
EB RESEARCH PARTNERSHIP
impact report 2019

Salim is a joyful, loving boy who is full of life. He was born in India in 2014 and spent the first three and a half years of his life in an orphanage there. In 2018, Salim's mom Laura was finally able to bring Salim home and complete his adoption. Salim has Epidermolysis Bullosa (EB). It ravages not only the skin we can see but also the inside of his body. He is one of 500,000 people in the world with EB. With no disease-modifying treatments yet available, Salim's day consists of painful baths and bandage changes, tube feeds, and countless doctors visits and medications. While EB is a diabolical opponent, Salim's mom remains hopeful, largely because of EB Research Partnership's commitment to heal and cure her son's life-threatening disease.

> *"Every day I wake up before the sun rises to start our day's fight against EB. I do my best to show Salim strength and bravery, even though he is far stronger and braver than I will ever be. The only way that I'm able to summon all that I am is both because of my immense love for my son and because I cling to the hope that EBRP provides us...hope for a cure. I read medical journal articles, scour clinical trials, watch talks given by scientists and researchers — so many of whom are funded by the generous donors of EBRP — and I am renewed with confidence and pride. We are so grateful for the collaborative, innovative, out-of-the-box thinking and research that EBRP funds."*
>
> *— Laura, Salim's Mom*

With you on our side, EBRP has embarked on a relentless mission to accelerate treatments and cures for EB. We have built a culture of urgency and innovation including launching the largest EB data project globally. EBRP brings together the often siloed academic, medical, and patient communities ensuring compounding benefits to each discovery. EBRP's Scientific Advisory Board carefully vets the most promising projects so we can invest in the most game-changing research and stack the odds of finding a cure in our favor.

Since our founding in 2010, we have funded more than 80 research projects, united a global consortium of 20 academic medical centers of excellence, and established the leading venture philanthropy model to create a sustainable investment portfolio. Our work in under a decade has already led to more than 10 times the amount of active clinical trials, including four Phase 3 trials — the first-ever Phase 3 trials for EB. We are now 70% of the way towards raising $25 million in our multi-year Venture Into Cures campaign. As research has advanced into clinical trials, the need for larger amounts of resources sooner is a challenge we embrace for all children living with EB. With your generous support, we are getting closer to making an EB free world a reality.

If we can do this, what does our future look like for Salim and the hundreds of thousands just like him? It means the daily bleach baths and hours of bandaging are no more. It means that a schedule once full of doctors visits can be filled with new adventures. It means no more fear. It means no more pain. It means never having to pronounce the words Epidermolysis Bullosa except for when celebrating its cure.

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 healing EB, and in the process showing the world how cures are found.

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 group of dedicated parents and Jill and Eddie Vedder, of Pearl Jam, EB Research Partnership (EBRP) is the largest 501(c)(3) nonprofit funding research to discover treatments and cures for Epidermolysis Bullosa (EB), a devastating and life-threatening genetic skin disorder that affects children from birth.

OUR MODEL

EBRP ensures sustainable funding for future EB research through our innovative venture philanthropy model. Instead of simply awarding 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. This means your generous donation has the potential to grow to multiples of its original value.

progress to a cure

80 Projects Funded
to date.

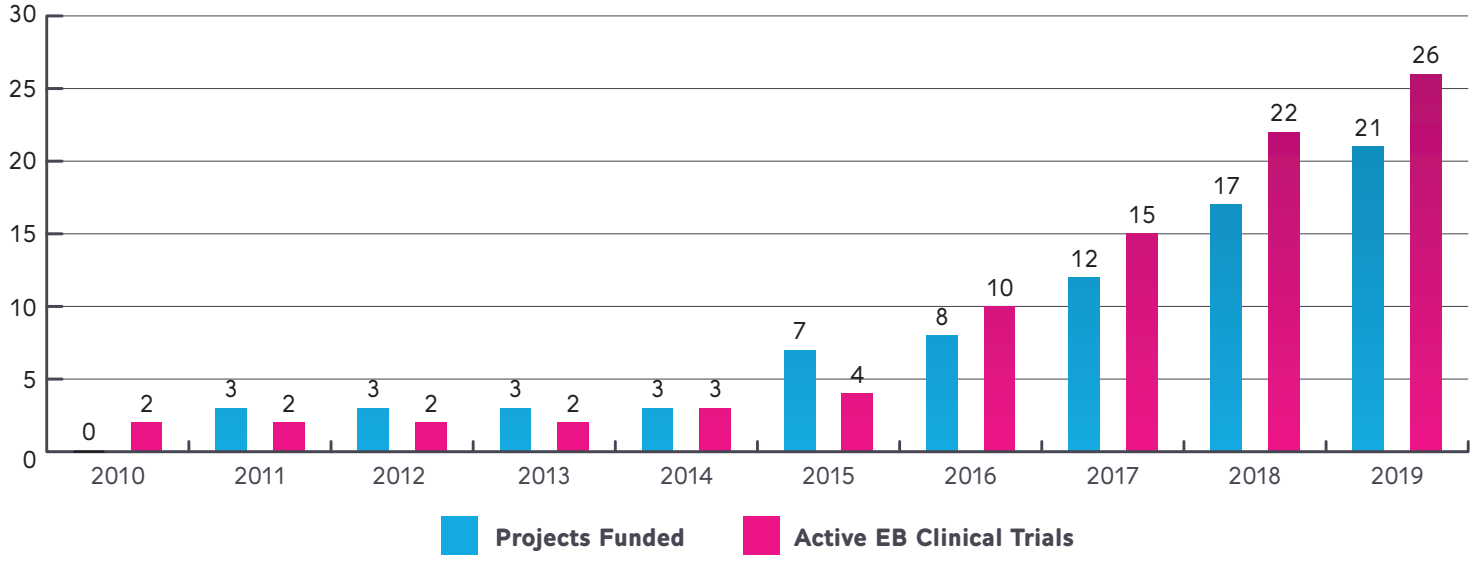
$40M Raised
to date.

Four Phase 3 Clinical Trials
in 2020.

Data from over 800 Patients at 20 Medical Centers
in our EB Clinical Characterization and Outcomes Database.

Nearly 90% of Revenue Goes Towards Research

EBRP's Impact at a Glance




research
and data

EBRP accepts grant applications biannually and awards funding to competitive projects with the 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 2019, the SAB recommended funding for nine new research projects and six project renewals. In addition, we awarded funds for three ongoing research projects and for our EB Clinical Research Consortium, totaling over $7.5M in awards. EBRP funded all efforts, securing matching funds from our partners EB Research Foundation of Australia, EB Medical Research Foundation and Cure EB.

2019 Newly Approved Research Projects

INSTITUTION	PROJECT NAME	PRINCIPAL INVESTIGATOR(S)	AMOUNT AWARDED
Columbia University	Development of a Drug Testing Platform for Recessive Dystrophic Epidermolysis Bullosa Squamous Cell Carcinoma Using Induced Pluripotent Cancer Cells	Joanna Jackow, PhD	$551,250
Neem Biotech Limited	Identification of Ajoene Analogues for the Treatment of Epidermolysis Bullosa	Tracy Nevitt, PhD	$112,400
University of Colorado	Developing an IPS Cell-Based Therapy for Epidermolysis Bullosa Simplex	Dennis R Roop, PhD Ganna Bilousova, PhD Igor Kogut, PhD Anna Bruckner, MD	$518,638
Universite a Quebec a Chicoutimi	Inactivation of Epidermolysis Bullosa Simplex Dominant Mutations by Allele-Specific CRISPR/Cas9	Catherine Laprise, PhD Lucie Germain, PhD Jacques-P Tremblay, PhD	$283,204
Centre for Human Genetics	Development of a Registry for Epidermolysis Bullosa in India	Ravi Hiremagalore, MD Gurudatta Baraka, PhD Arun Inamadar, MD Sacchidanand, MD	$35,620
University College Dublin	Development of Gene-Editing Therapy to Restore Type VII Collagen for the Treatment of Recessive Dystrophic Epidermolysis Bullosa Using a Topical RNP CRISPR System	Wenxin Wang, PhD	$117,053
Thomas Jefferson University	Epidermolysis Bullosa Community Cell Bank	Andrew South, PhD	$151,651
Thomas Jefferson University	An Immune-Competent Mouse Model of Recessive Dystrophic Epidermolysis Bullosa Squamous Cell Carcinoma for Preclinical Therapeutic Testing	Andrew South, PhD	$137,466
Stanford University	GMP Manufacturing of Autologous Esophageal Epithelial Cells for the Prevention of Esophageal Strictures	Anthony Oro, MD, PhD	$300,421
		TOTAL AWARDED	**$2,207,703**

2019 Renewals and Ongoing Research Funding

INSTITUTION	PROJECT NAME	PRINCIPAL INVESTIGATOR(S)	AMOUNT AWARDED
Thomas Jefferson University	Targeting Fibrosis for Recessive Dystrophic Epidermolysis Bullosa Therapy in Preclinical Animal Models	Andrew South, PhD	$159,054
University of Minnesota	Next Generation Genome Editing for Recessive Dystrophic Epidermolysis Bullosa	Jakub Tolar, MD, PhD	$1,000,000
University of Southern California	Optimization of Intravenous Gentamicin Treatment to Restore Functional Laminin 332 in Junctional Epidermolysis Bullosa Patients with Nonsense Mutations	Mei Chen, PhD David Woodley, MD	$254,300
Stanford University	Topical and Intradermal Recombinant Type VII Collagen Protein Replacement Therapy for Recessive Dystrophic Epidermolysis Bullosa: A Placebo Controlled Phase 1 Clinical Trial	M. Peter Marinkovich, MD Jean Tang, MD, PhD David Olsen, PhD Gerhard Bauer, PhD	$350,000
Stanford University	DISCOVER-JEB Study for Demystifying the Causes of Early Lethality in Generalized-Severe Junctional Epidermolysis Bullosa Due to Laminin-332 Mutations	M. Peter Marinkovich, MD Vamsi Krishna Yenamandra, MD, PhD Irina Gurevich, PhD Kerriann M Casey, DVM, DACVP	$127,000
Stanford University	Computational Drug Repurposing for Epidermolysis Bullosa Simplex	Joyce Teng, MD, PhD Kavita Sarin, MD, PhD	$187,782
Thomas Jefferson University	Targeting Fibrosis for Recessive Dystrophic Epidermolysis Bullosa Therapy in Preclinical Animal Models	Andrew South, PhD	$159,054
FIBRX Derm	Development of Human Recombinant Decorin Core Protein as a Topical Anti-Scarring Therapy for Dystrophic Epidermolysis Bullosa	Professor Jean Tang, MD, PhD	$1,250,000
Wings Therapeutics	Clinical Development of QR-313 for Treatment of Dystrophic Epidermolysis Bullosa	Mark De Souza, PhD	$1,500,000
University of Minnesota	Bioprinting Workstation for Epidermolysis Bullosa Therapy Development	Jakub Tolar, MD, PhD	$208,890
		TOTAL AWARDED	**$5,196,080**

Research Highlights

Title: Optimization of Intravenous Gentamicin Treatment to Restore Functional Laminin 332 in JEB Patients with Nonsense Mutations

Institution:
University of Southern California

Award Amount:
$254,300

Principal Investigators:
Mei Chen, PhD
David Woodley, MD

Patient Population:
Junctional EB

About: Junctional epidermolysis bullosa (JEB) is an incurable and fatal inherited blistering skin disease most commonly caused by nonsense mutations in genes coding for laminin 332, a crucial skin adhesion protein. As a result of lacking laminin 332, JEB patients have severe skin fragility, mucocutaneous blistering, and compromised wound healing. We aim to develop and optimize intravenous gentamicin readthrough therapies in order to create new laminin 332, improve wound healing, and improve the quality of life in JEB patients with nonsense mutations.

"The majority of JEB cases are caused by nonsense mutations, and gentamicin therapy works by suppressing these mutations to generate the missing laminin 332 protein. We envision that intravenous gentamicin may provide most JEB patients with a novel, low cost, minimally-invasive, and readily available therapy – simultaneously treating their multiple wounds and systemic symptoms."

— Mei Chen, PhD

Title: Clinical development of QR-313 for treatment of DEB

Institution:
Wings Therapeutics

Award Amount:
$1,500,000

Principal Investigator:
Mark De Souza, PhD

Patient Population:
Dystrophic EB

About: ~30% of patients with DEB have mutations in a part of the collagen VII gene known as exon 73. QR-313, a drug that can be applied to the skin, removes this part of the gene resulting in a slightly shorter but fully functional version of collagen VII. QR-313 is now being evaluated in DEB patients with exon 73 mutations to evaluate its safety, its ability to promote exon-skipping and collagen 7 expression, and wound healing.

"Our goal is to develop a non-invasive, topical, disease-modifying drug for patients with RDEB and DDEB to increase collagen VII expression, improve skin integrity and quality of life."

— Deborah Ramsdell, CEO

Research Highlights
Continued

Jefferson
Philadelphia University + Thomas Jefferson University
HOME OF SIDNEY KIMMEL MEDICAL COLLEGE

Title: Targeting fibrosis for RDEB therapy in preclinical animal models

Institution:
Thomas Jefferson University

Award Amount:
$159,054

Principal Investigator:
Andrew South, PhD

Patient Population:
Recessive Dystrophic EB

About:
Fibrosis is a major complication of EB and contributes to skin stiffness, esophageal problems, poor wound healing, and the eventual development of cancer. Dr. South has identified a number of drugs that are already FDA approved to treat other conditions, and this project will test these drugs in an animal model of recessive dystrophic EB.

"We have identified a number of promising drugs that inhibit fibrosis in patient cells in the laboratory. Here we will test these drugs in an animal model of EB to determine those that show the best improvement to health, fibrosis, and wound healing and do not show unwanted side effects with long term treatment. Once this work is complete, we can move directly to clinical trial because the drugs we are testing have already been used in people to treat other conditions."

— Andy South, PhD

Mariposa Therapeutics

Title: Identification of ajoene analogues for the treatment of epidermolysis bullosa

Institution:
Mariposa Therapeutics

Award Amount:
$112,400

Principal Investigators:
Tracy Nevitt, PhD

Patient Population:
EB Simplex

About:
EBRP formed Mariposa Therapeutics to focus fully on EB Simplex. This project involves the molecule ajoene, which promotes antimicrobial, anti-inflammatory, and wound healing activities. A screen performed on a library of almost 200 synthetic ajoene derivatives identified molecules that significantly ameliorate the molecular and cellular defects underlying EBS. The goal is to further progress lead compounds towards the development of a topical therapeutic for EBS.

"Our objective is to significantly improve skin resilience, increase the quality of life, and thereby reduce the burden of disease in EBS patients."

— Tracy Nevitt, PhD

Come Say “Hi”

Six-year-old Eli Meyer, who lives with Junctional EB, and his older sister Lily are on a mission to share their simple but astute message with the world. Lily noticed how strangers would stare at her little brother’s wounds, and the siblings banded together to encourage people that rather than just staring, they should “Come Say Hi” and learn more about Eli and EB. Armed with T-shirts and business cards, these amazing kids caught the eyes of EBRP Co-Founders Ed and Jill Vedder, who were compelled to help spread this important message. Eddie surprised Eli and Lily on the WE Day broadcast, which aired on ABC across the USA on August 9, 2019, with an original song and commended them for their inspiring work.

To match with Eddie, Eli, and Lily,
purchase your own Come Say “Hi” t-shirt at **homage.com/comesayhi**.

“A simple small word like that, two letters, it can smash down barriers”
— Eddie Vedder

Data

Data is a core pillar in realizing our mission of a world without EB. We aim to launch the largest EB data project imaginable to aggregate, centralize, analyze, and decode the clinical, genomic, and patient data underlying the disease.

EB Clinical Research Consortium

EB Research Partnership founded the Epidermolysis Bullosa Clinical Research Consortium (EBCRC) with leading North American pediatric dermatologists. The EBCRC, led by Dr. Anna Bruckner at Children's Hospital Colorado, 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.

2019 Awards: $279,686

Inventing a Platform

We've partnered with Amazon Web Services (AWS) to reimagine how rare disease data is leveraged to advance research and accelerate the path to cures by building a state-of-the-art technology platform that can be easily navigated by researchers and patients alike. Read this excerpt from a Global Genes case study on EBRP to learn more about our vision for this project.

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.


Inventing a Platform

Content taken from a recent case study on EBRP by Global Genes®

clinical landscape

Highlighted Clinical Trials

For the first time, there are four Phase 3 clinical trials in EB, the final phase before FDA approval, and more than 25 clinical trials in the pipeline.

Abeona Therapeutics: VIITAL Study — Phase 3 Clinical Trial of EB-101 for RDEB

In March 2020, Abeona Therapeutics dosed the first patient in the VIITAL Study, a pivotal Phase 3 clinical trial of EB-101, their gene-corrected cell therapy for Recessive Dystrophic EB. EB-101 treatment involves genetically correcting the Collagen VII gene in a patient's cells and returning those corrected cells to the patient via a skin graft. The previous Phase 1/2 trial showed durable wound healing from over two years to over five years. Abeona has received the Regenerative Medicine Advanced Therapy, Breakthrough Therapy, Rare Pediatric, and Orphan Drug designations for EB-101.

Castle Creek Biosciences: DeFi-RDEB — Phase 3 Clinical Trial of FCX-007 for RDEB

In early 2020, Castle Creek Biosciences initiated the DeFi-RDEB Study, a Phase 3 clinical trial of FCX-007, their gene therapy for RDEB. FCX-007 treatment involves taking cells from a patient, genetically modifying them to express collagen VII protein, and returning the cells to the patient via intradermal injection. In the previous Phase 1/2 trial, the therapy was well-tolerated and caused complete wound closure in 80% of patients. FCX-007 has received Orphan Drug, Rare Pediatric Disease, Fast Track, and Regenerative Medicine Advanced Therapy designations from the FDA..

Amryt Pharma: EASE Study — Phase 3 Clinical Trial of AP101 for EB

In April 2020, Amryt Pharma closed enrollment for the EASE Study, a Phase 3 clinical trial of AP101, their topical wound healing gel for treatment of Dystrophic EB, Junctional EB, and Kindler Syndrome. AP101 contains active ingredient Oleogel-S10, which was approved in 2016 in the EU for treatment of partial-thickness wounds under the name Episalvan. The EASE Study is the largest global Phase 3 clinical trial in EB, and the company expects topline data in late Q3/early Q4 2020. AP101 has received Orphan Drug, Rare Pediatric Disease, and Fast Track designations from the FDA.

Krystal Therapeutics: GEM-3 Study — Phase 3 Clinical Trial of B-VEC for DEB.

In July 2020, Krystal Biotech initiated the GEM-3 Study, a Phase 3 clinical trial of beremagene geperpavec (B-VEC), a topical gene therapy for treatment of Dystrophic EB. The trial aims to enroll 30 patients, who will be treated weekly over six months with either B-VEC or placebo on up to three wound pairs. The therapy aims to deliver a functional version of the Collagen VII gene through a viral vector directly to patients' skin. B-VEC has received Orphan Drug, Fast Track, Rare Pediatric, and Regenerative Medicine Advanced Therapy (RMAT) designations from the FDA and Orphan Drug designation and PRIority MEdicines (PRIME) eligibility from the EMA.

events

In 2019 $1,667,542 was raised

through events held throughout the country. Thank you to all event organizers, sponsors, and supporters for joining our mission to #HealEB.

2019 EBRP Events

Plunge for Elodie
March 2
Wellesley, MA & Cleveland, OH

All In For A Cure
May 16
New York, NY

Change for Charley
March 9
Chicago, IL

ACTion for Jackson
November 7
New York, NY

Believe in Brady
April 7
Houston, TX

2020 Virtual Fundraising!

Since we are unable to host our signature in-person events in 2020, your support for our virtual fundraising efforts will help drive our mission in this new events environment. Visit **ebresearch.org/virtual-fundraising** to start your own event and learn about new initiatives!

Events

ACTion for Jackson

November 7, 2019
New York, NY

Over 800 supporters gathered at the 10TH Annual ACTion for Jackson last November to further our mission. With their overwhelming support, $1.4M was raised to advance life-saving EB research and a new $3M gift from the Ann and Ari Deshe Family was celebrated that evening. *"We would love to see this disease gone, and we want to be a part of helping eradicate it—forever,"* says Ari Deshe. His family's contributions to heal EB now exceed $8M.

Change for Charley

March 9, 2019
Chicago, IL

The 2ND Annual Change for Charley raised $600,000 for EB Research. The Kauf Family hosts the gala-style event, emceed by ESPN's Scott Van Pelt, in honor of their daughter Charley, who lives with RDEB.

Rock4EB!

October 7, 2019
Malibu, CA

For the first time, EBRP joined our funding partners EB Medical Research Foundation for their annual Rock4EB!, a benefit concert for EB research. The star-studded evening featured a lineup of EBRP Co-Founder Eddie Vedder, who took the stage with Glen Hansard, and Adam Sandler. The event raised $1M, which was split between the two organizations.

financials

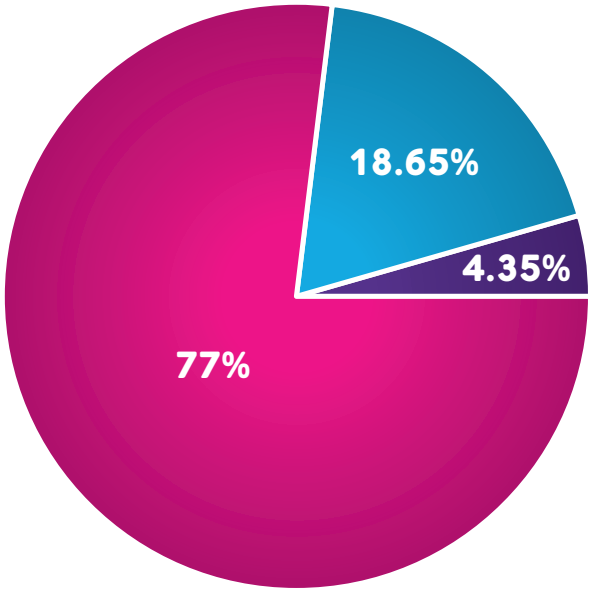
2019 Financial Summary

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 financials, please visit our website at **www.ebresearch.org**.

2019 EBRP Support & Revenue

$8,942,865

- Contributions **$6,886,155**
- Fundraising Events **$1,667,542**
- Other **$389,168**

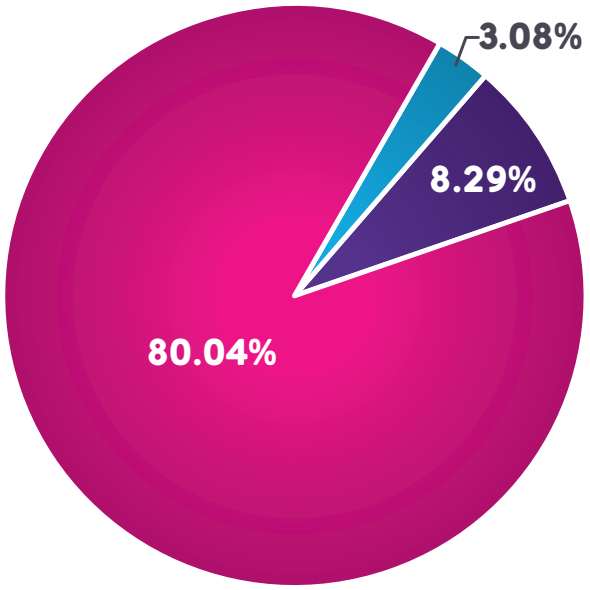


2019 EBRP Spending Allocation

$5,789,502

- Program & Research* **$5,918,609**
- Management **$205,942**
- Fundraising **$553,829**

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



Ending Net Assets: $16,108,085


leadership

Executive Board

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Co-Founder & Vice Chairman

Eddie Vedder
Co-Founder

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