

Title:

Parent's experience of administering vosoritide, a daily injectable for children with achondroplasia

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Appendix 1: Supplementary quotes

Theme	Quote	Participant
<b>Awareness of vosoritide</b>	<i>"the first time was actually already back in 2012...when my child was born. When [he] was born, we checked to see who would be the first, person to contact in Germany. And then we drove to [a physician]. And, um, I think [this physician] was the one who told us about it"</i>	Parent of 10-year-old, Germany
	<i>"That must have been at a full meeting from the achondroplasia centre. That was discussed for a few years, that was, I think, there was this first information meeting, as the phase III of the study began, so that must have been 4 years ago."</i>	Parent of 7-year-old, Germany
	<i>"I followed very closely... every 6 months or something, the news to see a bit how it was doing ... so I saw the doctor who followed us...and I showed her the articles by BioMarin saying that the treatment was available since December and so... that's it"</i>	Parent of 11-year-old, France
	<i>"it's always been totally dedicated to providing data and information about the study. We had numerous calls, and exchange opportunities regarding the children, and the families who participated in the study spoke, and there was even a ten-year-old who injected himself, and we could watch all that. They're totally engaged and really also totally neutral"</i>	Parent of 3-year-old, Germany
	<i>"I also remember that when I showed it to the doctor at [hospital] she told me "ah! How have you seen that? Can you give me a copy?" so apparently, she didn't have it"</i>	Parent of 11-year-old, France
	<i>"When the geneticist called me and told me that we needed to have an appointment... she was the one who started talking about it. She told me about treatments that were... in experimentation, that there would surely be things in the years to come. And then, that's all."</i>	Parent of 8-year-old, Germany
	<i>"...it is the geneticist who will be informed first if there are clinical trials, so I... every year, I hammer the geneticist by telling him "you remember well, if there is a treatment coming, there is a possibility of integration, if we have to go to [city], if we have to go to the other end of France we will do it, we will do it"</i>	Parent of 11-year-old, France
	<i>"All the information we have about it did not come from the doctor, but from the [patient advocacy group]."</i>	Parent of 6-year-old, Germany
	<i>"Through the official [association], there was an email for many groups of members and then... searching, we got all the descriptions of the</i>	Parent of 11-year-old, France

	<i>different centers... that followed it where we lived, which university hospital we should address."</i>	
<b>Treatment understanding and decision making</b>	<i>"I think if I wouldn't have informed myself before I would have liked another conversation with a physician. Because, um, I ... Through the association, you had been informed about the results of the clinical trial, but I haven't talked to a physician about what that may mean for my son."</i>	Parent of 10-year-old, Germany
	<i>"The decision was not greatly changed, you actually went there, and you were pretty certain that you wanted to do it. And we also talked in advance, before we were in the achondroplasia centre, also with [child]... at that point in time, so if your son is strongly advised against it by doctors and if they had given you good and understandable reasons for it, then we might have decided differently, or at least we wouldn't have decided in favour of it at that point. But that was not the case."</i>	Parent of 7-year-old, Germany
	<i>"The endocrinologist told her about the treatment in the same way he told me about it and... I asked her what she thought about it, she shrugged her shoulders and then she said "I don't know"</i>	Parent of 8-year-old, France
	<i>"I think he spoke to her- well... from a very medical point of view [laughs] and that she may not have understood everything, but in any case, she remembered the essential, that it would be injections. So, she was very apprehensive about the injections because she hadn't had many, maybe she had to do a blood test or two in her life so she... She was a bit afraid of the pain, so he had to reassure her about it by telling her that... Well, as it was going to be every day, she would get used to it and that... yes, it would hurt a bit, but that it wouldn't last long."</i>	Parent of 12-year-old, France
	<i>"We said, "this drug, and you have to receive an injection every day for a long time, but you have the opportunity to be bigger"...he said "Yes, of course, I want to be bigger" but yes... he is 6 years old... I think he didn't really understand it."</i>	Parent of 6-year-old, Germany
	<i>"We haven't really explained it beforehand. I mean we, um, he was two years old. He wouldn't have understood it either. We just said: "This is necessary for you to grow better.""</i>	Parent of 3-year-old, Germany
	<i>"I think for him it's mostly the height. He is happy about every centimeter. He wants to play soccer and the taller he is, the better he can play with others of his age. And, um, I think he hasn't thought of anything else for the time being"</i>	Parent of 10-year-old, Germany

	<i>"It involved him directly so, in any case, if he had doubts or if he hesitated... it was his decision to make. He is not 5 so..."</i>	Parent of 11-year-old, France
	<i>"And so, at the beginning she flat out refused, she said she didn't want the treatment because it scared her to... stop being a little person"</i>	Parent of 12-year-old, France
	<i>"I wanted to make sure that she wouldn't later say that there were extension treatments and she never found out about them, or "You didn't have me extended!" That's way I do all of this together with her."</i>	Parent of 13-year-old, Germany
	<i>"During the phase III of the study, scientific publications of the results of the phase II of the study came out. They were very interesting to read, and then the approval came at some point, and I know that if something is approved, then it's something that I trust. People who are more familiar with the matter than I have dealt with it intensively before it is approved"</i>	Parent of 7-year-old, Germany
	<i>"So, what motivated us was really thinking, well... because, since we see a lot of little adult people, we know that they experience a lot of pain in the back, legs, all sorts of difficulties, having to go through rather heavy operations, and so, what we liked in this treatment was the idea that we would be reducing uh... these medical issues."</i>	Parent of 12-year-old, France
	<i>"Long-term side effects were also something I thought of, that was also why I was a bit sceptical, but we were told that side effects due to the study were very unlikely."</i>	Parent of 6-year-old, Germany
	<i>"Because I think that the small size, there is some dependence for some things, they can't be independent for many things... "</i>	Parent of 11-year-old, France
<b>Training and initiation</b>	<i>"They contacted us, um, that we can get started. And, um, then we drove there and, um, received a good demonstration together with another, um, yeah, child with dwarfism and his parents. I think that made the whole situation less tense. And, um, yes, that was really good. He was also allowed to inject it into a cushion that, um, that took the fear away."</i>	Parent of 10-year-old, Germany
	<i>"She did it in front of us, she explained how we had to reconstitute the product and she showed us the manipulation... she explain that the injection needs to be done at 45 degrees... um... she showed us really how to secure the syringe, not to just put it however... she really explained it well."</i>	Parent of 8-year-old, France
	<i>"The first injection... it was at the hospital so that is the one I observed the nurse do."</i>	Parent of 8-year-old, France
	<i>"At first, given that the injection didn't go very well, in front of everybody and he didn't like much doing</i>	Parent of 11-year-old, France

	<i>it like that and then... I was clumsy the first time I did it"</i>	
	<i>"there was a curtain, so we couldn't see it, but he heard the cries and I think that fortunately he had done it first because otherwise he wouldn't have accepted it, after pain cries like that, it was terrible."</i>	Parent of 11-year-old, France
	<i>"I would have liked to have had someone next to me who could specifically guide my hand or show me where to reach, so how to reach, what angle to hold or how to stand."</i>	Parent of 6-year-old, Germany
	<i>"I need someone who can inject and show me how to hold the needle (syringe). I know some parents locally, just had the nurse come in for the first two weeks"</i>	Parent of 6-year-old, Germany
	<i>"The injection aspect... we think that it is a medical procedure and we discussed it with the private nurses, I think they even supported that, who told us that "actually if you want, we can continue to come, it is not a problem."</i>	Parent of 8-year-old, France
<b>Managing treatment at home</b>	<i>"... for the nurses when you are trained all that... I always have this feeling that for them it is all normal, you know?... But getting past the barrier of your child's skin, all that. Actually, it's more of that side kind of... The whole mental thing for parents"</i>	Parent of 11-year-old, France
	<i>"It is the fear of hurting him, not the fear of not doing it right, it is the fear of hurting him."</i>	Parent of 8-year-old, France
	<i>"It puts us in an even more stressful situation, we think to ourselves, "Oh, we have to make sure to do it well so that it doesn't hurt..." But then, sometimes we manage and other times, we're not doing it so well. Well, it's not that we're not doing it well, because she... she doesn't have any marks or anything on her skin, it's just that... sometimes it's more painful than others."</i>	Parent of 12-year-old, France
	<i>"After two or three weeks, you've got a total lock on it. It's very difficult at first, with these air bubbles. Knocking them out. But you get better at all that over time"</i>	Parent of 13-year-old, Germany
	<i>"We now use more a 30-degree angle than a 45-degree angle, a bit flatter that is. But making sure that it undoubtedly ends up in the fat tissue. And, um, we injected it fast because that's what's best for [child's name] because it hurts him both ways and, um [chuckles] like that it's better to do it fast because than it hurts for less."</i>	Parent of 10-year-old, Germany
	<i>"I think the path there is far more important – so that she is deciding – self-determinedly – how ... and when we're going to do it...I notice that her acceptance to receive the injection is much greater"</i>	Parent of 3-year-old, Germany

	<i>when I let her co-determine this whole procedure from the beginning. That helps enormously"</i>	
	<i>"The moment of the injection obviously is not nice, but by now we have reached the point, after months, that, um, he helps assembling the syringe. That he even thinks it's fun and he doesn't want anyone else to do it."</i>	Parent of 3-year-old, Germany
	<i>"We have also tried to involve our son a bit more, actually, he has started to prepare the mix alone, we assist him but... there you go."</i>	Parent of 11-year-old, France
	<i>"I think the attitude you take about approaching it – where you tell the child that it's necessary, she needs it, and it's important, and it's going to help her – you've got to have a basic attitude, and it radiates."</i>	Parent of 3-year-old, Germany
	<i>"In the beginning he got a piece of chocolate afterwards, so that he could be looking forward to that. After that we weaned him off of it bit by bit."</i>	Parent of 3-year-old, Germany
	<i>"It just becomes a part of the evening routine, just like brushing your teeth. So, I would say, I'm not asking about his motivation, to want to brush his teeth."</i>	Parent of 7-year-old, Germany
	<i>"I prefer to lose some efficiency from the treatment... by doing it sometimes 3 or 4 hours later... but making the most of the moment we are living um... rather than submitting absolutely to the schedule of 19h on holiday while we are doing something cool"</i>	Parent of 8-year-old, France
	<i>"When she's walking and holding my hand, I don't have to stretch my arm down so much, but it's a little easier. So, something certainly has happened."</i>	Parent of 13-year-old, Germany
	<i>"I don't measure him, it is just compared to his clothes and shoes... that we have seen that change more regularly."</i>	Parent of 11-year-old, France