Miguel Leal, MD (00:17):

Hello everyone. And welcome to the hypertrophic cardiomyopathy podcast, title, hypertrophic cardiomyopathy, not just an adult disease, the pediatric perspective. This is one of a series of podcasts from the American heart association hypertrophic cardiomyopathy initiative, which is a sponsored by Bristol Myers squib. I am Miguel Leal and I am a cardiologist and an electrophysiologist at the University of Wisconsin in Madison and I am honored to have two colleagues and a patient join us in today's podcast. I also have the privilege of introducing them all right now.

Miguel Leal, MD (00:49):

Our first colleague will be Dr. Seema Mital. Dr. Mital is a heart function and transplant cardiologist at the Hospital for Sick Children in Toronto, as well as a professor of pediatrics at university of Toronto. She leads the Sick Kids Heart Center Biobank with over 10,000 families with congenital heart disease and cardiomyopathy. She is also scientific lead of the cardiac precision medicine program of the Ted Rogers Center for Heart Research, where she spearheads the whole genome sequencing studies in cardiomyopathy.

Miguel Leal, MD (01:20):

She established an international 22 center study called Precision Medicine for Cardiomyopathy or PRIMaCY . This group published the first fully validated, sudden cardiac death risk calculator for childhood hypertrophy cardiomyopathy that is now available as a web-based digital tool. She was also vice chair of the 2020 AHH/ACC guideline for hypertrophic cardiomyopathy management. Dr. Mital has a strong expertise in leading a multi-center collaborative research and serves on the NIH Pediatric Heart Network executive committee, as well as the American Heart Association Scientific Publishing committee and the AHA Strategic Outcome subcommittee.

Miguel Leal, MD (01:56):

In addition to Dr. Mital's expertise, today we will be joined by Dr. Shelley Miyamoto. Dr. Miyamoto is the director of all the cardiomyopathy program, and she is also a transplant cardiologist at Children's Hospital, Colorado, and a professor of pediatrics at the University of Colorado. Her research program is focused on understanding mechanisms of pediatric heart failure with the goal of identifying novel therapies that could improve patient outcomes.

Miguel Leal, MD (02:20):

Dr. Miyamoto is also the chair of the American Heart Association, Lifelong Congenital, Heart Disease and Heart Health in the Young Hearts Council. And she is a member of the medical advisory board for the Children's Cardiomyopathy Foundation.

Miguel Leal, MD (02:34):

Last but not least, we have Ms. Allison Conklin join us today. And we would like to say that these podcasts cannot be complete without a patient's perspective. And that is exactly what Allison will bring to us today. If you want to learn more about Allison, in addition to listening to herself, her life story and journey is described in the American Heart Association website, including a very powerful narrative of how Allison became a hypertrophic cardiomyopathy patient and her journey thus far.

Miguel Leal, MD (03:02):

So without further ado, we are going to start with a very important background scenario here, which is exactly the causes of this disease and focusing on the pediatric population. And I would love to have Dr. Miyamoto start us here by helping us understand what causes hypertrophic cardiomyopathy in children. Dr. Miyamoto, thank you for joining us today.

Shelley Miyamoto, MD (03:23):

Thank you for having me and thanks for organizing this podcast. This is such an important topic, and I think understanding differences between pediatric and adult hypertrophic cardiomyopathy is certainly important. I think I would start with just making sure everyone understands what hypertrophic cardiomyopathy is. And HCM is a disease resulting in abnormal growth of the heart muscle in the lower left-sided pumping chamber or the left ventricle of the heart. So the heart becomes very thick and it becomes stiff.

Shelley Miyamoto, MD (03:55):

There are different patterns of HCM with some individuals having thickening of only one portion of the heart muscle, for example, isolated to the septum or the wall between the right and left lower pumping chambers of the heart while other patients have what's called concentric or symmetric hypertrophic cardiomyopathy, where all of the walls of the left ventricle are equally thick. The thickened heart muscle can be diagnosed with an ultrasound of the heart, which is called an echocardiogram. So that's the most common way that HCM is diagnosed.

Shelley Miyamoto, MD (04:27):

There's a wide range of causes of hypertrophic cardiomyopathy in children. The most common cause of HCM in children is through genetic variations in genes of the sarcomere. The sarcomere is the building blocks of the heart muscle. The, building blocks of the contractile elements of the heart, but there are other genetic diseases such as Noonan syndrome and neuromuscular genetic diseases, such as Friedreich's ataxia that are also associated with hypertrophic cardiomyopathy.

Shelley Miyamoto, MD (04:55):

In addition, there are diseases of the body's metabolism or the ability of the body to process fats, sugars and proteins like Pompe disease, which can also cause HCM. So I just want to end this answer with making sure it's clear also that it's very important to rule out reversible causes of thickening of the heart muscle before giving someone the diagnosis of HCM. So things such as high blood pressure and other forms of curable congenital heart disease can also result in a thick heart muscle. So it's certainly very important to have a thorough evaluation for all causes of thick heart muscle before diagnosing someone with HCM.

Miguel Leal, MD (05:32):

Thank you very much, Dr. Miyamoto. This is a great way to start this podcast because it is a complex disease and sometimes it's heart to really differentiate it from other common conditions that we may be encounter in clinical practice, as you just mentioned.

Miguel Leal, MD (05:45):

And it is interesting when we appreciate the history of HCM, how genetics has become a major factor in the management and evaluation of this disease. Something that was not the case when it was first reported several decades ago. So on that note, I'd like to bring Dr. Mital to our conversation.

Miguel Leal, MD (06:01):

Dr. Mital in your opinion, as far as genetic screening and clinical screening is concerned who should be offered screening tools, both clinical and genetic, and when should these tools be applicable or be offered to patients and their families?

Seema Mital, MD (06:16):

Thank you, Dr. Leal. So an answer to your question. Clinical screening with echocardiography should be offered to any first degree relative of a patient who is first diagnosed with hypertrophic cardiomyopathy. And this is true of children as well.

Seema Mital, MD (06:29):

A child from an affected family should get a screening echocardiogram regardless of their age. And this is because hypertrophic cardiomyopathy can be passed from one generation to the next. But in addition to clinical screening, genetic testing should be offered to any patient who has been diagnosed with HCM. If their testing reveals a disease-causing mutation, then all the first degree relatives should be offered testing for that particular mutation. And if relatives carry the mutation, they will need ongoing echocardiographic screening.

Seema Mital, MD (07:02):

But if the relatives do not carry the mutation, they are not at risk for developing HCM and they don't need any further echocardiography. It is however critical to do careful pretest genetic counseling to discuss the risks and benefits of a positive result, as well as the implications of a negative result in order to help families and patients make an informed decision about whether they want to get tested and when they want to get tested.

Miguel Leal, MD (07:28):

Thank you very much, Dr. Mital. The topic of genetic screening in genetic counsel in general is approached in addition to this podcast and other discussions that the American Heart Association has spearheaded because it is something that it is finally receiving the adequate amount of attention that it deserves as we are noticing in both clinical practice guidelines and also scientific sessions in general. So thanks for bringing us your expertise on that topic as well. Now let's bring our third speaker today, Ms. Allison Conklin and Allison, after we heard our two experts, Dr. Mital and Dr. Miyamoto.

Miguel Leal, MD (07:59):

I like to hear from your perspective now as a patient who has the diagnosis, give us a brief narrative of your diagnostic pathway thus far, including from when things started, when you were first told that you had this condition to how much diagnosis and therapeutic efforts have been invested in your life story.

Alison Conklin (08:17):

So, I was born with a heart murmur in 1980. I was never tested along the way or anything. My mother had been diagnosed with hypertrophic cardiomyopathy when she was in college. So, I am assuming early twenties. And she had one of the first septal myectomy at hospital, and I believe it was Boston. And so, she went through that. It was very early. I don’t think there was as much testing and all of that stuff, but it was when I was about 12 or 13, I was really active in sports. I was on the basketball team. I was always like super competitive in gym class. And I started passing out in gym class. I remember one

time particularly I was playing hockey and I completely just passed out. And another time I was in basketball and I felt like I was hyperventilating and they just kind of like brushed it off and said, just take a second, you're probably just out of shape, like whatever it was never taken too seriously, which I find now as a mother with two children.

Alison Conklin (09:12):

I have a different approach of my own kids. But back then, I wasn't really looked at until I did eventually pass out in the middle of a basketball court. And my mom took me to the cardiologist, her cardiologist, and they did an echocardiogram. They did EKGs, all of that stuff. And I remember sitting there as a 13-year-old being told that I had the same heart disease as my mother and that I could no longer be in gym. I could no longer be involved in field day. I could no longer be part of the sports teams that I had been part of. And suddenly as a pretty sheltered 13-year-old being told I wasn't allowed to be in stuff that I loved.

Alison Conklin (09:47):

It was a huge awakening. That was in February of 1994. And in July of 1994, my mother and I were in the kitchen in together and she was talking and then suddenly said she did not feel well and just dropped to the ground. And at this point I was 14 cause I had a birthday in between the diagnosis and this moment. And I remember I had taken like a CPR class as part of a babysitting thing at a hospital. And I like ran over to her and I'm trying, I'm begging her to wake up and I'm trying to bring her back to life. I called my dad at work, and I called 911, but there was nothing to be done. She had gone into a deadly rhythm and in 94 ICDs pacemakers defibrillators, they were not as available as they would be in six years when I would have one implanted.

Alison Conklin (10:32):

So, I lost my mom that day and it was definitely hard to be diagnosed with something and then losing mom months later. So, in 2001, my cardiologist sat me down and was like, Hey, we have got this new technology. And because you have said in death, running in your family, would you be interested in having a defibrillator implanted? And I wasn't even like a question. I was just like, sure. Yes. Because they had said, if your mom had one, she should still be here. And so, when faced with that, even though it can be terrifying to have something implanted in your body and you are like suddenly a robot or whatever, but I said yes, and that was a huge thing. But then fast forward a few years, it saved my life. It saved my life twice. Now it happened about five years ago and it happened a couple months ago where my heart went into or something it was VF and I didn't even feel the device go off because I wasn't even here for it.

Alison Conklin (11:23):

And I was with my own kids at the time. And so, it was kind of that same situation that I was in with my mother, except because of the technology. I'm still here to talk about it. That's a huge testament I think what's important about the American Heart Association and just raising awareness because these are things that should be looked at. And even me being on a basketball team, I feel like all kids should be looked at prior to joining sports. It's so incredibly important. So, then it happened again in October of 2021.

Alison Conklin (11:53):

So, I'm really grateful for all of that stuff. And now it's been decades that I have been diagnosed with it. We are now talking heart failure, level C and we're having some pretty serious conversations with my own cardiologist at this point. But that's the story,

Miguel Leal, MD (12:11):

Allison, thank you so much for sharing that with us. It's not only super generous and courageous of you to expose how this disease and its trajectory has impacted your life and your families. But it certainly brings a unique perspective to our conversation today, as we are discussing this topic with colleagues and physicians, nurses, nurse practitioners, physicians, assistants, patients, and their families, and many other people that are the target audience of these podcasts, I think message like yours resonate sometimes far deeper than any clinical practice guideline that one might read on one occasion or another. So once again, thanks. We'll, we'll bring you back to this conversation in a few moments at this point, Dr. Miyamoto I would like to bring you back into our discussion, our podcast, after listening to Ms. Conklin and the outcomes that you shared both involving her mother herself. Let me ask you this, Dr. Miyamoto, what are the clinical outcomes in children with hypertrophic cardiomyopathy?

Miguel Leal, MD (13:01):

We sometimes understand that this disease may present differently at different stages of life. And when children are diagnosed, naturally, they will be living with this disease condition longer than if somebody were to be diagnosed at a much older age. So is the clinical profile of the disease different in children, as far as the clinical outcomes are concerned.

Shelley Miyamoto, MD (13:21):

I would like to acknowledge your courage, Allison, in telling that story. Podcasts like this, and the ability to raise awareness is so much more powerful when individuals like yourself are willing to share their own stories and really make this an understandable problem that people need to be aware of. And Dr. Mital and Dr. Leal and I can speak to these issues. But the honest truth is that your story will carry a much bigger impact. And there was a couple things you said in that story that are really important. And I think we'll get to some of them, but the idea that getting dizzy and passing out when participating in exercise as a child is we usually tell our patients that's just never okay. And we know that now and I think your story demonstrates how much we have learned over the past few decades about HCM and hypertrophic cardiomyopathy.

Shelley Miyamoto, MD (14:14):

These were things that weren't good as well. So, thanks again for telling your story for children with hypertrophic cardiomyopathy, the outcome is pretty variable. And a lot of it depends on the cause of the HCM and on the age of the diagnosis. And so unfortunately infants with hypertrophic cardiomyopathy tend to have a worse prognosis than children who are older teenagers, for example, that are diagnosed with the disease.

Shelley Miyamoto, MD (14:41):

And in part that is because many times infants born with hypertrophic cardiomyopathy are suffering from more systemic illness and diseases. So inborn errors of metabolism for example, are presenting in infants and have other medical problems associated. So it's not just isolated to hypertrophic cardiomyopathy, but that form of hypertrophic cardiomyopathy and infancy does tend to have a worse prognosis.

Shelley Miyamoto, MD (15:05):

When we consider all children with hypertrophic cardiomyopathy. However, the outcome is really quite good compared to other forms of childhood heart muscle diseases.

Shelley Miyamoto, MD (15:14):

So overall 90% of children with hypertrophic cardiomyopathy survive for many years after their diagnosis. When you compare that to patients with dilated cardiomyopathy, for example, the outcome is actually much worse with many more children having a poor outcome. So children with hypertrophic cardiomyopathy can do quite well and can live very, very full lives. For those children with hypertrophic cardiomyopathy that develop heart failure symptoms. If those symptoms can't be improved with medicines or surgery, then certainly heart transplantation can be an option for individuals. So I think it really is a disease process that individuals can live with for a long time, but it is variable.

Miguel Leal, MD (15:59):

Thank you, Dr. Miyamoto. Dr. Mital, in terms of the two most dramatic features of this disease for younger patients, which is the risk of sudden cardiac death, which again, so well illustrated in Allison's testimony, but also the impact act on activities. And in terms of the recommendations that we as clinicians have to offer based on the best available evidence to our patients and to their families. Could you please share your expertise with us in specifically in those two topics?

Miguel Leal, MD (16:24):

So how do we manage this intrinsic risk of sudden cardiac death or sudden cardiac arrest that the disease brings with itself and how can we counsel patients, families regarding activity levels?

Seema Mital, MD (16:35):

Thank you Dr.Leal. That's a really important question that both physicians and patients struggle with. It was moving to hear Allison's story. And as Allison described sudden cardiac death as a catastrophic event. The heart can stop beating because of a serious arrhythmia like ventricular fibrillation. And if the child or the adult is not resuscitated than electric shock in a timely manner, they can die.

Seema Mital, MD (16:57):

But thankfully, sudden cardiac death is still a relatively rare event and it is preventable by using an ICD or an implantable cardio defibrillator. If one can recognize the early warning signals of who is at high risk of having such an event, not to say that that is and challenging, but there are some early wanting indicators which include things like having previously suffered such an event, but from which one may have been resuscitated.

Seema Mital, MD (17:22):

An unexplained fainting episode, non-sustained ventricular arrhythmia on Holter monitoring, family history of a sudden cardiac death event, particularly if it's occurring in young age. Severe thickening of the heart muscle, the dilation of the left atrium, which is the pumping chamber on the upper side of the left heart being genotype positive and genetic testing, and also having scarring in the heart on cardiac MRI.

Seema Mital, MD (17:47):

So having one or more of these kind of risk factors does increase the risk of having a sudden cardiac death, a sudden arrhythmic event over time. And as I said, this is preventable with an ICD. And what is an ICD?

Seema Mital, MD (18:01):

An ICD is a pacemaker like device that sits inside or near the heart. And it shocks the heart out of the bad rhythm if and that rhythm occurs, which children should get an ICD is an important question. An ICD is definitely recommended for any child who previously experienced a sudden death event but from which they were lucky enough to be resuscitated, but they are still at high risk for future events. And this is called secondary prevention ICD, but an ICD should also be considered in those who have not yet had an event, but they are still at risk for having it in the future because they have multiple risk factors of the kind that I just described.

Seema Mital, MD (18:38):

In fact, there are now online tools like the one we recently published called PRIMaCY, where a physician can enter their patient's risk factor into the tool and calculate the risk of such an event happen for their patient and using that to counsel the family and the patient. However, when making a decision or offering ICD for primary prevention, sort of to prevent future occurrences.

Seema Mital, MD (19:00):

It is very important to weigh the risk of sudden death against the risk of complications from an ICD particularly because younger children can have a higher risk of CD complications like inappropriate shocks or lead fractures or infections. So obviously this is a life-saving intervention, but you do want to balance out the risk benefit of doing this.

Seema Mital, MD (19:21):

So what is very important is that physicians engage in what is called a shared decision making discussion with patients and families to empower the families and give them all the information they need to make the right decision for their child.

Seema Mital, MD (19:34):

In terms of the question about sports and activity. There has been this fear that exercise triggers arrhythmias and hypertrophic cardiomyopathy, but to what extent it does that is not really proven. It's very hard to actually prove that in the literature, as I said earlier, sudden death events are rare and these events can occur during exercise, but can occur at, even at rest.

Seema Mital, MD (19:57):

Exercise is a very important part of a heart healthy lifestyle. That for children with HCM, like any other child, should be encouraged to exercise based on their own exercise tolerance. And this includes recreational exercise. It can even include low end intensity, competitive sports, but one of the challenges we face are that many teenagers are engaged in moderate to high intensity competitive sports. When they are first diagnosed with HCM, pulling them out of competitive athletics can be very traumatic for them and poses a dilemma for physicians.

Seema Mital, MD (20:29):

So as per the recent 2020 ACC/AHA guidelines, it is very important that physicians do a full medical assessment. When they're asked to counsel their patients about participating in competitive sports, but it is also their responsibility to explain to the patient that there is a small, but real risk of a sudden cardiac event. But we do not know yet how much higher the risk is with strenuous exercise or whether, and not every patient who engages in this will have that kind of an event.

Seema Mital, MD (20:59):

So ultimately with all that information, the decision rests with the patient and their lifestyle choices, but regardless of the type of exercise, it is really important to emphasize the need for warm up and warm down before and after exercise to stay well hydrated during exercise, to stop exercising, if you develop symptoms like dizziness or chest pain or palpitations, and to ensure that there is an AED on the sporting arena and people trained to use it, especially for competitive sports participation.

Seema Mital, MD (21:28):

Finally, I just want to add one comment, which is that there are no restrictions to exercise to competitive athletics for those who are only genotype positive but have normal echocardiograms. That is they have not yet developed the disease. Thank you.

Miguel Leal, MD (21:42):

Well, thank you. Dr. Mital it just highlights how complex those conversations are. And it is actually refreshing to appreciate there is no specific right answer here, but there is opportunity for an honest discussion between clinicians and their patients, including the families when, especially when you talk about pediatric patients. So thanks for sharing that with us.

Miguel Leal, MD (21:59):

Dr. Miyamoto, I'm going to bring this back to you with a slightly different topic within the mega topic of HCM. We all heard from earlier descriptions of this disease is the acronym HOCM or H-O-C-M or which is the hypertrophic obstructive cardiomyopathy. So, we all know that not every hypertrophic cardiomyopathy patients suffers from obstruction to cardiac flow, but many do. And I'd like to hear from you, what is special about this variety of HCM? How does one treat the obstructive form of hypertrophic cardiomyopathy in the pediatric population?

Shelley Miyamoto, MD (22:31):

Yes. Obstructive hypertrophic cardiomyopathy is a form of hypertrophic cardiomyopathy where the flow of blood coming out of the left ventricle is blocked in some way. The blockage of this blood flow can be due to the thickness of the heart walls themselves, but it's also often related to abnormal motion of one of the valves on the left side of the heart called the mitral valve.

Shelley Miyamoto, MD (22:53):

And so, the combination of that, the heart muscle and abnormal function of this valve can cause difficulty in the blood trying to exit the left ventricle. And as you said, not everyone with hypertrophic cardiomyopathy has this obstructive form, but many do. And the therapy for obstructive hypertrophic cardiomyopathy really is dependent on symptoms for the most part. So individuals with obstructive hypertrophic cardiomyopathy who have symptoms, whether that's shortness of breath, difficulty keeping up with their peers, chest pain, those types of patients can benefit from medical therapy.

Shelley Miyamoto, MD (23:29):

And there are more medicines that can be used, but the most common are beta blockers or calcium channel blockers. If in a patient who has obstructive hypertrophic cardiomyopathy, their symptoms persist, despite those medical therapies, we often well begin to have discussions about surgery, and it sounds like Alison's mother had a surgery called a myectomy, and that same type of surgery is done today in patients with obstructive hypertrophic cardiomyopathy has symptoms despite attempts at medical management.

Shelley Miyamoto, MD (23:59):

And so we do septal myectomy in children with hypertrophic cardiomyopathy. Where the surgeons remove part of that really thick heart muscle to make it easier for blood to flow out of the heart. So there are surgical options and medical options to treat obstructive hypertrophic cardiomyopathy, but both of those treatments really are reserved for patients with symptoms.

Miguel Leal, MD (24:20):

Oh, thank you for that, Dr. Miyamoto and Alison let's bring it back to the conversation here. If they're inspiring us with your first take on your journey, if you could give us a little perspective on what are the challenges that a hypertrophic cardiomyopathy patient is possibly going to encounter along the way, in terms of compliance to propose therapies or how you felt when the risks and benefits were being discussed with you in terms of a defibrillator implant or the medical therapies that you have been proposed. And you also mentioned that you are now looking at other very high level conversations with your clinicians regarding future steps.

Miguel Leal, MD (24:58):

So a lot of the listeners to this podcast are clinicians, but many are also patients. What message do you have for them not only sharing your personal perspective, but also with the learning that you have acquired, the knowledge you have acquired over the years dedicating your life to this cause?

Alison Conklin (25:14):

That's a great question. This month will be my four years since I had my septal myectomy, which I had in Boston as well. And I think just over my lifetime of growing up with this disease, losing a parent to this disease, it's been something that is definitely part of my every day because I take handfuls of pills in the morning and handfuls of pills in the afternoon, but it allows me a perspective of just appreciation to life and mundane days that might seem boring consecutively to someone else where I'm just thank you for this beautiful day where nothing is going crazily wrong. And that might sound a little like woo woo. But to me, it is really scary. The first time that my device went off, I was taking my son to get braces. I was at an orthodontist office. The second time it went off, I was doing laundry.

Alison Conklin (26:06):

It's definitely scary that there's something in my body. I have no control over. I can follow the rules. I can do the things that are given to me as guidelines, but essentially, I have no power over it, but I do in the way that I think about it. And I hope that makes sense.

Alison Conklin (26:21):

It's because we all can have these situations, be it terrible, but it's really, truly just the way choose to look at it. And I'm 41 now. And I have beautiful children who luckily enough, did not get the gene. And I consider myself honestly, very blessed do I wish my mom was still here. Of course, but I'm so grateful that there is technology and people like you, all that are putting the knowledge out there. And there are guidelines that have changed. And as Dr. Mital shared that you can now, like they maybe wouldn't have pulled me out of all sports back then in the nineties.

Alison Conklin (26:54):

Things are changing and it's for the better. And we never know what new technology is around the bench. I mean, obviously at this point with the heart failure that I'm in, we're talking in transplant. So, I know that's probably something that's going to become more real in my future. Hopefully not for, I would love it for not another couple decades, but there's also the hope of new medications. So, there's always something that's going to come around the bend. And I feel like there's always so many smart people out there working really hard on trying to make our lives easier. So as a patient, I think is really important not to get bogged down with the idea of right now, because you have no idea what people are working on and all the things that are kind of come out in the future.

Miguel Leal, MD (27:35):

Well, thank you so much. It's a perfect segue into my next question, which I'll bring Dr. Mital to the conversation. Alison has just mentioned the importance of discovery in our field, both in terms of surgical procedures. Procedures such as defibrillator implants, and also medical therapies as well. So Dr. Mital as we appreciate the historical evolution of treating hypertrophic cardiomyopathy patients. What can we look forward to as far as the future? Are there any emerging strategies for monitoring and for treating patients, especially children with hypertrophic cardiomyopathy, please share with us what you know, in terms of the latest and hopefully the greatest on this topic.

Seema Mital, MD (28:12):

Yeah. Thank you. What a great attitude Alison, toward something like cardiomyopathy condition. These are exciting times because there are a lot of new things on the horizon. There are many, I will just give you a few examples. For example, we are learning more about how to use genetic information to identify which patients are at higher risk for complications and maybe should receive an ICD or other interventions early.

Seema Mital, MD (28:36):

There are new monitoring devices like smart wearables and phone apps, which can be used to monitor heart rhythm more closely and recognize early warning signals. And there is new ICD technology like subcutaneous ICDs that don't require a surgery or a catheterization procedure. Not all of these are available for children, but the hope is that they will be in over time.

Seema Mital, MD (28:57):

There's also a lot of excitement about new drugs. Like mavacamten that act directly on the protein in the heart muscle that causes the disease. Again, these drugs have not yet been tested or approved in children. Even the early results in adults do look promising. And for some of the most severe cases of hypertrophic cardiomyopathy, like those caused by multiple mutations and myosin binding protein C3, clinical trials of gene therapy are beginning.

Seema Mital, MD (29:24):

So the future is bright as Alison and said, and as Dr. Miyamoto said earlier, the majority of patients can and will live a healthy life. And for those who do go on to have complications, new therapies are on the way.

Miguel Leal, MD (29:37):

Thank you, Dr. Mital. I think this is a perfect way to close. What has been a very insightful and certainly informative podcast. It is important for all of us to appreciate that the pediatric portion of patients with hypertrophic cardiomyopathy is a sizable amount of patients in a number that keeps growing. The more that we learn about screening mechanisms and diagnostic tools, it is also very encouraging to listen to a patient like Alison. To bring such a powerful optimistic message, a message of hope, a message of strength from somebody who literally has lived the consequences of a very complex condition that we keep working on. And there is a lot of research and invested knowledge that experts like Dr. Mital and Dr. Miyamoto who joined us today have been involved with. And it's really a privilege for all of us to listen to both of them, as well as to Miss Conklin.

Miguel Leal, MD (30:24):

And I would like to thank all three of them for their participation in today's podcast. Thank you all for participating in this podcast with me today, when a lot of important points about pediatric hypertrophic cardiomyopathy were made. This podcast is a part of the American heart association, hypertrophic cardiomyopathy initiative, which is sponsored by a Bristol Myers squib.

Miguel Leal, MD (30:45):

In closing, I would like to remind everyone who is listening to encourage your patients to play an active role in their medical care, by advocating for themselves and their family members to get any additional information. You have the option of visiting the American heart Association's hypertrophic cardiomyopathy websites for more education. Once again, thank you very much. And we'll hope to see you all in future podcasts.