Ep. 8: Rare Disease; Rarer Organization: How EB Research Partnership Drives Rare Disease Research

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Matthew Hittle: Hello and welcome to another edition of *OnAir: Health Care*, a podcast by Akin Gump that delves into the connection between health care policy and health care science. Today, we are joined by Michael Hund of EB Research Partnership. This is Matthew Hittle, senior policy advisor here at Akin Gump.

Dr. Mario Ramirez: And I'm Mario Ramirez, a consultant with Akin Gump.

Matthew Hittle: Mario, we've got a wonderful episode today. We're doing something a little bit different than we typically do. There are 400 million people in the world who've got a rare disease. Ten percent of the population of the world have a rare disease, and that seems to belie the term “rare” in “rare disease,” but many people have a connection to rare disease, whether they know it or not. Today, we're bringing on Michael Hund from EB Research Partnership, which is an organization designed to find treatments and cures for rare diseases.

Michael joined EB Research Partnership as CEO in 2017. Previously, he was with the Multiple Myeloma Research Foundation, leading a successful $100 million campaign that drove the achievement of the largest longitudinal genomic data set in cancer, 10 approved treatments in a decade, and the tripling of patient life expectancy. That is quite a background. He received his MBA from Yale and a degree in philosophy from the University of Kansas. He's a guy after my own heart with that. I have a philosophy degree as well. Michael, thank you for joining us today. We've talked about your background, all the cool stuff you've done, but could you please tell us a little bit about yourself behind the bio?

Michael Hund: Thank you, Matt and Mario, so much for having me today. Thank you guys for not only having an amazing podcast, but one that can shine a light on organizations like ours. Big, big thanks. I think you said it perfectly, Matt. The other things that maybe weren't in the bio: I'm a country kid. Hence, the University of Kansas. I grew up on a cattle ranch in the Flint Hills of Kansas, which I think is significant, because from a very early age, I understood the importance of a community and a community that can work together to accomplish really big, seemingly insurmountable goals.

Look, my life was changed when I was 18 years old. I got in a beat-up pickup truck and I drove from Kansas to the woods of Connecticut. I had learned about a camp for kids with serious illnesses—kids with cancer, sickle cell, HIV, and other life-threatening diseases—that was started by the late, great actor, philanthropist, entrepreneur, race car driver, Paul Newman. And his concept was, look, kids that battle very serious illness and disease oftentimes miss a lot of their childhood. This is particularly unfair or, in Mr. Newman's words, particularly unlucky.

He felt we ought to provide a camping experience where they can be kids and ride horses and play sports and be in a medically safe environment, but most importantly, meet other young people that were going through the same thing. That was really a life-changing moment for me. I worked for Paul and Hole in the Wall Gang Camp, an amazing organization, for a decade. But what I became focused on, being a very solutions-oriented person for as long as I can remember, is [*that*] disease is rough. The tough part of the job was experiencing the loss of life in young people and seeing the impact that that had on their families.

It was really that experience that made me want to go to the root problem of solving the disease itself, and the medical research. I joined the Multiple Myeloma Research Foundation (MMRF), as you mentioned in the intro. What was really unique about it, a mentor of mine—and mentors are so important, that's my biggest advice to young people is find yourself a or a group of really good mentors—was a woman named Kathy Giusti. Kathy was diagnosed with multiple myeloma more than two decades ago, given just a year or so to live, had a young child and was told to really get her affairs in order because research hadn't changed over really 50 years.

She said, "Look, that's a fate I'm not going to accept." She was a Harvard MBA, had worked in the pharmaceutical industry, and she really believed just because we're a 501(c)(3), that's a tax status, not a business model. We ought to operate like a Fortune 500 company. She was a mentor to me, inspiring me to say, look, if you want to accomplish big, bold, audacious goals like curing EB or tackling rare disease, you ought to have a really good business plan and a business education. She inspired me to get a business degree to focus on things like new innovative investment models that can accelerate research and development for rare diseases, and it was that journey that brought me to EB Research Partnership.

Dr. Mario Ramirez: Michael, that's an amazing story. I think a lot of our listeners are probably not familiar with EB, which stands for epidermolysis bullosa. I've taken care of some of these patients in the ER, and it's a devastating diagnosis. Can you tell us a little bit about what EB is and who the patient population is that's affected, some of the symptoms, and the state of treatment and cure for the disease right now?

Michael Hund: Absolutely, Mario. I had been in pediatric disease from Hole in the Wall Gang and working in blood cancer for a decade. I had never heard of epidermolysis bullosa. We'll call it EB for short because it's much easier to say. Having worked in pediatric disease for quite some time, I had never seen something quite as devastating as EB.

What is EB? EB is a life-threatening rare genetic skin disease. Most people forget that skin is the largest organ in the body, right? When your skin is under

attack, it affects about every single piece of our ecosystem as human beings from a health perspective, from a psychological perspective.

It's really a devastating disease. Kids are born with EB, and oftentimes the parents have never heard of it until their child is born missing sheets of skin. What EB does is it's caused often by one genetic mutation, which makes it a monogenic rare disease. In that mutation, it can be in different genes, but oftentimes in collagen VII it really causes the most core function of skin healing not to work in kids that are born with it. Kids that have EB, from the moment that they're born they face excruciating pain. Oftentimes it's compared to living life as a burn victim: constant wounds, full body bandages, treatment around the clock, a host of—not only the hospital that you're at—therapists and occupational therapists and physical therapists and dermatologists and clinicians and researchers. Life is spent really fully consumed by the disease itself. Kids with EB are called “butterfly children” because their skin is as fragile as the wings of a butterfly. Oftentimes you'll hear EB called the butterfly disease or butterfly children. Things that seem simple to us like eating, walking, drinking become monumental tasks for kids that battle this disease.

Something as simple as a child bumping up against the corner of a table can become a scrape and a wound, and a wound that never heals. This is the really tough part, the really devastating part. It's a brutal, brutal disease. The good news is because it's caused by one genetic mutation, we and the medical community that we work with believe that healing it, treating it, curing it by the end of this decade is a realistic possibility that we all race to.

Oftentimes that's done by a vast array and portfolio of approaches, things like gene therapy, gene editing, protein replacement, messenger RNA technology, exon skipping, even immune therapies, knowing that squamous cell carcinoma is one of the leading causes of death in EB. In summary, it's a really, really devastating disease that varies in life expectancy from one year old, to the least severe forms with children making it out of their teens and into their thirties. But as brutal as it is, it's solvable. It's solvable not only in the near term, not only in our lifetimes, but, potentially, by the end of this decade.

That's what gives EB Research Partnership, myself, the patient community, the family communities hope that life is too short not to work towards goals and big audacious outcomes that we believe are achievable. Here we have one in front of us that's solvable with the collective skills of the communities that we work with.

Matthew Hittle: Michael, one of the things that really caught my attention as I was learning about EB is the fact that in lieu of treatments or cures, patients really have one option, and that is intricate bandaging—rituals, almost like—every couple of days or every day or whatever the length of time is. These patients who are children and their parent caregivers who have full-time jobs and are working and maybe taking care of other children have to engage in this constant bathing to keep these wounds clean, and then intricately, depending on the severity of the disease, bandaging.

They become experts in first aid, I'm sure. Can you talk about what I imagine must be an enormous expense of these bandages and the process of keeping the skin clean, what role health insurance companies play here and other payers,

because as I said it must be extremely expensive in addition to being incredibly time-consuming because there is literally no other thing they can do.

Michael Hund: You're exactly right, Matthew. Look, you said at the top half, 400 million people with a rare disease. 400 million people with a rare disease, that's more people than cancer and HIV combined. It's nearly 10% of the global population, yet 95% of that population has zero approved treatments, and EB is one of them. We're quickly working to change that. But because of that, to your question, what's the reality of care today? There are no approved treatments for the disease. We say all the time, even if a parent is not a wound care nurse or a wound care specialist, they certainly become one very quickly because they have to be.

From the moment that a parent wakes up and has a child with EB, it's every single morning you don't know what to expect. Because wounds can happen so easily and so severely, it could happen in the middle of the night. The child could just accidentally scrape themself, and you wake up in the morning and you have a giant wound. Hours spent every single day in bleach baths, hours spent bandaging, doctor's appointments, because again, skin is the largest organ in the body. It's not just the skin and the wounds that you see, it's the effect on internal organs, osteoporosis, anemia, the immune system really being overworked just trying to care for and attend to wounds that can take place all over the body.

These parents have to become specialists in bandaging and wound care. The cost is enormous. It's not uncommon for us to speak to parents that are spending anywhere from $10,000 to $20,000 per month just on bandages alone. That can add up to hundreds of thousands of dollars every single year just in bandages, not to mention all the other care that's needed because of the effects and impact of the disease. It's incredibly expensive.

Something that we work with Akin Gump on all the time is that from a policy perspective, it varies from state to state, right? Some states cover bandages, other states don't cover the full bandages. Some states just don't have an understanding of the burden of the disease, really of how many bandages it can take. Certainly if you're not familiar with the disease, you may have a reaction to say, "There's no way that you can spend $200,000 a year on bandages," but it is the case. That's just the standard of care. That's in the absence of any treatment, just preserving the wounds, trying to treat the wounds to the best of your ability.

That's where we are today. Where do we hope it's going? When we started as an organization, there were only two clinical trials and they were both bone marrow treatments where the likelihood of survival was around 50/50 at best. It wasn't even a matter of “will the treatment work?” it's “will the child survive the surgery?”. That was a decade ago, which is pretty short, as you both know, in the grand scheme of medical research. In that decade, thanks to the work of EB Research Partnership, now we have 35 clinical trials. We have four phase 3 clinical trials for the first time ever. Of those four phase 3 clinical trials, three are gene therapies, and they're all ones in which EBRP is funded under a venture model.

So not only have we created this landscape where we've directly funded as an organization more than half of the 35 clinical trials, but because of our venture model, if you look at those three gene therapies that are now in phase 3 and, hopefully, will be approved by the FDA, those are things that we helped invest in

early on, but also more than doubled our investment and returned the ROI back to the foundation to fund more research until we cure the disease.

When we look of where we are, we still don't have that approved treatment. It's still incredibly burdensome for the patients and the families and the caretakers, but we're creating a future where we hope to see the first approved treatments in the near term, within the next year. After that, we are funding science and research projects that are beginning to enter the clinic that, by medical definition, are definitive and curative. Part of it is where we've been. We're in a much better place. Part of it is where we are, which is no approved treatments. The other part is where we're going, which is treatments on the very near-term horizon and right behind that, cures.

Dr. Mario Ramirez: Michael, you touched on the venture model there, which is pretty unique among rare disease models. Can you tell us a little bit more about what that model looks like, how it works and the role that EBRP plays in that ecosystem?

Michael Hund: EBRP has a really unique DNA as an organization that I think has led to this model and the evolution of the model. EBRP was started in 2010 by a group of parents that were just set out to save their kids' lives. There were other organizations, but there wasn't any organization just focused on solving EB, which means funding research and treatments and eventually cures that can impact the kids and families that live with it today. We were also lucky to have co-founders in a rockstar couple, in Jill and Eddie Vedder, lead singer of the band Pearl Jam, that have really helped expand not only the model, but the awareness for the disease.

Why the venture model? One of the founding groups of parents was the Silver family, and the long-term chairman of the board was a gentleman named Alex Silver. Alex and his wife Jamie's child was born with EB, Jackson. Alex was a Harvard MBA, worked in private equity in New York, so he understood finance and business models. This idea of just writing a check and hoping for the best didn't make a lot of sense to him and didn't make a lot of sense to the founders. The other part of how do you really build a model over time, it's sometimes from doing the wrong thing and learning from your mistakes.

Early on, EB Research Partnership had invested in at least one project that we helped to get into the clinic, we helped fund, and then eventually was a big commercial success, being bought by a household name pharmaceutical company. But the foundation that had funded it early on, as well as the patient community, didn't participate and benefit in that commercial success. So then the question is, how can we change this? How can we build a model that evolves this and addresses this challenge or this barrier? Ever since then, since about 2011-2012, we've raised over $50 million. We've funded 150 projects across the globe, all vetted by our renowned scientific advisory board.

What makes us unique is we didn't invent venture philanthropy. Groups like the Cystic Fibrosis Foundation certainly had much earlier and much bigger wins. But I think as a foundation, what we've done is institutionalize venture philanthropy in an investment model, meaning that every single one of those 115 projects that we funded has been under some sort of venture model.

What does that mean? Early on, we would fund universities that were working on EB projects. As part of our funding we would support these organizations with an upside that said, "If this is ever commercially viable, then EB Research Partnership participates in the success of that commercialization," meaning generating a return on investment back to the foundation to put towards future EB projects until we cure the disease and we're done. That's really how it started. In the last four years alone, how has that evolved? Twice a year we say, send us the most brilliant ideas to treat and cure the disease. Curative science will be prioritized. We want to see projects that have a plan to be in the clinic in the next one to four years.

That's a big part of what we fund now. Because of the landscape, that's realistic. We also evolved to say, look, you can be an academic medical center, you can be a public company, you can be a private company, you can be a startup company, a biotech or pharmaceutical. The structure doesn't matter. We're just looking for the best science and the best ideas. Because of that switch, we started to fund academia, but also private companies, also public companies. In the last four years alone, we've started four EB companies. We've been founding partners and founding equity holders in that model.

Last year, we took a couple of those companies, and we rolled them into a for-profit holding company of which the foundation is a significant shareholder. If that succeeds, and that's a portfolio approach of not only EB companies, but other diseases besides EB, if any of them succeed, that generates returns back to the foundation, again to fund more research until we achieve our mission. It's been core to our DNA and core to our fabric. I would make a strong argument that we've evolved that model to a degree in which it's a leader in the space, not only in rare disease, but all of medical research.

Matthew Hittle: And it's so different than most other rare disease organizations. I think many of our listeners, those who work on Capitol Hill and those who have, are very familiar with a wide array of rare disease and disease-specific advocacy organizations. Given how different you are, have you had a chance to partner with any of the current organizations that represent individuals with other rare diseases or those umbrella organizations that are out there? How much have you done of that, or are you out on your own?

Michael Hund: That's a great question. We feel a profound responsibility with other rare disease organizations. To go back to our stat, 10% of the global population has a rare disease. But in individuality, these are very small diseases. EB is somewhere between 30,000 and 50,000 people with EB in the U.S., around 500,000 worldwide. There are diseases that are much smaller than ours, as rare as that is.

We're in a unique position with venture philanthropy and the model for rare disease, because really if you look at some of the bigger disease groups, the big cancer groups, the heart diseases, diseases that affect a bigger part of the population, it's hard for those foundations to negotiate venture capital deals, mainly because there's so much other funding available. If we went into a university and tried to negotiate equity for a well-known cancer or heart disease or a disease that affects a bigger part of the population, it may not go very well because there's so many other places to get capital, whether it's government

funding or hundreds of foundations or just other opportunities. There's not as much leverage if you're a foundation.

When you're a rare disease, oftentimes, and this is the case for EB Research Partnership, we are one of the only sources of capital, whether you're a company or whether you're an academic bioinformatician or scientist working in the lab. It gives us the opportunity to say that any other business, any other sector, any other investor in the world wouldn't just make an investment and then not want any participation if it's successful. That doesn't make logical sense. So why should we?

This is applicable to most rare diseases. They're in a position to fight for every penny, to fight for every dollar, and really have good business models, which sounds so simple, but is so rare in the nonprofit space. We see patients as our shareholders. That's who we represent. Any investment that we make, we want to steward and be sophisticated in the same way because every penny matters, every dollar matters, especially when you're a rare disease that fights for every penny that we raise. It's a sustainable model that creates additional revenue sources from future opportunities. Because we feel that responsibility, we also feel very strongly about educating, teaching, and sharing that model.

Some ways that we've done it today, right now at Yale School of Management, a brilliant professor and thinker on health care, Dr. Gregory Licholai, is teaching a case on EB Research Partnership's venture philanthropy model, but also the efforts that we've taken to partner and build a first-of-its-kind patient-driven data platform. There's a case study that's being taught that's shared with other rare disease organizations, that's shared with young, brilliant business school students to think about how can we create this model, how can we take one stone and create ripples and have this model benefit other rare disease organizations.

I mentioned earlier Kathy Giusti, founder of the Multiple Myeloma Research Foundation. She put together a group via Harvard Business School and Bob Kraft called the Kraft Precision Medicine Accelerator. It was really cool. When we talk about innovative ideas, nonprofits thinking like businesses, the concept there was, let's take some of the leading medical research organizations across disease groups, and let's start a group to think about how we can transform and revolutionize health care, but let's bring in business leaders and leaders of sports teams and leaders of technology companies to think about how we can put speed into research and development for medical research, but also outcomes and deliver.

That's a group now, that's a very small group of leaders in the medical research space. Most of the organizations are household names. We're one of the smaller ones by disease size and foundation size, but one of the bigger ones as far as ideas on how to push and innovate concepts like venture philanthropy and evolve them into for-profit holding companies, into starting your own companies, into new investment models. That's another way in which we've kind of taken our model and tried to teach it in an academic sense between Yale and Harvard. We also partner with groups like Global Genes, that is a rare disease "corral-er," I guess you could say.

There's a case study through Global Genes now on not only the venture model, but the data model as well. And then we work with groups like the Milken Institute to attend the conferences, whether they be health care or otherwise, to talk about the model. I'm part of an amazing initiative called Changemakers, and they link leaders like myself with younger foundations that are just getting going so we can have formal mentorship and guide them on what we've learned. But the beautiful thing about mentorship, it's been a great program to be part of and work and teach other rare disease organizations, but I find I learn just as much from these new organizations starting out and the fresh perspectives they have.

So that's what we've done to date. I think looking into the future and what the next couple years hold, a sneak peek I suppose, but I think we'd like to think about how we can formalize that even more and institutionalize that even more to make sure that while we have our big, bold, audacious goal to cure EB by 2030, we can focus at the same time and in parallel on taking this model and helping other organizations along the way.

The rising tide lifts all ships. How can we do this in parallel as we pursue the cure for EB? We oftentimes say if we can cure EB, we hope that we can cure them all or at least help them make significant progress. We want to do even more of it than we do today. It's not only the business model. Because we are at the forefront of things like gene therapy and gene editing because we're a monogenic disease, we're entering, and I know you both know this, an era of medicine where we're moving towards scalable technology as medicine. Things like gene editing or CRISPR-Cas9 are scalable.

If EB can be a use case and first off the runway because we're monogenic, because we're observable on the surface of the skin, because we have a decade of experience doing this, how can even the scientific and clinical development work help other rare disease organizations as well? It's an exciting place to go. It's a massive opportunity. I'll take my moment to thank Akin Gump because Akin's really helped us think through that next chapter of the organization and really what our responsibility is to help other rare diseases.

Dr. Mario Ramirez: Thanks, Michael, that's really kind of you. You sort of headed off my question a little bit by talking about the crossover benefits to other genetic diseases. It strikes me that coming out of the pandemic, we learned a lot about using mRNA technology as a potential vector for gene editing. You talked about CRISPR-Cas9 and some of these other things. Are there any leading contenders within those technologies that look particularly promising thus far? Where do you think things are going exactly in the therapeutic space?

Michael Hund: That's a great question, Mario, and one that we think about all the time. I mean, progress comes in steps, which we all know. To look at first where we are today, there's 35 clinical trials in EB. That's remarkable for any rare disease and certainly remarkable given there were two a decade ago. If you look at the promise there, what's exciting is there's multiple approaches and multiple shots on goal, which is a great opportunity for patients that we have different approaches in the clinic, and we're going to find out which works best, but it's also a great opportunity to think about combination approaches and combination therapies.

If you look at the state of the union today as far as what's in clinical trials, you have everything from topical wound healing gels to the first generation of gene therapies, to protein replacement, to messenger RNA technology in EB—as you mentioned, the same technology that was used to create COVID vaccines—to squamous cell carcinoma identification through artificial intelligence, to immune therapies, to cancer treatments and oncology treatments that attack squamous cell carcinoma caused by EB. It's a really diverse portfolio, which is exciting.

The first off the runway, three of the four phase 3 clinical trials right now are gene therapies, and each one of the gene therapies has a different approach. One is a topical gene therapy, one is an injectable gene therapy, and one is a grafting surgical gene therapy. They're all taking different approaches. We hope that they all work, but there's going to be a tremendous amount learned about how they can work with one another as well. That's a real opportunity. The other phase 3 clinical trial is a gel that helps with wound treatment and is wonderful because it could be a standard of care the patients could use every single day without having to go into the clinic for something that's more invasive.

Protein replacement therapies are different than gene therapies because they're replacing that protein that doesn't work, the collagen protein. Exon skipping being another evolution of gene therapy and gene editing. Messenger RNA really delivering that correction to stimulate gene activity and in our case, healing of skin. And those are in the clinic now. Some of it sounds like science fiction, but it's a scientific reality.

What comes next? Last week, I had the great honor of visiting our partners out at the Stanford School of Medicine. Stanford School of Medicine now has a center called the Center for Definitive and Curative Medicine, which is pretty exciting to say, particularly when it comes from a place like Stanford School of Medicine in an academic sense. We like those words, “definitive and curative.” Along with some other leaders in the country like University of Colorado, they are preparing to, hopefully, enter the clinic within the next year to two years on gene editing. The way they describe these is definitive, permanently corrective, really fixing that gene mutation once and for all.

Some of the exciting things that are being done in the lab, quickly approaching patients, hopefully, as soon as possible, is actually just fixing that gene mutation. How can we fix something like a collagen mutation and now teach the skin and engineer the skin to generate collagen so wounds can heal? There's multiple groups looking at things like spray-on skin, which again, sounds like something you would see in a movie, but is a reality. You take things like iPS cells, you fix the mutation that causes EB, you create new skin, you can spray that on a patient. And not only is the hope that that would heal the wounds, but now we're into the territory beyond gene therapy, of regenerative medicine. The skin cells would continue to regenerate, and then hopefully the goal would be that we heal those patients.

When our founders started this organization from really having nothing to thinking that this is where we would be 10 years later, it's exciting, it's hopeful, it's optimistic, but we know that we're not at the finish line yet. These things energize us. I wake up every morning, as does our team and our staff and our board and those that help us like Akin Gump, and we're energized because these things are

here. Using the words “curative” and “definitive” and “permanently corrective,” I mean, those are things that we're preparing to support to enter the clinic.

There’s a lot of hope and a lot of optimism, but there's also this feeling of we can't turn back now. We're on that final chapter, that last mile, and that's the time when you really double down and put as much speed and urgency and funding and backing to get us across that finish line.

Matthew Hittle: Michael, one of the things you said at the beginning of this interview has really stuck with me throughout our conversation so far, and that is that 501(c)(3) is a tax designation, not a business model. Because everything you're talking about, all of this innovation is predicated on the assumption that EBRP is not a foundation or a traditional advocacy group, but rather it's a business, and you invest to then be able to reinvest. Now, there is a traditional funding mechanism for a lot of rare disease research that is, of course, through Congress.

Could you talk about some of the challenges or some of the work you've done when it comes to raising funds for research through Congress, NIH, CDC, etc.? I know that EB is on the list of the Peer Reviewed Medical Research Program at the DOD. Could you talk about that traditional approach, how you've used it and how it interacts with the venture philanthropy model that you use?

Michael Hund: Yeah, that's a great question, Matt. It's interesting, EB Research Partnership as a foundation has never received funding from government directly. It's a challenge. It's a challenge that I also think is an opportunity to address that there's so many rare diseases. If you look at the rare disease statistics, average diagnosis is five to eight years, half of rare diseases don't even have a foundation, very few ever cross the million-dollar fundraising threshold. It's difficult just from an organization perspective for the individual diseases really to unite. It's difficult for government to sort through 6,000 to 7,000 rare diseases and understand where the priority is.

The challenge isn't derivative of any blame on one individual, it's just the way things are. But we've really seen that start to change. I mean, first of all, you mentioned Department of Defense, the biggest funding that's gone to the EB community has actually been through the Department of Defense, which is interesting. You think it would be NIH or other government health entities, but it's actually been Department of Defense. A big reason is because wound healing and healing wounds has a lot to do with defense. It's made EB on the list of appropriations for very generous funding and awards.

Big shout out to our board member Ellie Dehoney, who works with Research!America, as well as the team at Akin Gump, really for putting this on our radar and making sure that we apply, making sure that we raise awareness, making sure that EB makes those appropriations lists. Because even in the last few years alone, there's been tens of millions of dollars that have been directed towards academic medical centers through the Department of Defense Peer Reviewed Medical Research Program, including some of the really innovative gene editing work that I described earlier at places like the University of Colorado and Stanford and Columbia and Thomas Jefferson University.

That's a great first step. There's funding that's been made available, maybe not from the government agency that you would be first to suspect, in the Department of Defense, but albeit one that's been incredibly generous and led

towards a lot of progress in the space. Looking forward, this is one of the things that Akin Gump has been just such a huge help for us to try to navigate where we should put our priorities and where we should put our focus.

What I have felt and what I have sensed and what I have seen is a lot of refocusing by the government at large on rare disease and how important it is for funding of rare disease, understanding agencies like the FDA, the large field that is gene therapy and gene editing. This has moved so fast. Like any disruptive technology, it's hard to understand and hard to keep track of, but there seems to be a real willingness by the government and various agencies to really want to understand that, to want to prioritize rare disease funding, to create opportunities for collaboration.

Again, as a cohort, more people are affected by rare disease than cancer and HIV/AIDS combined. But individually, these are really small diseases. How can we take that cohort approach and collaborate and whether that be some of the things that we've talked about, our model helping other rare diseases, technology platforms that can scale to other rare diseases, or just helping agencies understand that there's a really high unmet need? Again, 95% of 400 million people on the planet not having approved treatments is an opportunity to really innovate and shape policy around it. We have a great history of it, right? The Orphan Drug Act of the '80s. I mean, that was really instrumental of seeing a rapid evolution of just more approvals for rare disease treatments.

One of the beauties to bring all those ideas together and your question in some way, Matt, is what you said: business models, government engagement, private sector, public sector, how do we really bring those worlds together, and the Orphan Drug Act was a great example of the rare disease communities advocating to figure out a way to have more treatment approvals. How can we create strategy that benefits them? Companies need a way for taking rare disease therapeutics to market to be profitable and sustainable. We want to incentivize companies to develop in the space.

Regulatory agencies like the FDA need a way to unify these groups and do their part to help put speed to approvals and treatments. There was a great example of something that led to real change, and I think we're now at a point, and we talk about this often, of what's the 2.0 version of that? I think it has something to do with collaboration and sharing of best practices amongst leading rare disease organizations, building technology products that are open source and scalable so every single one of those 7,000 rare diseases doesn't have to create their own technology platform. It's very similar, the needs that we have as rare disease communities, and making funding available.

Groups like ours can go fight and just do everything we can, work every hour of the day to raise every dollar to keep our mission possible. We can think about how to leverage capital, whether it's from philanthropy or government funding or the private sector, to prove out some of these models in a way that we ensure that anything proved in one disease is scalable as a model, as a technology, as an approach that can help many, many other people that battle rare diseases.

Many of those things are starting to come to fruition. We think about it all the time. Again, just want to thank Akin because you guys have really helped us think through—there's a million directions you can run into when it comes to

government policy, but what are the ones that are going to have the highest impact for the patients that we serve? That's how we've always thought about our business model, and I think that's how we will need to continue to think about that great intersection of foundations like ours, biotech, pharma, academia, and government.

Dr. Mario Ramirez: Fascinating, Michael. I want to shift gears just a little bit because I know that EBRP was just recently recognized with the Horizon Prize, which is a huge deal. It's in part awarded by MIT. I was hoping you could tell us just a little bit about the award and why you guys were honored this year.

Michael Hund: Yeah, thank you, guys. It was a big one. We always get the highest charity ratings, and the case studies by some of the universities I mentioned are always good validation that you're on the right track. But this was certainly the biggest public display of an award and really just somebody championing our model on a stage like that. MIT Solve had partnered with Horizon Therapeutics to create an award. MIT Solve is a division within MIT that kind of does these “X prizes,” these big challenges, “send us your world-changing ideas and we'll pick the best one.”

Horizon Therapeutics is not a group we've worked with historically, but is a really smart pharmaceutical company that focuses exclusively on rare disease and their founder is somebody that has a personal journey with rare disease. Those groups combined. Really to summarize it as much as possible, they said, "Look, we want to know the most innovative technologies for rare disease to accelerate treatments and ultimately cures." They asked the world for those ideas. There were 200 applicants.

What's interesting, and to your point, Matt, about the intersection of business models for nonprofits, they sourced ideas from for-profit companies, from nonprofit companies. There were about 200 applicants from all around the world. They narrowed it down to five, of which EB Research Partnership was really honored to be one of those five. I believe we were the only nonprofit in that group and one of the only U.S. ones. We were invited to pitch our idea at the Concordia Summit, which is a great annual gathering of heads of state, global change makers, politicians and leaders from around the world. We pitched the idea on a Monday. We found out that afternoon we won, and then we had the opportunity to receive the award, which was a very generous grant, but almost bigger than the grant was the recognition from groups like MIT and Horizon on a stage like Concordia.

We had an opportunity to speak about our mission and our model in front of a room and a global streaming audience that was exactly the folks that we want to be in front of talking about what we do. It was a really, really big honor. One of the things that Horizon and MIT mentioned is why we were selected is not only because of the innovation of the technology, but because we went in as a patient research foundation focused on EB. Rather than just build a technology platform that mixes genotype and phenotype, meaning genomics and patient-reported outcomes, and puts patients at the center, giving them agency and control over their own health data, but connects them with the academic medical center world, the clinician world, biotech and pharma, and eventually seeks to help agencies like the FDA when they have to review lots of data to make approval decisions on treatments.

To not only do that for EB, but think holistically about building a product that could work for any rare disease, but just having EB be the use case as we build that, with the intent to really make this available to any rare disease organization as the need for good data platforms, which I would argue is just about every single one. It was an exciting moment for the foundation, for the organization, and I think just the start of really large things that we can do with technology. We've been lucky on that project to partner with Amazon Web Services, GeneDx, as well as Stanford Center for Definitive and Curative Medicine.

We've got a great team. We've got great partners, and this is something that really benefits patients. There's not a lot of resources for patients to have an easy way to curate their journey with the disease. It can be really difficult sorting through the Internet and social media and trying to find trusted, validated sources. We just started out with a big bold idea that said, what if navigating and curating your journey with the disease could be as easy as entering a destination into a GPS? But instead of left and right turns, it's the right treatment for the right patient at the right time.

That simple concept has evolved very quickly into not only something that can shave years off of treatments and approvals and cures for EB, but help many, many other rare disease organizations along the way.

Matthew Hittle: I want to put a finer point on that, Michael, because I think you're being humble. This platform that you've developed is really groundbreaking for rare disease. I come from a part of the country, Iowa, that has some very rural areas. I know you do too. I think about someone in a rural area who has a baby born with EB and is not expecting it at all because of no screening. The first they've heard of this disease is their baby who is in immense pain, and they just don't know how to deal. The first thing they do, like all of us when we're sick—sorry, Mario—is we Google it and we go to the Internet for answers.

With a total lack of reliable personalized information, it can lead not only to worry, but needless speculation on the part of the parents, and it just doesn't help connect their child to experts or connect them to experts any quicker. I think that your model here, this platform, is just so neat and, I think, has application to so many other rare diseases that are in the same boat as EB.

Now, I would like to finish by talking about the star-studded event that you are going to be holding here, I believe, in April, and you've held it in the past, called Venture Into Cures, which is your big fundraising effort. I'd love for you to tell our listeners about Venture Into Cures and some of the folks in Hollywood and elsewhere that you have joining you on that.

Michael Hund: As you both said, there were plenty of challenging things about a global pandemic, but there was also a lot of silver linings, and a large digital event called Venture Into Cures was one for us. The pandemic happened, we couldn't do fundraisers, we couldn't do in-person events. We said, look, we've got to do something. Let's figure out a way to continue to raise awareness, share the stories of families and patients that battle this disease, share the stories of the medical community that works day in and day out for solutions for the disease. So we started talking to Jill and Eddie Vedder and said, "What can we do, guys?"

We all started making some calls. The first year, we had had amazing stories of families, we had filmed content before, so we didn't have to go into people's homes and film, but we combined that with Judd Apatow and Bradley Cooper and Laura Dern and Billie Eilish and Chris Hemsworth and Jimmy Kimmel and David Letterman and Adam Levine and Willie Nelson, and of course, Jill and Ed and just got all these great celebrities to stand up for the cause. It ended up being our biggest fundraiser ever. It was seen by more than a million people in 2020. Further reach than we'd ever had before and bigger dollars than we'd ever raised before.

Of course, last year in 2021, we did the same thing. We were so lucky, the amazing actor and just all-around great human being Tom Holland, also known as Spider-Man, hosted the show. Jill and Ed were back with their leadership again, but brought in Ed Sheeran and Selena Gomez and Zendaya and just an amazing cast of people. It's humbling when you're in rare disease to get support. The real rock stars and celebrities are the kids and families that battle this disease and the doctors that work so tirelessly to treat and ultimately cure the disease, but it helps when you get names like that because it just widens your platform and widens your reach.

This year, November 20th, www.ventureintocures.org, 4:00 PM Eastern Time, we'll be having our third digital event. I won't leak any names just yet, but I can assure you that the talent will be perhaps one of the best that we've ever had. I say that with the celebrities, but I say that more for the kids and families and doctors that will be sharing their stories. So don't miss out. You can go to our website, ebresearch.org, to learn more or check out ventureintocures.org also to learn more. Please tune in. Akin Gump has been a supporter since the very beginning.

You'll learn a lot about EB, you'll hear some good music and see some familiar faces of those Hollywood and entertainers that you mentioned, Matt, standing with us on our mission to cure EB by the end of the decade and lead the way for the 400 million people with rare disease.

Matthew Hittle: That's wonderful. Thank you for talking about that. It's been a real honor partnering with you to help achieve treatments and cures for EB. I urge folks to go to EB Research Partnership's website, ebresearch.org. Just read through some of the stories of these children. Really tugs at the heartstrings, but it's important for you to see the faces of the children who are affected by this disease. It'll really give you some understanding as to why folks from all walks of life are coming together in an event like that. Michael Hund, who is the CEO of EB Research Partnership, thank you very much for joining us.

Michael Hund: Thank you, Matt. Thank you, Mario, and thank you, Akin Gump.

Dr. Mario Ramirez: Well, Matt, I thought that was just an amazing episode. I think the work that Michael's organization is doing is just incredibly impactful, and I'm fortunate when I get to split my time between Akin and my clinical practice and have a chance to see how policy plays out for some of the patients that I see in the ER. I thought his description of the funding model that they use and the approach to policy that they have is just great. I'm really hopeful to see where the organization goes. If they can really cure EB within the next decade, that would just be an incredible milestone for science.

Matthew Hittle: Mario, the one thing that really stuck with me was that their tax designation is not their business model, and that ethos allows them to go beyond the traditional "box" that is the rare disease organization. Of course, there are myriad benefits to those organizations and they do great things, but being innovative, I think, is the result of this mindset. I'm just so excited to see where they go with this new platform that they worked on with Amazon Web Services and for which they received the Horizon Award. I think they're going to do great things not only for EB, but for the rare disease community at large.

We'll definitely keep track of what Michael's up to. We're proud to partner with him here at Akin, and we'll keep our listeners updated on what's going on with him and what's going on with EB Research Partnership. As always, a special thanks to Sean Feely, policy advisor here at Akin Gump, for working with us behind the scenes on this episode. In closing, this is Matthew Hittle, senior policy advisor here at Akin Gump.

Dr. Mario Ramirez: And Mario Ramirez. I'm a consultant here with Akin Gump. Thanks for joining us.