



EB RESEARCH
PARTNERSHIP

impact report 2020





During 2020, the COVID-19 pandemic presented a great deal of global challenges and uncertainty. Despite this backdrop, EB Research Partnership did not stop—and will not stop—working tirelessly to advance our mission of funding vital, life-saving research.

For a decade, EBRP’s work has been laser-focused on supporting the brave researchers, doctors, healthcare professionals, and families battling EB every day. Their reliance on EBRP’s support inspires us to work harder than ever. While the world seemed to stop during these trying times, time never stood still for those living with EB. For many, the pandemic accelerated the progression of their disease. The resources EBRP provides to advance research have taken on an even greater importance as significant philanthropic resources are understandably focused on solving a global pandemic. Our dedication to the EB community of patients, families, and researchers has never been stronger. We think of Teya, who lives with Recessive Dystrophic EB. The pain that she suffers and the grit that she demonstrates on a daily basis are her reality, regardless of a pandemic or not. We are reminded of our obligation to Teya’s future as we forge ahead.

“EB is a rare and awful condition that affects her body inside and out. Seeing your child struggle and not being able to help much is the worst feeling as a parent. Most of Teya’s body is always bandaged. Teya’s life is full of challenges, but her strength and positivity prevail over the evils of EB. We, as parents, look up to her as she has shown us first-hand what being resilient is and how to stay positive. Currently no cure exists for Teya or her friends battling EB. With your support, however, there is hope!”

— Milana and Aleks, Teya’s Parents

In the face of adversity, we at EBRP did what we have always done, what families like Teya’s do every day: adapt, innovate, and persist. In 2020, we awarded \$7.2 million in research grants to 17 projects in five countries to fund life-saving treatments and potential cures for EB. Our total impact has increased to \$40 million raised for 94 projects, helping transform the EB landscape from two clinical trials in 2010 to more than 32 in 2021—a 15x increase from when we began our work. For the first time ever, there are four Phase III clinical trials in EB, which is the last phase before approval by the FDA. The possibility for our first approved treatment in EB is on the horizon.

Thanks to supporters like you, now is a critical moment in time when life-changing EB therapies are becoming not an “if”, but a “when”. With the current momentum, adequate financial resources, and our innovative venture philanthropy model, we are accelerating the pace at which meaningful treatments will be available to help Teya and others with EB. Each day we are getting closer to making an EB-treated world a reality, and in the process, are pioneering a model to accelerate potential treatments and cures for the 7,000 rare diseases that affect 10% of the global population. With you standing beside us, nothing will stop us in our mission to treat and cure EB.

We thank you for joining us on this journey, and provide this Impact Report to show you the meaningful difference your support has made in accelerating the path to heal EB.

Sincerely,

Michael Hund
Chief Executive Officer
EB Research Partnership

Alexander Silver
Chairman
EB Research Partnership



our mission



partner with us in our mission

**to further life-saving
research for EB**

OUR MISSION

Founded in 2010 by a dedicated group of parents and Jill and Eddie Vedder of Pearl Jam, EB Research Partnership (EBRP) is the largest global nonprofit dedicated to funding research aimed at treating and ultimately curing Epidermolysis Bullosa (EB), a group of devastating and life-threatening skin disorders that affect children from birth.

OUR MODEL

Working around the clock with offices in the US and Australia, EBRP ensures sustainable funding for future EB research through our innovative venture philanthropy model. Instead of simply writing grants, EBRP funds research projects in exchange for a financial interest in the work. If those projects lead to commercially successful therapies, we use the returns from our shares to fund additional EB research until a cure is found. This means your generous donation has the potential to grow to multiples of its original value, maximizing your impact.



progress
to a cure



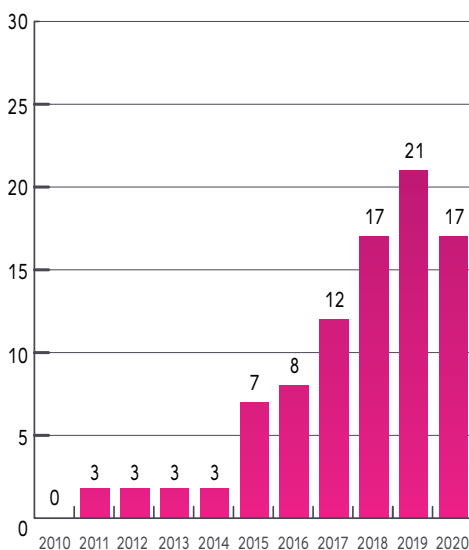
**94 Projects
Funded**

\$40M Raised

**4 Phase 3 clinical
trials in 2020,
the last phase
before potential
FDA approval**

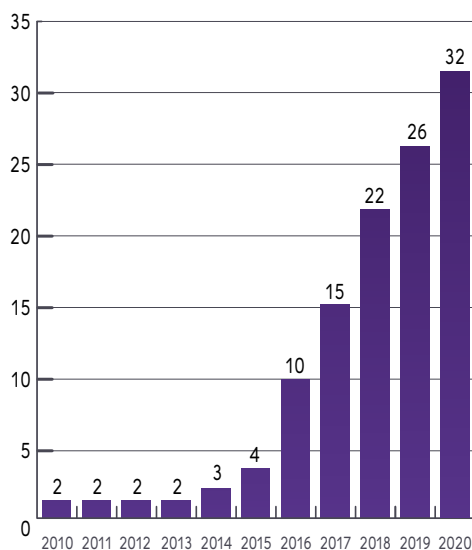
**15x the number
of clinical trials
in EB since
founding**

Projects Funded



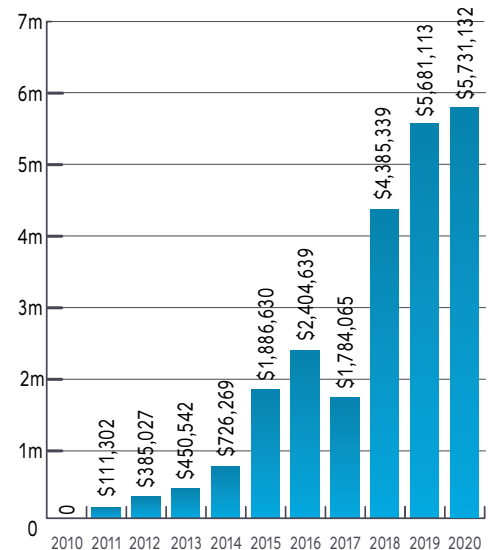
Projects Funded

Active EB Clinical Trials



Active EB Clinical Trials

EBRP Research Spend



Research Spend



résearch and data



EBRP accepts grant applications biannually and awards funding to competitive projects with potential to lead to treatments and cures for EB. Each application is reviewed by our distinguished Scientific Advisory Board (SAB) of experts in the fields of genetics, dermatology, basic science, and biotechnology. In 2020, we approved funding for nine new research projects and seven project renewals. In addition, we awarded funds to our EB Clinical Research Consortium, totaling over \$7.5M in awards. EBRP funded all efforts, securing some matching funds on four projects from our partners EB Medical Research Foundation and Cure EB.

2020 Newly Approved Research Projects

UNIVERSITY/ COMPANY	PROJECT NAME	PRINCIPAL INVESTIGATOR(S)	AMOUNT APPROVED
University of South Australia	Development of a systemic antibody therapy for the treatment of epidermolysis bullosa.	Allison Cowin, PhD	\$120,795
Stanford University	Evaluating the Natural History of Recessive Dystrophic Epidermolysis Bullosa Wounds	Jean Y. Tang, MD, PhD	\$498,023
Stanford University Amazon Web Services	EBCRC version 2.0: Investigator and patient portals on Amazon Web Services	Jean Y. Tang, MD, PhD	\$730,382
INSERM	Strategies for efficient and long-term engraftment of Mesenchymal Stromal Cells for the treatment of Recessive Dystrophic Epidermolysis Bullosa.	Alain Hovnanian, MD, PhD	\$144,351
Stanford University	An Anonymous Online International Survey of Prevalence and Impact of Cannabinoid Use in Patients with Epidermolysis Bullosa	Jean Y. Tang, MD, PhD Emily S. Gorell, DO, MS	\$ 35,986
University of Freiburg	Characterization Of Tissue Biomarkers For Fibrosis And Inflammation In Patients With Dystrophic Epidermolysis Bullosa	Dimitra Kiritsi, MD	\$73,522
King's College London, UCSF	Investigating the transcriptome of EB wounds at single-cell level	Alexandros Onoufriadis, PhD John McGrath, MD Raymond Cho, MD, PhD Jeffrey Cheung, MD	\$225,344
Northwestern University, University of Freiburg	Augmented Intelligence in EB: Using deep learning for early detection of squamous cell carcinoma in EB	Antonia Reimer-Taschenbrecker, MD Abel N. Kho, MD Amy S. Paller, MD	\$379,267
University of Minnesota	Autologous Revertant Mosaic Fibroblasts for Wound Healing in Dystrophic Epidermolysis Bullosa	Jakub Tolar, MD, PhD	\$1,000,000
TOTAL AWARDED			\$3,207,670

2020 Renewal Research Projects

UNIVERSITY/ COMPANY	PROJECT NAME	PRINCIPAL INVESTIGATOR(S)	AMOUNT APPROVED
FIBRX Tissue Repair	Development of Human Recombinant Decorin Core Protein as a Topical Anti-Scarring Therapy for Dystrophic Epidermolysis Bullosa	Jean Y. Tang, MD, PhD	\$300,000
University of Minnesota	Next Generation Genome Editing for RDEB	Jakub Tolar, MD, PhD	\$1,000,000
Tufts Medical Center	Development of a Collagen VII Eye Drop for the Treatment of Ocular Disease in Recessive Dystrophic Epidermolysis Bullosa	Vicki M. Chen, MD	\$336,315
Stanford University	Randomized Controlled Trial of a Neurokinin-1 Receptor Antagonist for the Treatment of Pruritus in Patients with Epidermolysis Bullosa	Albert Chiou, MD Jean Y. Tang, MD, PhD	\$495,480
Stanford University	Bridge Funding for iPS	Tony Oro, MD, PhD	\$800,000
Thomas Jefferson University	Targeting fibrosis for RDEB therapy in preclinical animal models	Andrew South, PhD	\$101,350
Wings Therapeutics	Clinical development of QR-313 for treatment of DEB	Mark De Souza, PhD	\$1,000,000
TOTAL AWARDED			\$4,033,145

**Total Combined 2020
Research Projects
Awarded: \$7,240,815**



Research Highlights



UNIVERSITY OF MINNESOTA

Title: Autologous Revertant Mosaic Fibroblasts for Wound Healing in Dystrophic Epidermolysis Bullosa

Institution:
University of Minnesota

Award Amount:
\$1,000,000

Principal Investigators:
Jakub Tolar, MD, PhD

Patient Population:
Dystrophic EB (DEB)

About: Some patients with DEB have naturally occurring unaffected patches of skin where the EB-causing mutation is corrected. This phenomenon is known as “revertant mosaicism”. Dr. Tolar’s lab is working on identifying and isolating these naturally corrected cells, growing them in the lab, and injecting them back into patients.

“Our goals are to continue to optimize gene editing technologies towards gene therapy and to create gene-corrected cells and topical delivery systems for therapeutic use. We are also working to comprehensively understand the unique cellular functions of RDEB squamous cell carcinoma tumors with the goal of developing immune therapies and rapid diagnostic testing.”

— Dr. Jakub Tolar



Northwestern University



Title: Augmented Intelligence in EB: Using deep learning for early detection of squamous cell carcinoma in EB

Institution:
Northwestern University and University of Freiburg

Award Amount:
\$379,267.00

Principal Investigator:
Amy S. Paller, MD
Antonia Reimer-Taschenbrecker, MD
Abel N. Kho, MD

Patient Population:
Recessive Dystrophic EB

About: Squamous cell carcinoma (SCC) is the most common cause of death for individuals with RDEB, shortening life expectancy to just 30 years. Patients must have regular skin checks at their dermatologist to remove any SCCs, however, distinguishing SCCs from normal EB wounds is difficult, even for experts. This lab aims to utilize deep learning and augmented intelligence as an early detection tool for SCCs in RDEB patients, improving disease management and prolonging lives.

“Squamous cell skin cancers are a common cause of death in adults with RDEB. Early diagnosis is critical. We plan to find patterns to facilitate diagnosis and identify best sites for biopsy. Ultimately, we hope to develop an app for helping families and physicians to interpret risk of cancer.”

— Dr. Amy Paller

Research Highlights

Continued



University of
South Australia

Title: Development of a systemic antibody therapy for the treatment of epidermolysis bullosa.

Institution:
University of South Australia

Award Amount:
\$120,795.00

Principal Investigator:
Allison Cowin, PhD

Patient Population:
All EB Types

About:

Dr. Cowin has identified a protein that contributes to inflammation, which leads to itch, wounds, and scarring in individuals with EB. She is creating an antibody that binds to this harmful protein, rendering it ineffective. Immunotherapy has been a helpful tool to combat several diseases, most prevalently cancer, and its potential to improve quality of life for the EB community is promising.

“Our goal is to develop an antibody therapy that simultaneously improves healing of blistered skin while reducing the severity of internal ulcerations of the digestive tract for patients with RDEB.”

— Dr. Allison Cowin



Title: Strategies for efficient and long-term engraftment of Mesenchymal Stromal Cells for the treatment of Recessive Dystrophic Epidermolysis Bullosa.

Institution:
INSERM

Award Amount:
\$144,351.00

Principal Investigators:
Alain Hovnanian, MD, PhD

Patient Population:
Recessive Dystrophic EB

About:

Stem cells from healthy bone marrow have shown exciting therapeutic potential for patients with RDEB, especially for internal wounds that are difficult to treat. However, most of these cells fail to engraft and target affected areas, so they are unfortunately cleared from the patient’s body. This project aims to improve the survival, engraftment, and homing of stem cells following injection as well as discover the best strategy for these cells to provide long-term systemic benefit to patients.

“Mesenchymal Stromal Cells (MSCs) have the potential to improve wound healing and skin inflammation in RDEB patients. We aim to overcome existing limitations by optimizing in vitro conditioning prior to their injection. We can determine the best strategy for long-term engraftment of MSC to ensure long-lasting therapeutic benefits in multiple cutaneous and mucous sites in RDEB patients.”

— Dr. Alain Hovnanian

Working Around the Clock to Heal EB



In October 2020, EBRP united with our longtime partners EB Research Foundation, based in Melbourne, Australia, to become the largest global organization focused on funding research to discover treatments and cures for EB. EBRF has come under the EBRP banner as “EB Research Partnership Australia.” Together, we will work around the clock to propel life-saving therapies into the hands of patients and families.



“Our shared mission to find treatments and cures for EB will be better served by uniting our organizations. We are looking forward to the great work our collective team will accomplish with combined resources and an established global presence.”

**— Scott Didier AM, Chairman of EBRP Australia
and Executive Board Member of EBRP**

EB Research Foundation was founded by the Wilkes Family when their daughter Tilly was born with EB in 2011. Once they adjusted to the realities of raising a child with this devastating disorder, they sprung into action to create awareness in their network and raise funds for a cure, much like the founders of EBRP. Our shared mission brought the two organizations together in 2018 to fund promising EB research, and we’re ecstatic to have established EBRP Australia this year.

Data

EB Clinical Research Consortium

EB Research Partnership founded the Epidermolysis Bullosa Clinical Research Consortium (EBCRC) with leading North American pediatric dermatologists. The EBCRC is made up of 20 prominent medical centers that contribute patient data to the EB Clinical Characterization and Outcomes Database (CCOD), which includes records on over 800 EB patients. Data drives progress, and EBRP is committed to accumulating the largest dataset possible to accelerate research for EB treatments and cures.



2020 Awards: \$299,021



Interviewing Jean Y. Tang, MD, PhD



Q | How has the landscape of clinical trials in EB changed since you began working with EB patients?

The landscape is so different and hopeful and exciting all at the same time. We are now witnessing at least four Phase 3 clinical trials in EB! 10 years ago, we had nothing!

Q | Why is data important to advancing EB therapies?

EBRP is focused on data that will eventually lead to new treatments for EB. For example, they funded a natural history study of EB wounds at Stanford. These data are critical to show the FDA that EB wounds cover large surface areas of the body, they are painful, and never spontaneously heal. They are different from diabetic wounds or burn wounds, and need new “rules” for showing treatment efficacy. We are also focused on patient reported outcomes like pain, itch, and quality of life that are important to measure in future clinical trials.

“ The landscape is so different and hopeful and exciting all at the same time. We are now witnessing at least four Phase 3 clinical trials in EB! 10 years ago, we had nothing! ”

— Jean Y. Tang, MD, PhD

Q | What makes you hopeful that we will find a cure for EB?

The world now knows about EB because of the good work, fundraising, and publicity from EBRP. Biotech and Pharma now care about rare diseases like EB and basic science advancements (like CRISPR technology, stem cells, etc.) that can now correct EB mutations. Protein replacement therapy like intravenous collagen is now being tested in trials. I am super hopeful that we will see an FDA approved drug for EB soon.

A photograph of a man and a woman in a clinical or laboratory setting. The man, on the right, is wearing glasses and a white button-down shirt, and is looking towards the woman. The woman, on the left, has long brown hair and is wearing a blue and white plaid shirt. They appear to be in a conversation. The background is slightly blurred, showing what looks like a laboratory or office environment. There are some decorative white lines (solid and dashed) overlaid on the image, and a red gradient at the bottom.

clinical landscape



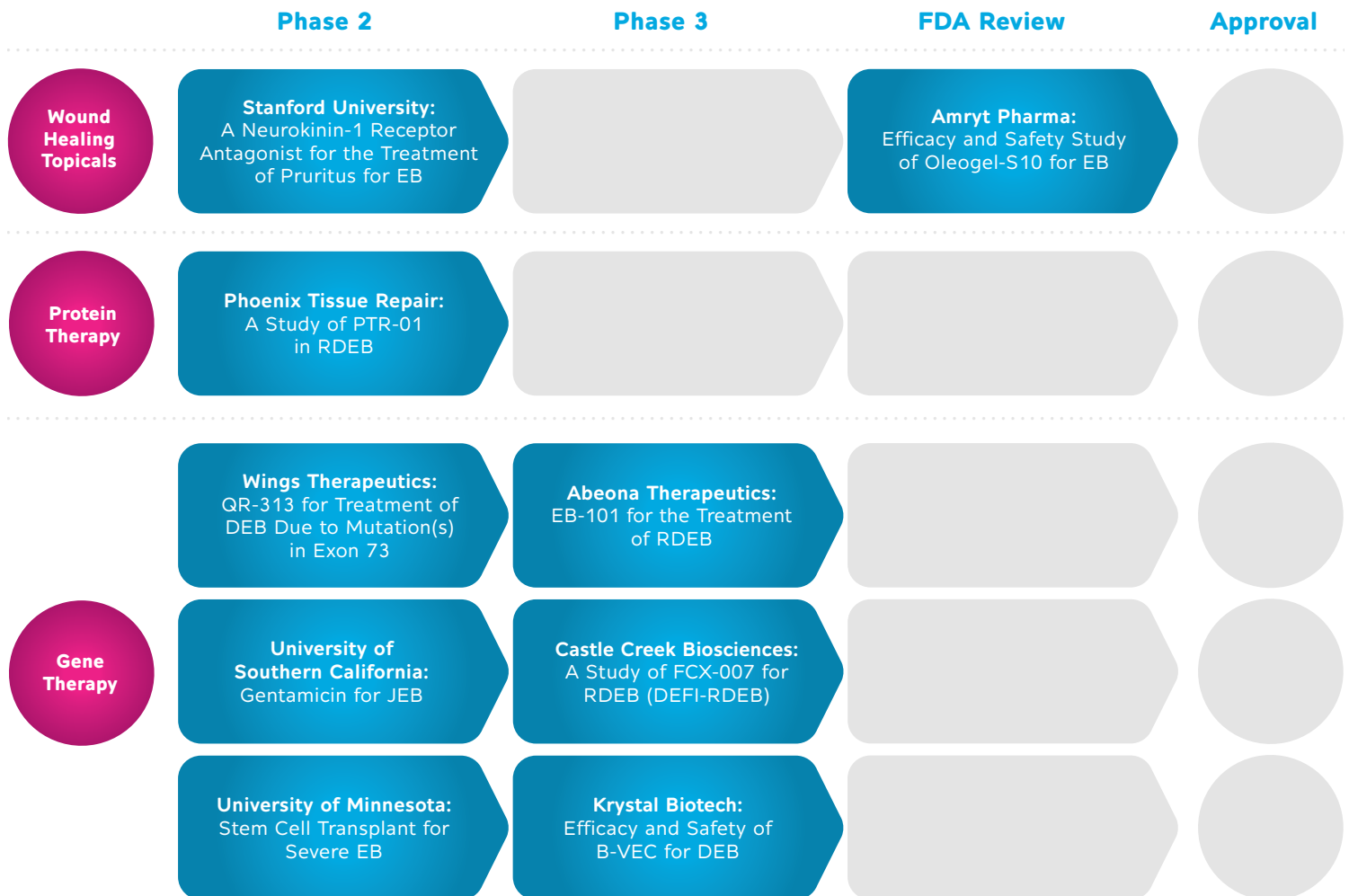
Since EBRP was founded in 2010, the number of clinical trials in EB has increased dramatically, from just two to over 30 today — a 15x increase. 2020 was a landmark year with four EB clinical trials in Phase 3, the final phase before potential FDA approval, for the first time ever.



Highlight: Amryt Pharma Files New Drug Application with FDA

In May 2021, Amryt Pharma became the first company to complete their trial, the EASE Study, and submit a New Drug Application (NDA) for Oleogel S-10, a wound healing topical gel for individuals with Dystrophic and Junctional EB, to the FDA. Amryt was granted Priority Review for this application, meaning the FDA shortens the review period to just six months.

Take a look at other major clinical trials in the pipeline that EBRP was an early investor in, with your support. These studies test a wide range of interventions from topicals to protein therapies and gene therapies.



A man with a beard and dark hair is sitting on a black metal stool in a dimly lit room, possibly a bar or a music studio. He is wearing a short-sleeved, button-down shirt and dark pants. He is holding a light-colored acoustic guitar and looking directly at the camera. The background is dark, with shelves of bottles visible. The word "events" is written in large, white, lowercase letters across the middle of the image. There are several white diagonal lines and dashed lines overlaid on the image.

events



\$2,434,952 was raised

through events in 2020. We are so grateful to all event organizers, sponsors, and supporters for joining us virtually to #healEB

2020 EBRP Events

Plunge for Elodie

March 7 & 8
Wellesley, MA
Old Greenwich, CT
Staten Island, NY
San Francisco, CA

All In For A Cure

July 8
Virtual Event

Venture Into Cures

November 18
Virtual Event

Take Flight 5K & Fun Run

August 16-30
Virtual Event



All in for a Cure



Take Flight



Venture Into Cures

Events



Venture Into Cures

November 18, 2020
Virtual Event

Last fall, EBRP co-founders Jill and Eddie Vedder hosted the inaugural Venture Into Cures, an inspiring virtual event featuring moving stories about individuals and families living with Epidermolysis Bullosa (EB) alongside our celebrity friends. With more than 500,000 viewers, the event raised more than \$2 million to fund EB research and pioneer our model to cure all rare disease.





Venture Into Cures Celebrity Friends

Judd Apatow
 Jon Batiste
 Alessia Cara
 Bradley Cooper
 Andra Day
 Laura Dern
 Billie Eilish
 Glen Hansard
 Chris Hemsworth
 Jimmy Kimmel
 David Letterman
 Adam Levine
 Gaten Matarazzo
 Keb' Mo'
 Lukas Nelson & Promise of the Real
 with Micah and Willie Nelson
 Luis "King Kong" Ortiz
 Adam Sandler
 Eddie Vedder
 Jill Vedder
 Renée Zellweger



Save the Date

This year's Venture Into Cures
will be on November 18, 2021

Learn more at:
www.ventureintocures.org



financials

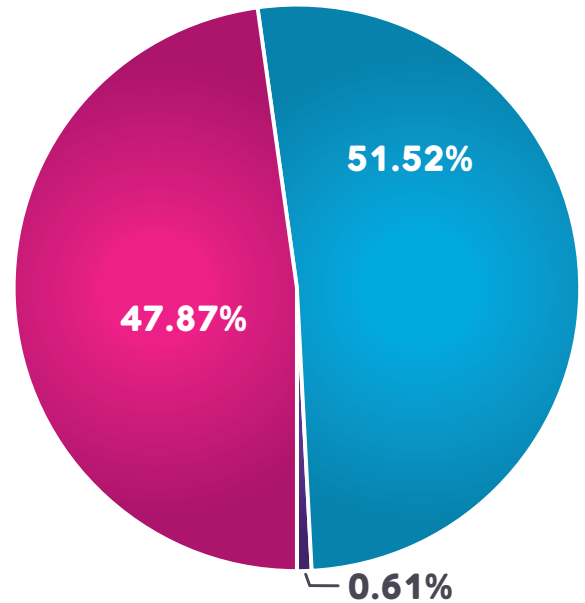


2020 Financial Summary

EBRP is committed to the highest financial responsibility and has received the top ratings from GuideStar, Platinum Seal of Transparency, and Charity Navigator, 4 stars. For complete audited financial information, please visit our website at www.ebresearch.org.

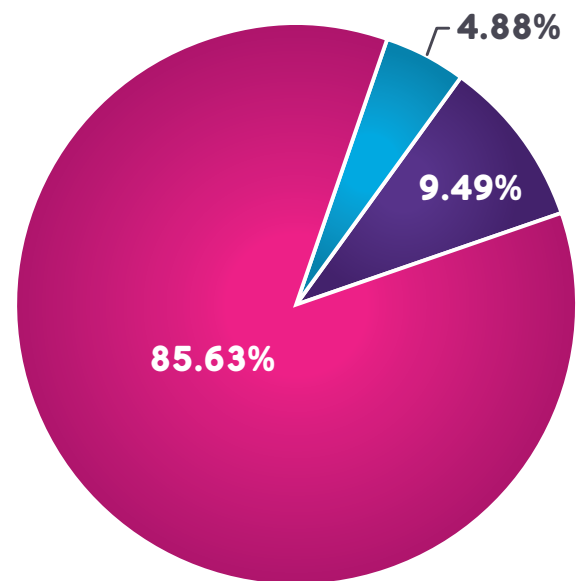
2020 EBRP Support & Revenue \$4,726,341

- Contributions
\$2,262,326
- Fundraising Events
\$2,434,952
- Investment Income
\$29,063



2020 EBRP Spending Allocation \$7,139,755

- Program & Research*
\$6,113,699
- Management
\$348,482
- Fundraising
\$677,574



*Includes funded EB research projects with academia and private/public companies

Ending Net Assets: \$14,784,158



'leadership



Executive Board

Alexander Silver
Co-Founder & Chairman

Jill Vedder
Co-Founder & Vice Chairman

Eddie Vedder
Co-Founder

Jamie Silver
Co-Founder

Heather Fullmer
Co-Founder

Ari Deshe

Scott Didier AM

Stephen Evans

Edward Grossmann

Directors

Eileen Attar

Tracy Baldwin

Jeffrey Berger

Mark Bomback

Chad Ceretto

Eleanor Dehoney

Daniel Deshe

Faye Dilgen

Richard Grossmann

Matthew Holmes

Michael Kahn

Emily Kubik

Jennifer Kauf

Kate Lee

Alexander Lemos

Abbie Levine

Joshua Paulson

Matthew Prince

Whitney Pollack

Monique Sock

Jared Stern

Rob Veres

Donna Whiffin

Staff

Michael Hund
Chief Executive Officer

Kiley Bergin
Vice President of Development

Craig Fox
Controller

Stephanie Ishoo
Director of Communications

Maxson Thomas
Development Associate

Michelle Hall
Senior Accountant

Scientific Advisory Board

Anne Lucky, MD, Chair

Greg Barsh, MD, PhD

Suephy Chen, MD, MS

Alain Hovnanian, MD, PhD

Christopher Sloey

Amy Paller, MD



EB RESEARCH
PARTNERSHIP

244 Madison Ave
Suite 104
New York, NY 10017

www.ebresearch.org

646-844-0902

info@ebresearch.org