



BUILDING A SUSTAINABLE ECOSYSTEM FOR PATIENT- CENTERED PHILANTHROPY IN MEDICAL RESEARCH

Announcer 00:04

Please welcome the panel on “Building a Sustainable Ecosystem for Patient-Centered Philanthropy in Medical Research,” moderated by Dougal Edwards, founder, Bright Arena.

Dougal Edwards 00:41

Good morning, everyone. It's great to see a few of you here for this morning. We're here obviously to talk about other sides of finance, much more around philanthropic giving. My name's Dougal Edwards, as was just introduced. I've spent the last 15 years focusing on repositioning research for investment, and a lot of that's basically through philanthropic and families and foundations. And today, we're going to take a moment really to start to look at how might we actually build sustainable, patient-centered philanthropic model for medical research. And whilst I won't introduce everyone individually on the stage today, I'd encourage you to have a look at their profiles online, because we basically have the whole value chain covered today. And I'm hoping within the half hour, 40 minutes that we have together, that we have the opportunity to at least go right the way from discovery through to access. And if there are any questions, maybe save them for the end. It'd be great to have a bit of a dialogue with yourselves in the audience. So, I'd like to start looking at philanthropy and medical research as a space that really does bring capital and patients together, with a sense of urgency. But very occasionally do we actually start to think about what that role of philanthropy could be, and what are the valleys of death that we as a philanthropic family could actually try to solve. And for me, one of the things that always brings me back to—whenever I'm talking to someone from a family office or from a philanthropic entity—is where do you see the gap and the value for you? And I know from David, our conversations, I'd be particularly interested to see where do you see philanthropy sitting in that value chain?

David Panzirer 02:36

First, I want to thank Milken for having me. My name's David Panzire. I'm a trustee of the Helmsley Trust, but more importantly, I'm a parent. I have three children. Two of my three children were diagnosed with Type 1 diabetes a decade apart. Just to level set, Type 1 is an autoimmune disease where your body kills its own insulin-producing cells. Insulin's a hormone that allows you to metabolize carbs. Without it, you die, period, full stop. It has nothing to do with lifestyle. It is an autoimmune disease. So, philanthropy is really my third career. I really fell into this, in that five months after my first daughter was diagnosed in 2007, I became a trustee of what's now an almost \$8 billion trust. Except I knew absolutely nothing about how to get a drug or a device or a therapy to market. I barely knew how to take care of my daughter, to be honest. So, very long story short, I traveled and learned, and I now have a team of 19 people that do nothing but Type 1 at the trust. We've invested over \$1.5 billion to date in Type 1 diabetes. And to answer the very specific question, we see the valley of death literally from academia or start-up all the way to phase 2. The big money is not coming in until phase 2 trials, and our ultimate goal is—whether it's philanthropy or nonprofit dollars—de-risk it, get to the big money. This is not a millions problem, it is a billions problem, and we don't have enough to solve. So our job is to really get to big pharma and the big VC monies. Basically, it's a whole continuum, unfortunately, from start-up down to phase 2.

Dougal Edwards 04:18

But it's a gap that's been growing over this period. Over the last 10, 15 years, that gap's just getting bigger. But from your perspective, Durhane, you see it from a different angle?

Durhane Wong-Rieger 04:27

Yes. Hi, I'm Durhane Wong-Rieger, and really want to thank everyone for the opportunity to be here. I'm based in Canada, so I head up the Canadian Organization for Rare Disorders there, but I'm really here in my capacity as the president also of the Asia Pacific Alliance of Rare Disease Organisations. And yes, I think as you were saying, I have two children, both born with rare conditions. There's no official definition of a rare condition, but it's usually one that affects small numbers of people. In Europe, it's less than one in 2,000. But really, there are about 7,000 to 10,000 rare conditions, and I think that's the huge challenge that we've got. And as you can imagine—you're talking about Type 1 diabetes—we're trying to do 9,999 additional conditions as well, not nearly as populous. And the challenge we have also, though—and you talk about the valley of death—I think especially when we're talking about Asia Pacific, and we're talking about the range, obviously from large countries like Australia and Japan, where we'll be this year for our conference, but also many of the lower-middle-income countries. And as I always say, the valley of death really is, for patients, is all the way through. Even if you get into the clinical trials, which is a miracle, even if you get a drug that comes to market, which is another miracle, and those are big gaps. Really what we would like to see more in terms of philanthropy thinking about is the whole patient journey. So, not just at the point of getting the therapy, but what do we do about making sure it gets to the patient? And as we all know, the worst drug that there is is one that never gets to the patient.

Dougal Edwards 06:14

That's quite a powerful message. And in fact, in Australia, I had a conversation just yesterday with big pharma, that sometimes only 20 percent of generally the number of accepted medications only makes it onto PBS in Australia. We've even got that problem, even in these rather large populations as well, such as Australia.

Durhane Wong-Rieger 06:37

Just a quick follow-up, and then we can move on. Of those 20 percent that get into the PBS, probably only about 20 percent of the eligible patients actually get access to that medicine. So, we can talk a little bit about some of the challenges from an access point of view, which is not just pricing.

Dougal Edwards 06:56

Mm. Thank you. Chris, hearing what David was saying there around the academic, the academia, the startup side of discovery, what's your perspective on that, and where do you see the value for philanthropy?

Christopher Laing 07:10

Yeah. So, I guess first off, I thank you. I'm Chris Laing. I'm vice dean for innovation and entrepreneurship at Duke-NUS, and thanks for having me in this conversation. My role at Duke-NUS is to oversee our commercialization programming. So, partnerships with industry, tech transfer sits inside my office. We do a lot of thinking about this, right? And Duke-NUS is a research-intensive medical school. Effectively, our faculty are discovery scientists, they're biologists. They discover new targets for drugs. They validate those targets. But we do not have the capability—most universities do not have the capability in-house—to develop something to the point where it's manufacturable or where it can be produced at scale and delivered to patients. And so we rely heavily on partnerships to do that. And most of the time, our partnerships are with industry, right? And our ultimate goal is how do we partner with industry that has those capabilities? And so, actually, I resonate very strongly with David's view, that for us, the gap typically sits between the validation of a target or maybe the early validation of a drug prototype through to the getting into the clinic, which is when we start to see industry interest and investor interest in those discoveries. And we rely very heavily, of course, on government support for discovery science. But typically—and actually Singapore does a very good job of this—but typically globally, government support ends at the discovery stage, and it doesn't typically take assets much further. Now, as I said, Singapore actually has a few very interesting programs that help us to bridge some of that gap. But our gap sits very firmly, I think, in the space that David talked about, which is: how do we bridge that gap to get interest from the commercialization engine?

Dougal Edwards 09:26

There was a golden age for government support, sort of in the '80s, where grant success rate for those academic researchers would have been almost 30 percent, one in three. Those numbers now are like one in 10, one in 12, so it's more like a lottery.

Christopher Laing 09:41

Yeah. Well, actually, you're raising a really interesting challenge—an increasing challenge—and it's one of momentum. And I think it's really interesting to have this group of people on the panel here, because universities and scientists are a community of largely project-focused teams, and those teams come together typically around a project when they have funding to do something. But when you get gaps in that funding, those teams disperse, and it can become very difficult then to bring those teams back. And so the importance of the continuum of funding, all the way from the beginning through to the end, has become increasingly relevant, I think, as these grants have become sort of less available or less accessible to scientists.

Dougal Edwards 10:33

You actually identify something that, Fook Kay, this is a space that you have explored extensively through Temasek, around the Health Collaborative. I wonder if you could sort of talk about maybe how that helps solve, or where that fits within the valley.

Fook Kay Lee 10:48

Thank you very much. I think three months ago, we decided to set up the Health Collaborative within the Temasek Trust because of the experience that we have with foundation. And indeed, there are many valleys that we need to cross. So, what the Temasek Trust Health Collaborative intends to do is to bring together the four Ps, very important: philanthropies—that one gives a lot of support—and the public and the private and the people together, to look at how we could actually bring as much of this good innovations into the people, and to benefit the people. So, I would like to talk about three examples. I think it's very good to have examples. Two years ago, we actually launched the Dynamic Resilience program with Wellcome Trust, and that's very much patient-centered research because it looks at how a patient reacts after a certain medical event, an extreme medical event. It could be a chemotherapy, it could be a severe infection, it could be a severe accident. And what are the factors that will promote recovery? And what are the factors that will actually cause more severe frailty in the person? So, these interventions that are necessary will be brought to the patients to ensure that they have this good recovery and continue with their well-being of life. The program is still currently ongoing, but what is so significant about this particular program is that it's very patient-centered, right? So, as much as possible, it will give that kind of outcomes to patients. And the second example I want to give is actually on a cancer treatment, which is based on ultrasound. It's basically at a pilot stage. And together with the Li Ka Shing Foundation, about a year ago, we brought it into Singapore, and worked with the national cancer institutes and cancer centers to actually look at how we could promote patient treatment with this very innovative solution, which is non-invasive. So, already, just over a few months, 10 patients have actually benefited from this kind of treatment. And finally, I would like to talk about how philanthropy can bring about more support from the health sectors from the region. So, in terms of looking at One Health, One Health is important because it impacts the whole life. It impacts from minus one, as we all know, to plus 100. So, how do we then bring about all these good surveillance programs together to give medical evidence to the health sectors, that these are the interventions that are necessary? And elevating the solutions from the valley of death into good outcomes for people. Thank you.

Dougal Edwards 13:48

Thank you, Fook Kay. I'm quite curious. With the recognition that there's multiple valleys of death, and actually you said there's one big valley of death. Where—other than obviously you have a lived experience within your families, and I'm thinking specifically Durhane and David. How do you drive urgency and alignment in philanthropy to get behind these missions? I've seen over the last 15 years of having spent this time with researchers and repositioning the funding, it's the same problem on the other side. And I just wonder, any examples of how you've been able to do that? Or is it still a struggle?

David Panzirer 14:25

Well, if you're asking me, I'd say it's somewhere in the middle. I think it's still a struggle. We have found generally if we try and get others to play with us—there's the old saying: if you ask for money, you're going to get advice. You're very rarely going to get money. So we have kind of took it upon ourselves that if we have an idea, we get it pretty fully baked, and we get out there and start doing it. And eventually, if we're successful, people will come on and try and play with us. Trying to get them to play early without the success—I could give you an example. When I first started in 2008 in Type 1 diabetes, I was asking very basic questions. Because I come from the business world.

I don't understand nonprofits, even though I'm now running a very large one. But the only way, if you're going to scale, is the business model. It's the only way to scale. But we started asking, where's the data on people in the United States to show how they were doing with Type 1? The data did not exist, which was mind-boggling to me, and I'm like, how do you know what you're—

Dougal Edwards 15:28

2008, this was?

David Panzirer 15:30

2008. You couldn't even base how people were doing. So we went to JDRF, which was the public charity in our space at the time, and we said, "We really need to figure out how to create some form of a registry. It's going to take a long time. It's going to take a couple of years before we have anything useful, and it's going to be super expensive." Because the EHRs or the electronic health records in the US, they don't speak to each other at all, so it's literally manual data entry. Very long story short, we spent well over \$150 million over the years.

Dougal Edwards 16:02

Wow.

David Panzirer 16:02

But we were able to get data, at least at the beginning, on a first few thousand people. I went to the FDA to present that data to them, and everybody thought Type 1 was a safe and managed disease. You take your insulin, you're all set. If only it were that simple. So, we were able to prove without a doubt that Type 1 was neither safe nor managed. Less than 30 percent of people were hitting their ADA-recommended targets, and over 10 percent of them were having a severe event within the prior 12 months. Severe event being defined as third-party assistance and/or hospitalization. So, it's real money. And the long story short is when we presented this to JDRF and said, "You guys should co-fund this with us," they really couldn't. And it's not a knock on JDRF—which is now Breakthrough, but I will always call them JDRF. It's not a knock on them, it's a knock on—the public charity model is inherently flawed, in my opinion. They are very short-sighted, and they're very optimistic, and they have blinders on. And we have 88 autoimmune diseases. How do you keep looking down the rabbit hole at one and not thinking there's something else to find if you go with a wider aperture? So, I think, it's a very long-winded answer to your question, but we don't waste a lot of time trying to get people to start with us. We just go and do, and then eventually they'll find us if we're successful.

Dougal Edwards 17:29

It feels like brute force, almost.

David Panzirer 17:31

Say again?

Dougal Edwards 17:31

It feels like brute force, that you go, "This is the route that we've got to go, and we will bring people with us when they're ready."

David Panzirer 17:39

Yeah. And, well, we put our money where our mouths are as well. So, we'll go and fund it and do it, and then eventually, others will want to join, hopefully because it's successful, and by the way, most of the things we do fail.

Dougal Edwards 17:52

When it's 10,000 of those, how do you build momentum alignment there?

Durhane Wong-Rieger 17:56

Yeah. Well, obviously, it's a very different scope, but in some respects, the pattern's somewhat the same. Many of the rare diseases that do have therapies, of course, come about through a commercialization process, right? But many of them, even the commercial ones, actually start with families, and it's a whole different process, right? And they start with, even families that do crowdsourcing. There are some major examples of very successful—cystic fibrosis, for instance, has been really successful in being able to become a very great fundraiser, being able to really focus in terms of being able to fund discovery for their diseases. We've got lots of examples. I don't know how many of you saw a movie at one time with Harrison Ford, called *Extraordinary Measures*, about a father who went out, and he wanted to get a treatment for his kids and drove across the country to find the researcher, to raise the monies, and just basically set them up in a lab and kind of locked them up in there and said, "Find this treatment." Right? So, it's very purpose-driven, right? And I think there, again—I mean, there are obviously big foundations—but not to lose sight of the little ones. A hospital for sick kids in Toronto, which we just learned is for the second year in a row, rated the best pediatric hospital in the world, not in Canada. But what they have is real specialties on what we call N-of-1s. So, when you talk about 10,000, some of these diseases—cystic fibrosis, hemophilia, big ones—some of them only affect maybe families. So, how do you get researchers, how do you get hospitals, even other foundations to be interested when you have only one? Amazing story about a father who had a child who had a very rare disease. He went to SickKids, and they said, "We think we can fix this for you. We can find you a gene therapy. You just need \$5 million." He went out and raised \$5 million. And he started with bake sales and garage sales and did crowdsourcing, and he got to four and a half, and they say, "Okay, fine. We'll start." They actually created a therapy for him. He found six other families in the context of when he was doing this, and he set it up so he could give it to other families. The good news is that his son did actually get the treatment, and it seems to have arrested the progression of the disease. The challenge was that: he couldn't give away the other therapies. They create it, and he says, "Give me six vectors." They had six families waiting, and FDA and Health Canada says, "Not so fast. Now that it's no longer just for you, we have to go through a commercialization process. You have to actually set up clinical trials. You have to apply for this." And suddenly, it became a real challenge, and they had to scramble like crazy to get some of these families treated before their children were too far advanced. So, I think it's always the same thing, though. But for us, from a specific—as you're saying—who's interested, who's willing to drive it, and who is really willing to make sure, though, at the end of the day, I keep saying, it's got to get to the patient.

Durhane Wong-Rieger 20:52

Cystic fibrosis did a great job. At one point, they said, "We got it. We think it's there. We need to get out of the business because we're not commercial." So they sold it to a company. Unfortunately, the drug became very expensive, of course. That's no knock on the commercial, the companies. We need them. We need them to do it.

They take a lot of risk. The real knock on—I think is the actual getting to the public, getting to use model. And that is, if you think about a rare disease, it's really designed for a small number of people. And if you are commercial and you've got, as we talked about, shareholders who have invested in it and they need to get their money back, well, you're going to have to price it in some way that you can get that money back. And unfortunately, in the real world, it's not just, "Okay, I got a treatment. Let me just give it to you." Even a gene therapy, right? There's a tremendous amount of work that goes into facilities and the risk, etc. At the end of the day, it's what you were saying, only 20 percent of plans will pick it up. Only about 20 percent of the patients will actually get access to it. If I'm commercializing, what am I going to charge for this? I'm not going to charge for what I think the potential population is. I'm going to charge for that small group that I actually can get. And guess what? I got my stockholders on my back who are saying, "We've invested in this for seven, 10 years. Where's our payback?" So I'm going to charge what we think—they charge on what they think is 10 percent of the community. So, we can talk later about what we think some of the suggestions are and some of the possible ways of getting around that. But we get it, right? The barriers are built into the system, hospital systems, treatment systems, and reimbursement systems.

Dougal Edwards 22:47

Yeah. Thank you. David and Durhane touched upon urgency and this need for translation to happen at speed and alignment with philanthropy to drive that. Sitting where you sit, do you see that at the bench with the researchers under your stewardship, or how do you see that translating into an academic setting?

Christopher Laing 23:11

Yeah. So, the answer to the question around urgency is a difficult one, right? Especially in the lab where I think our researchers feel so removed from the patient sometimes, right? But actually, if you don't mind, I'd really like to riff off something that you just said about the barriers being built into the system, because I think that's a really important characteristic for us to acknowledge. And I was actually going to answer your question a little bit by talking about maybe an observation rather than an answer, right? So, we're a medical school. We actually do a lot with foundations. We have a lot of interactions with foundations, and throughout all of the work that I've done in academic institutions, that's been true. My work has been largely around commercialization and the relationships with companies. My office's world and the development world at the university almost do not overlap at all, right? The Venn diagram of philanthropic support that overlaps with the development of assets that are moving through my office is very, very small. And it tends to be kind of—we've talked about this earlier on—it happens by chance. A great example is we have a project going on right now with great support from Gates Foundation. We have a technology for a fermentation process for a particular material. Gates is interested in this for infant nutrition in lower- and middle-income countries. We're interested in it with our industry partners for pharmaceutical development. And so, by chance, we are codeveloping components of this that can be used in each case. But I think coming back to what Durhane was saying, I've often thought about, okay, well, what is that really going to mean for the development? Because clearly the foundation has certain requirements that are built into the support that they're providing that relate to access, right? And as we are increasingly talking to commercial partners around this, they are starting to now bump up against some of those restrictions for exactly the reasons that Durhane gave, that they need to be thinking about, okay, how does this restrict my ability to monetize this for my shareholders? And I've often thought to myself—this may be a very naive observation—but I've often thought to myself, "Gosh, I wish we had a chance to have that conversation much earlier in the process." And what we're really doing is taking advantage of the opportunity when it arrives, trying to do the best we can around it, and then hoping that we can make a further transaction down the road work. But wouldn't it be great if we could have that conversation very early and find alignment? And I do agree with David's point as well. Asking for money early, you

get advice. I think we've all experienced that many times. I realize it's not an easy thing to establish early. But yeah, I think unless we actually do recognize that the barriers are inherently built into the system, we're not going to be able to overcome them.

Dougal Edwards 26:45

Yeah. Yes, David.

David Panzirer 26:48

Sorry, I was just going to jump in with a real quick thought, which is, I think one of the things that's often missing is you have to understand what industry's doing and understand the entire ecosystem, and if you don't know what industry's doing, you're missing probably the most important component in the whole pipeline. We, in Type 1, unfortunately, there's probably two handfuls of real companies that play in the space. So, we interact with them on a very regular basis, so that I understand and my team understands what's in their pipeline, and more importantly, what's falling out, because everybody's got a budget. And we have done a couple of projects where we've taken an industry problem and went to academia, solved it for them with industry scientists involved, all at the end of the day to move things faster. Industry's going to go at their own pace, and we want it yesterday. So if we can incentivize them to move faster, de-risk it with basically nondilutive capital, they're all over it. And I just think the key piece is understanding what industry's doing. If you don't understand the ecosystem, you can't have an impact on it.

Durhane Wong-Rieger 27:57

Can I ask a question, though? We kind of assume that to get these—and let's talk about advanced therapies. We're not talking about making large generics for large-scale infectious diseases. We kind of assume that it has to go into a commercial model in order for it to finally succeed, and maybe that doesn't have to be the case. I'll give a couple of examples. As I said, cystic fibrosis, they came to a point where they said, "Okay, we don't want to own it because we do not want to become commercial, so we have kind of sold it." What they did remain was tremendously interested in terms of getting it available, getting it funding, and making sure that it was, in fact, reasonably available. We have another example of—some of you know a gentleman with Castleman disease, anyway, a very difficult disease to diagnose. He was actually going to be dying from it. He actually decided to go into medical research. He became a doctor. He actually found his own cure. So, he found a cure for this disease, and he's making it widely available because he really is so committed to it. We have the same thing with a father who had two sons with a disease, and they found an off spin of an old poison, actually, that worked for it. The challenge they had is they couldn't control it. It did go into commercialization. And so, it really became... Even he himself was at the point of realizing his kids may not get it, even though he invested. He left his job, invested his own time to do it. So, I wonder if we cannot start with an actual model, as you're saying. Duke would be an ideal example. And I kind of said there's an amazing drug for SCID. Some of you know SCID? It's—talking about diabetes being an autoimmune disease—SCID are children who are born without an immune system. They're the bubble children you see, right? They usually live in a bubble. Actually, they found a gene therapy for this. There's not very many kids. What happened was that they tried to get it commercialized. They actually developed it. It's somewhat effective. It is, in fact, a CRISPR kind of therapy. Population is too small. They gave it to Telethon, Italy. They said, "You can have it. We cannot actually support making it commercial." And so they turned over all the rights to it. Children who are born with SCID can actually apply and get the gene therapy done. It's not free because of the process itself, but all they're paying—all they're paying for it, I don't mean to make a minimum about how much they're paying for it—but it's a different model. And I often think, let me get Duke. You could develop it, you get it

done to a certain point, and then when you've got it developed, Duke buys it back. And what we're going to give the commercial part is how much they might have made in profit. There were 20 percent of the 20 percent that might have gotten it. And you now own the whole patent, and you can make it available at whatever cost you want to a lot of people. So, it's just an alternative thinking, right? But the question is, how do we get those people built in, as you say, in the front? My goal is to treat all these people. I've got this amount of money, and I'm going to get this amount of money back commercially. I can get it from a small group of people that I charge a whole lot to, realistically, or I can give you your bit and now let me give the rest of it away.

Dougal Edwards 31:12

Mm. Fook Kay, do you have—

Fook Kay Lee 31:14

I think, yeah, I think philanthropy is very concerned with this. Because when we put in an effort to support a certain cost, we hope that the underserved communities will be able to benefit from it, and we want equitable access. That's our golden, so-called principle that we would want to work with. So, how do we do that? We have been working with other philanthropic institutions. I'd like to give a good example, like you know pathogen genomics, for example. You talk about, Durhane, about infectious disease, and pathogen genomics is actually the cornerstone of infectious disease. Just imagine, a few months ago, we have this hantavirus, that outbreak on a ship. And it goes round the world and maybe go to Cap-Vert, which is a very quiet port. And they do not have anything to ascertain whether this particular strain of hantavirus is indeed that contagious or not. So, that's why we feel that pathogen genomics should be made more available for everyone in the world. But how do we do that? And I think, well, philanthropy, Temasek, for example, work very closely with Chris' institution, Duke-NUS. It follow up on what Gates Foundation has actually done to build up genomic capacity and at the same time to also facilitate that kind of data sharing. So, one of our recent efforts together is actually to have an open grant call for lowering the cost of pathogen genomics and also make pathogen genomics less complicated in terms of you don't need a very knowledgeable scientist to operate it. Some port worker can probably do it as well. So, then we also want to make sure that companies have benefited. They've earned a lot of money from pathogen genomics, like Illumina, Oxford Nanopore, BGI, MGI. They all come into some kind of agreement to say that, "Look, we have to sign the kind of agreement, with the philanthropic organization and amongst each other to have a tiered pricing given to countries that need pathogen genomics." Then the thing that you mentioned about data sharing. Data is very important, right? Rare disease and so on. So, how to get all this data center? How do we get all these genomic medicine centers in the region, for example, and also globally to work together to share the data? I'm sure the data are there, but somehow it's not equitably shared. And I think it's very important, even from a rare disease point of view, diagnosed early, right? So, some treatments need to be done at the infancy, or even very early stage of life. And the pricing needs to be equitable. It's not like, I need a \$3 million treatment for just one dose of gene therapy for what spinal, what muscular atrophy, for example.

Dougal Edwards 34:11

Yeah. There's an interesting point, there's a presupposition here about the commercial model and the need for a commercial model behind this. But I know that Helmsley has backed a number of models that actually drive it through to access. And how have you kind of tackled that? Is it that business perspective that you've brought to it?

David Panzirer 34:31

Yes, in essence. We actually do a lot of work in sub-Saharan Africa as well. And ultimately, it's the ministries of health that you have to talk to. They guesstimate that 60 percent of people are dead before they're ever diagnosed with Type 1, and that's a guess because they have no clue. But to answer your question, Dougal, I do think at the end of the day, you have to start with the end in mind, right? So, if we're going to go into a country, the ministries of health, who's the ultimate payer, they have to lean in, or we're not going. We'll de-risk it. We'll show them. They don't have to put any skin in the game up front, but they got to commit to carry it forward. And we're happy to go and prove it. We're happy to fund all of it up front. But to Durhane's point, the differences between the haves and have-nots, or the under-resourced, it's not only different in access, it's different in need. And you talked about patient-centered. The first time I went to Africa, I went there with my own preconceived notions about, oh, they need the latest technology. They need this and that. 100 percent dead wrong. And until I actually spoke to people on the ground and said, "What do you need?" And I'll never forget, I spoke to this young man. He was 21 years old, although he looked like he was 16 because he was stunted from malnutrition, had Type 1, and he worked on his dad's farm. And he said, "I don't always take my insulin." Because they were using insulin that we used in the United States in the '80s. Which means, basically, you take your insulin, you have to have a meal within three hours, or your blood sugars are going to plummet. And if you have low blood sugar, your symptoms are very much like you're a drunk. And if you have a severe low blood sugar, you could die from it. So, Justin said, "I don't know if I'm going to be able to eat in three hours, so I don't take my insulin. I just run high so that I can work." If you had a modern-day insulin that actually you injected if you had a meal, as opposed to the old-fashioned one that they're giving to these poor folks in Africa, only because geography dictates it. And we have a pretty strong belief that although it happens everywhere, geography should not dictate your outcome, but it absolutely does.

Durhane Wong-Rieger

36:45

But maybe that's the point in terms of, as you say, developing it. Because if I'm developing it for a high-tech kind of environment, I can build all those things in. And we've got educated people, or at least families and even patients that are going to be aware and managing to do that. But if we develop it with, as you say, the characteristics of that population in mind. Quick examples is HIV and hepatitis C. Those were drugs that, for HIV, very complicated, really difficult. And it took a lot, as you say, management and monitoring and training people. I remember in the early days, they were training people with M&Ms so that they understood, "You take this, and then you take this, and then you take this." And yes, it worked, kind of. And now, of course, we've moved to a different way. Hep C was different. It was dead easy. It's like one shot, off you go. So, I think it's always thinking about what's an easy—one of the examples, because my daughter has thalassemia minor, but thalassemia major requires regular transfusions. Regular transfusions are difficult, especially if you're living in countries where you don't have a lot of access to good blood. But the biggest risk there is where you do have access, and you are able to do it. I think Pakistan, when I was there, the big problem is you build up iron, as you're taking the blood, you have to get rid of it. Chelating the iron actually was a horrible, and still is a horrible process because you have to wear a pump, you have to infuse the medicine in, it pumps out the iron. So, difficult. In Pakistan, it turns out the pumps were a real problem because there weren't enough pumps. Children had to go and live in the hospital because they had to share a pump. They end up being in the hospital—we have an oral chelator now, and I think that's the difference. Okay, now the world changes because people can actually take this and take it in the morning and can manage it. And in fact, it's quite cheap because you don't have to buy a pump. Governments and public systems can say, "Okay, we can invest." And in fact, the oral chelator is becoming cheaper and cheaper. So, I think if we were to think about what are the capacities of the people that we really need to serve, and to get to the point where you've got—you know, SMA, as you're talking about, is an interesting example. We started off with this god-awful, difficult intrathecal drug. We now have an oral option. So, can we move as quickly as we can to getting formulations that actually are manageable in that context?

Dougal Edwards 39:22

Mm. It's a fascinating point that we kind of circle around here, the ability, or the lack of its ability to do it right now, it's for philanthropic support and backing and families to align people behind these missions. And I find one of the things that's been particularly interesting over the last 15 years—I was chatting to Chris beforehand, your first exposure to Milken was a venture philanthropy conversation. My first exposure many years ago was, I believe, also a venture philanthropy conversation. And yet that's not something that's necessarily progressed. The sophistication of what happens in other capital markets hasn't made its way into philanthropy mainstream yet. The need for alignment of parties, government, industry. How do you see us trying to overcome that? And are there examples where we have succeeded? And what could we do next? Maybe if you have an opinion.

David Panzirer 40:23

I always have an opinion.

[Laughter]

David Panzirer 40:26

I look at it as, at the end of the day, like I said earlier, we're trying to de-risk to get to phase 2. What we've been doing now in Type 1 diabetes, we have the public charity. We're the largest private funder in the space, and there's still a huge gap to where we want to be and where we want to go. So, the idea is how can we tap other resources that's on the sideline? So, as an example, there's a T1D fund that was created as a single-member LLC off of the public charity JDRF. It raised \$100 million. It invested in a little later stage down the road. It grew to \$240 million. And then there's been a little bit of a kerfuffle with JDRF, which I'm not going to get into, but the point was this was a vehicle that other people wanted to play in. And we're now in the process of figuring out how to create a for-profit fund with a focus on T1D and autoimmunity. We've got a family that's willing to be the anchor. And the whole reason, again, is I've talked to a number of high-net-worth individuals, and they'll say, "My left pocket is my philanthropic pocket, my right pocket is my family office. I want to tap the family office pocket." It's usually way deeper, and if they have a personal connection to the disease, and you give them even the slightest chance of a return, they're all over it. So, to me, it's a model that has to be pursued, and we're in the middle of creating one now.

Dougal Edwards 41:56

Mm. We have just a few minutes. I wonder if anyone within the audience had a question before we wrap up? And we can keep going otherwise. Ah, yes, please. There's a microphone.

Audience member 42:12

Actually, it's not a question, but Durhane mentioned about the Harrison Ford movie. And I have a story that I shared yesterday, but I'm not going to be able to share it here. But my son had a brain tumor 11 years ago, and I was told that there was nothing to do because the tumor relapsed, and I didn't accept that. And I created an initiative that brings together 16 institutions across US, Canada, and Germany, and we already have two FDA clinical trials approved and more coming up. We don't care about IP. We care just about making it happen. We are not thinking about commercial. We're thinking about having something. So, I don't know how much it's going to cost. My drive is having a drug or having a treatment to save my son. So, it's a different model. Probably we'll have

opportunity to talk, or I'd like to talk to each one of you about it, but I just wanted to share, so it's not really a question.

Dougal Edwards 43:23

But it's urgency, again, driving the conversation. A very personal lived experience.

Audience member 43:29

Yes, exactly.

Dougal Edwards 43:30

Which I imagine we have driving your—

Durhane Wong-Rieger 43:33

But this is exactly it. There are lots more models there, and I think we've kind of locked ourselves into one model. But if we make it available, if we bring the families, if we bring in—as you're saying, there's nobody more committed than a family, a parent, who would do it.

David Panziner 43:47

The mother.

Durhane Wong-Rieger 43:49

I don't know. We've had great fathers, too, and you're real examples of it.

David Panziner 43:54

You want to get anything done, you get a mother.

Durhane Wong-Rieger 43:56

Well, it's true to some degree. But really, I think it is looking for different models and for philanthropy to think different. And if philanthropy is really directed towards that end goal, and that is we want to make it available. I love the idea, we're talking about philanthropy and medical research, but quite frankly—no disrespect to researchers—researchers ain't it. I don't really want to invest in a researcher. I want to invest in treatments for people. That's where we want to flip it over and think about it. And there are a lot more models that are there that are being pioneered. Brazil, amazing some of the work that they're doing in terms of collaboration with Philadelphia, in terms of how they're creating gene therapies. We look at what, quite frankly, China's doing. And they're setting up a whole medical tourism. And the idea is we will get minimum profit out of this because we want to make it available to as many people as possible. So, I think it's thinking differently.

Dougal Edwards 44:56

We have one minute, as I've just been politely notified. I wonder if there's just one thing that we could each leave the room to consider, as maybe around philanthropy and its alignment. Is there one thought you could leave with the room today?

Christopher Laing 45:09

Yeah. Maybe I'll jump in. I agree, there are a lot of different models. I do think that we can take examples of models that we're seeing. Cystic fibrosis is a great one, Fook Kay's example of the collaborative is a great one, but these are individual examples that are often a decade or more apart. How do we make it systemic? And I actually think that one of the ways to do that is this needs to be driven by the people who care or who should care. So, it's individual people who care about the patients, but it's also government. And you talked about the importance of having government involved. I think if we just have this conversation just amongst philanthropists, or just amongst universities, or just amongst companies, we're not going to change anything systemically. So, I do think that we need to make sure that conversation crosses over multiple different types of organizations.

Dougal Edwards 46:02

Mm. Any other points from the panel?

Durhane Wong-Rieger 46:07

Can I say real quickly, we believe really that it's not "Philanthropy is giving things away." Philanthropy is investment. And I think what you were talking about in terms of governments—I don't want to give you anything because you're not really going to respect it. I want to invest in you, and then you have a responsibility. What we talked about yesterday as well—the responsibility also goes to the patients, to actually honor that. So, think about philanthropy as investment, and investment that's going to allow for sustainability that people, countries, researchers, hospitals would take on themselves.

Dougal Edwards 46:41

Thank you.

David Panzirer 46:42

I would just add, at the end of the day, this is a business. We want to get a drug, a device, therapy, something to market. Three things have to happen. The regulatory authorities have to approve it, the payers have to pay for it, and industry has to believe that they're going to make money, so they do the manufacturing and distribution. Industry farms out the R&D now to academia. And we really need to...never take your business hat off in philanthropy. And if you remember nothing else, remember this, which is do more diligence than you did to make your money, to give your money away.

Dougal Edwards 47:16

That's a good point.

Fook Kay Lee 47:16

I'd like to end by saying that these are the concerns that philanthropies have because each of us have a different focus, and I think the best way is really to bring up a coalition of all these philanthropists, and it's happening right now. So, we talk about climate and health, we talk about certain diseases. This is alliance of philanthropy, and there's also financial advice given to philanthropies of how they could work together to support a common goal, for example.

Dougal Edwards 47:44

I'd like to wrap up by thanking Milken for continuing to hold a space for us to talk about philanthropy at these events. So, thank you.

Announcer 47:53

We hope you enjoyed the discussion. The next panel will begin shortly.

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