REWRITING THE RULES: SCALING INNOVATION FOR RARE DISEASES

Becky Quick 00:18

Hello everybody, and good morning. We are all here today to talk about rare disease, and I thought before we did, I could share a little bit about why I'm here today. I've been asked to come out here for a lot of years. I never do because the Berkshire Hathaway annual meeting in Omaha is before this, so I spend four or five of the days there before I come here, and it's just too long to be away from my family. But this year, when I heard about this panel, this was the reason that I really wanted to make sure I made the effort to come out here. What I'm hoping is, before we dig into this, just to share a little bit about our family story for why I'm here. Take a look.

[Video plays]

Becky Quick 02:35

So that's our family story. But the more we started telling that story, the more I realized that almost all people have a story. That if it doesn't connect back to rare disease, it connects back to some disease. But there are 30 million people in America who have a rare disease. There are up to 300 million people worldwide who have one of these diseases, and that personal connection is a force that drives so many of us, including everybody here on this stage. David, I think you've probably had the toughest path to where we are today. Your mother was diagnosed with glioblastoma when you were only 19 years old, and you were playing football at Georgetown. And then not long after that, your health changed too, and I was hoping you could talk about the before and after and how that molded what you're doing today.

David Fajgenbaum 03:25

Sure. Becky, thanks so much, and it's such an honor to be with this amazing panel, to be with all of you today. Yeah, I was a healthy undergraduate student—or healthy medical student. When out of nowhere, I became critically ill with this horrible disease called Castleman disease. As Becky mentioned, I had decided I wanted to become a doctor in memory of my mom. She had passed away from glioblastoma, and I promised her I would dedicate my life to trying to find drugs for patients like her. And then all of a sudden, I found myself in the intensive care unit, dying from a disease I'd never even heard about in medical school. I spent about six months hospitalized, had my last rites read to me. Nearly died on three occasions. And thankfully, I received a number of chemotherapy drugs that weren't made for my disease, and they somehow saved my life. I was able to return to medical school, but I was now on this mission to try to find a drug that could save my life, a mission that Michael and others in this room—I see Chris here is also on. You're on, Becky. And for me, the real unlock, which ended up being what saved my life, was realizing that there are drugs that are already available. Drugs at your local pharmacy that are made for one disease, that could actually treat additional diseases. And I eventually discovered a drug called sirolimus that was made for organ transplantation, that saved my life. And the moment that drug started saving my life, I have not been able to stop thinking, "If this drug could save my life that was made for another disease, how many more drugs are out there?"

Becky Quick 04:47

It was a drug that was on the shelves at CVS.

David Fajgenbaum 04:48

That's exactly right.

Becky Quick 04:48

It was right there in front of us.

David Fajgenbaum 04:49

How many more drugs are out there that could save more lives?

Becky Quick 04:52

Yeah. Nicola, let's talk a little bit about why you're here, but I think what you're working with are these

large data sets that are really pushing things forward to this point. Tell a little bit about your own personal story with this, and then where you think the promise is right now.

Nicola Blackwood 05:11

So, thank you, Becky, and it's great to be here as well and to see all of you here. I was sick my whole life, from childhood, all the time, in and out of hospital. It was very hard to track what the problem was. Many clinicians would pass me off to a psychiatrist or say we just weren't taking care of ourselves. My father is a clinician. I have the best background possible, and he just said, "I just don't know what's wrong." And eventually, 30 years later, I was diagnosed with Ehlers-Danlos syndrome. I remember the time, it was 20 minutes with the right neurologist and again, just crying because knowing what had been going on and that I wasn't crazy, I wasn't imagining it. But also, that there was a path to management, because by then I was really quite sick. And so, this led me to a fundamental belief that we have to abolish the diagnostic odyssey. We have to cut out that route and bring back diagnosis as early as possible so that we can characterize disease, and we can develop therapeutics. There's only 5 percent of rare diseases with therapeutics. So, I was really lucky. I got to be the health minister in the UK, where I made sort of national-level decisions. But now I chair something called Genomics England, where we created the Genomics Medicine Service for the NHS, which is whole-genome sequencing available for rare disease and cancer patients. They give permission for their data to be used for research. We have one of the most well-characterized multimodal datasets for rare disease and cancer in the world. And we engage with industry and researchers to access this. We have thousands of researchers in there. And the idea is you have the clinical diagnosis, which improves the individual life. You have the platform, which creates scale. And then the innovation of therapeutic and diagnostic can be driven straight back into the clinic as soon as possible. We need this infrastructure. It needs to de-risk not only getting to diagnosis, but also getting to therapy as soon as possible.

Becky Quick 07:08

And where do you think we are? Is this a moment in time? How does it compare to where you've been or where we've been, let's say, over the last decade or two?

Nicola Blackwood 07:19

So, the story of Genomics England started with a research project, the 100,000 Genome Project. It took eight years to complete that because the science wasn't quite there, the health systems weren't there. It wasn't clear how to integrate clinical genomics into health care and the diagnostic pathways and link those to actually helping patients. And once we managed to develop that into a platform service, which is the Genomics Medicine Service, we realized, okay, we can't stop here. We're going to decentralize that into the NHS, make it as quick as possible using new technologies like long-read sequencing, linked to short-read, which will just make sure that we can get to patients as early as possible. But what we know is there are windows when therapeutic value actually happens. And so, we're now trialing a national-scale program

on newborn sequencing. We'll recruit 100,000. We're in 71 sites now with 46,000 families and patients recruited. And this means you can diagnose at birth. And so, for example, if you had SMA, every moment counts to get to therapeutic. There are three therapeutics, but if you don't get those in the first 24 months, you can have a lifetime of disability, whereas you can actually cut that short. And more and more, we're finding therapies coming forward which can be curative, but only within the right timeframe. So that infrastructure and platform matters for both getting to patients on time, getting into the right clinic, and also, of course, developing the new therapeutics that we so desperately need.

Becky Quick 08:49

Right. And I think that early diagnostic and the ability to diagnose these things is a big deal for the drug companies as well. Will, I would say being able to find patients maybe where you couldn't before, find markets that you might not have realized that existed before. Tell us a little bit about what you're doing and, again, your personal connection to this, too.

William H. Lewis 09:08

Well, I got into this business because I lost my oldest brother to a rare disease. And what I always like to say in the context of that is he was given a one in 20 chance of surviving, and he did for a long time, and then we lost him. But today there's a 95 percent cure rate for that same disease. So, hope springs eternal from the loss and also from the learning that comes from the work that goes around that. And at Insmed, what we're trying to do is focus on creating first or best-in-class therapies that are going to have an intuitively obvious impact on the patient. We always talk about it as the holiday test, where you're sitting there at, God forbid, it's your family around the table at a holiday, and someone announces they have this dreadful disease. And you say, "I work at a company that actually makes a treatment for that," and you slide it metaphorically across the table, and you know that what you're giving them is going to make a big difference in their life. It's not just an additional medicine, or three times a day is now one time a day. There are business models for that. But I think the work that we're involved with, and certainly the work that Neil's involved with, is very focused on high-impact medicines for the, if you will, the forgotten diseases.

Becky Quick 10:20

I just want to know very quickly because I do want to have you build out on that. What was the disease that your brother had?

William H. Lewis 10:26

It was a kind of a specialized form of Hodgkin's lymphoma.

Becky Quick 10:29

Yeah.

William H. Lewis 10:30

But it was actually part of the treatment that ended up causing the damage that later caused him to succumb because he was heavily irradiated, and the fibrotic process that took place over time ultimately was what gave him heart failure and idiopathic pulmonary fibrosis and other things that collectively took him down.

Becky Quick 10:50

And now there's a cure for that or therapy?

William H. Lewis 10:52

Well, there's a treatment that is 95 percent effective. Yeah.

Becky Quick 10:55

That's amazing.

William H. Lewis 10:56

It is. It's wonderful.

Becky Quick 10:57

And I'm very sorry about your brother, but thank you for what you're doing, throwing yourself into this.

William H. Lewis 11:00

Listen, the raw truth is, whether you like it or not, everyone in this room is going to be touched by this industry, okay? That's just the nature of being human, and many of us probably already have been. And so, what I want, if there's one thing I can leave you with today, it's the understanding that many of the people that work in the rare disease community, particularly on the industry side, are deeply connected to the patients, oftentimes through personal stories. But if not, in a genuine way hoping to have that impact, and they've chosen this profession because there's never a doubt why you're getting up every day in the morning.

Becky Quick 11:35

And Neil, I know that is the case with you at BridgeBio.

Neil Kumar 11:37

Yeah. Well, I just wanted to build on these two comments because what Will said obliquely about once daily versus three times daily. Basically, we're at this conference. We are in this profound moment in time for rare genetic disease. The work that you guys have done, but if you think about it, when I was in graduate school, we were finding one causal variant a year. We're finding three a day at this point. Long-read sequencing, the declining cost of everything from data to the raw molecular data allows us to go after condition after condition, but it's a market failure at this point. And why is it? Because if you walk around these hallways, you're hearing about obesity, you're hearing about inflammatory disease, you're hearing about these monster markets, $20 billion plus, and that's all anyone is talking about. They're making antibody after antibody coming from China that's just a little bit more convenient. And I'm not saying there's not a place for that, but at this point, we don't have business models that can really address the promise of what's ongoing in rare genetic disease. And you open *Science*, *Nature*, *Cell* every week, the advances are huge. You would dream about this, but most companies are not going after it.

Becky Quick 12:40

You set up this very specific model. I don't know how many people here know about it, how many don't, but it makes perfect sense to me—this idea of a hub-and-spoke, this decentralized place—so that you can go after more and more diseases. And I think you've done 48 diseases over the last decade, which is pretty phenomenal about how you go. How did you come up with that idea? Where do you use it?

Neil Kumar 13:01

Really, it came out—I was a venture capitalist prior to, and we were building companies one at a time. It was very slow, and it was not really consistent with the amount of academic substrate that was coming out of the great universities in the US and Europe and that we could very quickly translate into medicines for patients. And mostly people don't build hub-and-spoke. It's a really awesome Roy event because

mostly in biotech, people build companies to sell. It's like a single asset. I hope to sell it to pharma one day. That's how I make my money, and I keep going. We had an eye to build a more generational company, and the hub-and-spoke model made a ton of sense because it allows us to be pretty cheap and efficient, so we can go after small markets in an economically viable way. Allows us to diversify because a lot of things do fail, but it also allows us to have focus at the level of each biology because an expert in Syngap may not be the expert in pantothenate kinase deficiency or ATTR cardiomyopathy. So, these different conditions need focus, but they need to be hooked up to a central chassis so that they can move much more quickly.

Becky Quick 14:01

Well, and much more cost efficient too.

Neil Kumar 14:03

Much more cost efficient and time efficient.

Becky Quick 14:03

Yeah.

William H. Lewis 14:04

And just to put it in perspective, for 15 years I've been at Insmed, and we've raised $6 billion and managed to get two drugs approved.

Becky Quick 14:12

Wow.

William H. Lewis 14:12

He's done 48. So just to put it in perspective, this person sitting to my right is probably one of the smartest people in our industry. Sorry to do that to you, Neil, but—

Neil Kumar 14:24

—That's okay.

Becky Quick 14:24

No, it's not. *[Laughter]*

William H. Lewis 14:25

Yeah. And he is trying to create a new paradigm, and with great success. And that, I think, I'm afraid, is what's needed. I took interest earlier in your story personally about how you came to be involved in this, but you went the nonprofit route and you're creating medicines, if you will, that are already there. So, AI, I think, folds into this. It's the hot topic of every discussion. But I think, to Neil's point and the work that Nicola has done, there is a massive amount of data and information that we are now going to turn our attention to that hopefully will create a new work stream and a whole lot of productivity for many more diseases to get to that 95 percent or greater cure rate.

Becky Quick 15:03

All right, Michael, let's talk a little bit about what you've done with EB, because it's absolutely amazing. You have raised over $80 million. You have garnered tons of attention with the Netflix documentary that you did, "Matter of Time," that brought a real focus to this. I know you have another documentary that you did too that brought to more broadly with rare disease. And to me, the thing that strikes me the most is you are figuring out how to work with other families and kind of show people how it's done. Tell everybody a little bit about this story and how you got involved.

Michael Hund 15:32

Yeah. Well, first, I have to take an opportunity to thank Becky Quick. The courage to share your story, share your platform, shine a light on rare disease are the type of things that are going to drive change in the space. So just a big thank you to Becky Quick.

[Applause]

Becky Quick 15:45

Thank you.

Michael Hund 15:47

I also want to thank this panel. I'm calling you all the health care avengers, or the rare disease avengers. I'm looking out at this audience, and I see a lot of rare disease avengers as well, and it's going to take all of us. Look, we're at Milken. I'm a trained economist, so just to size this market really quick, you heard Becky talk about it, 300 million people with a rare disease, half of which are children that may not make it into adulthood. Five to six years, the average diagnostic journey, when it takes us hours to do genomic sequencing, and we've gone from $100 million to hundreds of dollars to do this. The economic burden of rare disease annually in the United States has now crossed $1 trillion. Now, on the positive side, we talk about the progress. Thanks to the Orphan Drug Act and things like the Priority Review Voucher. Many people don't know this, but in recent years, half of the FDA approvals have had a rare designation to them. The market itself is approaching a $300 billion global market. So, there's opportunity. And I believe not only is rare disease the greatest unmet need in health care, I think we have the greatest opportunity to actually address this. I think where innovation is happening in rare disease and will impact all of medicine. So, what have we done at EB Research Partnership?

Becky Quick 17:03

And by the way, tell everybody what EB is for those who don't know.

Michael Hund 17:04

Yeah. So, EB stands for epidermolysis bullosa. It's a rare, fatal, devastating genetic skin disease. And often young people that live with it are called butterfly children because their skin is as fragile as a butterfly's wing. So, we were started with people just like Becky and David, parents that wanted to save their children's lives. I'm looking out in the room and see many of you here. But we were lucky. We had an ace in the hole. We had Jill and Eddie Vedder from the band Pearl Jam. So, what I love about that is we have a rock star, but we have rock and roll in our DNA, which means that we felt rebellious to the status quo. So how did we go from two to 50 clinical trials? We're proud to report we've had not one, not two, but three FDA approvals in the last two years. And quite frankly, we're just getting warmed up. So, we think a lot about models, and I think the rare thing in the nonprofit world is to run your organization like a business. Families deserve this. Organizations deserve this. And that's not the norm. So, we started to think about models. The brief version is that we call our model the venture into cures model. So, it's three things: data platform, collaborative infrastructure, and innovative investment models that include venture philanthropy. When we think about data platforms, platforms built by families, the data is owned by families, and the data is given back by families. So, we started something called Curator with GeneDx and AWS, and families get data back in real time. It's at-home genomic sequencing, so we went around academic medical centers where families can provide data and get real-time information. AI—while there's

still lots of caution—it largely is here. We've invested in squamous cell carcinoma analytics at Northwestern, so we now can predict two years before the clinician when squamous cell carcinoma is going to emerge, and that's something we've invested in for years. We've made investments in things like organoids, which sounds like science fiction, or skin on a chip. Now, we're not yet at digital twins in health care, but we as organizations need to build that data piping so we're prepared for an era where we will have digital twins. When we think about a collaborative infrastructure, it's not the norm for academics to share data. It's not the norm for even families or foundations to share data. So we have to incentivize those models with financial incentives. So, we built a 22-center academic medical consortium in North America, and one important rule, we don't put a check out the door unless you agree to sharing data with other partners. So that's the only way we will change collaboration. We also built pre-competitive consortiums where companies like Neo and Wills can see data at the same time. They can help foundations monetize that data philanthropically and have a fair shot at driving research and discovery. Finally, innovative investment models. For us, that's venture philanthropy. What that means simply is every single research project we fund is done under a venture capital model. So, I've done over 200 venture philanthropy deals. Every single one started with the university saying, "No way, we don't do that. We've never had one follow through." So, at universities, we have an upstream royalty. If it's commercialized, that comes back to the foundation so we can fund more research until we cure the disease. We kind of got semantical where we don't want equity in the IP, we want future commercialization rights of that equity, and that's enough to kind of get it done. When we invest in companies, we're like a traditional investor. We take shares in the companies, but our shareholders are patients and family. So, we want urgency, we want collaboration. We don't want things to sit on a shelf and be locked up. So, two short examples. The first FDA approval in EB is a company called Crystal Biotech. We invested early on. They were looking for capital. They didn't have a lot of investors. They got an investment from a foundation. We did about 3X return on our investment, although I wish I would've held it today. They have a $6 billion valuation, but we needed the cash at the time, and that became the first FDA approval that was supported by patients and investment. We invested in a gene therapy at Stanford. We took a royalty stake. It was acquired by a company a few years later. We did a 6X return. That went into clinical trials, and that became the third FDA approval. So, the final lesson there is my message to those that run foundations and patients and families, remember, you can demand economic rights. You can demand good investment models.

Becky Quick 21:09

Tell me how. Because you make it sound so easy, and I know by personal experience it is not. What do you do?

Michael Hund 21:14

Well, it's what you do every day, Becky. It's not taking no for an answer and being resilient. Again, every single venture philanthropy we've done, I get a giant red line back from the university that says, "We don't do this." And I pick up the phone and say, "Look, if you don't do this..." And rare disease, we're uniquely positioned to have leverage points. What do I mean by that? In our disease areas, we have the absence of capital. Nobody else are funding these. So usually, what it takes is actually that researcher or that clinician

going to their tech transfer office and say, "Look, if we don't work this out, no one else is going to fund my lab and I will go out of business and kids will suffer." So, it does come down to that ultimate leverage point. But we've also created win-win scenarios because we don't just come with capital. We come with biobanks and natural history studies and data repositories and connections to VCs and companies like Neo that can help get over that doubt, that valley of death that we don't talk about enough, right? Get it out of the lab—

William H. Lewis 22:10

—What I'll also say, you're hitting on a point which is really important. The patient needs to be in the room when the drug development is happening. And that has changed, I would say, in the last decade dramatically because of the success of foundations like the one you're representing, EB. That is a devastating disease. Cystic fibrosis is probably the best example because their investment in Vertex ended up creating a billion-dollar balance sheet at that foundation. And as a consequence, they can direct drug development, not just participate in it or be a voice on behalf of patients. So, I think, Becky, to your question, how do you get involved and how do you make this happen? It really comes down typically to a family who is affected by the disease, calling other families, using the social networks to build their consortium. We encountered this with the first disease we treated, which is called nontuberculous mycobacteria lung infection. And this was a group that was founded in Florida by the spouse of someone who had passed from the disease and just could not accept the fact that this was going to continue without intervention. So, they created a group and a foundation, and we linked arms with them, and now there's a drug on the market for it. There are other drugs in development. The ecosystem kicks in once there's enough critical mass, but getting there is not easy.

Becky Quick 23:23

Yeah. The idea of being able to not have to reinvent the wheel every single time for the advocacy groups for each of these 10,000-plus rare diseases is really appealing to me. That's the idea where I feel like, okay, you can really have a game changer and link all of these together. I don't know how to do that faster, how to do it better, but I love everything you're saying about it. I get it. It's right here.

William H. Lewis 23:50

But the challenge is it also goes right to the heart of why this is such a time-sensitive issue. We talk about do no harm in the medical community, but not taking action in the presence of these diseases is, in fact, doing harm. Waiting for the normal, traditional process to run, it screams at you as not making even intuitive sense. So that change needs to come. The voices need to get louder. I think there are early signs that we're going to see some of that change out of FDA and other regulatory bodies, but we're a long way from where we need to be. I think that's fair.

Becky Quick 24:21

You're talking about even just the idea of accepting these natural history studies, right? There's some question about that at the FDA right now, too. Do you accept a natural history study, or do you insist on having—

William H. Lewis 24:32

—Placebo controls?

Becky Quick 24:33

—Placebo controls on these things? Which is in some cases are completely inhumane in the things that we're talking about. In other cases, it's too small of a population to try and do those things, and it's really, really expensive.

William H. Lewis 24:43

Well, I think they get the priorities a little bit askew, right? The FDA is well-intentioned. They're very smart, capable people. They don't want something approved that isn't going to be safe and effective for patients in question. The counterpoint to that is it takes 15 years to get a drug from concept to approval, and we've got to cut that time down if you're talking about a disease like SMA that was mentioned earlier, where the mortality rate is almost 100 percent by age two.

Becky Quick 25:07

Right.

William H. Lewis 25:08

What are you protecting? I mean, at that stage, get in there, get early, try some different approaches, accept some risk, engage with the patients and their families, because the families are as affected by this as the patients, often more so, and bring that group together to say, "Okay. We accept that this is a higher risk pathway we're going to follow. We don't have time to run the animal studies. We don't have time to do a placebo control, and it's inhumane." So there just need to be exceptions made. And once we are comfortable making them, I think there will be a cascade of opportunity across the, as you say, more than 10,000 diseases, most of which don't have a single thing approved to treat them. So, it's not like there's a good alternative that we're using in the meantime.

Becky Quick 25:50

Right. Nicola, there's a lot of things that are happening here in the United States. What you are doing in the UK is particularly impressive with this database, but it seems to me like it's a much more fraught environment trying to figure out how to cooperate across the globe from different countries, too. I think you could probably speak to this a little bit. Neil, you might be able to too, with the whole idea of most favored nation when it comes to some of these drugs and what we're doing here versus what we're doing there. Nicola, why don't you talk about working across the pond and around the globe?

Nicola Blackwood 26:22

Yeah. Obviously, this is a sector where, without international collaboration, we will not make the progress that is necessary. Now, there are encouraging signs. We're seeing changes in the FDA to move to adaptive trials and other models. We're seeing the same in the UK in our regulator, the MHRA. They've just announced a new rare disease program and approach. It's more flexible than the FDA, which is obviously targeted to quite narrow areas, and the approach to developing it is also different because it's a consultative process which engages not only the regulator, the payer, the patient groups, and industry. And so, at the other end, I think we'll have consensus to be able to act because, as a former person who worked in government for a long time, one of the biggest problems is you set out your ambitions in a document, but there is not the comfort or the risk appetite in how to implement that. And so, at the moment, there's a gap between good intentions and plans and proposals and actually comfort with going over the dotted line and ticking the yes box.

Becky Quick 27:27

Right.

Nicola Blackwood 27:27

And I think that the rare disease community can play a really important role in supporting and explaining and engaging with those who need to develop the skill set within the regulatory environment. But the second thing I would say is it's not enough to speed up the path to licensing market authorization, because then what you're just going to have is a great big queue waiting to work out how you do reimbursement, and we've seen that elsewhere. So, we need to look at new and novel reimbursement models as well as novel trial models. In the UK, for example, we all recognize there's a failure around antimicrobial resistance, so they've introduced a de-linked reimbursement model, which means that you can get paid for the value that it provides into the system, regardless of the volume that's used. And two antibiotics have already been approved for this. They're being paid $10 million a year regardless of use, which is you can

see where the read-across would come into the similar market failure which we're observing with rare disease. It's not a complete read-across, but it's a place to start.

Becky Quick 28:29

Yeah.

Neil Kumar 28:30

Yeah. I agree with all that. Maybe I'll build on it in two separate chapters. I think one area that's been profoundly positive is our learnings from European countries and databases in and around diagnosis. Newborn sequencing, for instance, is way better in the UK than it is in the US. We've talked to Dr. Oz and others about this. We can do better. There are diseases for which if you're born in one state, you will die, and in another state, you'll pick it up, just because of what's on the RUSP for the newborn sequencing panel. So, I think that's all been good. I would say my impressions of Europe to date have been that it's getting more conservative than the United States. I'll give you an example. We just had a phase III readout where we hit every functional endpoint for a devastating muscular dystrophy, and the US is going to basically priority review that drug. In Europe, they said, "Well, you ought to run out the 36-month trial just to make sure, because that's how you designed the trial to start." It's just protocol. It's just bureaucracy. It really probably comes down to the fact that they don't want to pay for these drugs, and we're seeing a lot of that. So actually, five years ago, I remember talking about the fact that everyone approached regulatory in the same way. Now we have China, and I know everyone bemoans what's happening. But if I were a parent and I had a child who was dying from a devastating condition, the fact that you have an IIT trial and you can skip some of the toxicity work that Will talked about—we've had children die of Canavan disease waiting for our trial to be green-lit by the FDA. So, we have to have this conversation, and risk/reward is different in a deleterious Mendelian condition as compared to the fifth or sixth hyperlipidemia agent. So, I think those are the challenges. I think it's an ongoing discussion now, and hopefully it gets better, but there's a lot of noise globally right now. And in payment, MFN, I don't meet anyone in DC or elsewhere that says this feels like a fair system right now. At the same time, obviously as a small biotech, we partnered our first set of drugs with Bayer, AstraZeneca. I'm going to launch internationally at this point because I have to control pricing everywhere. That puts a lot of economic strain on the system, and then how do I think about that? And so, there's a lot to discuss right now that's not optimal for the rare disease community.

William H. Lewis 30:41

And I'll go one step further. We have a medicine that is approved right now in Europe and the UK, which we are not launching because of the possibility of MFN.

Becky Quick 30:49

Yeah.

William H. Lewis 30:49

It prevents us from bringing a medicine to populations that may benefit from it. And while this policy is getting worked out with best intentions, Europe pays less than the US, I completely observe and agree with that. Putting that burden on us is a little bit difficult. We can't get Germany to negotiate a higher price. The US government can, but we can't. So, we're really constrained in our ability to have impact on this issue, and we are absolutely controlled by it. So, it's a very frustrating circumstance for us to be in possession of something that we know can help people but not be able to get it to them.

Becky Quick 31:24

And what would it take the US government doing to fix that right away for you?

William H. Lewis 31:29

Well, I think they'd have to—and I would advocate that they exempt rare disease from this discussion.

Becky Quick 31:35

Yeah.

William H. Lewis 31:36

Because I think what we want is every incremental dollar that we can get invested in those areas. I think the other issue that they're just going to have to accept is that Europe is not in a position to pay as much as the US, and that's just a reality. The difficulty with the MFN policy now is if you move it forward in its current form, what's going to end up happening is we will stop selling our medicines abroad because it will bankrupt us in the US overnight if we are importing the price from Europe into the US. So, what will the consequence of that be? China will take the place and become the medicine chest for Europe and the rest of the world because they can do fast follower better than anybody. And by the time our drug was approved, there were three versions of it already up and running in China. That's how fast they're moving. And so, if we are prevented from making the incremental dollar in Europe because the price is lower, we won't sell there. China will move in and take over, and we'll be bankrolling their industry, which I really

don't think we want to do.

Neil Kumar 32:29

And I actually think there's another solution. What John Brooks was talking about, I think yesterday or earlier. I do think we can ask counterparties to pick up a bit more of ultimately what ought to be the profit pool associated with these medicines so we can go make more of them. And if you can have a hard conversation around that as a country to another country, or you say, "Hey, look, there's going to be a depot outside of that country then, but your patients are going to have to travel," that's an important discussion to have. I think the current system is not sustainable.

Becky Quick 32:59

At the administration level, you mean, though?

Neil Kumar 33:01

No—yes, at the administration level, they need to have the discussion.

Becky Quick 33:04

Yeah.

Neil Kumar 33:04

I don't think it's right. For instance, the first drug that I was involved in creating for hypertrophic cardiomyopathy is $120,000 in the United States, and it's $10,000 in the UK. That just can't be. It's not going to work. So that price point has to come up, or at $10,000, it's not MPV positive to make drugs for most rare conditions. And so, we have to think about what ultimately globally is fair, and I think the administration is working toward that, but we'll have to see.

Nicola Blackwood 33:32

But it's also about looking at what's actually economically rational, because actually, generally, approving these drugs is cheaper than paying for the untreated patient as they engage with the health system again

and again and again. So, we're having the discussion in isolation from the economic reality and the impact on patients, on the system, on clinicians, and so you always need to bring that back, which is what is so important about the rare disease community. And having the data infrastructure for diagnostics is one thing, but having it for post-market surveillance, health economics, demonstrating the value of the process, the argument in an essentially irrefutable manner, is where we need to get to.

Michael Hund 34:10

Just a specific example for exactly what Nicola just said. In the rare disease space, we have a 20th century payer system for now 21st century treatments. We're in this era of curative CRISPR gene editing therapeutics. We've seen this in sickle cell. We saw it with Baby KJ last summer. It is health care economics that requires systemic change, and what I mean specifically by that, I can tell you that the burden of one individual patient living with EB is over half a million dollars on the US health care system, and we conflate value with price, right? So, if we say a curative lipid nanoparticle CRISPR gene editing therapeutic is a one and done $3 million approach, we react to that. But any business leader, if I could tell you after six years, you'll save half a million dollars a year on the system, that actually makes economic sense. But we're a system that's engineered for things like GLP-1s, right? Regular payment streams, low cost, market exclusivity drops after 10 years. But I'm optimistic because of the models that Nicola talks about in other countries, but also, we can think about things that we see in other payer, pooled investments that we see for disaster response, amortization of payments over time when somebody leaves an employer and gets a new payer system, that's thought about if we pay for an expensive upfront gene therapy.

William H. Lewis 35:23

David, let me go controversial on you. Because one of the solutions that could be helpful, and I know the reaction this will engender immediately, is extending the patent life of some of the medicines to bring down the repayment of that capital to a longer timeframe. Right now, what's happening is that's getting shorter and shorter. I take note that last year, the trademark for Mickey Mouse finally expired after 100 years. Okay? So, anyone using that image had to pay Disney for 100 years for that image. And we're trying to create a medicine that will save a life, and we've got roughly 10 years to get reimbursed for the cost. The intellectual property cost of Mickey Mouse was someone with a cocktail napkin and probably a couple of cocktails. The cost for us is a little over $2 billion and about 15 years. It's not reasonable to ask us to get that fully reimbursed in 10 years' time. So, I would propose that get extended. Now, that runs counter because you're taking a lot of those generic medicines and turning them into meaningful cures now.

David Fajgenbaum 36:27

That's right, Will, and I think that while we're talking economics, I think it's important to talk about all these approved drugs that we already have. So, 80 percent of our FDA-approved drugs, those 4,000 drugs, are already generic, which means they're inexpensive. It means that we understand how they work, we

understand their safety profile, but those are the very drugs that no one does any research on. So, we have this incredible set of tools that can help save and improve lives, but unfortunately, no financial incentives to advance them forward. And so that's why I shared earlier my personal journey repurposing a drug that saved my life. But after we did this 13 more times in my lab at UPenn, repurposing drugs for patients with horrible rare diseases, that's when we started a nonprofit called Every Cure to try to take on this huge problem. And that's that 80 percent of the drugs that we have are generic, and no one's doing any work to find new uses for them. Since we launched Every Cure three years ago, we built a really powerful AI platform that scans every drug versus every disease, quantifies and identifies the most promising new uses for medicines. Not necessarily the best treatment for one disease that you point out up front, but really, what is the best opportunity to help people with the medicines that we have? And then we do the laboratory work, do the clinical trials. Then we go out and search for the patients to benefit from those medicines. So, we've got a drug that we're getting ready to take to patients for a rare disease called Rosai-Dorfman disease, a disease I'd never heard about before. Every Cure identified that lenalidomide could be a good fit for this. We've got 10 active programs. And so, from an economics perspective, there's no financial upside. You cannot make any money off finding a new use for these medicines. But you can save a lot of lives. You can help a lot of people with medicines we have, and they're inexpensive. And so, trying to come up with the right models here. We're fortunate that the US government, ARPA-H, led by Alicia Jackson, has invested $124 million into our nonprofit, which enabled us to build the platform, do laboratory work, and clinical trials. But it's sometimes called a missing market. And the estimate actually is that if we extended patent life by five more years, there'd be 200 more treatments that would be almost immediately available to patients. If we extended patent life by 20 years, it would be 800 new uses for medicines.

Becky Quick 38:35

So why does extending the patent life—because it almost sounded like you guys were going to be at odds. It sounds like you agree, extending the patent life.

David Fajgenbaum 38:41

Yeah, extend the patent life, they will—

William H. Lewis 38:42

—I wasn't expecting that answer. *[Laughter]*

David Fajgenbaum 38:43

Yeah. Well, so if you extend the patent life, they will have more incentive to find more uses for their medicines. They will not stop looking for new uses for medicines when the patent life is happening.

Becky Quick 38:54

So, you can team up with a company that has a lot of salespeople who can get into the marketing, to explaining it to doctors who are there on these things? Or what does that mean?

David Fajgenbaum 38:59

It's part of that. So, when a drug company pursues one drug, on average, they consider 15 to 25 diseases for that particular drug.

Becky Quick 39:08

Okay.

David Fajgenbaum 39:08

They pursue a handful of them, and they have to go for the handful most profitable opportunities, and they leave 15 to 20 by the wayside. And as science progresses, we learn about more and more ways that drugs might be useful in new ways. A great example is Viagra. Everyone knows that it was developed for heart disease before erectile dysfunction, but it's also a treatment for a rare pediatric lung disease because it shares the same mechanism in the body. The only reason that that was developed was because it was early in the patent life. And so, patent life is actually the thing here that leads to drug development.

Becky Quick 39:37

Okay.

David Fajgenbaum 39:37

And if we can—

William H. Lewis 39:37

—And you get leverage because we've already put in all the effort.

David Fajgenbaum 39:40

Yes.

William H. Lewis 39:41

All the heavy lifting. This is a profession. How absurd is it that we have to have global manufacturing, redundant, qualified, ready to go when 80 percent of the medicines are going to fail? But we're ready to produce them at quantity because the day that's approved, you've got to have it out there in the marketplace. And that global infrastructure is massively expensive, but it's absurd to have to go about that. Now, if you have made all those sunk cost investments and you have the ability to apply it to other diseases, absolutely we would be lining them up. I can tell you right now, we have a successor molecule to the one that was just approved that we are developing for different diseases because there is not enough patent life left on the first one.

Becky Quick 40:19

Yeah.

William H. Lewis 40:19

So we're going all the way back through the whole process all over again as a consequence of IRA, cutting more time off of the duration when we can recoup the cost of the investment. It's a frustrating situation, but this is how we try to work around it to make it still viable to develop the medicines to accomplish the goal that we all share, which is to have impact on patients' lives.

Becky Quick 40:39

But David, you're finding these things because you're using AI at the beginning to take every permutation of every drug and match it up to every disease profile that you've found with these things. What I think is really interesting about what you did, too, is you're coming at it from the bottoms up instead of the top down.

David Fajgenbaum 40:59

Yes.

Becky Quick 40:59

You're not doing it from a family saying, "Here's some money. Find a way to fix this." You're looking for the low-hanging fruit. You don't care what disease it is. And as a result, you're not really getting funding from the families either, which was a much harder route for you to go from.

David Fajgenbaum 41:11

That's exactly right, yeah. What we learned when we started Every Cure is that almost all medical research funders have specific diseases they care about. They're connected. I care about glioblastoma because my mom died from it. We all have specific diseases, but what we realized is in this entire system where we send billions and billions of dollars, there was no entity that was just looking for the low-hanging fruit. What drugs could help what people? And if you don't care what the disease is, you just want to help people, this is the way to do it. But it just was so surprising that there wasn't this sort of—I don't know what the right analogy is, but it's almost like you're looking for low-hanging fruit, or it's almost like the leftovers, the scraps that are not financially viable, but they can help patients. And I can promise you, as a patient who's alive because of a repurposed drug, we don't care if it's a new drug. We don't care what it was made for. We just want a drug that's going to help us. And when we get to see patients who are alive today on drugs that weren't made for their disease, it just drives this flywheel forward.

Becky Quick 42:03

You mentioned Viagra as one of the examples, but isn't Botox also another drug that you're looking at that might have something better than just ending wrinkles?

David Fajgenbaum 42:11
Yeah. One of our active programs is actually Botox for major depressive disorder. There's actually really strong clinical data that glabellar injection of Botox can improve mood and actually reduce major depressive disorder symptoms for three to four months. But it's not being developed for that, and so that's one of the 10 programs that are active right now.

Becky Quick 42:30

Okay.

Neil Kumar 42:32

Just to jump in on that.

Becky Quick 42:33

Go ahead.

Neil Kumar 42:33

It's a cool paradigm when you think about observing a phenotype like, okay, Viagra was an observed phenotype. GLPs actually were that, too, right? Okay, you've got weight loss. The other neat thing, though, in genetics is, people forget that statins came from a study of familial hypercholesterolemia.

Becky Quick 42:48

Yeah.

Neil Kumar 42:48

So there's a lot of the wonderful databases that you've been involved with that we can now start to use and say, "I have a drug for this target. Where are there overlapping pathomechanistic signatures in broader conditions?" And you can start to then elaborate on several different opportunities for any given therapy, which I think has to be the future, with IRA being a challenge there. But I think broadly, that has to be the future for a lot of these medicines to make them economically viable.

Becky Quick 43:17

I want to kind of weave some questions in from the audience at some point, too. Here's one that came up that I'm kind of interested in. This comes from a parent who says: "What advice do you have for parents

who are walking this journey every day, who want to bring more visibility to an ultra-rare disease, and who are eager to raise funds to advance treatments?" What would the advice of the panel be on this?

William H. Lewis 43:39

Well, the first thing I would do is go to the key opinion leader in the disease in question. Find out who the person is who knows more about this disease than anybody, and that is just a process of calling around and doing research. That individual can often take up the challenge of linking up with a company or through the tech transfer office if they're at an academic university to kick off focused development for that particular condition. That's the way that I see it happening most effectively right now. I think there need to be additional ways that make that more efficient. But there are stories where people go, and they find the cure that they need by linking up with a KOL and a company who's willing to take on the narrow indication.

Michael Hund 44:30

I was going to say, look, the reality is we can't accomplish our missions without raising the capital and dollars to do it, right? And this remains the biggest barrier for any rare disease family. I think it starts with storytelling, right, which you've done, Becky, and I think we've really focused on to personalize and humanize the stories. I think the second thing is trying to think bigger than your one disease. Again, this is a massive market inefficiency that every rare disease foundation, as small as they are, there's multiple foundations, there's multiple competing interests. So, trying to tell a story that's bigger than your one rare disease is incredibly important. So, if you look at our growth in revenue, now we have 90% of our revenue coming from people not even connected to EB, right, because they believe in the accomplishment. They want to be part of a team curing a rare disease. I think looking for collaboration, are there rare diseases that are similar? If you look at the therapeutic landscape, are we targeting similar therapeutics? It may be messenger RNA or gene therapy or immune therapy, so how can we collaborate? And now is a more exciting time than ever to think about scalability and platform technologies because we're seeing it in science, right? My friend, Annette Baker, is here from Children's Tumor Foundation. They just launched in the UK platform basket trials, right? So, we're going across multiple diseases. I'll give you some hot breaking news, Becky, because that's why we're here. We're preparing to announce a collaboration with Stanford University and Dr. Jennifer Doudna, the Nobel laureate inventor of CRISPR, that's umbrella trials. So, we have a CRISPR-Cas9 lipid nanoparticle that applies to multiple monogenic rare diseases. Why would we go five years here, seven years here, seven years here? We now have a green light from the FDA to run those umbrella trials, right? So, trying to find those adjacent and community rare diseases so we can do this at scale.

Becky Quick 46:19

Repeat that. I think that's really important.

Michael Hund 46:22

Yeah.

Becky Quick 46:22

And I don't want to skip by the breaking news aspect of this. What is so different about this? What does it mean?

Michael Hund 46:27

Yeah. So, this was a big barrier in the FDA for a long time. Not only did we have to run separate clinical trials for every disease, but we had to run separate clinical trials for every mutation. There are four mutations within EB. We now have been given permission to do this at scale, so we're piloting and trying to model where we're using that target, CRISPR gene editing approach. We're a monogenic rare disease. There are other ones. So, we have one subtype of EB called junctional. Children die at one years old. So, we also now have compassionate use. We saw it with Baby KJ last summer. We don't need to take five to seven years because we don't have time because children are dying. So, we can run these trials quickly. We learned it from Operation Warp Speed. We're doing the second subtype with recessive dystrophic EB, and we're in the process of identifying three other monogenic rare diseases so we can learn and do clinical trials at scale.

Becky Quick 47:15

Is that the equivalent of basically saying this is like a medical device? If we prove that it works we can use it for different targets to go after. If it's safe on this, it should be safe for all these other areas.

Michael Hund 47:25

In a lot of ways. And we're all trying to find safety. You can look at safety across multiple rare diseases and actually learn more about the safety and efficacy of that therapeutic. By the time you get into efficacy, you're looking to see how effective it is in different rare diseases, right? So, it's a new model that I think all of us as rare disease leaders, but it takes building that community. What are the diseases that are adjacent to us? What are the diseases that are using similar therapeutics to target? So really exciting news and more to come, but look forward to working with many other rare diseases on how the rising tide could lift all ships.

Becky Quick 47:57

Neil, how do you choose which diseases to go after? What makes an attractive target? What makes something you feel like, okay, we're going to say yes to this and no to these 17 others because you can't say yes to everything.

Neil Kumar 48:10

Yeah. Firstly, we have to believe we can either be first or best in class, and we have to have a deep relationship with the patient advocacy community from the get-go. That is really how we learn how we can best serve. The third thing is we really look at mechanism biology. We tend not to do anything unless we can really understand how you go from the genetics to what's happening in the cell, to what's happening in the human body and really map that out. That's what we believe leads to a higher probability of technical success, which is really required if you're going after smaller markets. And then the final is we do look at economic viability, but I think we have the approved product for one of the rarest conditions out there, molybdenum co-factor deficiency type A, which was 200 children.

Becky Quick 48:50

Yeah.

Neil Kumar 48:50

But we were able to run that phase three in $50 million. We got a pediatric review voucher that we sold for $110 million. So even though Wall Street didn't care that much about it, I could still go back and say it was economically viable. It was the right thing for investors, also the right thing for the patients that we serve.

Becky Quick 49:05

Because of the voucher that you-

Neil Kumar 49:07

Yeah, because of the voucher. So, we've been able to elaborate on all sorts of different business strategies to get in an economically viable way first or best in class medicines with high probability of technical success to the finish line, and that's basically all we look for. We could do any modality, any therapeutic

area, work with all sorts of different KOLs. We can start in phase three with a repurposed product like we have for achondroplasia, or we can start all the way at screen. Just really depends on the condition.

Becky Quick 49:35

David, is there a way for you to work with some of these drug companies? I know you do this all in-house with Every Cure, but if you link onto them, if they still have the patent protection on it, it seems like it'd be in their best interest to work with you on some of this stuff.

David Fajgenbaum 49:47

Yeah. We'd love to explore more ways to do that. So right now, our AI platform quantifies the likelihood of all 4,000 drugs to treat all 18,000 diseases. So, we generate 75 million scores from zero to one. And then we rank order those matches of every drug versus every disease to determine what's at the top of the list, what match scores a .999, and then we go after that. That's how we pick the disease drug to go after. It's based on rankings. Our medical team reviews about 1,000 AI-generated ideas per month. And so, I say that, and so we get all these promising opportunities. We have 10 active programs. We have another 15 coming down the pipeline and literally many thousands more waiting, and truly thousands of repurposing ideas waiting to help patients. But the reason I mention it is that eight of our—or sorry, nine of our 10 programs are with generic drugs. And that's, I think, one, a factor, just the fact that most drugs are already generic, so it's not too surprising that nine out of our 10, it's given that 80 percent are generic. But the one out of 10, and I'd love for us to explore ways to work with pharmaceutical companies moving forward. I'll share about one of our programs that I may be most excited about. It's a very rare condition called Bachmann-Bupp syndrome, which has only been described in about 20 kids thus far in the world. And these children have a mutation of gene called ODC1. And unfortunately, they have severe intellectual disability, developmental delays, and have a difficult time engaging with their loved ones. There was a drug made for African sleeping sickness 50 years ago called DFMO that inhibits ODC1. No one had ever even thought about the possibility that this African sleeping sickness drug could treat these kids, but it is an incredible inhibitor of ODC1. And of the six children or six patients that have been treated, five of them children, all six of them have benefited. The five children have had really striking improvements. And this is the kind of thing that we can go after as a nonprofit. We can work on Bachmann-Bupp. We expect we'll find a couple hundred patients around the world, but we want to make an incredible transformative change for the couple hundred we'll be able to help.

Becky Quick 51:46

Just on that exploration, your own story of how you found the drug that has cured you or has saved you to this point, you almost died five times in three years. A priest read last rites over you. Your family came to say goodbye to you over these times. And yet somehow you were still thinking through, and just the idea that you found this yourself. You had a disease—I think that it showed up in all the lymph nodes. And they were trying to use drugs that help the lymph nodes. You were smart enough to think further up the

chain, that it's not necessarily the lymph nodes. You went after a drug that went after the immune system's response itself. Talk about how you even thought of that.

David Fajgenbaum 52:27

Sure. Well, in my case, I had this incredible family, amazing. I had an amazing girlfriend at the time, who's now my wife. And I had made a promise to my mom that I would try to discover drugs in her memory. So, I had all these reasons to try to fight and see if I could find a drug that could save me. And I didn't have a billion dollars and 15 years, so I couldn't make a new drug. And so, the obvious next step would be to find an old drug. And I hoped so badly that from the experiments I ran, that I could find something. And the moment that that drug saved my life, as I said earlier, I just haven't been able to stop thinking. I got so lucky. I was a medical student at Penn. I had this amazing family supporting me. I had all the reasons in the world to find this drug. But there's a lot of other people who don't have these resources and opportunities. How do we find them so that some medical student doesn't have to get a disease and then go search for it themselves?

Becky Quick 53:19

Yeah. That's brilliant. If you haven't read his book yet, read it. I'm telling you. I think there's something else you put in it that really blew me away, and that was the idea that passive hope versus active hope. And I think we all have passive hope. What got you from passive hope to active hope, and what's the difference?

David Fajgenbaum 53:39

Yep. Excuse me. Passive hope was the kind of hope I had when my mom was diagnosed with brain cancer, was I really hope that her doctors are going to find something for her. I really hope I can go to the expert and it'll all work out. And she died from her cancer a year later. And in medical school, you have a lot of passive hope. Hopefully, we find something out. But active hope is where you say, if I'm going to hope for a treatment, I'm going to pull a Michael Hund and I'm going to work so hard to find treatments for EB. If I'm going to hope for this thing, what can I do today, tomorrow, and the next day to create the best environment possible for that hope to become reality?

Becky Quick 54:15

Bravo. It's something that I've put into my vernacular now, and I'm actively thinking about that.

Michael Hund 54:21

Love that.

Becky Quick 54:21

And I thank you for that. We only have a few minutes left, so I'd like to ask the panel if there's one thing you want to leave this group with. Let us know, what's the message we need to take away? How do we work together? How do we find somebody else to reach out to? And Will, I'll start with you. We'll just run down the line.

William H. Lewis 54:39
I'm incredibly optimistic about where we are at this point in time. We have this intersection of AI and its potential to sort through the massive amounts of data that is generated by our industry. There is clearly awareness and vocalization and asymmetric impact that individuals with these rare diseases can have that is creating outcomes that are, in some cases, curative, like cystic fibrosis. So, the paradigm is there for how this can work for everyone's benefit, and I think that just lays a foundation for us to all feel pretty energized about being able to go out and make an impact.

Becky Quick 55:13

Neil?

Neil Kumar 55:14

Great point. I guess I would say my one takeaway is that the numbers that we're all talking about don't really capture the importance of problems that we're working on. About 30 million Americans affected by rare genetic disease, but their families, their siblings, the longitudinal data that you have in terms of the cost to the health care system. But also, we met one of the young children that is alive today, maybe one of the first dosed in MO-CD type A, and he's eight years old. He's playing with his family. He's going to school. He would otherwise not be with us, unfortunately, given the devastating nature of the condition. You can't really capture that in numbers. I know we have to in society today, but he could go on to do almost anything. And I think we have to tell those stories. You guys have all told those stories very, very profoundly and proficiently, but we need to do more of that. Otherwise, this industry is just going to be a bunch of me-too drugs, and it's not going to be all that exciting.

Becky Quick 56:11

I loved what you said on a podcast, I think, where the podcaster was asking you and telling you how courageous you were, and you said you drafted off of the courage of others. And I think those are the families that you're meeting with all the time.

Neil Kumar 56:24

Definitely.

Becky Quick 56:25

Yeah.

Neil Kumar 56:25

That's quite right.

Becky Quick 56:26

We appreciate it. Thank you. Nicola?

Nicola Blackwood 56:29

I'll pick up on exactly that point. Everyone in this room, I think, knows how isolating it can feel to have a rare condition and how completely all-consuming it becomes trying to manage it, trying to get through in order to be able to cope. But don't forget that the outcome of that is that you have tremendous power within this system because you have knowledge and experience and expertise in your condition, which actually very, very few other people have. So, for us, we built the 100,000 with patients in the center of it. They helped us design it. We built our newborn sequencing program with patients at the heart of it. The MHRA is building a complete restructure of the regulatory process with patients at the heart of it. But it only works because of that hard-won, almost unbearable process that you have to go in order to get that experience and that knowledge. And this is something that your general regulator doesn't have, your decision-maker in government doesn't have. They only find out when you tell them, when you join

together with others in the community, and you explain the realities, and you set out that pathway to reform and change. And we've seen extraordinary steps already, and we're on the cusp of even more because the science is there. We now just need to de-risk the process to get us there, and it will be you that helps us do it.

Becky Quick 57:49

Thank you, Nicola. Michael?

Michael Hund 57:51

What I'm most hopeful about and optimistic about is rare is in fact not rare at all, right? More people than cancer and HIV combined. And while there's so much diversity within those 7,000 to 10,000 rare diseases, what patients and families want is shared. We want data, we want information, we want what's going to improve our child's life. We want to raise dollars, and ultimately, we want therapeutics, and one day, a cure. And what I'm hopeful for and the steps that we're taking, if we've got a model that works, and we only know that it works now because we've made so many mistakes, but we were just unafraid by the mistakes to focus on a vision, sharing that with other rare disease organizations. Because I'm looking out at so many parents and patients, like Becky and David. You are warriors. So, let's build that army across rare diseases, and you see a remarkable impact of what we can do together.

Becky Quick 58:42

David?

David Fajgenbaum 58:44

I'll just say that we have so many reasons to be hopeful. There are so many amazing leaders advancing new drugs, new technologies, artificial intelligence, so many reasons to be hopeful on the new drug development side. And I think thanks to artificial intelligence, thanks to the fact that this nonprofit, Every Cure, exists, so many reasons to be hopeful on the existing medicine side, finding new uses for medicines. Again, they might not be a disease you've heard about, but if you just want to maximize the impact for helping humans per unit time, per unit dollar, it's using all these amazing drugs that have already been developed, all those drugs that are already out there. And I'm just so excited for us to keep going one drug, one disease, one patient at a time.

Becky Quick 59:20

I could not have thought of a better panel, so I want to thank the Milken Institute for bringing these five incredible people together. I am grateful for your time, and I can't tell you how happy I am to be here. Thank you all.

Announcer 59:34

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