

## A Rare Disease Patient Registry

### Determining a Structure that Inspires Trust

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Michael Hund, CEO of Epidermolysis Bullosa Research Partnership (EBRP), had arrived at a fork in the road. He faced a decision on the structure of an entity he had created with cloud computing industry leader Amazon Web Services for a database of health information about Americans struggling with epidermolysis bullosa (EB). Should he spin out the new entity from EBRP into a for-profit venture? He had been besieged by offers from venture capitalists and investors to capitalize a for-profit company. Or should he stay with the tried-and-true model for medical charities and establish a non-profit to hold the database? Both options offered opportunities and challenges.

Hund was inclined to an entrepreneurial path. He had received an MBA from Yale's School of Management; his family tree included entrepreneurs; and he had founded a real estate holding company. At EBRP, the charity had used an innovative venture philanthropy business model to invest in companies developing EB treatments that would also earn the charity profits. In his three years at EBRP, Hund had raised \$22 million and inked over a dozen venture philanthropy agreements with industry for drug discovery. Several candidates were now in human trials. If they worked, they'd be the first prescription treatments for a devastating disease. In addition, EBRP occasionally had taken equity in its biopharma partners and had also incubated several start-ups. Given his entrepreneurial instincts and training, how should he organize a venture for gathering patient data with the world's largest cloud computing platform? How would the structure he chose for his company affect its success?

#### Big Health Data

Michael Hund's venture was just one of many aimed at transforming the \$30 billion health data market. For nearly 70 years in this country, anonymized data on patients had been bought and sold by hospitals, pharmacists, pharmaceutical companies, medical researchers and healthcare insurers for bioscience, drug development, medical billing, market research and public health studies. Over the past several decades, the market had exploded as the genomic revolution created exponential amounts of new data, and health information became more and more digitized. Powerful new players had pushed into the field, such as tech behemoths Google, Facebook, Amazon.com Inc., Microsoft Corp., Apple Inc. and IBM. These companies had bought up healthcare providers, insurers, and healthcare consumer companies whose electronic patient records they could store, manage, analyze and otherwise use for services and products. Since the data was anonymous, its use by third parties was not subject to national health privacy laws requiring informed consent. Consumers also began to understand that their health data was not just data, it produced profits for those who controlled it. Meanwhile,

Congress held hearings about trimming tech titans' monopolies. In 2020, the U.S. government brought monopoly lawsuits against Facebook and Google, claiming their business practices and data collection for web services harmed consumers by reduce privacy protections. Washington also started to investigate Amazon.com.<sup>3</sup>

As the fight for dominance intensified in the fast-changing health data economy, the aggregation and mining of consumer medical profiles became one of the most sensitive subjects in healthcare, pitting patients' rights to privacy against companies hoovering up vast quantities of data for old uses such as pharmaceutical sales and marketing and creating new uses in drug development, health smartphone apps, imaging analytics, mortality predictions, and COVID tracing. In patient registries, the data universe in which Hund was interested, the clash between privacy rights and commercial interests was especially acute. Patient registries – interactive compilations of data tracking patients' genetics, diseases, treatments and outcomes – were vital tools for researchers to learn about illness, discover new medications and improve treatments. Concerns about the use and protection of this data were intense because unlike the rest of the big health data market, where data is sold without the knowledge of consumers, the public must consent to join patient registries started by charities such as EBRP and volunteer their private health information to them.

Consequently, technology service vendors and pharmaceuticals that had relied on anonymized patient data and shaped the health data market now faced a new style of entrepreneur eager to decentralize data collection and put consumers in control of their health information. The decisions Michael Hund took for his new venture with Amazon Web Services would reveal whether he aligned with the incumbents or challengers. Hund leaned towards disruption.

## **Epidermolysis Bullosa Research Partnership**

Like most disease charities, the Epidermolysis Bullosa Research Partnership (EBRP), was begun by families with children suffering from the disorder. The inherited disease often kills before the age of five. It is so painful that parents in Europe have consented to euthanasia for their children.<sup>4</sup>

EB attacks the skin, creating blistering wounds that are hard to heal, and in other organs such as the digestive system and eyes. It has four main types, from mild to extreme, each with multiple subtypes. Mild cases do not hinder a child's development. The most severe fuses the hands into blocks without fingers and puts patients at risk for chronic skin cancers. Parents spend three to four hours a night bathing their children in bleach to disinfect their open wounds and bandaging them. Some babies are born without skin and others lose it later in life. Besides bandages, salves, anti-inflammatories, and pain medication, EB has no treatment.

Hedge fund partner Alexander Silver co-founded EBRP in 2010 with other EB families and Jill and Eddie Vedder (Pearl Jam). Two years later, Silver began engaging venture philanthropy to speed the development of therapies and won research contracts with industry to search for EB therapies. Silver oversaw EBRP in addition to his job in a Manhattan hedge fund.

In 2018, when Silver hired Michael Hund, EBRP had one paid staff member and had raised \$22.5 million over the previous seven years to accelerate EB treatments.<sup>5</sup> Some of the experimental therapies it had helped finance had progressed to human trials, notably, skin sheets engineered from patients' stem cells to replace their torn skin and gene editing therapies to correct mutations in the genes causing EB. The most common was the collagen gene COL1A1; mutations in it prevented the layers of tissue in the skin from cohering and healing wounds.



Silver recruited Hund from another charity which had also successfully exploited the venture philanthropy model, the Multiple Myeloma Research Foundation in Connecticut. Hund had run its \$100 million cancer fund drive in 2017 as director of development while attending the Yale School of Management on weekends. Since volunteering as a teenager at The Hole in the Wall Gang Camp, Paul Newman's summer camp for children with serious illnesses, Hund had known he wanted to be part of larger efforts to help alleviate or cure diseases.

As he drew up a strategic plan for EBRP and established himself on the job, reaching out to form relationships with grant institutions, scientists and wealthy donors, Hund thought about the way his previous employer's patient registry had furthered basic science and encouraged researchers to zero-in on a more than dozen new therapies that the FDA approved.<sup>6</sup> Commercialization of the discoveries also yielded profits for the charity, which re-invested them in research. But one thing bothered Hund: patients who consented to give the database their health information never saw their data again. Only researchers and industry could access it. For their gifts, patients got no immediate returns.

Hund saw this as a problem to solve and in 2019, with an introduction from a member of his board, he approached Amazon Web Services. He challenged the company to help build,

A first-of-its-kind global database for all rare diseases in the world that would guide patients to the nearest doctors, research studies, treatment clinics, trials of new drugs and patient support groups the same way GPS guides users to the nearest restaurant or gas station. I want EB to be the first runway study so that we can prove the model and then go disrupt the world with this product.

The registry would not only provide up- to-date information and support to those living with EB, it would also attract medical researchers to hone in on EB and rare disease to devise treatments.

Other EB patient registries already existed. The National Institutes of Health began the first in 2000, and with it, researchers estimated the incidence and prevalence of the disease in the U.S. for the first time. Other EB charities in the U.S. had also developed patient registries, as had a biotech. In rare disease, databases were collaborations between nations such as the U.S. (National Organization for Rare Disease, NORD), Europe (EURODIS), and Canada (CORD) that contained patient treatment-related data and patient biological samples. No one corporate structure was common to all: they were run by public companies, non-profits as well as private non-profits.<sup>7</sup>

Amazon Web Services was intrigued by the scale of Hund's ambition and on a summer day in Colorado in 2019, senior technical program managers from its Envision Engineering unit, which works on difficult, important problems, brainstormed with Hund on a pilot. The prototype, a portal for researchers, was finished in eight weeks. Hund populated it with data from an EB registry created by Mark de Souza, co-founder of a biotech which EBRP had bought. Hund teamed with another division of AWS to create a pilot patient portal and then began to consider how to find patients to upload data to the platform to give it medical and commercial value.

## Database Dreams

If Hund could find enough patients to donate data, the economics were compelling. The U.S. defines rare disease as any with under 200,000 patients. 25,000 to 50,000 Americans are thought to suffer from EB. Worldwide, the estimate is 500,000. Some rare diseases are even smaller, with only a handful of patients in different countries.



Because rare diseases affect a relatively small pool of people, pharmaceutical firms spend hundreds of thousands of dollars to identify enough patients to test novel therapies, the most important hurdle in drug development. A Phase 1 human trial of a rare disease drug may test only five people. The pivotal Phase 2 trial, whose outcome determines if the drug advances to one more trial before FDA review, may test 10-15. The final trial, called Phase 3, lasts a year or more and may test dozens or even a hundred subjects to prove the therapy safe and effective. For diseases like EB with many subtypes, recruiting patients with the necessary subtypes can be like searching for a needle in a haystack, Hund noted,

“One of those genetic subtypes is a fraction of that 30,000 to 50,000 EB patient number in the U.S. So you may be talking about two to 3,000 patients with this subtype. Of those two to 3,000 patients, how many are seen in an academic medical center? How many are in a major metropolitan area where clinical trials may be run and operated? How many sites are there for a clinical trial? Is there one in California, in New York and one in the Midwest? Are patients able to travel to the sites? Finding patients to share their data to advance clinical research is incredibly challenging.”

Enrolling sufficient numbers of human trial subjects for rare disease drugs is not only expensive and time-consuming, it can cost a company its chance to go to market. The biotech or pharmaceutical firm which cannot find enough rare disease patients for human trials in time for enrollment deadlines drops out of development and wastes its R&D investment. According to a recent study, this is one reason that 30 percent of Phase 3 trials fail. Johnson & Johnson, for example, stopped investigating a novel drug for the rare cancer mantle cell lymphoma in 2017 because it could not recruit enough patients to test.<sup>8</sup>

Hund believed these challenges gave his database considerable potential value. He argued,

We’d provide a turnkey solution to companies to get FDA approval faster and get to market faster. We’d give them a platform that’d take years for them to build on their own; locate and recruit patients for human trials quickly; collect and aggregate patients’ medical histories and track their clinical outcomes. For EB, for example, we’d give companies data on patient wounds, their level of pain, and treatment before they were given the companies’ therapies. Even better, we’d provide longitudinal data – how patients do over time with those treatments after the FDA approves them. And regulatory data on how much patients spend on daily care, on bandages for example, mixed with clinical data about outcomes. Companies and insurers use regulatory data to work out the price and reimbursement of a new therapy.

Hund estimated the patient registry would cost \$472,000 to build and about a million dollars a year to operate. EBRP had invested half a million dollars to build and configure it with AWS, an iterative process. Hund believed that the registry could realize a profit by year five with any of several models for revenue: subscription/per view/one-time payment access. Fees to industry and academic researchers can cover most of the annual operating costs of patient registries, though many created by patient advocacy groups are free. Hund hoped his could command a premium because customers would use AWS’s sophisticated rapid analytics, machine learning, and cloud computing to harvest insights and patterns from datasets. He could use a net present value formula to value it and choose its economic model once it was loaded with enough robust data to interest biopharma and researchers in paying to search it.

If he chose to create a for-profit patient registry with outside investor money, Hund knew that it would get up and running faster than as a non-profit, which would have to seek public donations and grants for operating funds. “With outside capital, a for-profit database would scale faster, too,” he said. And he could sell pieces of it to venture capitalists or private equity to raise operating funds.



## Finding the Willing

Would operating as a for-profit influence patients' willingness to donate their data? Hund decided to partner with a university for his pilot patient portal, "Direct to Patients," to find out. Stanford University School of Medicine managed a database of 100 EB patients that had been collected over 35 years by university doctors researching and treating EB. EBRP had good relations with the medical school, having helped fund several of its EB therapies in clinical trials. Hund proposed to Stanford that its professors lead the pilot patient portal. Stanford's institutional review board governing the university's research protocols approved the venture in 2020 as compliant with HIPAA and other federal regulations protecting patient privacy. Approval from the elite research institution was a coup; with Stanford dermatologist Dr. Joyce Tang and statistics professor Dr. Ying Lu as principal investigators, EBRP now had a green light to approach Stanford's EB patients.

The information Hund wanted from them was demographic (name, age, address) and medical such as diagnosis, prior treatment, co-morbidities, skin function, weight, medication, transplantations, antibiotics usage, their doctor's names and their medical records as well as outcomes, longitudinal and regulatory information. Patients would sign agreements stating that they'd authorized sharing their information on the registry, ensuring compliance with the federal healthcare privacy law, the Health Insurance Portability and Accountability Act of 1996 (HIPAA). Patients would then decide if they wished to donate data anonymously or link it to their names on the registry. The choice would give patients more control over their information than was usual for patient registries, which typically contain only anonymized data in order to comply with HIPAA law barring healthcare organizations and non-profits from sharing identified healthcare data. Patients would also sign consents for what information they wanted to share in the future, in order to eliminate the need for re-consents as the database grew. Such consents were protocol even for anonymous data in case an outsider managed to identify a donor, a small but not zero risk.

In the future, Hund hoped the interface would allow patients the right to edit their data, delete or opt out, for example, or change their minds and expand or restrict its use. But in 2021, that technology was unavailable. However, patients could specify whom they wished to share their data with-- academic researchers, pharmaceutical companies, doctors, clinics or other patients. In this way, patients would retain further control over the use of their information.

Besides demographic and medical data, Hund wanted to obtain genetic samples from consenting patients. He planned to mail free saliva genome sequencing kits to patients so they could test themselves and learn their EB subtype. A third-party genotyper would sequence the samples at an estimated \$1,700 a pop. Sequencing the DNA of 50 initial EB patients was projected to cost \$85,000.<sup>9</sup> The genotypes, a readout of the order of the letters in each person's genetic code that instructs the body how to maintain life, would then be matched with each person's health information.

Having both genotype (DNA) and phenotype (symptoms) data would make the registry more valuable to investigators searching it to locate genes that influence or cause disease and the associated mutations to target for new drugs. This was key for genetic diseases like EB because medications must be tailored to EB subtypes; not all medications for genetic diseases work on all patients, on account of the differences in their genes, called variants.

In his business plan for the registry, Hund anticipated recruiting 900 EB patients its first year, with genetic samples from some. In five years, he hoped to enroll tens of thousands of patients around the world. A governing committee would decide how the registry operated as it expanded, and a bioethicist or

healthcare professional would review it for data quality and correct errors that patients made uploading their profiles.

A sticking point was that each person's DNA is unique. Even if a patient donated their DNA anonymously, it could never actually be anonymous: DNA is a unique identifier of that particular person and no one else. The privacy challenge that collecting DNA for the registry posed could be solved in the short term by obtaining consents from each donor, clarifying that their genotype identifies them, or perhaps by blockchain, with anonymized links between patients' healthcare data and their names and addresses.

Another sticking point was that genotyping patients would reveal all their genetic secrets. For example, their risk for Alzheimer's or breast cancer. How would patients view giving a complete gene panel to the new company? Was this an incentive or a disincentive? Would patients consent for outside researchers to contact their relatives, for example, to trace inherited cancers? With 12-15 percent infidelity in the U.S., would patients welcome learning more about their family ties? Would they worry that giving a complete picture of their genetic susceptibilities to researchers could be used against them in health insurance or job hunts?

If he obtained genetic sequences for 50 EB patients as proof of concept, Hund anticipated opening the registry to 20 other universities in EBRP's research consortium, a standard tool in medical research. EBRP chairman Alexander Silver had established the consortium in 2012 to encourage collaboration among academic researchers studying dermatological conditions with shared patient data. Hund decided any of the 20 universities that joined his patient registry and entered data from their patients on it would be rewarded with free access. Only outside principal investigators and biotech and pharmaceutical companies would pay a fee.

The more universities that joined, the more data and the more publications, Hund believed: "No patients, no data, no answers," was a truism in medical science.

## Amazon Web Services

Principal investigators at the 20 universities in EBRP's consortium, however, reacted in ways that surprised Hund. Half saw the advantages of a registry with the world's largest web services company to speed up research with cloud computing, machine learning and rapid analytics and to fuel collaboration. The other half were skeptical. The extent of mistrust of Amazon Web Services among investigators was a revelation to Hund:

One camp said we could be so much better, using cloud computing, machine learning and data analysis, and we are willing to work with our institutional review boards to figure out how to do this with HIPAA compliance and privacy and shares. The other camp saw our teaming up with Amazon Web Services for data gathering as a threat--is the data going to be breached or leaked? What is cloud computing? Will we lose our data one day on the cloud?

Researchers and their universities were also concerned about credit for discoveries if they participated. Would they be credited for their inventions if these derived from pooled data from many schools? University reputations rest heavily on their scholars' research. In recent decades, royalties from faculty inventions have fueled universities' expansion: the HIV drug Zeret at Yale University is a famous instance.<sup>10</sup> Universities fiercely guard their intellectual property, seek the best returns possible and were in a position to bar their researchers from joining the platform.





Principal investigators also asked, “What is Amazon’s greater role in this? Is AWS going to take our data?” Some, Hund realized, didn’t trust the separation between AWS and the part of Amazon that delivered packages to their doorsteps. Others were concerned that Amazon.com had its own healthcare company, sold prescription drugs, operated health clinics for employees, and stored and managed their electronic patient records, while its subsidiary AWS stored and managed healthcare databases for competing healthcare providers and insurers. Many had heard that employees of Google had obtained access to tens of millions of patients’ medical records and names from partner Ascension, a health care insurer, in a widely publicized breach of privacy in 2019.<sup>11</sup> Could a similar disaster occur with AWS? What did Amazon’s ambitions in healthcare portend? Would it use studies shared on the database for new private label products? Researchers asked if an outage occurred or if AWS or the registry was hacked, would their data be compromised?

Faced with researchers’ worries and skepticism, Hund considered whether a for-profit company could succeed. The first obligation of a for-profit patient registry would be to its shareholders, not its stakeholders. Hund reflected,

I thought people would be thrilled that we have the largest cloud computing company in the world with us. I saw Amazon Web Services as a value proposition because we were bringing in the best and largest company. I learned that it was a little too scary for investigators not used to this company. It is actually not a value proposition to academics. I made this mistake early. It was a learning curve for me, quite frankly.

To win over doubters, Hund embarked on a round of discussions. He explained that AWS analytics and machine learning sped up research by chewing through data lakes in fantastically short order:

I tell them AWS is a utility. They just build the wire, the utility poles, to bring electricity into your home so you can work with a platform of your choice and ours is to cure rare disease. I explain that AWS works for the FDA, the Center for Disease Control, the FBI, the CIA, government agencies, thousands of companies and websites, and powers the apps on their cell phone. They are the very best in data management. AWS will have no equity in us. EBRP owns our database and the product. We are driving them, not the other way around.

Amazon Web Services executives agreed. “AWS will not use, or have access to, any of the data housed on the platform. AWS offers a breadth and depth of services across compute, AI, ML, database, networking and more. Whatever we build and launch as a service, EBRP will utilize as they see fit, just as any other AWS customer would,” an AWS representative noted.

On the other hand, EB patients and their families told Hund they had few such worries about credit or data security and privacy. They made it clear that they were willing to volunteer data and “do whatever it takes” to increase the odds of finding treatments for their disease. “I have not heard of one patient who said no,” Hund observed.

## Governance

When he evaluated a for-profit model for his new company with AWS, Hund saw little difference between it and a non-profit structure on the revenue side. Either could charge industry and outside researchers to analyze patient datasets. As time went by, the more EB patients who consented to donate their medical profiles and were tracked for longitudinal data, the more the database could charge and the more sustainable it would become, hastening scaling it to every rare disease in the world.

But as far as governance was concerned, questions about credit for breakthroughs made with its datasets and worries about data security, operational issues that the new company would have to resolve to succeed, seemed to be exacerbated by a for-profit company model. Managing the database to maximize profits for its shareholders clashed with Hund's goal of curing rare disease. A non-profit company, on the other hand, could set operational strategy exclusively for the purpose of finding therapies and cures.

In addition, Hund regarded governance of a for-profit company, whose board would be composed largely of venture capitalists and investors expecting every decision to maximize returns on their capital, as a potential nightmare. Since he might have to give a large portion of the equity in exchange for VC money and guarantee investors an ROI, the conflict involved in spending their money to discover a novel therapy or a cure when 95 percent of new drugs fail, might also harm the company's reputation and image and make recruiting patients to volunteer their data challenging.

For example, would VCs pull out if the registry was not profitable in five years? Or experienced a crisis? What if AWS and EBRP fell out? In that case, EBRP would own the registry but AWS would own its code. EBRP would have to find another partner to re-establish the platform. Would a for-profit board stacked with VCs give management enough time to cope with such a crisis? A non-profit board might be more forgiving than a for-profit board disturbed by the possibility that a setback could harm their investment.

After comparing the advantages and disadvantages of a for-profit and a non-profit model, Hund concluded the most important ingredient for the registry to succeed was trust. "This is a big barrier — there's lots of regulations and there's lots of mistrust. You have to think very carefully about how data is used and where it goes."

## Hybrid Possibilities

As he contemplated the need to assure patients of the privacy of their data on the registry in order to motivate them to offer that data and DNA to identify their subtype, find clinical trials and other patients, and possibly also help to usher in new therapies in 15 or 20 years, Hund turned over in his mind whether a hybrid solution existed. Would it be possible to launch the registry as a non-profit and down the road set up venture philanthropy deals with biopharma which would guarantee it a share of profits from any products created with its datasets? Such deals could be held as children companies under the mother non-profit. The "children" could re-invest the profits, which would make the registry sustainable and help scale it to other rare diseases without the need for outside investors.

Or would signing venture philanthropy alliances with biopharma, even as subsidiaries of a non-profit patient registry, raise red flags for patients and principal investigators about data security and credit again? Would these companies have exclusive rights only to data from patients who'd approved sharing with for-profit companies? Perhaps it would be better to spin out deals as privately owned start-ups. EBRP had experience launching three such fledgling companies, whose equity it had shared with founders and managers.

The hybrid scenario broached a further consideration: should patients share in any monetization of therapies or other products derived from their data? "This is a very touchy question," Hund allowed. U.S. medical history contained infamous examples of injustice on this very question. Henrietta Lacks, for example. Lacks was a poor Black woman whose cancer cells were taken for study in 1954 without her consent, cloned and given to laboratories throughout the world, which then developed therapies from them without Lack's estate receiving a penny.<sup>12</sup> Hund leaned against offering patients a share in



monetization, no matter whether the registry was a for-profit or non-profit, although he had not yet decided whether patients who donated their data gave up ownership of that data to the registry. That key decision required more discussion with patients and a legal review.

Again, Hund asked himself, “Does outside capital help advance my goals? It’s not totally off the table,” he mused. “But if we did take outside money, the patients and the universities would have to approve it. Another scenario is, maybe it’s not VC money. Maybe it’s pharmaceutical money from companies testing compounds for EB that would be acceptable to universities and patients.”

Mulling his options, Hund watched EBRP’s staff and volunteers leave for the day. The EB “Direct to Patients” platform debuted in two months and Hund would have to put his decisions about the structure of the new company with Amazon Web Services to a board vote very soon.

This case has been developed for pedagogical purposes. The case is not intended to furnish primary data, serve as an endorsement of the organization in question, or illustrate either effective or ineffective management techniques or strategies. Dollar amounts have been disguised for proprietary reasons.

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## Endnotes

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