Dawn

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Dawn, mother of Vayle (age 16 months). Vayle has Canavan Disease.

TITLE: A Canavan mom on learning the diagnosis and hearing that there was nothing to be done.

Description: The mother of a 16-month old talks with Courageous Parents Network about how she first understood there was no treatment for her daughter's Canavan diagnosis.

Transcript:

Dawn: My name is Dawn. My daughter, Vayle, is now 16 months old. She was diagnosed with Canavan Disease just a couple of days shy of turning six months old, so we're coming up on that one year mark that all parents know all too well about diagnosis day. But yeah, she is a beautiful, wonderful, perfect little girl with a horrible disease, but she is wonderful.

Interviewer: Dawn, when they finally did tell you that it was Canavan, what did they share with you about what that would mean for your daughter, for Vayle.

Dawn: They said that most people with Canavan Disease won't live past their fifth or tenth birthday. That she would likely --- she wouldn't sit unassisted on her own, she wouldn't crawl, she wouldn't walk, she wouldn't talk. She would probably require a feeding tube. She would possibly lose her vision. Just nothing, that there is no treatment. Treatment is treating symptoms. Then you're sent off with like a --- I'm not blaming doctors or anything, but it's kind of like you've received this horrendous news that you can't even fathom, but it's not like --- here's the different things we can do and the different things we can try and you tell us what you want to try and what you don't want to try, this medication or this medication. It was like – okay, now go home and just live your life and do whatever.

I mean what do I have to do, what are the different treatment options or anything, what do we do, what do we try out, and there was none of that. I remember calling people from the hospital, and I was like my daughter is really sick. It's like -- okay, what does she have? God forbid, is she diagnosed with cancer or something. What is it? It's like my daughter is really sick, but with that comes we're going to stay in the hospital and we're going to do this, this and this. No, we went home the next day.

Interviewer: It is a very unique space. The diagnosis for which there are no treatments where there is nothing in that moment that you can be offered. It takes a while.

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TITLE: A Canavan mom on learning gene therapy was on the horizon and getting information from the company. "Information is being shared the way it needs to be shared."

https://courageousparentsnetwork.org/videos/a-canavan-mom-on-learning-gene-therapy-was-on-the-horizon-and-getting-information-from-the-company-information-is-being-shared-the-way-it-needs-to-be-shared/

Description: The mother of a 16-month old talks with Courageous Parents Network about how she first learned of potential gene therapy for her daughter and how the Sponsor company is keeping the Canavan community informed.

Transcript:

Dawn: I got to a point where I was looking for things or someone found something and I started going down the rabbit hole search and saw that there was research going on that could potentially come to gene therapy.

Ten months ago, here's the worst news you can possibly get and go home and love your baby. Now, you know I'm not a New Year's person, I don't care about New Year's, it was like a Tuesday, no big deal. Now it's like --- here's a new year and potentially a year that's going to really mean something. So there is some hope out there right now, but I'm cautiously optimistic.

Interviewer: I am cautiously optimistic for you. What is your understanding --- where are you learning about the state of where it is, when it might open on clinicaltrials.gov? Until it does open, no one will know what the criteria are for participation, but how are you staying current with where it stands.

Dawn: With the company that's doing one of them, they've been very forthcoming with the information that they're able to provide. I reach out to them on occasion asking status updates or they'll reach out to me just saying – we haven't spoken in a little while, just wanted to give you a heads-up.

Interviewer: Wow.

Dawn: Yeah, it's been great. But always it's been just as much information as can be shared but that's also public information. I think not trying to over promise or set – it's certainly setting expectations, because any parent who is hopeful for something is looking ahead like I can't wait for the first half of this year to be over, right. But I've had a very great experience directly with the company, and then there are also patient advocacy groups, that both companies are keeping informed. And then that's being shared whether it's on their website or on Facebook groups and stuff like that. So I feel like the information that needs to be shared is being shared in the way it's supposed to be shared.

TITLE: A Rare Disease mom: Just tell me, what do I need to do??????

https://courageousparentsnetwork.org/videos/clinical-trials-a-rare-disease-mom-just-tell-me-what-do-i-need-to-do/

Description: The mother of a 16-month old expresses the sentiment that all parents of children with rare disease feel: There must be something I can do.

Transcript:

Interviewer: Has it been hard – has it been hard for you as a mom to be patient with the pace of progress?

Dawn: Initially yes, because again here is this news and what do I do, what do I need to do, how can I help. What do you need me to do? I remember asking – do you need me to raise \$2 million, \$10 million. I don't know how I'll do it, but I'll do it. So just tell me you need that and I'll do it. So yeah. I think initially that was hard. Or do you need me to write letters to people in government. I think I even asked – do you need me to go meet with the FDA, I'll do that. Initially again, as a parent what do I need to do. I imagine it's the same if there is something going on at your kid's school, how do I help? Do I join the PTA? Do I go talk to the teachers? Just on a very different level. So do I need to fly to DC and talk to the FDA. I was told that that wouldn't help at this point. I think initially it was that, and now it's I mean you got to put your --- again, it's not in my control. It's in everyone's best interest for it to move along as quickly and in the right way that it's supposed to, so I just have to trust that that's happening and just parent my child.

Interviewer: That's very wise.

Dawn: It's hard.

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Interviewer: It's hard but at least you're not --- that effort and that mindset might keep you from some anxiety that you should be doing more, protect you from the anxiety that you should be --- that there is more that you should be doing when in fact there is probably not more you could do.

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TITLE: Clinical Trials: A mom considers her risk aversion. "For me it's more about whether this is the right thing for her."

https://courageousparentsnetwork.org/videos/clinical-trials-a-mom-considers-her-risk-aversion-for-me-its-more-about-whether-this-is-the-right-thing-for-her/

Description: The mother of a 16-month old shares how she is thinking about what she hopes will be the upcoming opportunity for her daughter to participate in a gene therapy trial and what will determine her decision to participate.

Interviewer: Have you thought about, or what have you thought when you think about the risk, because there's never been anything like this for --- so as a Phase I, II trial, it will really be an experiment in assessing safety. Safety first and foremost and then secondly efficacy. So how have you thought about that?

Dawn: To be honest, risk wise, I'm not adverse to any potential risks, because without it the risk is too great. Without it, she won't live, endure young adolescence. So to me, I know that there is enough going on behind the scenes to --- it is being done safely. I'm not afraid that something is going to happen, and immediately there is a respiratory arrest or something like that. I'm not concerned with that. I think my biggest concern with risk is maybe doing let's say she can be included in the trial and I choose to say --- yes, I want my daughter to be a part of this, and then what is that treatment going to do for her. I think my fear of that risk factor is more --- I only want to do this for her if it's the right thing for her in the very long run. I don't know that we'll know those answers before it's time to decide. I don't know if that makes sense.

Interviewer: That did make sense. What I think you were touching upon is do you put her in this trial or do you see if there is another one that's more effective? How do you know that this is the most effective one? Is that what you meant?

Dawn: Well, that's another one though.

Interviewer: That's another thing.

Dawn: Because they also learn from this one, and then maybe in a year or whatever, they perfect dosing or they tweak this or tweak that, and then if you hadn't done it the first time, because I don't think you can do it again. Then maybe if you just waited, instead of being one and a half, maybe she was three. So there is definitely that, because this would be the very first time and extremely experimental. Again, as a parent with this hopeful outlook, it's like this is a treatment. No, it's not a treatment. This is a trial with a hope for treatment. So that's another thing. I think my thing is more --- and this is hard and this is going to be one of the harder decisions, if it's a decision that I have to make, whether or not I want her to be included. Because I know that there is another decision up to the – just the protocol and whatever criteria. What do they hope to see happen? What improvements do they hope to see? Is there any --- will she be able to walk with a walker or talk or anything like that, that maybe kids without the treatment won't be able to do. What are those improvements and what are those things? Because in my mind, I don't know, I'm in a way, when it comes to deciding whether or not it's something we're going to move forward with, which is hard to think of because if you don't do it, they're definitely not going to, right.

TITLE: Clinical Trials: A mom – "I hope I get to make that decision whether to participate and that I make the right decision."

https://courageousparentsnetwork.org/videos/clinical-trials-a-mom -i-hope-i-get-to-make-that-decision-whether-to-participate-and-that-i -make-the-right-decision/

Description: The mother of a 16-month old articulates the challenge that parents of children considering early stage clinical trials face: what is the right decision for my child, in the absence of a crystal ball.

Transcript:

Dawn: But I think that's one of the other things too is I'm going along again cautiously optimistic, that it's going to be my decision to make. Then you just get this fear of what if I don't get to make the decision, because of whatever, because the different criteria, this, that or the other, which again sets you back to diagnosis day when you're given this horrible diagnosis, and you're told that there are no treatments. Then for probably the last nine months, I'm hopeful that there is a treatment. Again, it's until you know the criteria, until you know who's been accepted, until you know whatever. I hope I get to make that decision, and then I hope I make the right decision. But there are different things that are going to happen even getting to that point of whether or not it's a decision I even get to make.

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Interviewer: Yeah, no crystal ball.

Dawn: No.

Interviewer: I really appreciate how you're talking about the whole --- the parents agency and decision making, because we at Courageous Parents Network, we talk a lot about decision making, because when we're caring for children who are medically complex and living with serious conditions, there are lot of decisions that we do have to make. Types of care, types of medical interventions, and in most of those instances, we are empowered as the decision makers. In fact, we are kind of burdened with the decision. But you're actually flagging that this is one of those decisions that at the end of the day is entirely out of the parents' control, because of the criteria for qualifying.

Dawn: Yep.

Interviewer: Who is part of your decision making, caregiving team for Vayle?

Dawn: I think like – I think there is two physicians that will be on our end, her primary care physician or her pediatrician and our neurologist that I think I'll try to take the mom emotion out of it and really drill down to expectations, and again in speaking with the medical team from the company's side as well. Then I mean ultimately it's me, right. I think ultimately it's that mom gut, that intuition that's just going to tell me – it's hard to think that I would say I'm not going to do it, but I know that has to be one of the alternatives. I just hope I'll just know.

TITLE: Clinical Trials: A mom on her child participating in a natural history study

https://courageousparentsnetwork.org/videos/clinical-trials-a-mom-on-her-child-participating-in-a-natural-history-study/

Description: The mother of a 16-month-old with Canavan describes the process by which her child is participating in a natural history study

Transcript:

Dawn: Right now there is just a little bit of buzz. I guess if you go on clinicaltrials.gov, there is the natural history study which is open. So there is a little buzz around that. Hey, who's enrolled, who's going to

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this site, who's going to this site, who has PT coming to them, this, that and the other. So that's just where a little bit of the buzz is. Hopefully that leads into the buzz for a potential trial, because everyone in our community knows that the natural history study, which I knew nothing about ten months ago of what a natural history study was, is that this is all necessary and required in order for the trial to get approved and to move forward. So knowing there is a little buzz around that and people are enrolling and everything, that to me is good buzz.

Interviewer: Yeah. No, it's true. That's another part of the learning curve is what – the whole vocabulary of these things is long and cumbersome. The whole natural history study as an avenue, one of the critical stepping stones towards the trial. Has Vayle participated?

Dawn: Yep. She's enrolled in the natural history study, and we've already had one visit. Our third PT visit is coming up.

Interviewer: What happens in one of those studies? There weren't any when my daughter was alive.

Dawn: Essentially it's a collection of all the medical data. So for us, it was like when I enrolled, six months of data that she had from that point from her diagnosis on. I mean there are some children who are 20, teenagers, so the amount of medical data and medical records that they have. I think Vayle had over 400 pages in just the few months of records that we had, so it's a lot. I think it's a collection of ---here's how I understand it, it's a collection of the data of just what happens to a child with Canavan Disease, so that they can show the FDA, here's what will happen if there is not any treatment done. As I understand, it's to then aid to help with approvals of a drug gene therapy, and then to prevent having the requirement to do a placebo group. Because without doing anything, here's the natural progression of this disease.