The Lead in Peds

Transcript: Season 1, Episode 5 – Mission Critical: The Importance of Pediatric Research

Host: Dr. Nathan Kupperman
Guests: Dr. Catherine Bollard & Dr. Sallie Permar

Dr. Nathan Kupperman (00:00):
The quality of care we deliver to children in the United States and around the globe is directly affected by the quality and quantity of research we conduct. And the big impactful research that we have accomplished in this country so far has mainly been supported by the United States Federal Government. It has kept our country at the pinnacle of innovation and has provided us the ability to deliver expert care to the most vulnerable in our society, our children. Welcome to The Lead in Peds. I'm Dr. Nate Kuppermann, Chair of Pediatrics and Chief Academic Officer at Children's National in Washington, DC. Today, pediatric research is under attack. We're seeing proposed and in-progress cuts to federal funding that directly impact pediatric research, especially in areas like rare diseases, childhood cancer, emergency care, and long-term developmental studies. These aren't just numbers on a spreadsheet. They represent missed breakthroughs, delayed treatments, and lost opportunities to improve and to save children's lives.

(01:02):
Today I'm honored to welcome my colleague Dr. Catherine Bollard, Chief Research Officer at Children's National and Director of the Center for Cancer and Immunology Research. She's a global leader in pediatric immunotherapy whose work is driving some of the most innovative treatments in childhood cancer and immune system disorders. Later in the show, we'll hear from Dr. Sallie Permar, Chair of Pediatrics at Weill Cornell Medicine and one of the nation's foremost experts in maternal and infant immunity. Her research has been critical in advancing our understanding of how to protect newborns from serious infections. She's also a leader in training the next generation of researchers, which is now at risk due to proposed cuts to pediatric research funding at the NIH.

Welcome Dr. Bollard. Or I'm going to call you Cath cause on the show we tend to use first names. So, I'll call you Cath and you can call me Nate. And before I start, I want to ask you a question since we're both in the same line of work, that is, we're clinician scientists and we are facing the struggles of chasing our next research dollar. What I'd like to ask you first is how do you decompress? How do you sort of take the stress off each day before you come to work?

Dr. Catherine Bollard (02:21):
I mean, great question, Nate. will say that the pandemic really taught me the importance of taking care of oneself. So, self-care is really something that is now really important to me. And as a cancer doctor, I'm aware that it is really important to take care of your body and your mind. So, I've been really focused a lot on ensuring we do the exercise, we do the mindfulness, we do the yoga, but I do like to keep my mind active, and I know you and I share a bit of a passion for the New York Times games. So, I will say that I do use that also as a stress release every morning, and I have to have a lot of tools in my toolbox these days. That is for sure.

Dr. Nathan Kupperman (03:07):
It's interesting, Cath, because first of all, I share the concept that we all need a lot of tools to keep ourselves balanced. We share some of the tools. I start my day with, again, the New York Times gamut of games, and I do a little bit of mindfulness, thankfuls in the morning. And then I have to end my day with a round of Colbert and Jimmy Kimmel, and then I'm in a good state anyway. So anyway, it's important to think about these things as we pursue our stressful careers. But today we're going to talk about the research infrastructure, the importance of pediatric research in this country. And we know that pediatric research is fundamental to elevating the care of children in this country, providing hope to families where hope is much needed and it's being threatened, it's being threatened by cuts to our federal funding, bureaucracy, et cetera. So, I'd like to ask you first, what are you seeing day to day with regards to that and what are your patients saying and what are your first general thoughts about that?

Dr. Catherine Bollard (04:09):
So, I think it's important to know where I came from. I came to this country from New Zealand because it offers hope to patients who had no hope. And I came to this country because a friend of mine from high school died of her cancer. But what was worse, she didn't die of her cancer. She died of a second cancer from all the treatment she'd received. And so, I came to this country because I knew we could do a better job to develop new therapies that could only kill the cancer cells and not healthy cells. And I've enjoyed over 20 years of really amazing funding, especially from the federal government that has truly impacted children's lives. And I know that children are alive today because of the therapies that we've developed. But where we are right now is that it is really hard to obtain additional funding.

(05:11):
I know that we have clinical trials going on right now and that if we don't get additional funding, we may be turning patients away. And this is obviously completely heartbreaking to me because we are seeing these successes in the clinic and if we can't finish the work we started and we can't really impact on the lives of the patients we serve, this will be devastating on so many levels. And it's particularly poignant for me today because I just literally received an email from one of the patient advocates who works with us on developing these new clinical trials using our

immune system to kill cancer. And he told me today that this would have been his son's 22nd birthday and my oldest daughter is also the same age, 22, and he knows that his son would be alive today if he could have had access to these really amazing cutting-edge therapies that we and others are developing. And so, all of that is under threat with this loss of federal funding.

Dr. Nathan Kupperman (06:22):
It is a sad irony that we're in an era of such tremendous breakthroughs in therapies and you look to the next decade and its sort of incredible the potential of what we have to do. And this is now when we are facing these really important challenges. And one of the things that I struggle with is that if you look at the federal funding for research in the care of adults versus research in the care of children, for children, it's much less and much less even than the proportionate number of children in our country. In fact, about 22% of our country is children, but NIH funding, it's five to 10%. So, this gap is an important gap. How do you explain that? What are your thoughts about that gap and what are the consequences for child health?

Dr. Catherine Bollard (07:14):
I can specifically talk about this through the National Cancer Institute because you are correct. It is around about 5% of funds are given to specifically to pediatric cancer research. And the reason is really because the numbers that pediatric cancer and many pediatric diseases are considered rare diseases. So, we struggle with, well, are you going to have the biggest impact for the greatest number of patients because you're dealing with a rare disease? Whereas our argument as pediatric physician scientists is that you are investing in the child's lifetime. So, this is, while there's small numbers, you are going to be impacting a huge number if you look at the overall years saved from these children who we treat every day.

Dr. Nathan Kupperman (08:07):
When I go and advocate in Congress, I always point that out that is that you're not comparing apples and oranges as you save a five-year-old. You're saving 85 potential years of life. And so really true. I want to talk a little bit about clinical trials. As you know, it's funny, we do very complimentary types of research. You're a translational researcher. I'm more of a clinical epi and clinical trialist, but we both do clinical trials. It's interesting in that spectrum that we talk about a lot from bench to bedside. The clinical trial is just a fundamental piece, right? Taking those initial discoveries, then doing the trials to know whether we can really turn them into treatments and it's a fragile ecosystem, that particular piece. Just want to get your thoughts about, again, these federal funding challenges, particularly on the critical trials infrastructure.

Dr. Catherine Bollard (08:59):
Yes. I mean, I think this is really important to emphasize that there's a lot of funding that goes to the hidden importance of what we need to support these really complex bench to bedside clinical trials. And while it seems intuitive just to fund the actual research per se, if you don't have all

that infrastructure built into that grant, then it's just not possible to implement. We're not just treating cancer in a test tube. We are really truly moving these discoveries from bench to bedside. And all of that is, as you say, under threat right now because the infrastructure is as important as the individual research projects.

Dr. Nathan Kupperman (09:53):
How would you encapsulate what's at stake if we really lose funding for that clinical trials infrastructure? And how does that impact, I mean your families, families of the patients who you treat, do they come to you asking for clinical trials? How would you summarize the impact?

Dr. Catherine Bollard (10:12):
Children's lives will be lost. That is pure and simple. I get emails and calls from patients from all over the country every day, other doctors in the country every day asking for access to these novel therapies that we have developed. And that's what's at stake here. And we know we have saved children's lives from the therapies that we have developed through NIH funding. And if that is lost, we know that we can no longer save these children who have lost all other hope for other standard of care medicines.

Dr. Nathan Kupperman (10:54):
Wow. Cath. That was a really important statement of why clinical trials are so important, poignant and very powerful. But even as we push the boundaries of treatment, we must also focus on building up the next generation of pediatric researchers. We're thrilled to be joined by Dr. Sallie Permar, chair of pediatrics at Weill Cornell Medicine, who's dedicated her career to safeguarding newborns from life-threatening infections through maternal and infant immunity research. She also has directed the now terminated NICHD Pediatric Scientist Development Program for the past seven years. So, Dr. Permar, welcome to the show.

Dr. Sallie Permar (11:36):
Thank you for having me.

Dr. Nathan Kupperman (11:37):
I think the last time I saw you, you poured a bucket of ice water on my head at a meeting in Hawaii. It was the ice water challenge for youth mental health awareness. How did that feel for you?

Dr. Sallie Permar (11:52):
Well, that's right, Nate. I think it really shows how far you and I both will go to advocate for children and their health. And you were lucky we were in Hawaii with that ice bucket, I will say.

Dr. Nathan Kupperman (12:06):

It's a very good point. I would have normally perforated something after that, but it was in Hawaii, and it was for a super great cause, as you know, for child mental health. So anyway, great to have you here. And as you've been hearing, we've been talking about the challenges that we're all facing as physician scientists with the federal funding bureaucracy and as a means of introduction, I'd like you to go ahead and just tell us a bit about your research and separately about the research training program that you've led for seven years at the NIH.

Dr. Sallie Permar (12:40):
I am a pediatric infectious disease doctor and a researcher, and I got excited to be in the field that I'm in today when one of our former pandemics that's still ongoing today, I was introduced to the HIV pandemic when I was in college and I got an opportunity to travel to a part of the world that was being severely impacted in Sub-Saharan Africa. And I thought, how does a place come back from such a devastating epidemic that was really devastating, the adult generation killing the parents of young children and then children were also becoming infected and even dying. And so, I thought right then I think what would turn this pandemic around would be a vaccine. And so, I went to work on that. And being a physician scientist is the way that you best know what are the most important conditions that are facing children and families and health at large, and what are the solutions that are going to work?

(13:43):
So, I've worked on the HIV vaccine, I've worked on the Zika vaccine, the cytomegalovirus vaccine, anything that will create health from the very first start of life is what I'm interested in. That has really led to me also feeling it is really important to bring up the next generation of scientists and of pediatrician scientists and like you all just spoke about how important children's health is to the rest of health, the rest of our lives, our population at large, the biggest impact on your health for life is in the first few years of life really what your trajectory of health will be. So, I got excited about bringing in the next generation of pediatricians who would do research and to train that group because it's not an easy transition to go from being an all clinician to also taking on the craft of research and doing it well and getting it funded as you've already talked about.

(14:44):
And so, I got the opportunity to lead the nation's most coveted program around training pediatricians to be scientists. It really was the honor of my career to lead that program. It existed for 40 years, started by the department chairs of pediatrics 40 years ago, was funded by the National Institute of Child Health and Development and has had major success in starting many highly respectable and successful careers of pediatricians who do research, over 270 have been trained on the program. The alumni of the program fill the leadership of many parts of the pediatric field, but also other research fields like cancer immunology. They've been secretaries of health, they lead biotech, they lead many of the organizations that are tasked with our health all

because of this program. And unfortunately, it was terminated earlier this year by the new priorities of the NIH and our federal government.

Dr. Catherine Bollard (15:48):
So, Sallie, I share your passion for the growth and development of our next generation of pediatric physician scientists. I think we both agree they're almost a dying breed and the PSDP was so instrumental in really bolstering and growing that very, very important and talented group. So, with the loss of the PSDP and all the other funding challenges that we've discussed, how do you see there's a pipeline growing forward? How can we preserve that critical talent and really make sure that we have that pool of creative amazing physician scientists? Because honestly, from my point of view, at the end of the day, what's at stake is the patients we serve.

Dr. Sallie Permar (16:42):
In order to create that health to make sure that our children are as healthy as they can be to combat some of the chronic diseases that do start in childhood, like mental health concerns like obesity. We have to have great pediatrician scientists. And so, it really is in concert with many of the priorities of all our leadership in health want as well is having a workforce that is most well suited to take that on. And so, I should say that while the federal funding of the pediatric scientist development program was the backbone and really provided two thirds of the slots that we fund about eight or so scholars every year, it's highly competitive. We have been able to continue it on some level because we have a number of co-funding organizations like the American Academy of Pediatrics, like the American Pediatric Society, like the March of Dimes, like the Burroughs Wellcome Foundation.

(17:41):
So, there are other types of foundation, some of our private support that went alongside the public funding. And then our institutions, our institutions like our hospitals, our medical schools, they are co-funders in this work as well. However, what happens if you don't have that funding stream to preserve the time of these pediatricians training to be researchers is the pressures that most physicians feel. They will also start to feel that you need to see more patients because that's how our healthcare system runs is on a volume based for the most part. So, if we have those pediatricians who we've already invested in to be researchers, if they can't continue their work, they need to go see more patients with their time. And that is a disservice to all of us because while the patients that they're seeing at that time will be served, we won't be serving the next generation, the next need, the next cure, the next discovery, the next disease that we can eliminate from our country, from our planet, from our memories, is lost when we don't provide the time that it takes to really train our researchers out of a physician population.

Dr. Nathan Kupperman (18:56):

I just want to highlight for our listeners, there is one other level of challenge for us as pediatrician scientists is that as we all know, there are fewer medical students entering the field of pediatrics. In fact, this past year, a year ago, one third of the pediatric residency training programs in the country did not fill sort of unheard of, and there's a lot of potential reasons, but the obvious reason is that physicians who care for children earn substantially less than physicians who care for adults. Again, this needs to be fixed at a federal level, and then it's from that pool that has already somewhat dwindled that we find the physician pediatrician researchers that we need to train into careers around pediatric research. So, I don't want to have our listeners just feel doom and gloom, but these are just the realistic challenges that we face. So now with that as a backdrop, I want to turn to both of you and to say, okay, let's look at solutions. What gives us hope and what should our institutions, policy makers and even the public be doing to help us as a nation really combat this situation that could really lead to adverse consequences for our children?

Dr. Catherine Bollard (20:19):
I think philanthropy is going to play a major role. We are already, many of us in the research community are getting calls from grateful parents, philanthropists who want to see how they can fill the gap. So that's really unbelievable in terms of that sort of impact on our patients, I think we are going to have to develop more creative partnerships with industry. The same problem that we were talking about with rare diseases does require some creative partnerships, but it has been done, and I know that there is certainly interest in different areas. So, I think we just need to really explore everything. And then I think we really need to appreciate the power of advocacy. And Nate, you are a terrific advocate. I know Sallie and you are really making great strides in this area. But also, our patients, they are incredible advocates for the work that we are doing, the work that has saved their lives. And I think as a team, we're stronger together. So really, pulling all these resources together. I know you want to end on a positive note, Nate, but the bottom line is, without the federal support, I think we won't be the powerhouse that we used to be. So, while we are going to be engaging all these other avenues and options, I think we should also remember and be mindful that we will need federal funding to really be able to make the impact that we so desperately want to impact.

Dr. Nathan Kupperman (22:09):
Absolutely. Sallie, how about you? What are your thoughts on that?

Dr. Sallie Permar (22:12):
Cath couldn't have said it better. All of those avenues we're all thinking about, we're thinking about redefining what we're doing so that it can be most marketable to the next way that we're going to fund research. So, we're all pivoting now, but we are advocating because like Cath said, this is going to impact lives, children's lives, every single one of our communities. It's economics, its research, provides more dollars into the community than it requires, its jobs. And

it's the innovation that makes it so that when you have a devastating diagnosis, you have a great hospital to take your child to or your loved one to that will have the unique therapy that you won't get at just any hospital, or you wouldn't be able to get in other countries. We're losing that opportunity. So, my solution is also we have to train the next generation of advocators.

(23:13):
So, that wasn't a thing that I really learned in going through all of my training, but that is something I'm thinking about right now with all of the training that we're already doing of the next generation of pediatricians and researchers, is to weave in the advocacy from the very beginning and the communicating about science, communicating why it's so important, what we do as researchers and physicians, to not only the lives of the patients we're treating, but lives of the community and of our country. So, I think we will all be different after this time and where we end up with the investments that the US will make. But I can't agree with Cath more that it has to include publicly funded research for us to remain at the cutting edge of health and innovation.

Dr. Catherine Bollard (24:01):
And Nate, can I just say, I think because Sallie and I are completely aligned on all this, but the bottom line is research gives hope to patients, to many patients where hope has generally been lost and there's no economic value you can give to that. So, at the end of the day, this is why we're all here.

Dr. Nathan Kupperman (24:24):
You both are super insightful and super articulate about these critical issues. So, what I'd like to do is just put a little brief summary around what we've talked about, and I'm going to give my line of hope, innovation and research in pediatrics is really at risk. We've already seen the partial dismantling of the research infrastructure and that risk not only innovation and research, but it critically also risks the next generation of investigators, that we've heard. And both would have tremendously adverse consequences, not only just for health care, but for our society at large because we're a society of innovators. And I'll just remind the listeners, as I said earlier, children are 22% of our population, but they're a hundred percent of our future. They're our future doctors, lawyers, military, engineers, teachers, they're everything. So, we all communally should be very invested in reversing this trend. And what I will put out there to our listeners and my thoughts just echoing what both of you have said, one of the great roles of social media and a podcast like this is to try to educate the population. And it's really important that I think the world and the country are aware of what's happening. And really everyone should be advocates for the future of our children. And with that, I just really want to thank both of you for joining me in the podcast today. Super enlightening, two powerhouse leaders of pediatric research. I really appreciate it.

Dr. Catherine Bollard (25:59):
Thank you, Nate. It was an honor to do this with you today. And thank you, Sallie. It was an honor to be here with you too.

Dr. Sallie Permar (26:05):
Thank you. My only disappointment is that I wasn't there in that cool studio with you all on this The Lead in Peds

Dr. Nathan Kupperman (26:12):
Cath, Sallie, thank you both again for your time and for joining me for this important conversation. Pediatric research is often the only hope for children and their families facing devastating diagnoses. Investment in pediatric research is an investment in the future of our country. Healthier children become healthier adults, reducing the burden on our healthcare system and strengthening our communities. And thank you to our listeners for joining this episode of The Lead in Peds. Please subscribe to our show wherever you get your podcasts and learn about what we're doing to raise the bar of care for all children here at Children's National and around the world.

** This podcast has been edited for clarity. Some content may have been altered to enhance the listening experience. **