



EB Research Partnership Venture Philanthropy Model

Epidermolysis bullosa (EB) is a group of rare genetic disorders resulting from a lack of the proteins that bind the skin's two layers together. Without these proteins, the skin tears apart, blisters and tears off. This leads to excruciating pain, disfigurement, and wounds that become infected and may never heal. Blisters can appear in response to minor injury, rubbing or scratching and, in severe cases, can occur inside the body.

There is no U.S. Food and Drug Administration approved treatment, so the disease is managed with bleach baths, full body bandages, and frequent surgeries. Life expectancy for the most severe forms ranges from a few months to 30 years of age. So, the need for treatment is urgent.

Powering Research with Revenue

EB Research Partnership (EBRP) was founded in 2010 with the goal of finding a cure for EB - and finding it quickly.

"We want to inject rocket fuel into advancing life-saving treatments for EB and we're willing to fight for it,"

- MICHAEL HUND, MBA, CEO OF EBRP

In the past 10 years, EBRP has raised \$35 million and funded 65 projects across the globe. The number of clinical trials in the space has grown from 2 to 20 and they expect five phase 3 clinical trials next year.

Their innovative **venture philanthropy** business model is the engine behind this research program. In a nutshell, **venture philanthropy** takes venture capital principles and applies them to rare disease research. EBRP negotiates equity, such as shares or warrants for stock, with companies and academic medical centers when they enter into partnership agreements. So each research investment offers a potential revenue stream.

EBRP has support from generous family donors including rock star Eddie Vedder of Pearl Jam and his wife, Jill. They know this is a piece of good fortune and want to use their platform responsibly by maximizing the benefit to patients and sharing the mission of curing EB with the world.

"We don't just write checks and hope for the best," Hund says. "We source the best ideas, get recommendations from our Scientific Advisory Board and invest in meaningful therapies. We come to the table and negotiate deal terms, whether it is with a university, biotech, or publicly traded company. So, when the product is commercialized, we share in the profits and pump it right back into our research." ■

A Case Study in Venture Philanthropy

Three years ago, the group found a university with an innovative gene therapy product and decided to fund it for \$500,000. Two years later they helped accelerate the therapy into clinical trials and received interest from a publicly traded biotech. When the intellectual property (IP) from the university was acquired, EPRB exchanged their equity in that company for a financial return of \$3 million which went back into research.

“Every single piece of research we fund is under this model. We have standard term sheets, so if something is commercialized, we’ll share in the profits.”

– MICHAEL HUND, CEO OF EBRP



Hund focused on medical research models while he was doing his MBA at Yale. His expertise in technology, fundraising, and negotiation was a perfect fit for the culture of EBRP. Alexander Silver, MBA, the father of a child with EB, chairman of the Board of Directors, and a co-founder of the group, is also the founder of a New York City investment firm. His philosophy was: “Why wouldn’t the foundation get a share of the deal?”

Venture philanthropy gives patients a seat at the table in drug development. Not only does 90 percent of every dollar raised go directly to research, but also the return on investment can multiply the impact of the original donation. ■



DATA DIY TIP

Like venture capital financing, **venture philanthropy** requires expertise and can involve more risk than traditional methods of dispersing funds. But potential pay-offs are also greater.

For more information on venture philanthropy, check out FasterCures’ report, “Honest Brokers for Cures,” a series of interviews with leaders of organizations that use venture philanthropy principles for biomedical research.

<https://www.fastercures.org/assets/Uploads/PDF/HonestBrokers.pdf>

EBRP's Strategic Collaborations

Fostering collaboration is another key component of EBRP's strategy. "We had three top universities come to us with an innovative project for EB with the potential for a cure," says Hund. "They all applied separately, but there were synergies. They all had the same target and same approach, but each university had a different expertise. One was an expert in gene editing, one in manufacturing and one in 3D printing of skin. We went back to them and said we would fund on the condition that the three universities come together and collaborate. They agreed and formed the iPS Cell Consortium."

The consortium has developed a technology for using small skin samples, isolating iPS cells and correcting the EB gene mutation with gene editing, and then creating new sheets of skin to return to the patient. Because the technology can also be used for wound care, the consortium later received a \$3.8 million Department of Defense grant. In awarding the funds, the grantors noted that the collaborative nature of the proposal was an important factor in helping them decide in their favor. ■

Expanding the Consortium

In addition to the iPS Cell Consortium, EBRP has also worked to establish the EB Clinical Research Consortium, a collaboration of 21 universities which now has a data set of 800 patients.

"If you get one center to buy in, there is usually a domino effect. You don't want to be the only university that won't share their data."

– MICHAEL HUND, CEO OF EBRP

Navigating university policies and procedures across multiple organizations is always a challenge, Hund notes. But negotiation skills and understanding your leverage can help.

"Economic incentive is part of the language they speak and most of the time foundations are the primary funding sources for these rare diseases," he says. "The key factor is representing patients. They are our true north. With patients as our stakeholders, over the last 10 years, we have been able to figure out win/win scenarios that motivate universities and industry to want to participate." ■



Inventing a Platform

EBRP is also focusing on streamlining data collection by working with Amazon Web Services (AWS) to create a platform that will house the data and make it accessible to all stakeholders.

“We gathered researchers, clinicians, industry, and patients together to help design the platform using AWS’s working backward model,” Hund says. “They told us we couldn’t leave until we agreed on a one-sentence problem.”

“This is what we came up with: What if we could navigate the research and patient journey as easily as you enter a destination in your GPS, only the destination would be a cure for EB.”

“We want to take large-scale patient data sets, combine them with biorepository information and genotype and phenotype information, all of which now exists in silos at different universities,” says Hund.

“There will be one state-of-the-art technology platform, providing security, HIPAA compliance, a network of partners and tools, machine learning, artificial intelligence, and rapid analytics. Rather than rely solely on the more time-consuming approach of having researchers enter all of the data, we also plan to go directly to patients.”

“All of that data will be given back to the researchers. That’s the motivation for them. If you contribute data, you can get it right back along with data from 20 other centers.”

They envision a social element, so researchers can follow other researchers, clinical trial managers can be linked to one another, and patients can follow progress.

“Since patients contribute data, we want them to be able to learn from it, too. Patients can log on and see, upon the moment of diagnosis, the best doctor within 100 miles. They’ll see what other patients with their genetic subtype are doing for treatment and what the outcomes have been.”

“We’re moving quickly to get an eight-week rapid prototype built. This will be piloted at a select group of universities. Once we have proof of concept, we will scale it to the 21 institutions in our consortium.

Beyond that, there’s an even bigger aspiration. “Eventually,” says Hund, “we will make it available to all rare diseases.” ■

KEY TAKEAWAYS

1. Giving advocacy groups a financial stake in treatment development accelerates outcomes.
2. Governance should represent the people who will benefit - patients, researchers and industry – so all should participate in discussions.
3. Fail fast and keep evolving.



DATA DIY TIP

Amazon Web Services (AWS)'s Working Backwards Method

Working backwards is an approach that AWS uses to develop new products. The goal is to keep the customer's needs in mind. Product managers typically start by writing a press release and a set of FAQs that focus on the customer's problem, why current solutions fail, and how the new product will be better. Some things that may be included are:

- A **summary** of the product and its benefits to customers
- The **problem** and why this is a good **solution**
- A **quote** from a **spokesperson** or hypothetical **customer**
- Description of **how to get started**
- **Call to action**

These materials are circulated internally to get feedback so the idea can be evolved and perfected, long before there is an actual product that will be released. Although AWS applies "working backwards" to product development, it's a strategy that advocacy groups could apply to a wide variety of strategic planning decisions.



CHECK YOUR KNOWLEDGE

Which of the following best represents the concept of venture philanthropy?

- Venture capital firms raising funds for charitable causes
- Home-grown fundraising efforts, such as 5Ks organized by a patient advocacy group
- Structuring research investments and partnerships to allow for a potential revenue stream for a philanthropic organization
- Using online and social media-fueled crowdfunding, such as Kickstarter

Answer: C

