

Speaker 1:

Welcome to The Eye on the Cure Podcast, the podcast about winning the fight against retinal disease from the foundation Fighting Blindness.

Ben Shaberman:

Welcome everyone to The Eye on the Cure Podcast. I am Ben Shaberman with the Foundation Fighting Blindness, and glad you could join us today. And I'm really excited about our guest for this episode. His name is Michael Kalberer, and Michael has, or is I should say, participating in a clinical trial for an emerging treatment. And we're going to have Michael tell us about that experience. And Michael is coming to us from Long Island, New York, and welcome to this episode of Eye on The Cure Michael, it's great to have you.

Michael Kalberer:

Good afternoon, and thank you for having me. Hi everyone.

Ben Shaberman:

Michael, before we get into the trial and your experience in the trial, can you take a minute or two to just tell us about yourself and what you do in Long Island?

Michael Kalberer:

Absolutely. I'm a resident of Mineola Long Island who lives with both Cerebral palsy and Leber Congenital Amaurosis. I am currently an entrepreneur and small business owner of my company, Michael J. Kalberer Presents Inc. The mission of my company is to enhance the communicative and interpersonal experiences of people with disabilities rendered by licensed professionals of all types. So for example, when I go to a doctor and I'm accompanied by my personal care attendant, the natural tendency is for people to address the person accompanying me and not me. So I teach interdisciplinary staffs of healthcare facilities, educational facilities, licensed professionals like social workers, psychologists and their interdisciplinary staff, cultural competence and practical application on how to approach individuals with disabilities. So that's what I'm doing here on Long Island. I'm also a licensed, master's level, social worker in the state of New York. So while simultaneously trying to build my company, Michael J Kalberer Present, I also am pursuing full-time employment in the field of social work.

Ben Shaberman:

Well that's a remarkable background. You have a lot going on for anybody and it's so wonderful to hear that despite the challenges you have with cerebral palsy and LCA that you're being so productive. Can you tell us just a little bit about what it's been like to deal with having these dual challenges of CP and LCA?

Michael Kalberer:

Well, I did not know I had a retinal disease condition until about the age of 33 when I was officially diagnosed with what we thought was Retinitis pigmentosa. But in retrospect, I was labeled as legally blind at approximately the age of between 10 and 12. So I learned at a very young age the value of altering and controlling my external environment and also the value of advocating for myself. One of my first experiences in advocating for myself was my pursuit of speech and language pathology therapy

within my school setting. I went to a school exclusively for individuals with disabilities called the Henry Viscardi School, now called the Vicardi Center in Albertson, New York. People who would listen to me would recognize that my speech is very intelligible and clear. So the natural question they would ask would be, "why do you feel you need speech and language pathology services?"

Well, because of the cerebral palsy, my body has involuntary muscle spasms or reflexes that are sometimes exacerbated when I'm trying to project my voice. So living with the dual disability, to bring my answer back to your question and not go off on a tangent, has been a challenge. But living with the transition to blindness or inherited retinal disease has been made easier in many respects because I feel like because of my physical limitations, I've had to become a better communicator and more accepting of help from others anyway. Although there have been challenges in pursuit of employment, both post secondarily high school wise and post-collegiate that are still presenting themselves. That I would love to address in a further question. But I do feel like my transition to blindness or living with inherited retinal condition has been made easier in some respects by the fact that I already live with a physical disability, which requires me to receive physical assistance.

And because of the fact that I knew because of the cerebral palsy in my case, I was not going to be able to do things like drive at the age of 18 anyway. I know for the blind community or the visually impaired community, sometimes the lack of ability to drive or the lack of spontaneity is a barrier. But that barrier was already present to me at a very young age, and I knew I was not going to be able to drive. So I've always believed that I identify myself as Michael and as a person first and a person with disabilities second. My conditions don't define me, I control them, I control how they're labeled by others. And to the greatest extent I possibly can, I control how I'm received by others and how others perceive me. I always try to greet people and look at every interaction as an opportunity for me to connect with the world outside on a micro level, person to person. And on a macro level, how can I help society become better? How can I become a productive member of society?

Ben Shaberman:

That's a really compelling story and perspective that you provide on your situation and you do communicate so well and articulately. It's very impressive and I congratulate you just on your positive attitude and your desire to not only help yourself but better society. So-

Michael Kalberer:

Thank you very much.

Ben Shaberman:

Oh, you're welcome. So let's transition into the clinical trial that you're participating in. How did you come across this trial? And for our listeners, the trial is for a CRISPR/CAS9 therapy. It's a gene editing technology that's been developed by the company Editas, and it's in an early stage clinical trial. So back to you Michael. How did you learn about this study?

Michael Kalberer:

My connection with FFB began in approximately 2015 when I became part of the New York Vision Walks. I have captained the team for several years and was given the opportunity to attend as a participant and a speaker, several Foundation Fighting Blindness functions. At one of those functions, in approximately 2017, a doctor was giving a presentation on the differential diagnosis or diagnoses of retinal pigmentosa and LCA. I listened to his presentation prior to giving my portion of our panel

discussion that day. After giving my portion of our panel discussion that day and presenting the patient perspective of what it was like living with an inherited retinal disease and Cerebral palsy, we were given the opportunity to break for breakfast.

At the breakfast, I was able to have the doctor on the shoulder and humbly ask him to help me butter a bagel, which gave me five minutes to talk to him and say, "Hey, Dr. Romero during your presentation, I was noticing that I have about nine symptoms consistent with the LCA portion of your tree diagram and about maybe two to three or four symptoms of the Retinitis Pigmentosa', I apologize. So I said to him, "Can you please help me try to find out whether I have Leber Congenital Amaurosis or Retinitis Pigmentosa? I understand they're genetically similar. How do I go get genetic testing?" So I went to a hospital in New York, New York Presbyterian genetic lab with Dr. Steven Tsang and his staff, and they took some blood and spun it and genetically tested it. Three days after the presentation, which was on a Saturday. So on a Tuesday, three days after that presentation in 2017, they found my genetic mutation, which corresponds with a diagnosis of LCA 10 called CEP290. That's Charles, Edward, Peter, 290. So we knew that in about 2017.

Fast forward to March of 2019, I was given an opportunity to speak at a Dining in the Dark Event in New York City, [inaudible 00:10:04] an Events coordinator for the New York Chapter of Foundation Fighting Blinds. During my speech, which was a stretch goal speech intended to raise funds for the foundation and launch a new app. I spoke about my experiences living with LCA 10. But at that speech in March of 2019, I met doctors from Mass Eye and ear. They told me that they were going to be beginning another trial for the treatment of LCA 10 using gene editing, and alluded to the fact that I should expect to hear from them within a year. The doctors at the event from Mass Eye and ear, the March 7th, 2019 event said, "Expect to hear from us in about a year", February, 2020, rose around just prior to the pandemic.

I contact people from Mass Eye and Ear using my Retainer Tracker patient data registry to get their information. I was lucky enough and fortunate enough to register for pre-screening testing up in Boston for the Brilliance Editas CRISPR/CAS9 trial. February of 2020 I go up there, all the expenses paid by the sponsor as is commensurate with clinical eye trials, get pre-screened, find out that I'm a candidate to be in the first cohort of the Editas Brilliance EDIT-101 CRISPR/CAS9 trial. Covid happens, we have to hold the whole world shut down for about nine months, in my case. September of 2020, I have the surgery, the gene editing treatment, my experience with FFB being at the forefront of connecting people with retinal degenerative conditions to doctors like Jason Comander and Eric Pierce and staffs of hospitals like the gracious and warm and welcoming staff of Mass Eye and Ear Brigham, that my connection to them would not have happened had I not made the speeches for FFB. And had I not met people representing Mass Eye and Ear at a speech in New York, in Queens New York, on March 7th, 2019.

Ben Shaberman:

Michael, that's a great story. It's wonderful to hear how you trying to help FFB with events and fundraising came back to benefit you in getting into the Editas trial. So-

Michael Kalberer:

Then that's what FFB does. FFB not only connects people medically. I'm very proud of the fact that I've been able to help people connect socially at different fundraising functions, bowling events, dining in the dark events, a luncheon that I put together for friends prior to going to my trial. I'm a very proud and I hope valued participant with FFB, with the Foundation Fighting Blindness.

Ben Shaberman:

Well we're very pleased that you are such an active person with our organization, you do so much for us. So if I understand correctly, you were treated on September 20th of 2020, is that correct?

Michael Kalberer:

My surgical day was actually September 3rd, 2020.

Ben Shaberman:

Okay. So what was that day like?

Michael Kalberer:

Days leading up to it, there was natural anticipatory anxiety. It really hit home for me when I met Dr. Comander and Dr. Pierce actually was able to use humor that the doctor implemented to ease my anxiety and answer questions and make me feel welcome and comfortable. When I go to Mass Eye and Ear now I feel like I'm visiting my home away from home. But Dr. Comander, Dr. Pierce and the entire staff at Mass Brigham were able to recognize my ability to understand and for lack of better term right now, my ability to understand the progression of the LCA. How I identified with it and how I tried to control it. And the use of humor by referencing Monte Python when we were reading the bad things that could happen to me during the surgery, immediately made me like Dr. Comander more than I already did.

And knowing that I had nothing to lose and everything to gain just by my understanding of how LCA was progressing and having the autonomous ability to consult with my doctors as a team and family as a team. And to be able to have them concur with the selection of what I thought was my weaker eye, not only by medical testing, but by actually being able to visually see the progression of the disease. It made me feel like I had full autonomy in the decision making process while still having a team of great surgeons and great people behind me. So there was absolutely no hesitation for me when I signed the consent forms. I'm like, "I'm in, where do I sign? Let's go". Because I've always wanted to be a part of something greater than myself. And I thought that even a chance at the ability to regain some sight was like the chance to cash in a winning lottery ticket. That's how I equated it.

I remember giving an interview to NPR radio the night before my surgery, while in a hotel up in Boston. And during that interview, which did not air until after my surgery, I had the chance to meet colleagues and friends from Mass Eye and Ear Media relations as well as a reporter by the name of Rob Stein. And that's where the emotion of what was going to happen to me literally the next day took shape. It was kind of a mixture of gratitude, trepidation, nerves, and praying for my surgeon. While also feeling like the luckiest person in the world to even have the gift of opportunity to regain some sight. But before I even regained vision, from meeting the people at Mass Eye and Ear, I had a newfound perspective and perception of what LCA meant to me and what my ability to be part of the Brilliance trial and Editas trial could bring to medical science and to me and my family.

Ben Shaberman:

That's interesting. All the different emotions you had going in, I can only imagine how overwhelmed, at least at certain points you must have felt. So you had the surgery September 3rd, 2020. And this is, by the way, during the middle of a pandemic.

Michael Kalberer:

But by the way, just as an aside, I've never felt safer in a hospital.

Ben Shaberman:

Okay. I'm glad you made that point. So how long did it take before you had an awareness that something had changed with your vision?

Michael Kalberer:

After swelling went down and so on and so forth, I remember a day in late September where I was able to see what I would describe as perceptible, just noticeable differences between the height of a sandwich and the height of potato chips on my plate. I would say that was about two to two and a half to maybe almost three weeks post surgery. That was the first incremental change but that happened in the perception of what I'll describe as shadows. There have been further advancements in my ability to perceive light increased visual field to the point where prior to the surgery, my central vision was that between a QTIP head and the size a dime circumference. And now I would say that my visual field is the size of between a dime and a quarter. And the underwater sensation, which would often accompany the progression of LCA for me prior to the surgery, that underwater sensation is now gone in my right eye.

Ben Shaberman:

That's wonderful.

Michael Kalberer:

There has been slow and steady incremental improvements that are allowing me to see differences in light perception. Slight visual field increases in terms of central vision from the outside of my visual field in. I see bursts of color sometimes and I can most definitely identify food on a plate for me, which probably means in a funny way, I'm probably going to gain weight because I can now more easily finish meals and identify things like my glass of beverage beside my plate on a table. So my taste buds are a little happier and my waistline and stomach are probably going to get a little bit bigger. So it's just been an awesome experience.

Ben Shaberman:

That's great. It's wonderful that you are enjoying some of the more regular things in life just because you're able to see them better.

Michael Kalberer:

Yeah.

Ben Shaberman:

So you said your vision improvements have been incremental and now it's been more than a year since you had the surgery. Do you feel like you're still gaining vision, have things leveled off? Where would you say your vision is at now?

Michael Kalberer:

I feel like it's an evolutionary process, not to use too scientific of a term, but I feel like it's a process that is, I hope, continuing to evolve. But the fact that I can go into a restaurant and have a more enjoyable experience rather than a more trepidatious one is amazing. But I'm hoping for more advancements in my vision and progress in visual recovery. But if I don't get a single thing more, I already considered this a victory because of what I've gained back both visually and emotionally. To be part of something

greater than myself, and to know that because of my contribution and others' contribution to the study, that children are now able to get the treatment in higher dosages because of the safety and efficacy of what we were rendered and the people following me were rendered. That is a huge contribution, and that shows me that I'm definitely part of something greater than myself. While I still want to regain enough vision that would minimize or change my level of needing someone else to accompany me and be more independent, I can now do things like walk around my home relatively independently without bumping into too much stuff. So that's awesome. So I consider a small but significant victory, which I think can lead to even bigger and greater successes.

Ben Shaberman:

That's wonderful, Michael. And I'm so delighted to hear that you're enjoying and making use of your improved vision. But I think something that's important for our listeners to understand is this trial was the first ever CRISPR/CAS9 therapy that was applied directly to the human body. So that's a pretty big milestone. And as you've told everyone, Michael, you were in that first cohort at the lower doses when they're still really learning about really the safety and the efficacy of the therapy. So-

Michael Kalberer:

Yes, I can safely say that I was the second person in the world to receive the treatment.

Ben Shaberman:

Wow, that's a remarkable contribution. So thank you for being a pioneer and the vision improvements that you've experienced are well deserved. Just one last question. What has been the biggest challenge, would you say, or what's been most difficult about being in this trial?

Michael Kalberer:

The biggest challenge is really tempering my expectations, tempering the expectations and hopes of my family and friends and not being hypervigilant to changes in my vision. And really staying cognizant of making sure that any progress in my vision doesn't cause alterations in my attitude. And really staying even throughout the process and just keeping that positive mental attitude that I've had. Because I really do truly believe that positive mental attitudes can have great benefit on medical outcomes. But I look most forward to just returning to the pursuit of employment and growing my company. And to just return into being Michael outside of the CRISPR/CAS9 trial. I have done a lot of media outreach, both on behalf of the media relations department of Mass Eye and Ear, and local Fox News affiliates and National Public Radio, which I love, because I love to get the story out here for the benefit of the science, not necessarily my own personal benefit. But if I can change the attitudes or minds or hearts of people living with Retinal degenerative conditions and their families to show that they can perceive themselves from a position of strength rather than a position of deficit.

And even one more person hears about medical technology and science like CRISPR/CAS9 or the foundation of Fighting Blindness or the great staff at Mass Eye and Ear and gets connected with opportunity to make their lives better in the future. I've done what I've set out to do. It's great. Going up to Boston is great. I'm 14 months to the day after my surgery and I've experienced so much emotional intrapersonal growth from the experience that I wouldn't have changed a thing about this. I would make the same decision 1,000 times out of 1,000. I look forward to continuing to return to Mass Eye and Ear for my 18 month follow up sometime in the spring of 2022. Look forward to continuing to grow my company and use my assistive technology to continue to pursue employment either in the field of social

work or doing consulting work and presentations for entities that need my help and would benefit from my skill set.

But it's all about just taking every day, literally opening my eyes open, visually perceiving what I can see with my eyes, but observing it with my heart and taking it in and just working, being happy with what I have while working toward what I want and making contributions for a greater good for the cumulative people with inherited retinal disease and blindness causing conditions. But I have never felt like I've had to be cured or fixed. And I don't think people with blindness should feel as though they have to be cured or fixed either. Alleviating medical symptoms and getting a gene to turn on cures my eye. It restores my vision and clarifies my purpose, but it doesn't fix who I am as a person. I was never broken, my eye was. My spirit may have been temporarily, but whatever doesn't kill us, make us stronger. So I congratulate all of you on the successes that you have accomplished and I know you will continue to accomplish, but there's a reason why our eyes are placed in the front of our heads. Look forward, don't look back, confront every challenge and embrace every opportunity.

Ben Shaberman:

Michael, that's so well put. And I congratulate you on having such a strong emotional perspective on your situation and you clearly are a strong person who has grown tremendously through your situation and receiving this therapy. And I know you've talked to a lot of media and I appreciate you talking to me here for Eye on The Cure. I want to remind our listeners that if you have a comment or a question, you can email it to podcast@fightingblindness.org. Again, that's podcast@fightingblindness.org. Michael, thanks for taking the past half hour or so to share your story and your very enlightened perspective on your vision and who you are as a member of this world. Any final thoughts or words before we say goodbye?

Michael Kalberer:

Yes, if anybody in the community of listeners to this podcast would like to contact me, Ben, I would like you to humbly ask you to put my website and email address in the show notes of the podcast.

Ben Shaberman:

Okay. And I will have you send those to me through email and we will put those in the podcast description.

Michael Kalberer:

Absolutely. Everybody, enjoy your Thanksgiving coming up. Veterans Day is also coming up, so God bless our troops and their families and just continue to believe in yourselves and enjoy life. Have a great day.

Ben Shaberman:

Well, so well put Michael. Thanks everyone for joining another episode of Eye on the Cure, and we look forward to having you back for another episode in a couple of weeks. Bye-bye.

Speaker 1:

This has been Eye on the Cure. To help us win the fight, please donate at foundationfightingblindness.org.