Oklahoma Innovations Radio Show

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Guests: **Sanjay Bidichandani**, Oklahoma Children's Hospital

>> From the OCAST Radio Network, this is *Oklahoma Innovations*, a weekly science and technology radio magazine, brought to you as a service of OCAST, the Oklahoma Center for the Advancement of Science and Technology. OCAST is the state's only agency whose sole focus is science and technology. The OCAST mission is to identify and fund promising research and technologies that allow Oklahoma to compete in a global market economy from our own backyard. This program features some of Oklahoma's most gifted scientists, inventors, entrepreneurs, manufacturers, educators and business leaders who all have one common goal: developing technology based economic growth for all Oklahomans. Now, here are your hosts Gary Owen and Tessa North.

[Music]

>> Thank you Andy and welcome Oklahoma to another edition of Oklahoma's science radio magazine, *Oklahoma Innovations*, and my co-host, Tessa North is really excited again this week because it hadn't been that long ago we did some research discussions on children, we're going to do it again. And this week we're going to talk about genetics.

>> Fascinating. I'm very interested to hear what our guest has to talk about today. I know this is something that we got a little bit of introduction to when we were pregnant with our twins.

>> Oh really?

>> It will be interesting to see as we go forward.

>> Yeah.

>> What he has to say for us today.

>> And have you had your genetics tested?

>> I have not, but--

>> OK.

>> There have many instances where things have come up, and I thought maybe that's worth looking into.

>> Well what we're going to talk about today with our researcher at the University of Oklahoma College of Medicine, and I'm not going to pronounce his name yet, I'm going to let him do it first, but he is going to be talking to us about the genetic process and research. We all have unique DNA, and sometimes in the lab they can tell what kind of diseases we might be prone to get down the line, so-- and then of course if there's symptoms of a possible disease now, why they'll be able to test that out in genetic research. And we're going to be talking about that a little bit later. Very fascinating stuff. So who's our Oklahoma spotlight this week?

>> Today we have a spotlight that comes to us from the Oklahoma Medical Research Foundation just across the street from our office. Recently, scientists have published a landmark paper that sheds new light on MS, multiple sclerosis. This paper marks the first collaboration between research scientist Dr. Robert Axtell and physician Dr. Gabriel Pardo, who are combining their

expertise to understand how a particular MS medication alters immune cells in a way that suppresses the disease and its symptoms in patients. This new information gleaned from the study will also allow clinicians to look for potential markers in patient response so that treatments can be specifically tailored to individuals. This study represents a significant milestone in OMRF's efforts to accelerate translational medicine, a discipline that's often referred as to a bench to bedside approach.

>> That's easy for you to say.

>> Bench to bedside. Thanks to OMRF's world-class Multiple Sclerosis Center of Excellence, clinicians are co-located with basic researchers, allowing physicians and scientists to work hand-in-hand to learn what does and doesn't work in treating MS patients. By enabling researchers and clinicians to work closely with one another and share findings, patients can receive new treatments more quickly, so if somethings not working, the doctor can go to the research scientist and say "Hey, what can we do to tweak this treatment?"

>> Wow.

>> Thanks to this collaborative nature of the MS Center of Excellence, this paper is the first step in understanding how some MS medications work and ultimately improving the lives of patients with the disease. So a lot of progress is made over there at OMRF, and a lot of opportunity for increased progress as clinician's are working right alongside scientists.

>> Outstanding. Outstanding. Well in our Innovations in History This Week, how long do you think the QWERTY board has been around? Do you have any idea?

>> Twenty five years.

>> No. Would you believe the QWERTY board has been around since for nearly 150 years?

>> Whoa.

>> Yes. In June of 1868, and I know people just stopped their cars and went "What?" In June of 1868, the first commercially successful typewriter was patented and that featured a QWERTY board. This keyboard design was used to help prevent jams caused by neighboring keys being pressed at the same time, thus speeding up typing ability. The QWERTY board keyboard has been popular since 1878, and remains the most common keyboard layout today. Now a lot of people think of that as the little boards we slide out from our phones, you know, they call that the QWERTY board, but actually-- isn't that fun? You didn't know that did you?

>> I had no idea.

>> Well if you have seen some of the old manuals typewriters, which was way, way, beyond-- she's going "I don't." You've probably seen one--

>> Typewriter? What's that?

>> Yeah. That's right. See, most young people they don't know what it is, but, so, that's OK. You do know what a type-- but right now a lot of people who are listening to this program that used to work on the electric typewriters; they'll have an appreciation for that as well, all right. Well let's introduce our guest. He's the Associate Professor at the University of Oklahoma College and Medicine, Biochemistry, Molecular Biology, and Pediatrics. That's the label we've gotten. And he's the Section Head of Genetics and he's got a big list of credentials there. So let's welcome-- you know, I'm going to let you pronounce your name for the first time, and then after

that we're going to call you Dr. B because you have one of those names. You're from India right?

>> That's right.

>> That's your heritage, so tell us how you pronounce your name because every time we see a name like this we go "Oh, I don't want to mess it up."

>> It's actually said just the way it's written. It's Sanjay Bidichandani. Dr. B is just fine, yeah.

>> I like that. Bidichandani. OK. Sanjay, right?

>> That's correct.

>> OK. All right. So tell us a little bit you and how you came to Oklahoma.

>> So I came to Oklahoma actually in 2000. I came here to setup my research lab. This is after medical school, after getting a PhD, and after finishing my fellowship training in Houston at the Baylor College of Medicine. I came here, setup a lab in the biochemistry department, moved up to full professor, and then left for two years and went to the Muscular Dystrophy Association as their Vice President for Research. And in 2013, actually at the very end of 2012, I returned mostly because the Children's Hospital Foundation recruited me back to come as the head of Pediatric Genetics, and somehow I called the CMRI, Claire Gordon Duncan Chair in Genetics.

>> The CMRI, Claire Gordon Duncan Chair of Genetics. That's interesting. Go back to working for Muscular Dystrophy because that had to be some interesting research. Did you ever meet Jerry Lewis?

>> Yes. So, before that, the research that I do is on a condition called Friedreich ataxia, and it's one of the forty different neuro muscular diseases that the Muscular Dystrophy Association cares for. So they do a lot of research and they have many clinics that care of that.

>> I see the connection now.

>> So that was the connection, and actually in 2010, I think it was, about five years ago, I actually got to be on the telethon with Jerry Lewis to explain some of our research and help in the fundraising. And then of course while I was at MDA then I got to meet with him several times.

>> So that was a long, long, worthwhile project for Jerry, and of course everybody knows come Labor Day, we're all going to hear more about where the research has gone, and where are we today with muscular dystrophy? And it's obviously progressed quite a bit thanks to all of the donations that have been made to research.

>> Right. So there's been a lot of changes in the past two decades, where the genes have been found for most of the conditions, the pathogenesis has been figured out for most of the conditions and now, there is several drugs that are in clinical trials for many of the neuro muscular diseases.

>> Well, Tessa and I have reviewed a lot of the bio information, and I know as many of our listeners know she's a relatively new mom. And she has had some experiences with her family and at Children's Hospital, and one of the things that we both talk a little bit about is the fascination of research on the genetic level, and getting down to the DNA. Kind of give our audience an overview if you will about how this all works. Genetic, you know, the genetic level,

and the DNA and how you uncover-- the-- if you will, I guess on the chromosomal cellular level of our makeup.

>> Sure. So the simple way to think about this is our body is made up of proteins, OK? And we have in every cell of our body about 20,000 genes that code for these proteins, OK? And these genes can have spelling mistakes in them, which is what causes several genetic defects. These genes are also packaged into 23 pairs of chromosomes, and you can lose and gain chromosomes as another way to have genetic defects. So you know about Down Syndrome? In Down Syndrome kids instead of having 46 chromosomes, they'll have 47. They have an extra 21. Then you have people who have sickle cell disease for example, or cystic fibrosis. They don't have missing or additional chromosomes; they actually end up with small spelling mistakes in genes that cause these conditions. So the things that we do in the clinic is to first identify people who we think may have a genetic condition, and then we take their DNA, all their cells, and test them in the lab to see if they have these spelling mistakes, or extra chromosomes, or missing chromosomes.

>> And there's more of this kind of research going on, right, on the genetic level?

>> Oh absolutely. In fact, there are just newer types of tests that are being developed, but I think we'll just step back a little bit and think about this in another way also. So we know that about 50,000 babies are born in the state of Oklahoma, and all of them go through a newborn screening test. So this is where they take a little, you know, heel prick and they'll get some blood spots, and these blood spots are tested for several conditions, many of which are genetic conditions. So many people are actually are already getting genetic tests without realizing they're getting them. And these individuals would have been identified, let's say for cystic fibrosis, or sickle cell disease, at a lot later stage if we were not doing it at the newborn stage. And the reason it's done at the newborn stage is one, it's cost effective to do it, but two, we can identify them really early and actually start giving them counseling and start doing interventions that can make their lives a lot better.

>> So you just mentioned counseling, what sort of counseling might go into-- are you talking about like emotional counseling, genetic counseling, behavioral? What sort of counseling are you talking about?

>> Thank you for asking that question. So in a genetics team we have typically a clinical geneticist who's an MD, and then we have a genetic counselor who actually is specifically trained to do counseling for genetics. Now, the thing that's important to realize is that any other field of medicine when you get a diagnosis, that diagnosis is your diagnosis.

>> Right.

>> In genetics when you get a diagnosis, it's a diagnosis that has huge implications for the rest of the family, OK? So many times you can then tell if there are brothers and sisters who are at risk for this, or are other family members that might be at higher risk of having a child with a certain condition. So the counselor will sit with the family, explain to them some of these complicated things as to what the diagnosis means to start with, but then what it means for the extended family members and actually bring them in and talk with them as well.

>> So-- sorry-- go ahead.

>> So I was just going to say that we actually train genetic counselors in our department and we have about four genetic counselors that get trained every year and we have eight at any one time,

because it's a two year program, but we graduate four every year. And many departments are now using genetic counselors because of the large number of genetic tests that are done.

>> Right. That was actually my next question for you. So, as a geneticist you have an MD or some sort of doctoral degree, what sort of credentials I suppose other than the two year training are-- is required for a genetic counselor?

>> So the two year training is actually a graduate program, so that's a master's program. So they're obviously graduated with a biology degree typically, and then they'll do two years rotating in different clinical sections. They'll go into the diagnostic lab. They'll have experience with, you know, social issues and counseling, that sort of thing. And when they finish they actually get certified, so they have to take a board exam, pass the board exam, and then they get certified as genetic counselors to actually practice in the state of Oklahoma.

>> Very cool.

>> This is interesting stuff. I'm going to start calling you Dr. B because I don't know that I can pronounce your name for the entire time. That's challenging. That's a big mouthful there. We're talking with Dr. Sanjay, and his name is actually Dr. Sanjay Bidichandani, is that right? OK. I'll try to roll that off my mouth here. And he's the Associate Professor with the University Of Oklahoma College Of Medicine. We've got to take a little break. We'll come back and we'll get a little deeper into his research, and find out just what kinds of genetic disorders they uncover at the College when we return on *Oklahoma Innovations*.

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>> Pancreatic cancer is the fourth leading cause of cancer deaths, with a median survival range of only six months. As an oncologist, I see far too many families suffer from the effects of this terrible disease. We need better treatment options for patients.

>> With the support of the Oklahoma Center for the Advancement of Science and Technology, the researchers at Core Biotechnology have what they hope will eventually be a treatment, even a cure for pancreatic cancer. They have identified a protein that if blocked may prevent tumors or keep them from growing. With help from OCAST and i2E, the team at Core was recently awarded an SBIR research grant to enable them to continue their research and move closer to a treatment for pancreatic cancer. If you're a researcher or a small business in Oklahoma, and are considering applying for federal SBIR funding, contact OCAST toll free at 866-265-2215, or visit us on Facebook or our website at ocast.ok.gov.

>> Now in its 19th year, this is *Oklahoma Innovations* on the OCAST Radio Network.

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>> We're talking about genetic research and primarily in children this week on *Oklahoma Innovations*. Dr. Sanjay Bidichandani, I hope I'm rolling that out. He's a Professor of Pediatrics at the University of Oklahoma's College of Medicine.

>> Dr. Bidichandani, Dr. B, one thing that I'm interested in knowing about is how did you get into this very interesting field?

>> So, first of all, it is a fascinating field and lots of new things are happening in this field. And actually went I went to medical school, which is way back, not much was known. I mean at that stage the human genome was not sequenced, many genes were not known. And then when I

started to do my PhD, at that stage I actually got into genetics because a lot of new genes were being identified, and actually I was part of a group that found one of these genes in 1996, for Friedreich ataxia. And at that time I was in Houston at the Baylor College of Medicine, and the field just sucks you in, because at that point the human genome was about to be sequenced. But I think more than anything the field has continued to grow in leaps and bounds. So way back it was just about identifying genes. Now that we've sequenced the human genome and new technologies have developed for therapies, we actually had a really landmark time where we're doing two really important things. We've got new technologies for doing diagnostic tests that would have been considered science fiction until, you know, just a few years ago. And then some of the new molecules that are being taken into clinical trials now, there small molecules that can be small DNA strands, small R and A strands, new kinds of proteins, all of these things are in clinical trials now, again in the realm of science fiction. So whatever made me decide to get in the field, I consider myself really lucky that I am in this field, just given how it's progressed.

>> Well, you know, so you've mentioned that these therapies and testing sound like something out of science-- sounded like something out of science fiction in the past, it sounds kind of scary to me to think about getting, you know, my whole genome sequenced. What on earth goes into getting genetic testing? Is it very invasive? Is it really simple? Should I be terrified of it?

>> So the most typical type of genetic test is you get a blood sample from somebody, and you will test for a specific gene defect, or a specific chromosomal defect. So you don't just say "I'm going to sequence the whole genome, or sequence part of the genome." What you do is you make a clinical diagnosis and based on that you make an informed decision to do a genetic test. Now, having said that, we also have the technologies to do either whole gene genome sequencing, but more commonly whole x-ome sequencing. Whole x-ome sequencing is just saying "You know what? We don't understand what 98 or 97% of the genome does, so we'll just focus in on this two or three percent that we know actually is functional and we understand what it does, so if we do find something wrong in this two-- three percent we might be able to figure what this person has." OK. And so those are new tests that are being developed. There's no-- we don't take biopsies, and I mean those are done in rare circumstances, but it's typically a blood sample or a cheek swab, or things like that.

>> Wow. That sounds really amazing.

>> So do you see in the future where we normally go to the doctor for our typical physical and they take blood for various tests, average tests, you know. Do you see this part of the testing process eventually for those who request it, to say, you know, whether your test is age, or you're my age, or your age, or Debbie Cox, our producer's age. I mean a call falls in different categories to say "You know, I just want to know, OK, I'm getting older now, what are my risks? What's in my genome? What do I need to be prepared for? What is my DNA saying? Am I going to get cancer? Am I candidate for this? A candidate for that?" Is that something you see in the future, because not everybody wants to get that done right now because it's expensive, but--

>> So I think there's two ways to think about this from the academic, you know, perspective, or the medical practice perspective. You really need to be diagnosing the condition, or taking a family history and figuring out that this person is at a higher risk of developing a certain condition and then test in this sort of rational manner, OK? And now that you bring up this point, I think it's important to talk about something new that's been going on and it's called direct to consumer testing. So there are many companies out there that they're not based on academic

medical centers where they reach out to the general public and say "You know we can test you for any of 50 different conditions and we'll send you a little container. All you do is get a cheek swab, put it into this and send it to us, and we'll tell you, you know, whether you're prone to certain conditions." Now, people are obviously curious, right? It's some form of genetic aspect--

>> Well especially if there's medical history of uncertain conditions that's hereditary, so.

>> Absolutely. So if there's a medical condition that we want to diagnose, typically people will come to a clinical geneticist or a clinical department and be seen, and we would do a genetic test under those circumstances. But we don't just the cast the net wide and just say "out of curiosity let's try to find out what you're predisposed to."

>> Yeah. Because there's obviously a reason, which when you talk about children right now, I mean you're testing for birth defects, you're testing for connective tissue disorders, cancer predisposition, neurological conditions, hearing loss, skeletal disorders, what else? I mean-- I see you going down a bullet list here of all kinds of things that we don't think about when we have babies.

>> So, I mean, one to two percent of newborn kids will have a genetic condition.

>> Wow.

>> So it can be obviously single gene defects. It can be chromosomal defects. It can be biochemical genetic defects, and, you know, now that we're talking about it, I think it's important to realize that here in Oklahoma in the College of Medicine, we have three certified diagnostic labs that are doing a total of about 5,000 tests every year, looking at either single genes, or chromosomes, or biochemical defects. And I don't know if we're going to talk about this, but we now have, you know, an expanded genetics lab facility. We used to have this really small genetics lab, and now we have three times the space. They've got 20 people testing all kinds of conditions, and actually with the help of the Children's Hospital Foundation we are now trying to bring some of the newer technologies for testing into our lab, so we can be, you know, absolutely current for the kids of Oklahoma.

>> Wow. That's just fascinating. We are coming up on a break. And congratulations on having that wonderful lab, because we know that the bigger the better, and the more tools you have to work with the better. And we want to also get into talking with some of your colleagues and some of the students that are working in your research, because I'm sure it's fascinating as well. We'll be back with our guest from the OU College of Medicine, Dr. Sanjay Bidichandani when we return on *Oklahoma Innovations*.

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projects, and provided support to hundreds of Oklahoma based companies. The investments made in these businesses yield high returns for our state by strengthening and diversifying or economy. Advancing innovation is investing in a positive future. That's what OCAST is all about. For more information, call OCAST toll free at 866-265-2215, or visit us on Facebook or our website at ocast.ok.gov.

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>> Research and development, technology transfer and commercialization, creating high paying jobs in Oklahoma, is what OCAST is all about. This is *Oklahoma Innovations* on the OCAST Radio Network.

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>> Welcome back to *Oklahoma Innovations*. Thank you for joining us. We are talking about genetic research at Children's Hospital and the OU College of Medicine. The facts about Children's Hospital, the Children's Hospital Foundation is the only organization in Oklahoma whose sole focus is the advancement of pediatric research and education. And since its inception, Children's Hospital Foundation has raised $100 million to fund pediatric research, education, and clinical care activities at the Oklahoma Health Center. Today, Children's Hospital Foundation has endowed 35 research chairs in the Oklahoma University Department of Pediatrics, and our guest this week, Dr. Sanjay Bidichandani is one of those researchers, and you obviously-- this is one of the things that you are most proud of.

>> Absolutely. First of all, I wouldn't be here if the Children's Hospital Foundation hadn't recruited me.

>> Sure.

>> To come here. I would have to say that working with my predecessor, Dr. John Mulvihill, and Children's Hospital together, the Children's Hospital Foundation together, actually setup a world-class genetics department, or genetics section, we call it, in the Department of Pediatrics. And we have not only grown in terms of our clinical outputs, we see about 1,700 patients in an outpatient basis every year. We'll see about 750 of them for genetic counseling. We do more than 5,000 genetic tests, so there's a lot of clinical load that we now cover. And so this is great for obviously for the children of Oklahoma. But I would say the biggest contribution that the Children's Hospital Foundation has made is in terms of building up our research base. So we

have now, you know, really top names, so we've got D. K. Sanghera who's a world leader in the genetics of diabetes, who's working in our section. We have Klaas Wierenga and Susan Hassed who have used some of these whole x-ome sequencing methods to discover brand new genes that cause extremely rare conditions in people who would not normally have been diagnosed before. And of course John Mulvihill, who is a world leader in neurofibromatosis, and tubular sclerosis and is part of our faculty. My research is focused on Friedreich ataxia and we can talk about that if you want.

>> We'll do that here in a bit.

>> But that is again another area in which we are supported in a big way by the Children's Hospital Foundation. I'd just like to add one more thing here that's just that the Children's Hospital Foundation has funded 35 endowed professorships. OK. So, you'll see my name is the CMRI Claire Gordon Duncan Chair, so that is one of those 35. We have five out of these 35 that are dedicated for genetics research and genetics clinical care. We've got two of those positions filled, so we're in place now to recruit three new world leaders who can come here, again, thanks to the Children's Hospital Foundation. And the Children's Hospital Foundation also has funded about 100 different research programs. It is where you imagine an endowed chair, you see a person there, but what you don't see is the research programs that are going behind the scenes, right?

>> And you don't hear about them much.

>> You don't hear about them, yes. And so we have in the genetics section 24 research projects that are going on, not all of which are funded by the Children's Hospital Foundation, but our main genetics focus is funded by the Children's Hospital Foundation. And so a lot of work comes through because they've been great partners with us, and we've had great people who've been able to step-up and actually use some of these resources.

>> And their affiliations. We haven't talked much about their affiliations. For example, the Children's Miracle Network, which we have mentioned in previous programs, that is an important one. You talk about some of the other colleagues that you have around the country that are tied into similar research in what you're doing at very credible research centers and hospitals. So a lot of great things going on in Oklahoma, and again unless you get on a program like this, you just don't hear about it much in the media, right?

>> Let's talk a little bit about the research that you're actually doing because as both you and Gary have mentioned nobody hears about the research, but obviously it's of a paramount importance, especially with it comes for talking about treatments for the future. Can you talk a little bit about what you research? What is Friedreich ataxia?

>> Yeah. So Friedreich ataxia is a neuro degenerative condition. It affects about one in 25,000 people, typically from the teenage years, so you'll have kids who are fine up to maybe about 10 to 12 years of age, and then they start stumbling and falling and then by mid-twenties, they'll be in a wheelchair. And typically by their mid-thirties or early forties they will succumb to the condition. So it affects people in their prime of their lives. And it typically affects the coordination. That's what Ataxia means. It means you're in-coordinated. So they have slurred speech, they cannot walk in a straight line, and so from finding the gene, which is what I did during my fellowship training for this commission, and then setting up my lab here at OU College of Medicine, we started to figure out what that mutation actually does, so trying to

understand the pathogenesis of this condition. And then more recently now we've been working on actually coming up with therapies for this condition. So we have figured out exactly how this gene is turned off by this mutation, and we've got drugs now. They're small molecules that can actually make the gene come back on.

>> Really?

>> Let me just say what that small molecule-- so I won't tell you too much about the chemistry of this.

>> That's fine.

>> But when we say small molecule that is code for saying if you take-- you can take it orally, OK? And it goes all over the body including to the brain, OK? And so this takes away the whole discussion about how will you get to all the cells that are in the brain or in other parts of your body that may be affected? So we're really lucky to have had-- to have discovered this small molecule that actually turned the gene back on.

>> That's fascinating.

>> What sort of other research is going on over at the Health Sciences Center in your department?

>> Yeah. So I mentioned Dr. D. K. Sanghera who's a world leader in trying to understand the genetics diabetes. Now, we all understand that diabetes, you know, there's an epidemic of diabetes going on, OK? A lot of it is tied into behavioral things like diet and exercise and things of that nature, but what's not recognized enough is that there's a genetic predisposition to getting this diabetes. So not every obese person, not every person who's not doing exercise will develop diabetes, but some people will be more prone to doing so. And so in D.K. Sanghera's lab she's creating the whole genome to identify these susceptibility genes, and has already published some really high, you know, high level papers in nature genetics, some of her top journals in collaboration with many people all around the country. And I mentioned earlier that Susan Hassed and Klaas Wierenga they are using this whole x-ome sequencing method. And I said well that was early but it's just basically sequencing about two percent or three percent of the genome, and you do this after having suspected a certain condition, done a genetic test, it comes up negative, you do another genetic test, it comes up negative, you do one of these low, sort of, resolution scans of the genome, that comes up negative, and then you have no other recourse, and you say well now they're going to sequence this one to two percent of the genome. And that's not something that was available, you know, even a few years ago. So now we're able to come up with diagnosis along those lines.

>> I want to go back to your Friedreich ataxia for just a second because we may have some grandparents and parents out there listening to this and go "Give us the symptoms, the early stage symptoms on this."

>> Sure. So you typically have-- ataxia means incoordination. And what will happen is you'll have kids who you can imagine, they don't have loss of power, so their muscles initially have normal strength, but they're not able to walk in a straight line, and they'll typically trip on things and eventually they won't be able to do, you know, their writing will start to suffer because it takes a lot of coordination. Their speech will start to get slurred, and then in later stages they'll also start developing cardio myopia, which can actually present as heart failure, or arrhythmias

of the heart. And you can then also develop diabetes, so this is sort of the whole spectrum of this condition.

>> And how does this compare to something like MS and some of these other muscular disorders? Is there a mis-diagnosis with this kind of disorder?

>> Oh no. If you see a neurologist they will know, yeah, because first of all it's a progressive condition, so you will not suddenly go from having ataxia to not having ataxia. In MS you can have, you know, sometimes it has a remitting, sort of it goes back to normal and then it comes back in a worse way.

>> About what age does this seem to appear?

>> So Friedreich's will start typically in the early teens, and it will get progressively worse where they'll have a really tough time walking past the age of 20. And so we meet a lot of families all around the nation actually. So I'm on the Board of Directors of FARA, which is the Friedreich Ataxia Research Alliance. That and my role on the Medical Advisory Committee of the MDA, so I get to meet a lot of the families who have, you know, this condition. And we also have people here in the state of Oklahoma who come visiting us in our lab to see what latest research is going on.

>> So I'm interested-- something that you just mentioned made me wonder this. I know that the Genetics Program at the Health Sciences Center is world-class. It's really become a very well known. Do you see clients from patients that are from outside of Oklahoma that come here seeking your expertise?

>> Yeah. So we will have people come in from the surrounding states. I mean, obviously, we compete to some extent with Dallas and Houston on our South and St. Louis to our North, but we'll have people coming in from Arkansas from Southern Kansas, from Northern Texas, especially if they have certain conditions that they feel that there's more expertise over here.

>> We're talking with Dr. Sanjay Bidichandani. He's the Professor of Pediatrics with the University of Oklahoma's College of Medicine. We're talking about all kinds of genetic research. Fascinating what he's doing and we have a lot more to talk about when we return on your Oklahoma Science and Radio Magazine. Stay around. I think you'll find this next segment interesting as well on *Oklahoma Innovations*. Don't go away.

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>> This is one of the longest running weekend radio talk shows in America. *Oklahoma Innovations* on the OCAST Radio Network.

>> As a police officer one of the most dangerous parts of my job is arriving on a scene where an armed suspect has barricaded himself, or where we suspect some type of booby trap. We're most vulnerable when we don't know what kind of explosives or weapons are on the other side. It can be deadly.

>> Tactical Electronics, an Oklahoma based company invents, manufacturers, and sells tools such as under door cameras, and video fiberscopes that are used by law enforcement officers, military, and counter-terrorism personnel around the globe. The tools allow areas and packages to be inspected from a safe distance, which reduces the risks of injuries and death. With the support of OCAST, the company is developing imagine recognition software that scans packages, and in milliseconds identifies what's inside. OCAST is advancing science and

technology that not only improves, but also saves lives. For more information call OCAST toll free at 866-265-2215, or visit us on Facebook, or our website at ocast.ok.gov.

[Music]

>> Genetics touches virtually every medical condition and the need for genetics knowledge is expanding. Physicians who can diagnose genetic illnesses and analyze genetic disease data are in short supply. Just a short decade ago a scientist began the quest to enhance their ability to provide this very specialized care for patients with conditions such as cystic fibrosis, autism, birth defects, mental retardation, and extremely rare genetic conditions as we've been talking about today. And today hundreds of Oklahoma's children are receiving sophisticated treatments and prevention measures due to ensure a better health outcome for Oklahoma's children. Genetic counselors assist individuals and families in understanding the diagnosis, treatment and management of a genetic disorder. When appropriate, they may refer patients to a specialist for treatment of a specific condition. And when we say we're in short supply, you know, hopefully a university campus like the OU College of Medicine and people like Dr. Sanjay Bidichandani, Professor of Pediatrics are leading new future scientists down that path.

>> That's right and Dr. Bidichandani had mentioned at the very beginning that even since he's started studying genetics the field has grown by leaps and bounds. So I'm interested Dr. B in knowing-- getting your thoughts about where do you see the field going in maybe the next 10 or 15 years.

>> Yeah, so I think that's a really important question and, you know, as opposed to most specialties in clinical medicine where in other specialties you'll do a diagnosis, you'll have a treatment, some treatments work, some don't work, some work better than others, but in genetics, typically you will do a diagnosis and then you will have sort of preventive methods, or other kind of interventions, but we don't really have therapies for most of our conditions. And so most of the treatments are based on supportive care, surgery, nutrition, you know, that sort of thing. But I think what's important to recognize is that we actually are at a unique time, I think, in history where we have several hundreds of molecules, hundreds of drugs literally, that are in clinical trials in phase one and phase two trials. So our goal obviously is to give the best healthcare possible, right. The best preventative care possible, but at the same time, best therapeutic care possible is becoming-- is going to be possible over the next five or ten years, because hopefully many of these molecules will progress through clinical trials. And the metaphor I like to use here is that in the past, maybe 10, 20 years ago, we would say it had sort of more of a pie in the sky type of strategy, and now I say that there are many pies, they're in the oven, right? Some will bake right. Some will burn. Some will not bake. And so over the next two, three, four, ten years, maybe 15 years, we will have several of these molecules actually enter clinical practice.

>> Do you know what I appreciate about scientists like you, is you take a young mother like Tessa, my cohost, who has twins, you're giving whereas opposed to my generation there was no real hope. If you have a young mother like Tessa who may have a child who might develop a disorder, and we hope that never happens, but what you're giving young parents today is now you're giving more promise and more hope because of all of this wonderful new technology in the testing cycle.

>> I mean cystic fibrosis is a great example, isn't it? So they have one common mutation that causes cystic fibrosis, but then you have several other mutations that cause cystic fibrosis. And

you may have heard the term personalized medicine, so now there are molecules that can actually be used based on the known mutation. Same with Duchenne muscular dystrophy, Becker muscular dystrophy, Friedreich ataxia. Once you know the mutation, you actually have a molecule that targets whatever it is that mutation is doing wrong. And so this is a whole new realm of genetic-- of actually medicine in general.

>> Where do you see your research going? I mean where would you like to see your research expand to say in the next five or ten years? I mean you've been doing obviously doing this quite a while, and you've seen the progress. Obviously a lot of things would help accelerate the research is the development of new tools in the lab, the discovery of new pathways to get to where your goals are in research. Talk about that.

>> So, you know, I mentioned earlier on in the program that whatever made me decide to come into this field, you know, fascination, whatever it was that where I am right now, I'm in a very lucky position because the molecule that we've now found that works in Friedreich ataxia, at least in cells and in mouse models, has not completed a phase one trial. Now phase one trial remember is just looking for safety, OK? So it's come out as safe. Now remember this molecule never existed in nature, so you don't want to just give to people and, you know, start to see if it's going-- yeah, hopes for the best. And so what's happened now it's come out as being safe, and what I can hope for is for that molecule to actually progress through clinical trials and actually become a therapy in the next five years, let's say four.

>> Talk about your students because that's a part of what you're doing.

>> Oh yeah. So, first of all, our section is involved in training of medical students, PhD students, pharmacy students, and medical residents. OK. So we teach human genetics and medical genetics at all of these levels. We also train PhD's to actually do research in the lab. Post-doctoral fellows actually do research in the lab, many of whom I can say are doing actually award winning research, OK? But the flagship program we have is our Masters in Genetic Counseling Program, and it's the only program in the state of Oklahoma. It's actually a very competitive program, so it's hard to get in to. It takes about two years and then you graduate and then you do a board exam and you certify as genetic counselors. I'm proud to say that not only do they do great clinical care, they also, all of them, do research projects, many of which get published or presented at the national level. But, at the same time, these guys get recruited four or five months before they finish their-- before they graduate. So we're turning out people that are going to be, you know, supporting not just kids in Oklahoma, many of them go all around the country and take care of kids with genetic conditions.

>> I think it's really interesting that an underlying-- not even underlying-- a theme throughout this entire discussion today has been research, and as Gary mentioned that's not something that we almost ever hear about when we think about the treatment at least of diseases and disorders. So I think that's really cool that there's a lot of research going on behind this. Obviously that is what is needed to ensure that we have a better future for our children. And one thing that I think is really interesting is that you've actually been funded under OCAST, twice in the past, I think, several years ago, but it's just really encouraging to see that there's so much research going on over there to work on curing some really detrimental diseases for our children.

>> If I may just first of all thank the two of you for doing this program because I think one of the things we probably don't do that well is to communicate with the public to tell them all the latest things that are going on in genetics. So thank you for doing that. Two, I would again like to

thank Children's Hospital Foundation for making so much of this research actually possible. And I've only been here for two years in this role, so I cannot take, you know, full credit for all the things that have happened, and I think due credit goes to John Mulvihill, Terry Stahl who's my chairman, and our Dean, Dean Andrews, whose actually, you know, shown some of the leadership to make some of this research happen here in the College of Medicine.

>> I know this is probably discussed among colleagues, and I'm sure you go to seminars all over the country, and you collaborate a lot about where genetic science has come from and where it's going, and what the future looks like. Can you paint a picture for us, not just in children, but in all ages because science, when you look at research in general, we're looking for therapies for everything from heart disease to cancer and of course, lots of mental and physical disorders that we haven't even talked about today. Where is this whole thing going? You know, I can think a decade ago that I remember when the genome was discovered and you guys found the actual combination, you know, which helped DNA research. Where is this going 10 years from now? What do you envision as a genetic scientist? What do you hope will be the next big discovery?

>> So, two things. First of all, ten years from now we will hopefully have a shelf of molecules when somebody comes in, we'll do a really quick genetic test. We will be able to pick up, you know, something from the shelf and actually give them a therapy that's personalized to their mutation. So that's something that used to be a pipe dream, today the pipe is a little shorter than it used to be, so I think that's one thing. The other big thing is, you know, we obviously-- we as the genetics community after having sequenced the genome, realized that, you know, the genome is actually a lot more complicated than just looking at a string of ladders. So I'm partly involved in some of this research, but it's mostly going on all around the world, where we now recognize that it's not just the genes themselves, but how they're packaged in the cell that determines whether they are functioning or not functioning. So you can actually have quote unquote, "A genetic defect, i.e.; a gene not working, not because there's a mutation there, it's just packaged wrong. And so now there are drugs that are coming out to actually modify the packaging of these genes, but there so much new to learn. Now that we've got the basic thing, the sequence out of the way, is-- its that's what I mean by everything is growing in leaps and bounds. And one more thing if I can say, you know, for many genetic conditions one of the first things we do, and we do this as responsibly as possible, is we make an animal model for a genetic condition. It's typically mice, because they're easier to study, but we study worms and geese, and things like that also. And we've been able to cure many of these animal models and so hopefully the next step is the shelf with drugs for humans.

>> Sanjay that's just fabulous stuff. Thank you so much for being our guest this week, and we're certainly proud of University of Oklahoma's College of Medicine and all the wonderful things that the Children's Hospital Foundation is doing as well. We appreciate everybody being on this program. We've got to go. Tessa I'll see you next time on *Oklahoma Innovations*. Have a good week.

[Music]

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