VENTURE INTO CURES

An Initiative by EB Research Partnership

Building a Faster Path to Curing Epidermolysis Bullosa

Lives Changed:

Meet Brady the Brave and His Parents.

When “Brady the Brave” was born, his parents were shocked to learn that his feet were raw and missing skin. After two weeks in the NICU, Brady was diagnosed with Recessive Dystrophic Epidermolysis Bullosa (RDEB).

Brady’s skin is so fragile that it comes off his body with the slightest touch. Simple scrapes become severe wounds. He requires daily wrapping in full body bandages. Routine tasks such as eating, walking, and sleeping become monumental daily challenges. Yet Brady and his parents are fighters. “Our family keeps high hopes that Brady will be able to do most of the things that ‘normal’ children can do.” We are working diligently to fund the research and clinical trials needed to improve the lives of Brady and hundreds of thousands like him.

— THE PROBLEM AND SOLUTION —

For the **500,000** People Living with Epidermolysis Bullosa, the Question of a Cure Isn’t “If.” It’s Only “When.”

50 Research Projects Funded

10x The Clinical Trials Within a Decade

1 Organization Putting a Cure Within Reach

Through an innovative approach to research, EB Research Partnership (EBRP) is changing how EB is treated and how cures are found.

Epidermolysis Bullosa (EB) attacks the body’s largest organ — skin — and not just the skin we see but also the connective tissue and organs inside of us. It results in wounds that never heal, disfigurement, blisters, bleeding, and ever-present pain. It is lethal for many.

If that sounds awful, imagine how it feels to the children born with it.

One in 227 people carries the defective gene that causes EB. Fortunately, most never express the disease. For those that do, EB means skin can peel away at the softest touch, like the wings of a butterfly. Everyday pleasures — eating, sleeping, and walking — can bring fear and pain.

We have a plan to defeat EB. It’s a plan that not only promises to change the lives of families fighting this disease but also to change the way rare disease treatments are found. Our plan will change the lives of millions. We are pioneering the model to cure disease.

This isn’t just a dream. It’s real.

We’re building it. Today.

Join us.

Some people say my skin is like the wings of a butterfly because it can break easily. Butterfly wings are fragile, but they also help them fly. I may have EB, but I'm going to keep on flying.

— Eli
EB Patient

Everyday Impact:

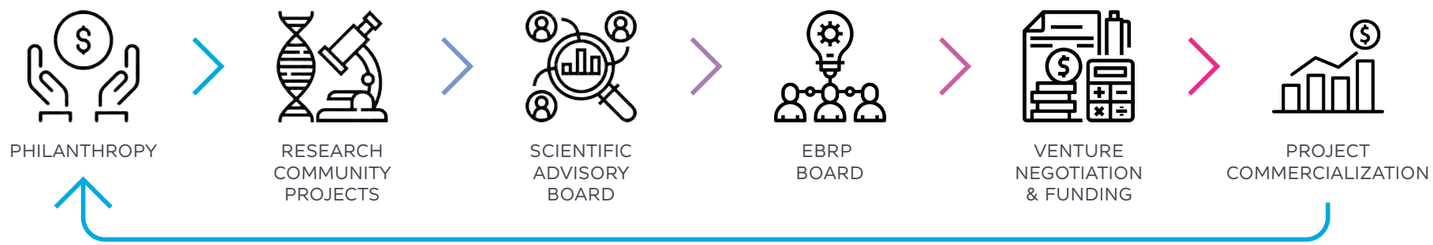
Eli and the "Come Say Hi" Movement.

In many ways, Eli is a just a normal kid. He loves playing outside with his siblings, building Legos, drawing, and telling stories. But what makes Eli different is what most people see first. "When people look at me, I think they ask 'what happened to me?' I tell people I was born different. But, we're all born different." Eli and his big sister, Lily, decided to make a change. "We want people to know that if someone looks different than you, instead of staring, they should Come Say Hi," she says. Together, they have started a global movement to raise awareness and impact the search to cure EB.

— THE VENTURE INTO CURES METHODOLOGY —

Investing Like a Venture Capitalist. Collaborating Across the Medical Community. Driving Results for Patients.

Think of Venture Into Cures as a mutual fund of possible EB treatments. We go out, find the most promising research projects, and fund them in exchange for a financial interest in the work of university or business. When those projects have commercial success, we take the returns from our shares and plow them right back into more promising EB research projects. That means every dollar invested in Venture Into Cures is multiplied — potentially many times over, making every dollar count again and again.



95% of Rare Diseases Have No Approved Treatment. Our Model Changes That.

Epidermolysis Bullosa is a perfect case study for our model. As a rare disease, it struggles for funding because it's not well known. As a dermatological disease with clear physical indicators, the effects of treatments can be seen and studied more quickly. The work we've laid out to date has already opened numerous pathways to a cure. We are first off the runway for not only our business model but also innovative gene therapies that will help many other diseases soar to cures.

21 Institutions, One Focus: Curing Epidermolysis Bullosa.

Central to our model is collaboration and shared data. We established the EB Clinical Research Consortium (EBCRC), so every research team has the opportunity to benefit from the others.

— THE VENTURE INTO CURES MODEL —

Sharing. Collaborating. Curing.

After years of hard work, we have reached the threshold of a far more promising future for children born with EB. Leading researchers believe that both life-changing treatments and a cure are within reach. Now is the time to accelerate our efforts. To realize our vision of a world without EB, we're building a research model based on three core pillars.

> *"Our work in skin and stem cell biology has opened up therapeutic avenues we never thought possible."*
>
> *– Tony Oro, MD*

A Comprehensive and Shared Data Platform.

By launching the largest EB data project imaginable, we will aggregate, centralize, analyze, and decode the clinical, genomic, and patient data underlying the disease. Among the contributing initiatives are as follows:

- **EB Clinical Characterization and Outcomes Database (CCOD)**
- **EB Natural History Study**
- **EB Direct to Patient Registry**

Seamless Collaboration Between Research Entities.

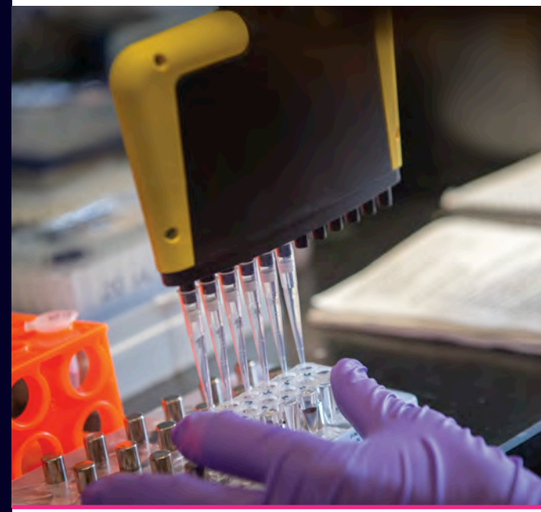
By bringing together the often siloed academic, medical, and patient communities, we are breaking down barriers to cooperation and ensuring compounding benefits to each discovery. Collaborative initiatives include the following:

- **EB Clinical Research Consortium (EBCRC)**
- **EBRP Scientific Advisory Board (SAB)**
- **EB Community Council**

Investment in the Most Hopeful Research Projects.

By carefully vetting and investing in the most propitious research projects with help from our Scientific Advisory Board, we stack the odds of finding a cure in our favor. Potential therapies fall into three categories:

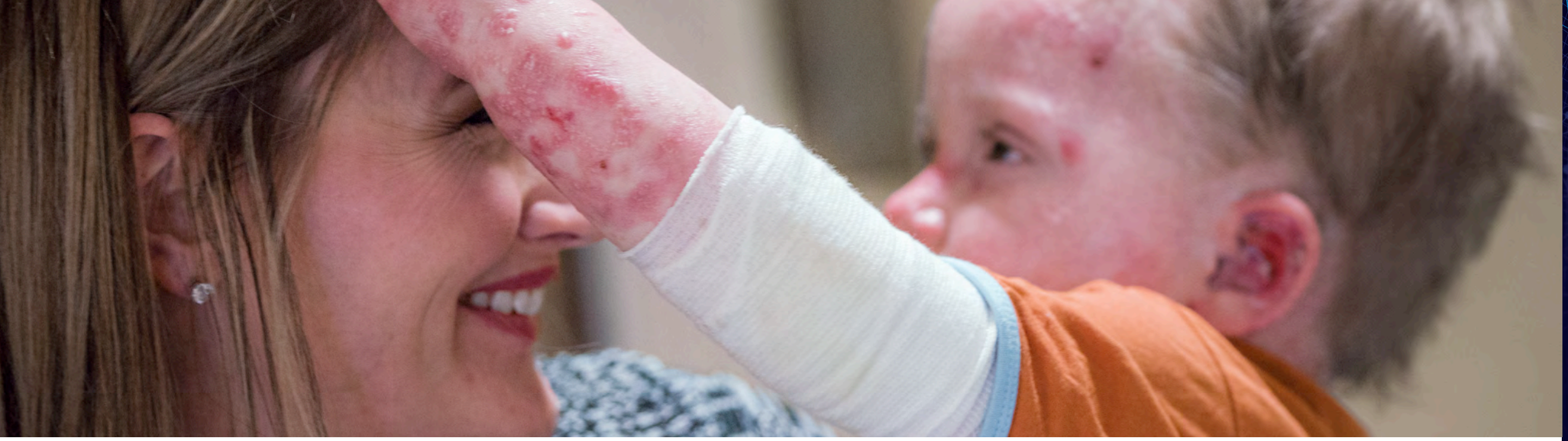
- **Disease Controlling**
  Therapies that stabilize the effects of EB, including topicals to minimize itching and blistering or speed wound healing.
- **Disease Changing**
  Therapies that enhance the body's ability to fight the disease, including gene and protein therapies.
- **Disease Curing**
  Therapies that achieve the ultimate goal of a definitive cure, including exon skipping and gene editing.



Collaborating for Cures:

iPS Cell Consortium.

A testament to EBRP's founding principle of collaboration, we established the EB iPS Cell Consortium with leading research teams at Columbia University, the University of Colorado Anschutz, and Stanford University. The Consortium studies cutting-edge induced pluripotent stem (iPS) cell technology that can reprogram patient cells to iPS cells and correct the mutation that causes EB. This research is at the forefront of medical innovation and has received additional support from the US Department of Defense and the California Institute of Regenerative Medicine. Fully realized, it will allow patients to produce their own cells, which are gene edited and infinitely reproducible, effectively curing EB. Further, EB is the case study for this platform technology that scientists believe holds the key to curing thousands of other genetic diseases



— RETURN ON IMPACT // FOR EB AND BEYOND —

Return on Impact.
Is There any Better Kind of ROI?

The biggest obstacle to curing EB isn't science — it's funding.

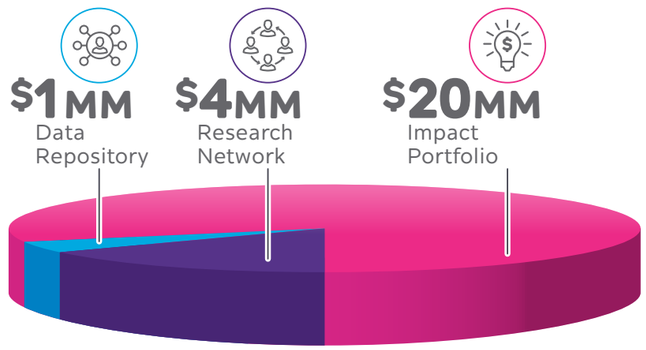
EB Research Partnership has a proven history and plan to scale. In under a decade, we've funded 50 projects, increased the number of EB clinical trials by 1,000%, formed three new companies, and struck nearly 50 venture deals with businesses and universities.

Curing EB today. Helping 7,000 rare diseases tomorrow.

350,000,000 people suffer from rare diseases globally. That's more than are impacted by cancer and AIDS combined. It takes an average of eight years to even get a diagnosis. Half are diagnosed in childhood, and 30% of those children don't live to see their 5th birthdays. 95% of these rare diseases have no FDA-approved treatments. 50% don't even have a foundation of research or support.

Venture Into Cures is raising $25MM to cure EB now, and change the way cures are found forever.

Investments supporting our innovative research model are being targeted as follows:



The Time to Act is Now.
We're Looking for Investors Who See What We Do — A Better Way.

$3,000,000

THREE YEARS

$500,000

Proof Positive:

Turning a $500,000 Investment into $3,000,000 of Impact.

A high-profile university was developing a gene therapy for EB and applied for a grant from EBRP. After review by our Scientific Advisory Board, we chose to fund the effort for $500K. A public biotech company then acquired the rights to the intellectual property to manufacture the treatment. In exchange, they issued EBRP stock in their company. The stock went on to multiply well beyond our initial investment. Within three years, we sold our shares for $3MM and redirected that revenue back into additional EB research.

Driven by Science. Directed by Business Leaders. Fueled by Philanthropic Investors Like You.

Combining the expertise of our esteemed Scientific Advisory Board, executive business leaders, research consortia, and parent advocates, we have accomplished much in a short time. Along the way, we've refined our model and optimized our methodology and are now ready to scale Venture Into Cures. Our goal is a cure for EB – and a new hope for developing treatments for other rare diseases.

Driven Donors:
Ari Deshe

Ari, as well as three of his children, were born with EB Simplex. The Deshe family has generously invested millions of dollars to take an active role in advancing a cure. After conducting an extensive search with their philanthropic advisors, EBRP was chosen for our ability to deliver the highest impact in achieving a cure for all forms of EB.

Scientific Advisory Board:
Anne Lucky, MD

Dr. Lucky chairs the EBRP Scientific Advisory Board (SAB) of leading doctors, scientists, and researchers. The Board's expertise spans dermatology, genetics, rare disease, and clinical trial development. The SAB evaluates and scores every project we fund, offering guidance so that EBRP can focus on projects that can put treatments into the hands of patients within three to five years.

Board of Directors:
Alexander Silver, MBA

Alex chairs our Board of Directors. This entity brings together business leaders and parents on a mission to save children's lives. Alex combines his background as a founding partner of a New York City investment firm with the heartfelt commitment of an EB father. Working with his Board, he applies the methodology of impact investing to the pursuit of a cure for EB.

Foundation Leadership:
Michael Hund, MBA

Michael works with his team to keep EBRP tightly focused on executing the foundation's innovative, venture-funded research model. His entire career has been dedicated to transforming healthcare — a process he honed at the Yale School of Management. Recently, Michael helped drive the FDA approval of 10 treatments that tripled life expectancy for patients with a rare blood cancer. He now brings this expertise to bear on curing EB.

Research Community:
Anna Bruckner, MD

Dr. Bruckner is the driving force behind a global community of more than 50 researchers and their EBRP-funded research projects. Her role is to champion collaboration across all the 21 academic centers of excellence in the EB Clinical Research Consortium. By knocking down barriers to cooperation, she empowers each researcher to use shared data for a shared purpose — to deliver hope and healing to those battling EB.

2010
- Jackson Gabriel Silver Foundation established

2011
- 1st research project funded
- EBCRC established

2012
- 1st patient enrolled in CCOD
- HealEB formed

2014
- JGSF and HealEB merge to form EBRP

2015
- EBRP Scientific Advisory Board founded

2016
- iPS Cell Consortium established
- 30th research project funded

2018
- 800th patient enrolled in CCOD
- 20+ ongoing clinical trials in EB
- EBCRC grows to 21 hospitals

2019
- 50th research project funded
- Five clinical trials primed for Phase III
- Venture Into Cures launched

NEXT
- 1st FDA-approved EB teatment
- 1st FDA-approved EB curative therapy
- Venture Into Cures model adopted by other rare diseases

Ten Years. Many Milestones. And Plenty of Room to Grow.

"It's about the most insane skin disorder you can imagine. And when you realize it also affects the internal organs, then you see it as diabolical."

— Ed Vedder
EBRP co-founder

Rocking the Status Quo:
Meet Jill and Ed Vedder.

Jill and Ed knew they had to take action when a childhood friend's son was born with EB. They combined their commitment to catalyzing global change with Ed's powerful platform of being the singer-songwriter for one of the world's biggest rock bands: Pearl Jam. Together, they are not only co-founders of EBRP but also leading ambassadors sharing the mission of curing EB with the world.

— FACES OF EB —

By Fixing a Single Mutation, We Can Change the Lives of Many.

It's incredible the suffering a single genetic mutation can unleash. Yet, for those who suffer from EB and other genetic diseases, it is often just that — a single mutation.

Through tireless research, scientists have identified the mutation that leads to EB. They believe they know how to fix the problem. And they believe that this fix is applicable to many genetic diseases.

What is needed now is clear. Access to data. The ability to collaborate and share knowledge. The resources to do that work quickly.

EBRP is committed to making this possible.

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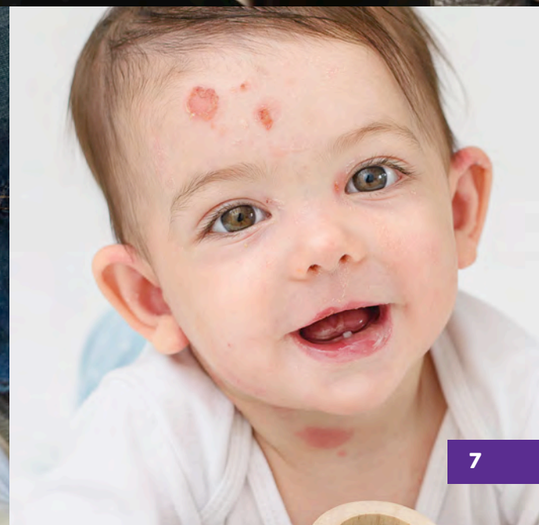
Join us on our venture into cures.
Your investment will be transformational.



Founded in 2010 by a group of dedicated parents along with Jill and Ed Vedder (Pearl Jam), EB Research Partnership (EBRP) is the largest 501(c)(3) nonprofit dedicated to funding research to treat and cure Epidermolysis Bullosa (EB), a group of devastating and life-threatening genetic rare diseases affecting children from birth and attacking the body's largest organ: the skin. EBRP combines rigorous and collaborative scientific research, astute business discipline, and a unique philanthropic investment model. Together we are accelerating the path forward to cure EB, and in the process, we are pioneering the roadmap for all disease.

To get involved visit ebresearch.org

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