

## O'Leary Videos - Transcript

### Lower third:

Michelle and Joe, parents of Logan (11) and Matthew (8). Matthew has Batten Disease/CLN2 and was one of the first children to be enrolled in a clinical trial for ERT at the age of 4.

<b>1</b>	<p><i>Title: The road to diagnosis ... Do we wish we had known earlier?</i></p> <p>Parents of two sons, one of whom has CLN2 (Batten disease), talk about their son's road to diagnosis -- at first they thought it was epilepsy, then autism. Going from that to a genetic diagnosis was devastating. Yes, we want early diagnosis but in some ways it was pretty nice to be clueless those first 3 years.</p> <p>***</p> <p><b>Joe:</b> .... we were dealing with things before we even got the diagnosis [0:06:00.4] you know we were thinking autism we were thinking you know developmental delays and all this other stuff and so we were kind of going through the motions of having to try to accept that he was not going to be like how Logan turned out.</p> <p><b>Michelle:</b> we followed the path that a lot of the Batten families follow where I should say specifically CLN2 families because his first seizure was at three and that's what a lot of CLN2 kids start at 3 with seizures. So at first we thought it was an epilepsy diagnosis; his sister had epilepsy as a child. So we didn't really think much of it. And then as we were seeing more and more things play out in school with the language delay, [0:07:00.8] and he was just struggling in general then we started looking at an autism diagnosis which a lot of CLN2 families get as well. So we were following the path that many families had. And it's funny you talk about the that lost piece during the time that we were going through all of this I was actually taking a class on spectrum disorders online. And I am reading a book and its going through all of these different genetic disorders and I am reading and going well at least we are just dealing with autism I mean it would be so much worse if we were dealing with one of these where he has got stuff and then he is going to lose it and the little did I know this was probably eight months, six to eight months before --</p> <p><b>Joe:</b> At the end of the day nothing is going to prepare you for that type of diagnosis I mean you are just not going to be prepared for that because you are just don't -- you know I said to her we lived a sheltered life so to speak and a just a little bit naive to you know rare diseases and you know -- [0:08:00.8]</p> <p><b>Michelle:</b> About naïve --</p>
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	<p><b>Joe:</b> So and then it came so –</p> <p><b>Michelle:</b> nowadays they are talking about early diagnosis especially for these diseases where there are treatments and I was just at a conference recently and spoke about early diagnosis and someone asked me if you could now have had him tested at birth and knew which would have taken away our three years of just living life not knowing that he had this rare disease, would you have done it? And I was like oh my gosh -- what an amazing question because like we were like he said we were naive, we were clueless for the first three years you know and an epilepsy comes up and it's like okay well we can manage this then autism comes up and -- half the populations autistic.</p> <p><b>Joe:</b> Albert Einstein was autistic -- I sent you an email, look Albert Einstein was autistic.</p> <p><b>Michelle:</b> So it was like put things in to an interesting perspective because it's like yeah we want an early diagnosis but living three years in that like just joy of -- pretty nice too [0:10:00.2]</p>
2	<p>Title: <i>With the diagnosis comes the need to DO SOMETHING!</i></p> <p>Parents of two sons, one of whom has CLN2 (Batten disease), talk about the urgency parents feel to do something from the moment of diagnosis on -- participate in a trial, raising money, supporting other families.</p> <p>***</p> <p><b>Joe:</b> If we can do something to help Matt do something that will just help maybe not Matt but somebody else you know what I mean like just doing something. When we got the diagnosis we walked out of MGH going [0:18:00.4] -- there is nothing we can do about this initially. Because we were told well there is maybe a trial going on in Germany but I don't know if they are taking any patients. So we came home going, alright we took a punch and we got up and we said alright you are going to Germany. That's the mindset that we had very early on –</p> <p><b>Michelle:</b> I was just going to show up knock on the German door [cross talk]</p> <p><b>Joe:</b> You are going to Germany, we will figure out the logistics. Like that was the conversation we had in bed and you know like I said the timeline of getting the diagnosis to actually finding out that there was a trial starting in Ohio, the whole time from the diagnosis to now we have been doing something. We were able to do something and then as a byproduct of that we have done some small things in terms of raising money for the BDSRA, we go to the conferences and everything. So it's a life changing event and you get a hand dealt to you and you figure out how you play it.</p>

3	<p>Title: <i>Clinical Trials: Our son is on the cutting edge. He IS the data.</i></p> <p>Parents of two sons, one of whom has CLN2 (Batten disease) and was one of the first children selected for a clinical trial, talk about what it means to be one of the first families in a clinical trial. "There are so many families that came before us, that made a sacrifice. We're in uncharted territory now."</p> <p>***</p> <p><b>Michelle:</b> So we have always had the [00:22:00.0] attitude of take his blood, take whatever you need to help -- you know the fight really. That was never a question and even with the trial we know that there are certain things that the enzyme treatments are knocking out for Matty like his vision. But we have told them right along, whatever we can do may not be for Matty. But if it's going to be helping other kids in the future that's what we are going to do. A lot of that comes from; we keep referring to the BDSRA which the <i>Batten Disease Support and Research Association</i>, which is the national support group. Hooking up with them was vital; you know I think it's vital for any family dealing with rare disease. To hook up with any sort of whether it's national or local support group. We saw these families that came before us. May be you can speak to that.</p> <p><b>Joe:</b> [00:23:00.0] Oh, just at the conference and what not? Yeah. These parents that have suffered such horrific loss is coming back to the conference year after year and -- you know their children were studied, which led to the research that a lot of the enzyme replacement therapy that Matt gets. So it's difficult to kind of - - you know we were very-very -- we could be in a totally different situation right now than we are in right now. There is so many more families that came before us that had to sacrifice for us to actually to you know have our son. It is a burden, it is like a -- you feel guilt because, a little bit of guilt because you are not following the path that so many [00:24:00.0] other kids followed. We are in an uncharted territory now with him, because there is no data on these children that are getting the enzyme replacement therapy. It is happening now.</p> <p><b>Michelle:</b> He is the data.</p>
4	<p>Title: <i>Our son and family's road to the clinical trial. No man's land.</i></p> <p>Parents of two sons, one of whom has CLN2 (Batten disease), talk about how lucky they were, with Matt being the right age, to qualify for the trial. It was all about timing. And then they had to be ready for anything -- to drop everything and move for the trial. There was no guarantee. So many 'ifs'. So much waiting. Nerve wracking. "It was only when his port was being put in that we felt it was official."</p> <p>***</p>

**Michelle:** So this is where like the -- meant to be part comes in, because we called Margi who was head of the BDSRA at the time and told her about our diagnosis. This was right at the beginning of September. It was right after that appointment really. So in our conversation we said we had heard something about a trial in Germany and she said the FDA, and I'm getting Goosebumps right now, the FDA just approved that trial in the US on August 15. [00:32:00.0] So this was literally weeks after the trial approved in the US. We said how do we get our name on the list? And she said if he qualifies you are in. So at that point she connected us to the neurologist Dr Daylor Reis who was at Nationwide Children's in Ohio in Columbus. We started talking with her and then all of a sudden the ball just started rolling and --

**Joe:** I said we -- I remember you gave a call saying there is a trial starting in Ohio. We can do Ohio. That's a lot better than Germany.

**Interviewer:** We were like map questing. What are some midway meeting points?

**Joe:** We can do Ohio. Let's relocate Ohio, that's fine. So we are assuming like we hoping to grasping on to anything we can hold hope to at that time. Right, I get a call. I'm at the doctor's office, getting a physical. I'm like we can do Ohio, that's fine.

**Michelle:** So we are having heart palpitations --

**Joe:** So we send all his [00:33:00.0] info in. They want video of him, we get them the video, all this medical records, and we send it over to them. I think we found out, we sent that over in September I believe.

**Michelle:** Yeah.

**Joe:** It was like couple of weeks after. And on Columbus Day, I sent an e-mail out to the doctor just to get an update. Because we are just in kind of a limbo, I think we got a call a day later, or two that he was in. So --

**Michelle:** And then it was like pack your bags because we don't know when this is going to start. Maybe as soon as Thanksgiving, before Thanksgiving?, because they were still in that trial. I mean that period of having to go through the board, with the hospital, get everything approved. So they were in that mode too, of like let's get this moving.

**Joe:** And Dr Emily had said that it's been -- this has been in the works for like eight years.

**Michelle:** It took that long to get it approved in the US to actually go to trial.

**Joe:** Yeah. [00:34:00.0]

**Michelle:** So in terms of actually qualifying and we didn't -- there was no real promise. We didn't really feel like we were in the trial week until he was in surgery, getting the port put in.

Because it was if he qualifies, but then there were kids that would have qualified and then had lost some skills. So by the time the trial actually got going, they no longer qualify. So we really, the entire time it was -- we would think of it as hopefully this is going to work out. And it wasn't until it was December 5, that he had his port put in that we felt Okay, this is official. Even when we are moving down to Ohio, we are not moving down even when we are going down to Ohio. That was the first time that neurologist was actually seeing Matty in person. She had seen videos and all of that. But she hadn't seen him in person. [00:36:00.0] So we knew that there was still that possibility that she could see him in person and say, no he is not right.

**Joe:** And there was --

**Michelle:** It was, it was just painful, it was very difficult. But I think it goes back to what Joe had said earlier, doing something. We felt like we are doing something.

**Joe:** We could focus on that.

**Michelle:** We could focus on that. There was hope there that we were able to hold on to.

**Joe:** And the trial was actually supposedly going to start in October and then it got delayed into November. And then November became December, and we called Dr Emily and said, all these kids are falling off cliffs from what I have seen. What about Matt? She said that he had may be a "milder" form, that it wouldn't affect him that much. But we are hearing the delays and like seeing how quickly the kids regress. It was [00:37:00.0] terrifying because it's like we are on the cusp of actually doing something and at the same time awaiting. Like you said, until we were there, like we could -- you know -- we are at the Target -- I remember we going to the Target -- like -- right? We are at the Target or whatever and --

**Michelle:** Yeah, because we made the decision that Matty and I would move to Columbus and Joe would stay here with Logan. So we weren't interrupting Logan's schooling, Joe gets to work; I was able to take time from my job. So we were literally moving Matt and I into an apartment in Columbus. So yeah, it really it wasn't until we were really and at that point your son is in brain surgery. And that was the first time you can relax [laughs] which is very, doesn't make any sense, but at that moment. And then the other thing that struck us in that way we realized how lucky we were was, we were thinking Matty was going to -- it is a clinical trial, he is going to [00:38:00.0] be -- you know even for a rare disease -

	<p>one of fifty, one of thirty. He was one of the three in the US. And so when we heard that I just broke down. Because it was that Oh my gosh, it may not have been to him. It was that moment so; it is heart wrenching to go through. But to go through it and come out on the other side, and know we were getting a treatment for our son that ended up, you know doing what they said was going to do. We would do it a million times again. No question.</p>
5	<p>Title: <i>Clinical Trials: We really felt like we had the golden ticket.</i></p> <p>Parents of two sons, one of whom has CLN2 (Batten disease), talk about some of the emotions they felt knowing that their son was in the trial while other children they had come to know and care for were not. "We felt guilty, because why Matty and not someone else? We understand it rationally but our hearts think something else." They understand the FDA process, the business and costs of the research and industry.</p> <p>***</p> <p><b>Michelle:</b> we really felt like [00:40:00.0] we had the golden ticket and we were one of 3 families at that conference that had it. And our son was still walking around and he was still seeing and he was still getting all his needs and wants met through his communication. We really felt like, it was just really difficult and I know that sounds weird to say like, it was difficult for us, when we look around and see their kids there in wheel chairs and no longer seeing. But we really felt guilty, because why Matty? Versus why wasn't it someone else? We came very close to the family who have two children with CLN-2, the older daughter got into the trial, the younger son didn't. I was -- they became part of our family. We were down there and it was -- it's so heartbreaking to watch a child that we know, could be [00:41:00.0] benefiting from it. Not because there was three spots and he just missed it.</p> <p>We get it from the rational money of that -- the economic. We understand it. But I know, our heart it thinks something different obviously. But we know -- I mean we joke around saying Matty is worth more [00:43:00.0] than we were ever going to be worth in our lifetime, because of what it has cost for this trial, for him alone. So we understand that you can't just say let's take every kid we can and try this out for free. We also understand that something that is not FDA approved, you can't just hand it out like candy either. Because then that's bringing on all sorts of more issues. God forbid something goes wrong. So the rational part of our brain gets it, like it is -- I mean when it comes down to it, it is a business. The pharmaceutical companies, although the one we have worked with has been fantastic. They are talking to their board; they are about their stocks like it is a business. We always joke around saying in a million years we never thought we would be praying for a pharmaceutical company. Like when it first [00:44:00.0] came up there was another trial that had not been very successful with gene therapy for CLN-2. Then there was this newer one with the pharmaceutical company, and then I said to Joe, we don't want to go with them. They are going</p>

	<p>to make us dependent on their stuff. Don't trust the pharmaceutical companies. We haven't had that experience with them luckily.</p> <p><b>Joe:</b> And we have done our own research too. We looked at certain -- your brother too was always in tune with the investor calls and stuff like that. You know saying these companies have they have a good track record. You know what I mean. So we did some research on our own too. And you know just -- paid attention to that stuff.</p>
6	<p>Title: <i>Clinical trials: Weighing the risks; A Leap of Faith</i></p> <p>"We were taking a leap of faith because there was nothing else there."</p> <p>Parents of two sons, one of whom has CLN2 (Batten disease), talk about how they considered enrolling their young son in the trial: they learned from a previous trial in Europe. "When you get a diagnosis that your child is going to pass away, the side effects are not a top priority. It goes back to doing something. Let's do this."</p> <p>***</p> <p><b>Joe:</b> So the decision making process with the trial and deciding to go forward with it was really -- we were lucky enough to actually get a little bit of data from the overseas trial that was taking place with the 19 children. Was it 19 or 22? 19 children. So we had heard through word of mouth that the kids that were on the trial were taking the medicine fine. There was some minimal side effects such as what? A rash?</p> <p><b>Michelle:</b> Light fever, rash.</p> <p><b>Joe:</b> Yeah, so there was some -- there was some side effects from it. But nothing threatening. So we did have luckily the benefit of understanding that, I mean when you get a diagnosis that your child is going to pass away. The side effects are not really a top priority. [00:46:00.0] Because you are just trying to do whatever you can to get him well. So you know we -- it was more of like we were taking the leap of faith no matter what, because there was nothing else there.</p> <p><b>Michelle:</b> We have literally said, Okay none of the kids have died in this trial. So we are in. Because you know --</p> <p><b>Joe:</b> Yeah, it goes back to doing something. We actually had a chance to do something and we could focus on that and just keep tunnel vision, just focus on that. So all the other stuff like getting her out to Ohio, getting them an apartment, getting them furnished and set up and situated, that was all side stuff. We will deal with that, it's just let's get him there, let's get him going and let's do this. So all</p>

	<p>the other [00:46:57.8] stuff, side effects, [00:47:00.0] logistics, all doesn't matter, let's do it.</p> <p><b>Interviewer:</b> That was four years ago?</p> <p><b>Michelle:</b> Yeah.</p> <p><b>Interviewer:</b> Since it started? So he has been now three and a half since December. So three years he has been on --?</p> <p><b>Michelle:</b> Four years.</p> <p><b>Interviewer:</b> Four years?</p> <p><b>Michelle:</b> Four years, yeah.</p>
7	<p><i>Title: Clinical Trials: You adjust your life and just do it.</i></p> <p>Parents of two sons, one of whom has CLN2 (Batten disease), describe the ERT protocol that first year living away from home for the trial -- time spent in the hospital, CSF draws, weekly schedule, getting to know the ICU staff. "When you're in the middle of something, you just do it." Mom notes that you never know how long the trial is going to take.</p> <p>***</p> <p><b>Michelle:</b> So the first year, he was going in every other week for enzyme treatments. There was some weeks where he was staying for one to two nights in the hospital. [00:48:00.0] We would be in the ICU when he got the treatment. There were some weeks where we may have to stay extra because they would do CSF, it is a cerebrospinal fluid draws, they would do it at 24 hours and they do it at 72 hours so we may have to go back on a Saturday. They access his port to do the CSF draw, but it was just every two weeks. I would pack the bag, we go in the hospital. You know we got to know the entire ICU staff. And it just became just like -- you know you are dropping your -- I'm not dropping him off, I'm there with him the whole time. But it just became like, that's just our schedule. We enrolled him in a school down there, so he was still going to school for part day, half days. So on the days he wasn't at the hospital he was in the school. So we just created as normal environment as we could for him. You -- it's like [00:49:00.0] when you are in the middle of something, that we look back at it now going wow -- you know when people talk to us they are like, wow that must have been really hard. You just do it you just adjust life. You just do it. That's how it was for Matty, and we were lucky too, because he is tremendous like does such a nice job. so he made it easier too in that sense.</p> <p><b>Joe:</b> Yeah, he naps during his infusions. So he is sleeping through two hours of his infusion. And then --</p>

	<p><b>Michelle:</b> We would have to go through every six months MRIs, EEGs. That part was difficult because it's taxing on his body. But once again that was every six months and we would have to do that. So a big challenge for us was getting on the clinical trial, it was a year according to the original protocol. Then as we are getting closer to the end of the [00:50:00.0] year all of a sudden we are being told, well it is not over yet. So now in our mind we had separated the family for a year and now we are saying, Oh my god we can't do this for another year. We thought it was a year. We could wrap our head around a year. So that was definitely information I would have loved to have known that -- okay it says it is a year but it may last up to five years. We can't tell you. That was one thing I look back on and say I wish we had known that, because we may have all moved down there. We don't know. But once again they were that able to work with us and we started commuting after a year from New Hampshire down to Columbus every two weeks. So you know, they were able to work with us and make adjustments so, I mean would you like to have your kid in a clinical trial and with a Neurodegenerative disease? It's you know we feel like we got the best treatment we ever could have received.</p>
8	<p>Title: <i>Clinical Trials: Relocation and impact on the sibling</i></p> <p>"How much did that year apart affect him? We don't really know."</p> <p>Parents of two sons, one of whom has CLN2 (Batten disease), talk about how they managed the year apart -- when they were separated during the trial -- for their older son who remained at home with dad; what they explained to him, how they supported him, how they managed to meet up periodically.</p> <p>**</p> <p><b>Joe:</b> So while they were away for a year, how we handled it with Logan was like -- it was kind of like an adventure. We made it fun, we explained to him that Matty's got a disease and the only place he can go and get help for it is in Ohio. We all went out. Right? I'm trying to think of this now, we all drove out in the van. Right?</p> <p><b>Michelle:</b> Initially just the three of us went out and then Logan came after Christmas with my family.</p> <p><b>Joe:</b> Right, yeah. Sometimes I forget, like we took so many trips. But ultimately we worked it out so that it was like an adventure, because Logan at the time was what? Six?</p> <p><b>Michelle:</b> No.</p> <p><b>Joe:</b> He is eleven now, so is he -- that's 6 or 7.</p> <p><b>Michelle:</b> Yeah, just about to turn 7.</p>

**Joe:** So you know, he is not at that point where you can say full out right what is happening. So we just tried to frame it and say we are going to see mum and daddy as much as we can and we are going to take trips. It's going to be fun; we are going to do lots of activities. But we need -- what is it? Do your job. Because to take like at least I will say now like in retrospect, how much did that affect him. We don't really know, you know because we don't really talk to him about it like in depth. Because we don't see like --

**Michelle:** We bring it up --

**Joe:** But we don't know how much that affected him that year we just don't -- you know and I -- maybe it didn't affect him and we are just paranoid but if that was me at that age and having my mom taken away from me and my brother I can't imagine -- he's stronger than I will ever be. Both the boys are, so you know we did -- one of the things that was helpful was we were allowed to make quarterly visits out there. They would fly us out to visit with them. To call my vacation time, worked it out. We would -- we met up in Niagara once. [0:54:00.2] We met up at HershyPark another time in addition to flying out we went out for Christmas, we spent a full week out there at Christmas.

**Michelle:** There were travel restrictions for Matty and I with the child initially so we weren't able to just fly home very month or anything like that. So it wasn't until we were there for quite a bit a time. I think thanksgiving may have been the first trip we took home. So it's almost a year before you know I mean he was a commodity, he was a really important guy that they didn't want to just throw on a plane, open to germs and everything else.

**Joe:** Yeah we did a make a wish trip too. We always had a full week out in California [cross talk] so we tried to meet up as much as possible while they were there. So he could have one on one with her. You know because that was very important to keep them connected still [0:55:00.8] you know because that's a long time to be without your mom at that age. I mean -- and he got through it I mean there was never a moment I felt like -- I mean yeah a little bit but like he was in - - he is happy you know Logan is happy you know and he understands his brother deals with a lot but he is a good kid and he is tough and resilient just like Matt is.

**Michelle:** He always says he would never choose another brother. You know because sometimes it makes us sad, now that our kids are older and we are watching friend's kids play together and to not have that sibling piece and Joe always reminds me Matt is who Logan knows, that's all he knows. He doesn't know having another brother. He doesn't know you know the camaraderie that brothers can have. He knows Matt in their special relationship so that's -- it's important to keep in mind because that can be something that's really hard for families to have a child with disabilities. [0:56:00.2]

9	<p>TITLE: <i>CLN2: How the ERT is delivered.</i></p> <p>Parents of two sons, one of whom has CLN2 (Batten disease) describe how his port goes into the ventricles in his brain and how the infusions are delivered. The side effects in his care are minimal which makes things easier. The port has to be replaced every couple of years.</p> <p>**</p> <p>so Matty's treatments it's a little different than the typical enzyme treatments or infusions. His port is actually right in to his ventricles and I would describe it looks like a jelly fish almost. Where it's got a little cap and then it has a little catheter that goes right in to his ventricles. And so you know and it's just a little needle that goes in and they wrap it up good and the issues are obviously any sort of contamination at the site is always a concern. Because when you are dealing with the brain you don't want foreign entities in there at all. [0:59:00.1] But unfortunately because it is right in to brain I don't know that this is going to be something that will be in and in-home treatment at anytime or anything. They need everything to be oh my gosh I am blinking -- sterile. They need everything sterile and so there are those risks. You know they always test the CSF, they draw that and they always test that to make sure there is no foreign bodies or anything. So those are really the biggest risks are the access in the ports and making sure that there is nothing there. Beyond that, you know just they give him Zyrtec before every infusion just in case there is rashes. I think Matty had maybe one or two rashes and generally it was because of the sheet in the hospital not because of the actual infusion. Low grade fever does happen with a couple of kids, there have been some kids that have gotten physically sick, vomiting. [1:00:00.4] Matty has never dealt with any of that stuff. But once again those kids that would vomit they would just give an anti-nausea medication. So the side effects are very limited and they are manageable. So that definitely makes it easier to -- we are not bringing our son in to get this in treatment that he is then like lying on his back, not himself a few days. He is back to school the next day you know so that obviously makes things easier as well because he is still himself through the whole thing. Doesn't knock his body for a loop or anything.</p> <p><b>Joe:</b> And then every couple of years he has to have the actual port replaced because of the number of accesses. So that is something we have to you know go in -- have and go under the knife, have the port replaced.</p>
10	<p>Title: <i>Clinical Trial: Our expectations – to buy more time.</i></p> <p>Parents of two sons, one of whom has CLN2 (Batten disease) share that their goal with the treatment is to slow down the disease progression and maintain quality of life so as to have more time with their son. "There are things that are coming that we will have to deal, not everything is fine." Dad stresses the importance of getting information, including from other parents, to set realistic expectations.</p>

	<p>**</p> <p>I think at the end of the day with everything, the diagnosis and everything we just wanted to buy more time. You know that was -- if we could have more time with Matt you know like the way he is and that's really what I tried to simplify it down to -- I mean we said that to each other all the time. We said if we could just have more time with him. Whatever that was, if we could slow this down [1:04:00.2] and just have more time with him. It was worth it.</p> <p><b>Michelle:</b> And the actual clinical trial, they focused on ambulation, they focused on how many steps the kids could take and they focused on language. And I think there was one other thing -- I can't remember off the top of my head but it was really speech and walking. So they weren't talking about eyesight, they weren't talking about you know slowing of seizures or any of that stuff. So we really you know from the pharmaceutical stand point we are just looking at trying to slow the progression of the disease. So they can walk for a longer period of time, they could continue their speech --</p> <p><b>Joe:</b> And by no means did we -- because this was the first treatment for it but in no means were we thinking oh this is going to have a regular life -- we never had that expectation going in to it. We were just trying to see if we could slow things down [1:05:00.6] and buy more time. And then whatever else came along we were going to deal with it you know and one of the big things obviously is the vision. His feet are turning in, so recently we had him have a procedure done where they lengthen the tendons in his feet, so his feet can be flat again. So there are things that are coming that we have to deal with you know but first and foremost he is still walking, he is still talking. So he can communicate with us, he gets his needs met. I think now with a drug to market and I have talked to Michelle about this a lot, there needs to be you know parent advocacy so to speak of -- okay you know you need to speak to someone who is actually been in the trial -- not been in the trial but been through treatment I should say. And can give you kind of an idea of what you are going to be dealing with because again a lot of the things now you get your information you look online [1:06:00.5] and you get it like that -- oh, my son or daughter has CLN2, oh there is a treatment Brineura, oh lets go get it and he or she will be fine. No, there is going to be things that pop up that you know you have to come to terms with and you know and we got as much information as we could when we started it but now with a lot of kids being on it there is so much more information that could be shared that you know just a family calling you know and a parent that's been through it to kind of say, okay here is what happens and here is what the port getting put in is and you know some of the difficulties that you are going to kind of have to deal with as you go along here.</p>
11	<p>Title: <i>Clinical Trials: Not everything is fixed. Outcome isn't a 'cure-all.'</i></p> <p>Parents of two sons, one of whom has CLN2 (Batten disease) and is receiving ERT in a trial, talk about how there are still things the treatment cannot</p>

address. Their son is losing his vision, for example. Things are going to keep coming up.

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**Joe:** I think the biggest thing that I would want parents to know is that -- the vision. You got to come to terms with the vision. The vision -- this isn't going to help your child with their vision. It may slow things down a little bit but the vision piece is not something [1:09:00.8] that is going to be affected by the replacement therapy, enzyme replacement therapy. And you know -- but when you kind of look at it from a stand point of -- well they are losing everything you know without treatment you know like you kind of get a level set but I think that's one of the big things is that -- that's going to be very hard for us I mean we deal with it now, but it's still hard for me to deal with because I mean I -- the thought of him losing his vision is horrifying you know but the thought of losing him is outright is horrifying as well. So you just kind of get a you know deal with the best you can. But it is you know -- he is legally blind already. And there is times where you can tell he is having difficulty seeing something. His eyes are shifting a little bit more now than before. So I think that's one of the big things is to get it out, right out the gate [1:10:00.8] that you know this is going to hopefully help your child but the vision piece is something you got to really understand and that they are working on doing an enzyme replacement therapy for the eyes. But it doesn't -- it's not going to help Matt. It's a ways off but they are working on it. Very much so like before Matt, the replacement therapy wasn't available for the children period and then you know the disease progressed. So it's you know it's the timelines that you know it wasn't there for their children but it was there for Matt, the vision is not there for Matt, but it might be there for other children down the road.

12

Title: *Clinical Trials: You still need to be an advocate for your child.*

Parents of two sons, one of whom has CLN2 (Batten disease), offer advice to parents about participating in a trial -- know the timeline, advocate for your child and your family. They need us, the child in the trial, as much as we need them, the company delivering the treatment. "We get to speak up."

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**Michelle:** So I think for participating in a clinical trial, I think knowing time line is important you know we were definitely surprised when after the year was up they said oh no you are going to stay for a little longer and you know -- but we were able to work that out. I think that to remember that you are your best advocate for your child and if something is not going the way you feel like it should be going, that doesn't mean there is not control over it you know to work with the company or the hospital or whoever you are working with speak up you know. We did feel like bowing down to everyone involved because [1:12:01.3] we felt it was a gift for our son but they kept reminding us, he is a gift to us. You know they need us just as much as we need them and that was something that we didn't

	<p>get initially. We really felt like thank you, but you know they needed Matty. I mean he went to the FDA, they really needed him. So –</p> <p><b>Joe:</b> He was in the approval meeting.</p> <p><b>Michelle:</b> So that was a really important thing for us to wrap our head around that you know we got to speak up you know and when it came to the idea of me living in Ohio for another year we –BDSRA once again helped advocate for us but then they were also patient advocates within the pharmaceutical company who said no, this family shouldn't be living apart. There is no reason we can't do this. So there are a lot of people that are looking out for us as a family [1:13:01.0] and for Matty, first and foremost and I think that's really important to remember because I think sometimes we think that okay we are doing this and we just have to follow their directions and don't speak up.</p>
13	<p>Title: <i>Clinical Trials: Expectations – Holding on to Hope.</i></p> <p>Parents of two sons, one of whom has CLN2 (Batten disease) and is receiving enzyme replacement therapy, share how they think about the future.</p> <p>We are optimistic. There are other treatments coming down the pike, such as gene therapy. And in the meantime, we're helping the children that come later. "Hopefully we're the last family that will have been told their child is going to die." Mom describes the shift in the disease community now that there is an approved treatment and gene therapy trials on the horizon. The outlook is so different now.</p> <p>***</p> <p>Joe: we are optimistic, which is great because you are generally not and historically you are generally not optimistic with the disease like this and there is other stuff coming down the pipe. So enzyme replacement therapy might just be a part of other treatments that he will be getting down the road such as hopefully fingers crossed gene therapy that they are working on right now. [1:16:00.2] Because to us I think gene therapy is much more you know stable thing if it can be done correctly. So you know there is hope for us, you know we have hope. And other families have hope I mean and then the other thing we said to is that even if it doesn't work out -- maybe they get something from this that helps other children you know and they won't -- we said -- hopefully we are the last family that's told their son is going to die from this disease. Because that's one of the worst things you will ever go through and you know now when they go in they get the diagnosis from a doctor, there is a treatment, we can start this. It's not go home and pray.</p> <p><b>Michelle:</b> And I feel like you really see the difference going to the yearly conference because the first couple of years that we were there when we didn't know what was going to happen with the trial and everything. [1:17:00.8] There is this just weight that everyone shares and now with having an approved treatment and having CLN1 and CLN3 are starting trials for gene therapy and</p>

there are other trials that are going on. There is just -- people are feeling lighter, they are feeling like there is so much more hope and instead of being told, go home and hug your child which so many families have been told that with rare disease. We are being told, go home, keep your child strong you know go to OT, PT, Speech -- Matty does all those things on a weekly basis you know we are being told to fight whereas in the past it was more like just spend time with your child and make them comfortable.

**Joe:** We were in an IAP meeting; they were talking about adding services at a years old where normally the conversation is -- [1:18:00.7]

**Michelle:** Taking services away, some schools will do that because they will say well they have declined so much you know it doesn't make any sense to continue this service. So I feel like you know one of the things when we first got the diagnosis we were on the BDSRA website and they have a memorial page and just pictures of children who have passed and adult you know ranging the CLN's. and I don't know why may be I am a self injurious behavior or something, I was looking at the site when we were first dealing with the diagnosis and I kept saying that Joe, all of these kids were born before 2010 this is what Matt has going for him, He was born in 2010, moving forward this is -- this page is not going to be this big and so that was -- we just feel like it's just all meant to be and that's what's given us hope [1:19:00.5] and that's what we hold on to. Like you said in the very beginning, you can't prepare yourself; you know my dad is very much of a preparation guy. And you know it's only going to slow, it's not going to stop the disease and we keep saying you can't prepare yourself for what a nerve degenerative disease is going to do for your child so why not hold on to hope? Why not look at every day, taking one step at a time vs. trying to prepare yourself for the very worse. Because the very worse is still going to be the worst, there is no way around it. So you might as well just hold down to hope.