00:37:32)

**Stacey:** Okay. And what about the Everesti tablet? Anyone that you would say just you know, they're just not a candidate for this?



(00:37:33 - 00:37:34)

**Doctor:** For ballbar function.



(00:37:35 - 00:37:58)

**Stacey:** Mhmm. K. Alright. Got it. And then in terms of the, Zolgensma being indicated up to age seventeen, you know, again, would you, would you make your families aware of all these potential changes even if they weren't necessarily your preferred option?



(00:37:59 - 00:38:00)

**Doctor:** Yeah. Of course.



(00:38:00 - 00:38:14)

**Stacey:** Okay. Alright. Is there anyone in particular that you would see just being a really good candidate? Like, in your mind, these would be the really good candidates for Zolgensma over the age of two.



(00:38:14 - 00:38:23)

**Doctor:** Someone who, like, didn't have newborn screening done. And then they found out later that they have SMA.



(00:38:23 - 00:38:41)

**Stacey:** Mhmm. Okay. Alright. Got it. And, anything else? Any other type of patients, who you feel just would be a really good candidate for, Solgen's mouth if it had the indication for older older children?



(00:38:42 - 00:38:44)

**Doctor:** No. Not that I can think of.



(00:38:44 - 00:39:01)

**Stacey:** Okay. Alright. So, you know, how often is insurance coverage an issue, you know, for patients getting on a drug? Is that something that happens often or or not so often in SMA?



(00:39:02 - 00:39:04)

**Doctor:** It has not been much of an issue lately.



(00:39:05 - 00:39:09)

**Stacey:** Okay. Lately, so has something changed or improved? Can.



(00:39:09 - 00:39:09)

**Doctor:** No.



(00:39:09 - 00:39:09)

**Stacey:** You.



(00:39:09 - 00:39:09)

**Doctor:** I.



(00:39:09 - 00:39:09)

**Stacey:** Say.



(00:39:09 - 00:39:09)

**Doctor:** Just.



(00:39:09 - 00:39:10)

**Stacey:** More.



(00:39:09 - 00:39:10)

**Doctor:** Think.



(00:39:10 - 00:39:10)

**Stacey:** About that?



(00:39:10 - 00:39:14)

**Doctor:** I have not had much of an issue. I have not had issues getting coverage.



(00:39:15 - 00:39:15)

**Stacey:** Okay. So.



(00:39:15 - 00:39:16)

**Doctor:** Yes,



(00:39:15 - 00:39:22)

**Stacey:** You're able to put patients on whichever therapy you or they want to be on. Coverage is not the obstacle. Okay.



(00:39:22 - 00:39:23)

**Doctor:** Correct.



(00:39:23 - 00:39:42)

**Stacey:** Alright. It It sounds like you have at least experienced obstacles with that in the past. What happens? Like, do you, like, do you get involved to to try to push for coverage or do you move on to the like, what what tends to be the procedure if you do get pushed back in terms of coverage?



(00:39:42 - 00:39:54)

**Doctor:** I would try to push back or get the prior authorization as needed, do an appeal if needed. But, usually, things have been lately, it's been pretty simple to get the.



(00:39:54 - 00:39:54)

**Stacey:** Okay.



(00:39:54 - 00:39:55)

**Doctor:** Need.



(00:39:55 - 00:40:21)

**Stacey:** Got it. Alright. Alright. And then just, before we move to our next page, thinking a little bit more about the, SPINRAZA in combination with Zolgensma, because I know that, you know, the combination was appealing to you. All all patients, or would there be, like, certain reasons in your mind for for wanting to go to a combination?



(00:40:24 - 00:40:30)

**Doctor:** I would definitely say if they have suboptimal improvement in their motor function on just one therapy.



(00:40:31 - 00:40:32)

**Stacey:** Mhmm. Okay.



(00:40:33 - 00:40:50)

**Doctor:** But if a family would just like to try it to see if it gives them extra benefit in terms of motor function or maybe we don't know how long this the drugs are actually going to last. The effectiveness is going to last. So family just wants to try something else in addition.



(00:40:49 - 00:40:50)

**Stacey:** Mhmm.



(00:40:50 - 00:40:51)

**Doctor:** Fine with me.



(00:40:51 - 00:41:21)

**Stacey:** Okay. Alright. Got it. Thank you. So, I'll let the team let me know if there's anything you'd like me to circle back to, but for now, I'm gonna go ahead on to our next page. And, doctor, I wanna share a, hypothetical profile for the Evaristi tablet. So go ahead and review. By the way, do you do you need me to give you a minute for anything? I feel like something maybe is pulling your attention. If you need a minute, just let me know. I'm happy to give it to you. Okay.



(00:41:21 - 00:41:22)

**Doctor:** No. No. We're.



(00:41:22 - 00:41:22)

**Stacey:** Alright.



(00:41:22 - 00:41:22)

**Doctor:** Fine.



(00:41:23 - 00:41:26)

**Stacey:** Yeah. So take a look at the the profile.



(00:41:56 - 00:41:57)

**Doctor:** Okay.



(00:41:58 - 00:42:05)

**Stacey:** So talk to me about your overall impression of what you've read here about the hypothetical Everest d tablet.



(00:42:07 - 00:42:13)

**Doctor:** It seems very comparable to any other tablet medication that would be taken by a kid or an adult.



(00:42:15 - 00:42:19)

**Stacey:** How how so? Tell me what like, in what sense is it comparable?



(00:42:19 - 00:42:32)

**Doctor:** Like the size, the like, how you store it, you know, once daily dosing. It seems very straightforward.



(00:42:32 - 00:42:48)

**Stacey:** Mhmm. K. Alright. What else? What else? Just in terms of your overall impression, you know, when we talked about it before, all you had was, like, a little line on that chart. So,



(00:42:49 - 00:42:49)

**Doctor:** Yeah.



(00:42:49 - 00:42:55)

**Stacey:** Yeah, curious to know more about just what you're how you're thinking about this, how you're feeling about this potential option.



(00:42:57 - 00:43:12)

**Doctor:** I I don't feel very strongly about this option. I think that, it's a nice it's a it's a potentially a good add on therapy, but, I don't think by itself it's like a wonderful therapy.



(00:43:12 - 00:43:13)

**Stacey:** Mhmm. K.



(00:43:13 - 00:43:15)

**Doctor:** Just not as effective as the others.



(00:43:15 - 00:43:43)

**Stacey:** Yep. Got it. Okay. Understood. Alright. So, we can certainly think of it in that context as an add on therapy. What, you know, what are the pot you know, are there positives here? Are there negatives that are coming to mind? Like, just what, yeah, pros, cons, positives, negatives that are popping for you here.



(00:43:46 - 00:43:59)

**Doctor:** I think that kids who are who are, young even, like, up till age, like, seven or eight are not gonna want to take a pill tablet. They are gonna prefer a.



(00:43:59 - 00:44:00)

**Stacey:** Mhmm.



(00:43:59 - 00:44:03)

**Doctor:** Liquid. So that is the negative to this.



(00:44:03 - 00:44:26)

**Stacey:** Got it. Well okay. So I'll use that as a segue to talk about the administration options. So there's two options here over on the right hand side. So the first option is to swallow the pill with water as you would any other tablet or pill, and then the second option is to disperse the pill in water, in bottled water.



(00:44:27 - 00:44:27)

**Doctor:** Mhmm.



(00:44:27 - 00:44:42)

**Stacey:** Kind of you swirl, and then once it's fully dispersed dispersed, it it can be consumed, you know, as a as a liquid. So interested, in your thoughts about having two administration options here.



(00:44:43 - 00:44:53)

**Doctor:** Yeah. Then you once you put it in bottled water, then you have to make sure that they drink it off. So that's always a problem. You can't, like, force a two year old to drink everything.



(00:44:55 - 00:45:04)

**Stacey:** Got it. What about at different age ranges? Would you have that same concern for a five year old, a seven year old, a ten year.



(00:45:03 - 00:45:04)

**Doctor:** Yeah.



(00:45:04 - 00:45:05)

**Stacey:** Old? Okay.



(00:45:06 - 00:45:07)

**Doctor:** Maybe not a ten year old, but, like,



(00:45:07 - 00:45:08)

**Stacey:** Okay.



(00:45:07 - 00:45:10)

**Doctor:** A five through eight year old, probably. Yeah.



(00:45:11 - 00:45:31)

**Stacey:** Got it. Okay. Alright. So that's helpful. Thank you. What else? As as you see those parameters around administration, what else is standing out to you in those, a and b in those corresponding steps underneath?



(00:45:39 - 00:45:44)

**Doctor:** There's just kind of a lot of like, under b, there's a lot of extra steps, a lot of reading involved for a parent.



(00:45:44 - 00:45:46)

**Stacey:** Mhmm. K.



(00:45:49 - 00:45:53)

**Doctor:** It would seem much easier to me to just do the liquid that is already available.



(00:45:54 - 00:46:10)

**Stacey:** Okay. Got it. Alright. So then so okay. So that's helpful. So the storage so this, this can be at room temperature. It I mean, is that meaningful at all in your mind?



(00:46:13 - 00:46:14)

**Doctor:** It's better than having to be refrigerated.



(00:46:18 - 00:46:43)

**Stacey:** Well and and but I'm curious. So as so I'm very much hearing what you're saying. So in this option, if they wanna put it in liquid, they've gotta go through some steps. They could, you know, obtain it as currently, it's already in liquid form. They don't have to do all that, but one has to be refrigerated, one doesn't. So I'm I'm curious, like, kinda how, you know, how that scale, kinda weighs out in your mind.



(00:46:44 - 00:46:50)

**Doctor:** The refrigerated part doesn't make much of a difference. A lot of my patients take medications after you refrigerated, and that doesn't really make.



(00:46:49 - 00:46:50)

**Stacey:** Okay.



(00:46:50 - 00:46:50)

**Doctor:** Much.



(00:46:51 - 00:47:01)

**Stacey:** Okay. Got it. Alright. Thank you. Okay. So then not not a huge advantage that this is at room temperature.



(00:47:02 - 00:47:02)

**Doctor:** Right.



(00:47:03 - 00:47:21)

**Stacey:** Okay. Alright. So then in terms of, swallowing the pill, what what do you see in your patients? Again, you're seeing typically patients up to ten years old. So, you know, when when are patients able to you know, how how many of your patients can swallow a pill? What kinds of.



(00:47:21 - 00:47:21)

**Doctor:** Not.



(00:47:21 - 00:47:22)

**Stacey:** Ages?



(00:47:21 - 00:47:22)

**Doctor:** Very again.



(00:47:22 - 00:47:23)

**Stacey:** Okay. Not.



(00:47:23 - 00:47:23)

**Doctor:** Yeah.



(00:47:23 - 00:47:23)

**Stacey:** Very many.



(00:47:23 - 00:47:23)

**Doctor:** This.



(00:47:23 - 00:47:24)

**Stacey:** Okay.



(00:47:24 - 00:47:26)

**Doctor:** Yeah. Very unlikely.



(00:47:27 - 00:47:54)

**Stacey:** Okay. Alright. Got it. Okay. Great. So if just hypothetically, if the Adverse two tablet needed to be swallowed, if the option to mix it in water was not part of this, how would that affect your perception of this option overall?



(00:47:55 - 00:47:59)

**Doctor:** Then I probably wouldn't prescribe it to anyone under ten.



(00:47:59 - 00:48:21)

**Stacey:** Okay. Alright. Got it. Okay. So thank you. So what is your impression here of the Avrissi tablet in terms of its, effectiveness? How do you feel the effectiveness of this would compare with the current liquid option? What kind of impression are you getting about that?



(00:48:22 - 00:48:24)

**Doctor:** I hope it would be the same.



(00:48:28 - 00:48:47)

**Stacey:** Is this, giving you like, do you what what impression, though, is the profile giving you? So it is, discussing bioequivalence there on the left hand side. And curious, you know, what again, what impression that's giving you or what assumption, you know, you would would come away with?



(00:48:48 - 00:48:50)

**Doctor:** Yeah, I I assume that it would be the same.



(00:48:50 - 00:48:52)

**Stacey:** Mhmm. K.



(00:48:52 - 00:48:58)

**Doctor:** I, if, yeah, I would expect that it. Would work exactly the same.



(00:48:58 - 00:49:12)

**Stacey:** Okay. Alright. So is there anything missing from this profile in terms of the efficacy of the Evaristi tablet, something you would wanna know, but it has not been included on this page?



(00:49:14 - 00:49:15)

**Doctor:** No.



(00:49:16 - 00:49:32)

**Stacey:** K. Alright. Any questions? You know, if we if we had, you know, an an expert here on the line with us, you know, what are there any questions that you would ask that person about the Everest tablet?



(00:49:33 - 00:49:35)

**Doctor:** Any additional adverse effects?



(00:49:38 - 00:49:54)

**Stacey:** Okay. Got it. What else? Any other questions that you would ask if you could? Okay. Alright. And in terms of how you feel the tablet's efficacy would compare to SPINRAZA.



(00:49:53 - 00:49:54)

**Doctor:** No.



(00:49:55 - 00:49:59)

**Stacey:** SPINRAZA's efficacy, what assumption or thought do you have about that?



(00:50:00 - 00:50:02)

**Doctor:** SPINRAZA seems to work better.



(00:50:02 - 00:50:03)

**Stacey:** Okay.



(00:50:03 - 00:50:03)

**Doctor:** In general.



(00:50:04 - 00:50:11)

**Stacey:** Got it. So same same feeling that you've had would apply here to the tablet. Okay. Alright. What.



(00:50:11 - 00:50:11)

**Doctor:** Yeah.



(00:50:11 - 00:50:21)

**Stacey:** About the safety of the EFRIZZ d tablet? What, again, what assumption or impression are you, forming about the safety of this based on what you're seeing here?



(00:50:22 - 00:50:39)

**Doctor:** Yeah. There's no, adverse effects, like, listed on here or anything, so I don't know. I would assume it's the same, but, like, is this are there black box warnings now? Are there, something is there something that I need to be aware of? What's the main side effect?



(00:50:39 - 00:51:00)

**Stacey:** Got it. Okay. So if you can assume if I ask you to assume that safety is the same that you could expect from the current Everest d formulation, how do you feel the safety, of the Everest d tablet would compare to SPINRAZA in your experience?



(00:51:04 - 00:51:11)

**Doctor:** There's less I mean, Spinraza has risks of infection and, yeah. And so there's always risks with doing something that's, like,



(00:51:11 - 00:51:11)

**Stacey:** Okay.



(00:51:11 - 00:51:15)

**Doctor:** Injecting into the spinal cord. So I think there's more risk with SPINRAZA, but it's.



(00:51:15 - 00:51:15)

**Stacey:** Okay.



(00:51:15 - 00:51:16)

**Doctor:** More this. So.



(00:51:17 - 00:51:24)

**Stacey:** Got it. And from everything you said, it sounds like the the greater efficacy outweighs.



(00:51:25 - 00:51:26)

**Doctor:** Of course.



(00:51:26 - 00:51:26)

**Stacey:** Okay.



(00:51:26 - 00:51:27)

**Doctor:** Yeah. Of.



(00:51:27 - 00:51:28)

**Stacey:** Well,



(00:51:27 - 00:51:28)

**Doctor:** Course.



(00:51:28 - 00:51:39)

**Stacey:** I mean, tell tell me about that. I mean, certainly, I could be having a conversation in another disease area and the safety might outweigh. So in SMA, can you say more about why the efficacy does outweigh.



(00:51:39 - 00:51:39)

**Doctor:** Yeah.



(00:51:39 - 00:51:44)

**Stacey:** The safety concern with the the route of administration with the.



(00:51:44 - 00:51:54)

**Doctor:** Yeah. Family want families are going to want to use a medication that actually treats the disease and does show improvement in such a severe.



(00:51:55 - 00:51:56)

**Stacey:** Mhmm.



(00:51:55 - 00:52:15)

**Doctor:** Disease that can cause complete motor devastation. A patient could be completely non mobile, not able to walk, not able to sit up or talk, things like that. Preserved cognitive function. So, if a a daily tablet is going to provide some benefit but not very much, then it seems pointless to give,



(00:52:15 - 00:52:16)

**Stacey:** Mhmm.



(00:52:15 - 00:52:41)

**Doctor:** Own if it's when there's a medication that, yes, it's, like, more risky, but everyone does lumbar punctures and intrathecal, spinal taps that happen so frequently, and we the risk of infection. We minimize the risk of danger with that by making sure that it's a sterile environment and giving, and giving the patient some if they need some, you.



(00:52:41 - 00:52:41)

**Stacey:** Mhmm.



(00:52:41 - 00:52:48)

**Doctor:** Know, relaxing relaxation, like, anxiolytic, sedation before they go through with the procedure.



(00:52:48 - 00:53:15)

**Stacey:** Okay. Alright. Got it. Okay. So I wanna go back to our allocations here. Just now that you've had a chance to go through some details on the Everesti tablet, does anything change? Do you do you view your hypothetical future prescribing any differently than you did when we completed this page the first time?



(00:53:15 - 00:53:16)

**Doctor:** No.



(00:53:16 - 00:53:43)

**Stacey:** Okay. Right. Got it. You had indicated that it's the combination idea that is most meaningful to you. We talked about, you know, coverage issues that you have not been experiencing really any coverage issues lately. What you know, do you do you expect that would be the same in a combination therapy situation, or would you.



(00:53:44 - 00:53:44)

**Doctor:** I expect.



(00:53:44 - 00:53:44)

**Stacey:** Expect.



(00:53:44 - 00:53:44)

**Doctor:** There.



(00:53:44 - 00:53:44)

**Stacey:** Any.



(00:53:44 - 00:53:44)

**Doctor:** To.



(00:53:44 - 00:53:45)

**Stacey:** Additional.



(00:53:44 - 00:53:45)

**Doctor:** Be It.



(00:53:45 - 00:53:45)

**Stacey:** Barriers?



(00:53:46 - 00:54:04)

**Doctor:** Will be there expect there to be a lot of difficulty, and I expect there you must we're probably going to have to document some sort of decline or nonprogression in improvement or lack of improvement on one medication requiring another medication?



(00:54:05 - 00:54:39)

**Stacey:** Got it. Okay. Alright. Thank you. Alright. So I'm gonna let my team know that we are, closing out here in just a moment. Let them know if they have any questions. Ah, we do have one that has come in. So, you know, if you were to experience access issues, you know, in in terms of what we just talked about, would you look to the manufacturer for support? What kind of support could the manufacturer provide to to help with those potential issues? What would you need or want?



(00:54:42 - 00:54:48)

**Doctor:** From the manufacturer. If if I'm not getting it approved, is that what they mean?



(00:54:47 - 00:54:58)

**Stacey:** In terms of access issues for, you know, for combination therapy, as you said, you thought it would be more challenging than it is right now, that you would encounter some barriers. And so.



(00:54:58 - 00:55:08)

**Doctor:** I guess I would like to know why exactly it's not being approved and then what what it would take to get it approved. So maybe the manufacturer, like or, like, what what needs to be documented for it to get approved.



(00:55:11 - 00:55:18)

**Stacey:** Okay. So that you can be submitting the the the correct or the effective information to facilitate the approval.



(00:55:18 - 00:55:19)

**Doctor:** Right.



(00:55:19 - 00:55:29)

**Stacey:** Okay. Got it. What else? Would you want the manufacturer to have any greater involvement in that process or.



(00:55:30 - 00:55:30)

**Doctor:** Sure.



(00:55:30 - 00:55:31)

**Stacey:** Provide.



(00:55:30 - 00:55:31)

**Doctor:** If.



(00:55:31 - 00:55:32)

**Stacey:** Any other support?



(00:55:31 - 00:55:36)

**Doctor:** They help with if they're able to help with appeals or, yeah, that would be helpful.



(00:55:37 - 00:56:08)

**Stacey:** Okay. Got it. Alright. So I'm keeping an eye to see if any additional questions come in. So doctor, you you know, after viewing all of the material today, is there anything else that you'd like to share with me about any of the potential future options that will be available in SMA, thoughts about those, any other wish list items, just anything else that's coming to mind.



(00:56:08 - 00:56:12)

**Doctor:** No. I I just hope that the the coverage does happen, that it's it's.



(00:56:12 - 00:56:13)

**Stacey:** Mhmm.



(00:56:12 - 00:56:17)

**Doctor:** Able to covered for both for combination therapy. I do think that would be effective.



(00:56:17 - 00:56:23)

**Stacey:** Got it. Okay. Alright. Thank you so much, doctor. It's been a pleasure. I appreciate your help.



(00:56:22 - 00:56:23)

**Doctor:** Thank.



(00:56:23 - 00:56:23)

**Stacey:** Today.



(00:56:23 - 00:56:24)

**Doctor:** You. Yeah.



(00:56:23 - 00:56:24)

**Stacey:** Have a.



(00:56:24 - 00:56:24)

**Doctor:** Thank.



(00:56:24 - 00:56:24)

**Stacey:** Good rest.



(00:56:24 - 00:56:24)

**Doctor:** You.



(00:56:24 - 00:56:25)

**Stacey:** Of your day.



(00:56:25 - 00:56:26)

**Doctor:** You too.

