**Pursuing Diagnoses and Sequencing with Gay Grossman**

# Transcript

Stan

Hello and welcome everyone to the n-Lorem podcast series. I'm Stan Crooke. I'm chairman and CEO of n-Lorem, and I'm your host for the podcast series. Today we have a special guest that I'm sure many of you already know. Gay Grossman is joining me. Gay, her husband Steve, and her daughter Lilly have been strong supporters of n-Lorem from the very first, and Lilly is a patient that we have accepted for potential treatment at n-Lorem. Gay, welcome. Thanks so much for joining us.

Gay

Thank you, Stan. I'm really happy to be here to talk to you today.

Stan

Well, great. Well, it's good to see you again and I'm sure many, many people who are listening already know your history because you've been involved in rare diseases now for well, I suppose, since Lilly was born. But maybe I'll do a quick rundown and then you can tell us what you're doing these days. I know you've changed jobs recently. As I said, Gay has been involved in rare diseases for many years and founded the ADC5Y Society, if I recall correctly, and ADCY5 is an adenylyl cyclase that particularly gain of function mutations cause severe movement disorders. And thanks to Gays efforts and those of others, we know a lot more about this disease today than we did a few years ago. She was head of patient advocacy at Neurocrine is it Neurocrine? That was at Neurogene, and now has just moved to Citizen, if I understand it correctly, as responsible for patient engagement, if I recall. So Gay, welcome. What are you doing these days at Citizen? I know that's an exciting place. I think one of our other parents works there as well. Kelly Dalby, if I remember correctly. So, tell us about that.

Gay

I’m, yes, very excited to have joined the Citizen team just four weeks ago and I joined Citizen to help them with their engagement with families. So, a lot of the work that is being done at Citizen patient advocacy organizations are joining, and they're signing up to have their medical records accumulated by Citizen so that patients have access because we all know that patients do own their medical records. But sometimes it's challenging to get those medical records, so citizen helps families get those records and put them in the palm of their hands so that if they're traveling around and seeing other physicians, they can share their records widely, they can engage in research with that information and pharmaceutical companies that are studying the disease can also have access to these records if the patient decides to share them.

Stan

Yeah, yes. You know, we've been involved with Citizen now for a while ourselves, and I think the effort to assure that every, particularly nano-rare patients from my perspective has full access to all the medical records in a fashion that they can use, it is really a critical step, and a number of our patients benefit from having, you know, very detailed medical histories. It's a shame that a parent or a patient has to do that themselves, and hopefully one of the policy changes that we engineer in the coming years is a much more thoughtful way of accumulating medical records and making them accessible to parents. But I imagine it sounds like you're really doing much the same as you did at Neurogene, that is, involved in engaging patients in the process.

Gay

Yes, working with families is something that I've always wanted to do, and that I'd like to do. I think that, you know, my daughter is 26 years old now and I have been through a lot of the process of, you know, just learning about the gene that's been identified as you know, Lilly was undiagnosed for 15 years. So, in that 15 years we traveled around to a lot of different institutions, and what I would do is contact them afterwards and try to get the medical records and sometimes it was easier than others. I knew the people in medical records at my local hospital. I knew a woman, you know, first name basis. I would go in every once in a while, and I would get all the records copied, I would have them in a notebook. And really, at Citizen what we want to do is enable families to get rid of that notebook and be able to have it electronically so they can share it more easily. But I would go to the local copy store, and I would copy those records and I would put them in a big manila envelope, and I mailed them to institutions all over the country that were trying to help us. But it was, you know, you can imagine it was a laborious task to try to keep everything organized and just try to make sure that it got to the right place. And also, I was sharing it with, you know, Lilly's information on it when really it should have been de-identified some places that I was sending it, and you know we can do that now, but this is one of the advantages I think of being in the game for 26 years, things change. And what I can help families do more easily today than what I had to do a couple decades ago is something that I really like to do.

Stan

That sounds wonderful. And you said a couple of very important things that you know are very common histories. First, it was 15 years. And of course, Lilly benefits from advances in technology and the technology to genomically sequence has come of age and gotten cheaper and more available. But we still believe that the vast majority of nano-rare patients are never diagnosed. And in our first 173 patients, we have patients who, one patient who was diagnosed within a month, and another that took 36 years. And our average time is about five years and obviously that's vastly too long because during that time patients were progressing and while you were doing all that, you had to try to take care of Lilly and manage your healthcare and try to learn what the heck is going on. When did you first begin to worry that something was wrong with Lilly?

Gay

I remember being concerned when she was about six months old, you know, because she, you know, she had trouble even balancing on four extremities. Her arms would often collapse, her head would, you know, crash onto the floor, and she often had bruises on her forehead. The first time I actually have it on paper. She was eight months old. That was when she had her first ear infection, and they had taken her for a follow up, and that was the first time that it was written on paper that I can find that I asked about it. And I remember that appointment clearly that I actually sat her down on the exam room floor. And I said, "Now, now look what she does." And I handed Lilly a magazine that was hanging on the wall. And she took the magazine and she, in a very controlled way, rolled back, and she could hold the magazine in her hands, and she could turn the pages, so her dexterity was quite good. But I said to the doctor, "You know, it just, it seems like she doesn't want to sit up and she does this all the time, she plays on her back, and she can hold a board book and turn the pages like she's doing now, but she just doesn't ever sit up." And she seemed more complacent, then things were difficult for her, and she could stand and hold on to something and, you know, move around. But she would never let go. And she didn't actually start the movements until she was a little bit older. She was probably three years old when she started waking up in the middle of the night, and that's really the key to this disorder. The children or the patients are woken or are awoken at night because of the movements, and we've actually done the research to prove that they're waking up because of the movements. And as it developed through her body, you know, when she was little, I remember thinking it was a behavioral issue that she just wouldn't go to sleep. And so, you know, we would watch as she got older and her body got bigger that it almost appeared like the mass of her was getting bigger, so things got more difficult. So, you know, children will use those plastic grocery carts, you know, to walk behind and Lilly could do that as long as I put a gallon of water in the bottom of it so that it wouldn't tip, but she could walk with doing that. But it just got more difficult as she got older and the movement started to move from her legs and it traveled up her body as the years went on, and the worst of it was, you know, it got all the way up to her face and her full body was affected by it before we were able to identify a compound to stop it.

Stan

So, going back to the first visit to the pediatrician, how did the pediatrician react to your concerns?

Gay

Well, you know, I'll tell you the end of the story. I had a lot of pediatricians.

Gay

This pediatrician in particular, she was helpful. She tried to help us. She did pull out the Denver study, and she went through it. And she said, "Well, you know, really the only thing that she's not doing on here is these gross motor skills. You know, she's eating correctly." and a lot of times they'll look at how a child is using the muscles in their mouth to identify if there's a problem, and she wasn't having any problems with that, and she could feed herself, you know, on a high. And there was a lot she could do. And this was again before the movements had started. So, it was really difficult to identify it. But when the movement started, that's when I started to really get worried, because then it became very clear that something was not just a delay, that there was actually something that was the matter, and then I got busy identifying different physicians in my city that saw children for you know, different specialties that were not just a neurologist but were other specialties that perhaps could, you know, an orthopedic who could perhaps recommend something that, you know, did she just need some orthotics on her legs? But you know, early in the years, I think what they tried to do was reassure me as a mother and to let me know that, you know, she'll grow out of this, and maybe she needs a few physical therapy visits. And of course, I did all of those, but that didn't help.

Stan

No, and that's, you know, common. And it makes sense because parents do worry about development delays and often the development delays are nothing to worry about. And that's one of the real challenges of these patients is recognizing a patient that has something significant going on versus, you know, just the natural variability and when you talk about movements, you're really talking about sort of ballistic uncontrolled movements I suppose. Is that the right way to describe it?

Gay

That's right. I often identify, I help people picture it. If you picture someone having a seizure, a grand mal seizure, and their body is very stiff and every one of their extremities is affected and it's much, that's what it's like. Her body will actually go through the exact same cycle of movements, so if she's going through a time we call that, you know, kind of a time of crisis when these movements will happen, if she wakes up in the middle of the night and I go into her room to help her, and her legs are kicking very, very stiffly, and then her left arm will swing around to almost hit her face. I know I can count how long it will last and I can actually know that the next time I go into her bedroom for a movement to happen, it will go through the same cycle, her legs will move the same way, her arm will move the same way, and it will last about as long, and it will go on, you know, all night long. And sometimes these movements will happen when we were kind of in the thick of it, it would happen eight to ten times would be typical. That would actually be a normal night, and a very bad night would be every five minutes where there would really only be a minute or seconds in between, and they would last minutes long. And that was really, really challenging. You know, Stan, I used to think of times when I would have the opportunity to talk to someone like you about this, because you can imagine being up all night. I thought about this a lot, and I remember certain parts of this series of my life then, and I remember hearing on the radio about them, you know, sequencing the first genome. And I remember looking at Lilly and thinking, I wonder if this is what we'll need to find out what's wrong, which is what we did need. And I remember connecting, you know, my husband and I are very good at networking. We always have been, we have been in sales, and so we are very good at making connections, and we would talk to everyone, and we would share our story, and you know, I just remember thinking, what will it take to find the right person who can help. And so many nights, I would think to myself that we have just fallen through the system and we're not able to find anybody to help us. So, I never thought it would be 26 years, but I'm glad that it's happened, that we've connected with you and those at n-Lorem, and other people who have helped us along the way to get to this point.

Stan

Yeah, I'm sure that there were many nights when despair was about the only emotion you could feel. I certainly know I went through many of those nights with my son, and those are tough. It's tough to be hopeless, I suppose is the simplest way of saying it.

Gay

Yeah, I mean, I like to do lists, and it was very challenging for me to have my to do list get very short because I ran out of options.

Stan

You bet. And so, you mentioned you found a medicine that helped her. Probably on a step back, adenylyl cyclase is one of the key sets of proteins that you have that you use to respond to stimuli and alter then cellular activity and among the things that adenylyl cyclase does is it regulates cyclic AMP levels in neurological cells that control movement. And so, it's a rhythmic kind of problem that Lilly and other patients experience, and the pattern may be different for different patients, but it will be the same pattern repeated over and over again. Being an adenylyl cyclase, things like caffeine and theophylline and so on can cause potential problems. So, does Lilly have to watch her caffeine intake or anything like that?

Gay

No, so caffeine is what helps her.

Stan

Sometimes it helps, sometimes it doesn't I guess, but anyway.

Gay

So, caffeine, we've identified as the compound that does help these patients and it works much like Ritalin for ADHD, it turns back the overexpressed gene. And although Lilly doesn't drink caffeine, she does take it in a capsule form four times a day. And if she wakes up in the middle of the night, she does the opposite of what any of any of us would do. She actually takes a caffeine capsule, and she will be back to sleep in 20 minutes.

Stan

Does she or you know, a lot of the diazepam's have been used? I guess, right? But is that something that has helped her?

Gay

Before we really knew the gene, we used a lot of medications that are often used for seizures to try to dull the movements, and that's what they did was they would dull it, but they never fixed it. It was, unfortunately it would dull her even during the day, and really, Stan, we never really realized how much it was dulling her during the day until we were able to eliminate all of those things. And I can tell you the day that she stopped taking the last one, it was actually when she came home during COVID and she said, I really want to stop taking this, and we stopped it, and then the next morning I could honestly see it was like her eyes were clear. And I said to her, "I don't know how you went through school with all of these medications you were taking." And she said, "I feel so much different, I feel so much clearer, and I just think more clearly." Well, of course, any of us would, but all of the families have tried medications like that, but they don't work like caffeine and some of the other compounds that have been identified.

Stan

Yeah. And you know, many of our patients have severe seizure disorders that are unresponsive to all of the seizure medications, and sadly there are no anti-seizure medications that don't have meaningful side effects including just dulling you, and Lilly, of course, is a very bright young lady. How's she doing these days?

Gay

She's doing great. She's recently started a position at a company called Disability:IN, and they're hired by corporations to help them accommodate the workspace for people with disabilities. So, she's really enjoying the interaction she's having there at this new company.

Stan

And she went to UCLA, right?

Gay

She went to Whittier College, and she majored in political science, and she minored in English.

Stan

Well, you know, it's wonderful that she's overcome all those challenges, and that you and Steve and she are in a very different place from where you were for, I'm sure, many years. How did you happen to get Lilly sequenced?

Gay

Well, we have always made sure that we know what's happening in San Diego with research, and we were able to find out about a study that was happening. And you know, I always say that we learned a lot by families, and I was contacted by a family I know that told me about a research project that was going on locally, and they were doing whole genome sequencing. And my first thought was, oh, geez, this is the time when they're going to tell me that because I didn't have more children I can't be involved in the study because I don't have a control of, you know, I didn't know how it would be done. So, I called them, and I said, "You know, I want to let you know up front that Lilly doesn't have siblings." And she said, "Well, all we need is the trio." And I said, "Well, I have that." And she said, "Are you sure you can get the blood from Lilly's father?" And I said, "I'm positive I can get the blood from Lilly's father." So, we went ahead into the study and Lilly was patient number one, and they were able to identify the gene.

Stan

And your story is far, far more positive than many of the stories we hear, and with us, I guess the next sort of thing that would be good to talk about is, you know, what are the major lessons that you learned? I imagine you're going to say, what I believe strongly is that we must introduce genomic sequencing into newborn evaluations. And we have to do it now, not five years from now, today.

Gay

I am one of the biggest proponents of genetic testing that you'll find. It's very challenging for me to hear about the families that go without genetic testing, or the ones that get some genetic testing, but maybe not the right genetic testing, which is why whole genome sequencing is so valuable to us. The descriptive diagnosis. I think we used to get a diagnosis of epilepsy or cerebral palsy or mitochondrial disease, and we had to stop there. We didn't have any other options. But today we have genetic sequencing where you can find out what causes the symptoms of cerebral palsy, epilepsy, mitochondrial disease and I think it's so important that parents understand that if you have a diagnosis like dystonia or the ones I previously mentioned, you're not really getting the true diagnosis. You're getting a symptom, and it used to be a diagnosis, but today we can we can know so much more.

Stan

I've been arguing for quite a number of years that it's time to discard the names of diseases that are all hundreds of thousands of years old. They're archaic. They don't teach anything. And of course, the one plus that nano-rare patients have is they have a definable, understandable genetic cause of their disease. So, once we have that, we can go to work. Obviously, that's step one, and so that is a central lesson that I think everyone involved would carry forward. And certainly, at n-Lorem, we're trying to drive that banner forward and sadly it's happening in lots of countries other than the US right now, and it's time that it happened in the US, and I hope that day comes soon. When you had the sequencing, what did you have to get done before you understood the nature of the mutation? Was it a gain of function mutation? Was it a loss of function mutation, that sort of thing, which is often something that is missing in our applications, and we have to then go try to figure all that out?

Gay

Yeah, there wasn't a lot known when we got the diagnosis. We went to have a meeting with our neurologist who supported us being in the study, and we knew the name, and that was it. We knew ADCY5. We were given one paper about a family that had a facial twitch. And we've never been able to find that family. We knew that Lilly was obviously more affected as she depends on a wheelchair. She needs 24/7 care even though she's not affected cognitively, she still needs support physically. You know, a lot of people still say, and I know you've heard this too Stan, they still say, well, you know, what's the point of getting a diagnosis if you don't have a treatment, but really you can't get a treatment if you don't have a diagnosis.

Stan

I mean, people want to know what's wrong.

Gay

People want to know what's wrong, and we were traveling all over trying to find out what was wrong and, you know, trying to find a diagnosis is one thing, and we were so good at our pitch to try to find this diagnosis, but as soon as we had the diagnosis and we were given this one paper, I remember Steve and I were, we said to each other, "Well, now we need a new pitch, we've got to figure out what this is," and so we really got to work. The first thing that we did was contact the authors on the paper and find out if they knew any other families. If they knew anyone doing research. We agreed to any publicity that scripts translational research wanted to do on our story, and we said, "You can connect us with any journalist that you want to, and we will be available to do a story, but you must put our website." And we had Lilly presented at the Movement Disorder Congress in front of 4000 attendees. We had our neurologists share the disease. We were very fortunate to do that because that really opened the doors for us, and people started coming out of the woodwork. We were able to connect with one of our lead researchers, who's in Paris, and we really sat down to say, how can we find these families? You know, it's hard to do research with one and we only had one Lilly. So, we did all of this publicity so that we could find the other 99 families, and we did that. Now we have over 300 patients with the gene mutation. The exact variant is much smaller, which is why we're working with you at n-Lorem. We don't have a a large contingent that have the exact variant that Lilly does. But Lilies is the most common, and you know our hope is that if we can help Lilly, then we can help the other few that have the same variant, and then we can help the greater community of ADCY5, and then perhaps we can then overlay that on to other diseases.

Stan

It's absolutely right, and obvious, and I think there's a ton of data also showing that diagnosis saves money, and it also it's mistreatment and misdiagnosis which is a very dangerous process that that we see a lot of. And one of the things that that we're experiencing in various communities is, you know, real disappointment that their child isn't the first to get treated. But again, I think the message that you just gave is absolutely right, everything we learned applies across the board, and if we can help one patient with, or group of patients with a particular variant, we can help others often, and all of that advances in understanding of the disease and also advances making the world aware of the problems these patients have. These are patients who are uncared for by our system today. It's not an indictment of the system. The system was built for common diseases, and this is the opposite end of that spectrum. Any other lessons that you want to share with people after 26 years? You must have a giant notebook full of important lessons.

Gay

One of the things that I try to convey to families that are newly diagnosed, and they contact us, and they do contact us all the time. I just had a family reach out to me last night. Actually, I often wake up and I look at my cell phone, and today there was a message from a family in Australia, newly diagnosed, and the first thing I want to do is connect them to other families in Australia, which I do. But one of the things that we talk about on the family calls that we have is the drug development process and just how challenging it is that this is science, it's not black and white. And this is still experimental, and I think some families, many families, they get the diagnosis, and they think you know, what's the treatment, where is the treatment. And when can we have the treatment and a cure? And I don't use the word cure. You know, I will be very hopeful if there's a treatment that could help Lilly and I. I know I can't get the 26 years back, but if we can somehow alleviate the symptoms, and have her and other people live as typically a life as they can, then I will feel like we've done our job, but I think that you know a lot of what I do with our foundation and the families that we work with is really educate them about where our place is in the drug development process, and doing things like keeping our medical records, you know, and that's what I'm talking about with working with Citizen now is, you know, I'm enrolling my own community in Citizen so that we can get the medical records, and we can make sure that we can truly understand that the retrospective data that's available to all of us without having to travel and go to a study. Encouraging families to participate in other studies that we're doing, we're doing a brain imaging study right now and it's, you know, it's challenging to go through studies like that, but that's how we learn about the disease and if I can just relay that to other families, the importance of what they're going through now and sharing that information, that is what's going to help us with the babies yet to be born, who will be born with the same variant.

Stan

You bet. And you know, one of the questions that I had when I began n-Lorem was that I knew that the patients by the time they would make their way to us would be very advanced. And as the disease advances, it becomes harder and harder to treat. But we now, after actually a very limited experience, know unequivocally that in many cases we can help patients. We have one patient with a form of ALS who actually died and had to be resuscitated who's now doing marvelously. And we have another who's improving in multiple domains, not just movement, but so on. And both were extremely advanced, and we know from SPINRAZA that if we can get to patients before they become symptomatic often, they'll grow up like normal, healthy human beings. And so, n-Lorem is the tip of the sphere. Treatment is always the tip of the sphere, and that we can help some means that others can be helped in due course. And I think you said another very important thing, and that is drugs don't cure, they treat. But if you take your Lipitor every day, your risk of heart attacks is dramatically reduced. And I think there is so much misuse of the word cure. And we know even today with gene therapy, it's clear that it doesn't last forever. So, there's really no such thing as a drug today that cures. What we can do is treat, but treatment can be extraordinarily effective, and our goal is to get these patients earlier, treat them more effectively, and prevent them from having to return from developmental delays which we're seeing. We're seeing patients who experienced developmental delays actually begin to recover some of those and that again is the cost for I think substantial hope and that's a message that I think ought to get out there and we're doing our best to get it out, so it's been a really interesting chat for me, and very, as it has been every time we've interacted very, it's a learning process for me to see all that you've accomplished, and had to go through to accomplish it, any questions for me about n-Lorem or anything else that's going on?

Gay

Well, I'd love to hear a little bit about n-Lorem today compared to a few years ago, like what kind of things have you been able to abbreviate since you've done this a couple of times, and how will that affect our experience?

Stan

I think as you know, when I started n-Lorem, I thought by now we'd have a handful of patients, and I'd plan to run n-Lorem all volunteers for the first two or three years. And, you know, the applications are complex. And the first day we had a website, we had several applications, and so I think the biggest difference is that the demand has been greater than we expected. And we've been able to raise the funds to grow much more rapidly than we expected to respond to the demand. And so, today we have about four times the capacity that we had just a year ago. And our goal is from treatment on average from making the decision to treat, actually that first dose, do that in 18 months or less, and that we're not doing that. Yet that's very disappointing to me. Just the demand has overwhelmed us. And some mutations, like Lillys, are particularly challenging, and they take longer, but our goal is 18 months, and we now have expanded enough that we're beginning to work our way through the backlog and that's very exciting. We've also learned that just finding institutions willing to take care of these patients is a real challenge. And so, I think that was a shock. But now we've identified multiple institutions and we're identifying more and more that will be available to help our patients. And then this year, I think we can reduce cost and time by about 30% and even today gives thanks to all the support that we get, the cost is about 40% less than it would be without support from various vendors. But every minute counts. Every minute counts, and so our goal this year is to cut the time by 30 percent. And I think they're very straightforward ways to do that. We're moving toward it, and we're also investing now in basic research so that we'll be able to make better ASOs for allele selective problems and do it faster. So, I think we've just begun, and the technology continues to advance, and our understanding of how to use it. These patients continues to advance. And as that comes forward, we'll be able to treat more patients and we'll do it faster, and we'll do it cheaper. It's astonishing that we can talk about treating a patient for $1,000,000 or so for life; that alone is an amazing achievement. And we're doing that. But we can be better.

Gay

And I think you know, there are a couple of things that are exciting to me about that. $1,000,000 sounds like a lot of money, right? But it's not, and I can tell you that we know that has been spent on just Lilly many times over already in her lifetime. So, imagine if we could have introduced something like this when she was much younger. But, you know, now we're here. The other thing that is very exciting to me is we worked very hard as a foundation to be able to do initial research and the fact that we were able to coordinate having our fibroblasts sent to n-Lorem to help in your research is so exciting to me because it shows that we as parents and our group and the people who helped us get to the point of having those fibroblasts and getting them to you and they're actually being used in order to get us to a treatment is so exciting to me.

Stan

But you know we can't do this alone at n-Lorem. We have many, many partners and that partnership extends to the FDA. It extends to patients and parents. It extends to all the vendors and companies that support us and all of the donors that support us and that's the only way we're going to make progress here is link arms and work together to make fundamental changes in our policies and our processes. And you know, one of our goals this year is to really begin to get to insurers, to explain to them how much money they could save if they can help us get to these patients, and you know, just end of life care is millions of dollars. Never mind all the misdiagnosis, mistreatment, misreferrals that every one of these patients goes through. Incredible waste of time, incredible waste of human potential, an incredible waste of money, and it all has to change. And I think we're beginning to get traction in many, many quarters. And with help from people like you, I'm optimistic that sooner rather than later, we're going to see real changes. That helps us do better here.

Gay

I agree. And we've had a lot of people help us. You know, the company that did the fibroblasts, they did them for free for us, and that's really how we function as a foundation. We work with the people who advise us, like Nick Schork has been incredibly helpful to us. And any time we get to the point of next steps, and we go back to our board, and we say what do we need next, and then our next question always is who does that? Who does that and who do you know who does that? And then we go talk to them and we find out how we can do it, and that's what we did with the fibroblasts. And it was so exciting when we were able to call that company and say, hey, we actually have a use for them. Can you get them over to n-Lorem? And it was just a couple of phone calls and then they were delivered to you, so you know, that's really exciting because all the time that we've put in to learn about this and to get people to work together, it's shown that it can work and people do work together and they, you know, I can't wait until this comes to fruition, and really does have her first dose.

Stan

Yeah, you know, I realized late in life that basically I've been a dream merchant most of my career. And doing n-Lorem was not what I planned, but it has turned out to be, again, infinitely rewarding for me personally. And one of the great privileges I've had is to meet so many wonderful patients and parents and you and Steve and Lilly are in that cohort. So, please do say hi to Steve and give Lilly a hug for me, and as you know, we're working hard to bring treatment to Lilly. We'll keep working until we get it done.

Gay

Well, thank you, Stan. I can't thank you enough, and your team has been so helpful to us, and we've really enjoyed getting to know everybody.

Stan

Thanks so much. Thanks everybody for listening. Tune-in next time for whatever I do next. Thank you.

Narrator

n-Lorem is a non-profit committed to discovering and providing personalized experimental treatments for free, for life to patients with genetic diseases that affect 1 to 30 patients worldwide, referred to by n-Lorem as nano-rare. Many of these patients progress and die without ever achieving a diagnosis. This is where n-Lorem comes in. They do the impossible by providing hope, and for those that they can help, free lifetime treatment. For more information about n-Lorem or today's episode, visit nlorem.org. Any questions can be sent into podcasts@nlorem.org. Search n-Lorem on Twitter, Instagram, YouTube, LinkedIn and Facebook to connect with us. This video is hosted by Dr. Stan Crooke and produced with the help of the following professionals. Thank you for watching.