EB RESEARCH PARTNERSHIP

impact report 2017

When “Brady the Brave” was born, his parents, Chris and Eileen, 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), meaning that Brady’s skin, his body’s largest organ, was so fragile that it would come off his body with the slightest touch. Simple scrapes became severe wounds, daily wrapping in full body bandages was required, and routine tasks like eating, walking, and sleeping became monumental daily challenges. Yet Brady, now two years old, has an indomitable spirit. He is a fighter, and so are his parents.

> *“Our family keeps high hopes that Brady will be able to do most of the things that ‘normal’ children can do. EB Research Partnership is working diligently to fund the research and the clinical trials needed to improve Brady’s quality of life and give him the chance to ‘be a kid.’ Treatment would mean Brady could walk and possibly even run! We will not give up and we are grateful for all who believe that together we will heal EB.” – Eileen*

At EB Research Partnership, we stand with Brady and all those battling EB. Thanks to your support, we are pursuing our mission to rapidly accelerate treatments and cures for EB and, in the process, pioneering a new venture philanthropy model for how rare diseases can be cured.

Since 2010, we have raised over $25 million to treat and cure EB. We have partnered with brilliant researchers and doctors, assembled a world-class scientific advisory board, created a collaborative medical research consortium and shared dataset among 21 institutions, and funded a research portfolio of more than 50 projects. EBRP supports research spanning all forms and approaches of healing EB, including exon skipping and gene therapy. When we started in 2010, there were only two clinical trials for EB. Today, there are nearly 20. While our goal is to cure EB, the research we support has the potential to help treat or cure the estimated 7,000 rare diseases that impact 10% of the global population.

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 a cure and life-changing treatments are within reach. To make this belief a reality, we need to accelerate our efforts. In the next three years, we aim to double our efforts, raising $25 million in half the time to maximize the possibility of success for those living today with EB. While this task is daunting, we believe that it can be done. Because if not us, then who? If not now, then when? The time to act is today, and the responsibility is ours. Together we can partner to make our vision of an EB-free world a reality.

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

Sincerely,

Michael Hund
Executive Director
EB Research Partnership

Alexander Silver
Chairman
EB Research Partnership

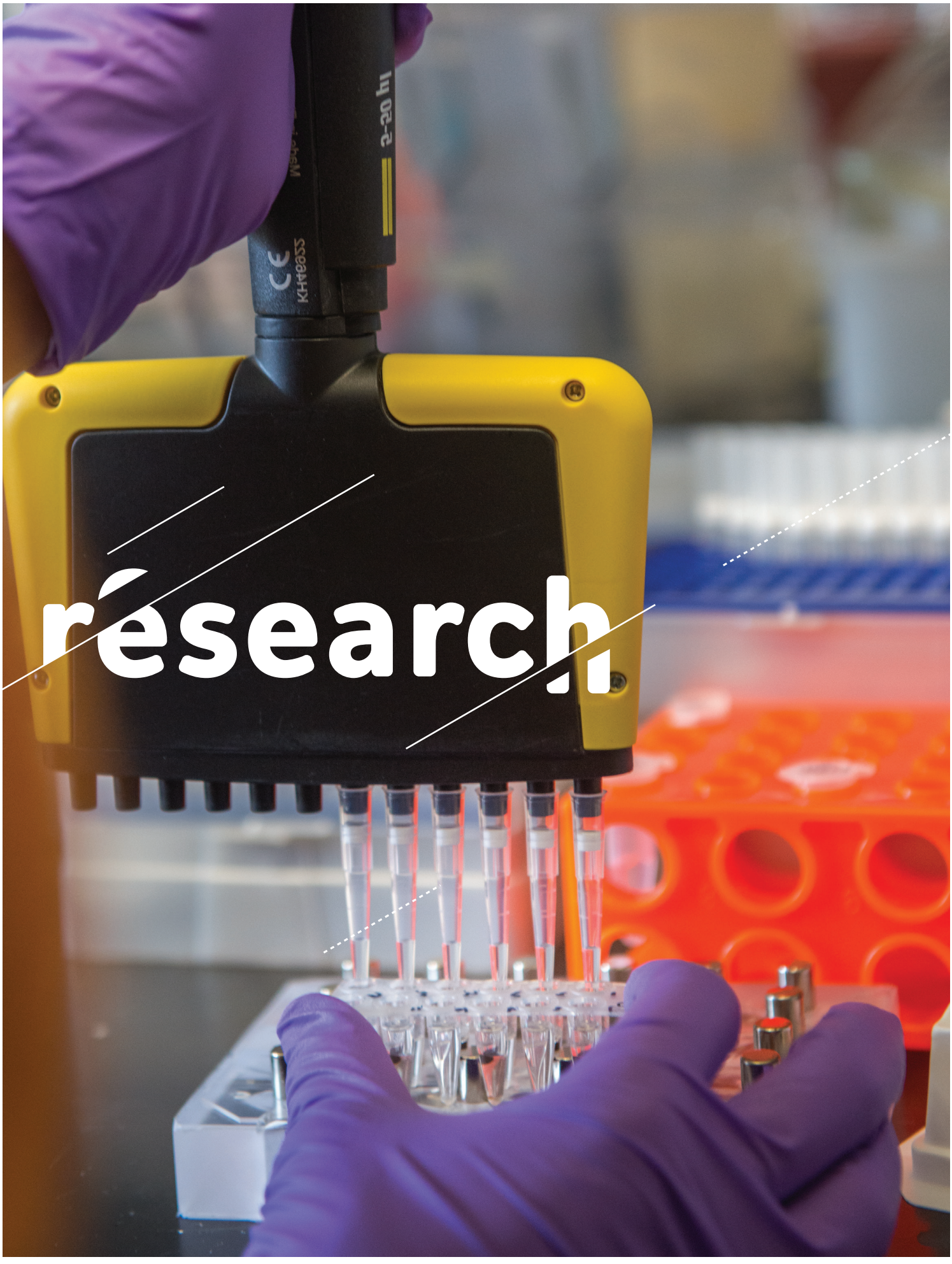

our
mission

Founded in 2010, EB Research Partnership (EBRP) is the largest 501(c)(3) 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. EBRP works to treat and cure EB as quickly and efficiently as possible and fulfills our mission by partnering with non profit and for-profit organizations, foundations, individual donors, and the EB and research communities.

EB Research Partnership utilizes an innovative venture philanthropy model, leveraging concepts from principal investing and applying them toward achieving philanthropic goals. When making a grant to a research project, EBRP retains the added upside of generating a recurring donation stream if the therapy or product is commercially successful; then, EBRP can use this revenue to fund additional research.

partner with us in our mission

to further life-saving research for EB


research

EBRP reviews grant applications biannually and awards funding to competitive and innovative research projects with the potential to lead to commercially feasible products and therapies to treat and cure EB. The applications are evaluated and scored by EBRP's Scientific Advisory Board (SAB), which is made up of experts in various research and clinical fields and which recommends the best projects for funding. In 2017, EBRP awarded grants to 11 new projects totaling $2,746,094 — securing matching funds in partnership with the EB Medical Research Foundation and Cure EB.

2017 Newly Funded Projects

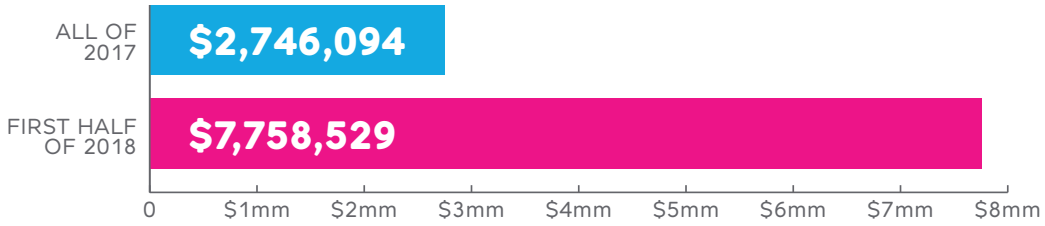
INSTITUTION	PROJECT NAME	PRINCIPAL INVESTIGATOR(S)	AMOUNT AWARDED
University of Southern California	An Evaluation of the Feasibility of Aminoglycosides to Induce Premature Termination Codon Read-through and Restore Functional Laminin 332 in Nonsense Mutations Associated with H-JEB	Mei Chen, PhD David Woodley, MD	$178,500
Stanford University, University of Colorado, and Columbia University	Epidermolysis Bullosa (EB) iPS Cell Consortium	Anthony E. Oro MD/PhD Dennis Roop PhD Angela Christiano PhD	$842,333
Stanford University	Computational Drug Repurposing for Epidermolysis Bullosa Simplex	Andrew A. Radin Joyce Teng, MD, PhD	$127,310
University of Colorado	iPS Cell Biobank for EB Patients	Dennis Roop, PhD	$83,790
Pediatric Dermatology Research Alliance	Stigma, Anxiety, and Depression in Children and Adolescents with Skin Disorders	Amy Paller, MD Sarah Chamlin, MD	$10,000
Thomas Jefferson University	Targeting APOBEC for RDEB SCC Prevention	Andrew South, PhD	$349,089
Tufts University	National Epidermolysis Bullosa Eye Disease Survey	Vicki Chen, MD	$38,325
University of California, San Francisco and Thomas Jefferson University	Deep Sequencing Diagnostic Field Lesions in Recessive Dystrophic Epidermolysis Bullosa Squamous Cell Carcinoma	Raymond Cho, MD Andrew South, PhD	$200,000
Stanford University and Krystal Biotech	Engineered COL7A1- HSV-1, KB103 Applied Intradermally/Topically as a Treatment for Recessive Dystrophic Epidermolysis Bullosa	Peter Marinkovich, MD	$770,000
Tel Aviv Sourasky Medical Center	Improving Wound Healing in Epidermolysis Bullosa Through Modulation of the Skin Microbiome	Eli Sprecher, MD	$85,000
University of Freiburg	Perturbed Proteolytic Activation by Kallikrein Family-Proteases in Recessive Dystrophic Epidermolysis Bullosa: New Prospects for Therapies	Dimitra Kiritsi, MD Alexander Nystrom, PhD Georgia Sotiropoulou, PhD	$61,747
		TOTAL AWARDED	**$2,746,094**

2017 Ongoing Project Funding

INSTITUTION	PROJECT NAME	PRINCIPAL INVESTIGATOR(S)	AMOUNT FUNDED
University of Minnesota	Gene Editing of Type VII Collagen Mutations	Jakub Tolar, MD, PhD	$279,667
Seattle Children's Hospital	Development of a Foamy Viral Vector to Express Col7A1	Andrew Scharenberg, MD	$67,487
Stanford University and Immusoft Corporation	Cell Reprogramming of Autologous Cells as Treatment Strategies for RDEB	Peter Marinkovich, MD Jean Tang, MD, PhD Eric Herbig, PhD Scott McIvor, PhD	$253,781
Stanford University	Phase 1 Clinical Trial of Injected C7 Protein vs Placebo for Wound Healing in five RDEB Subjects	Jean Tang, MD	$181,376
Stanford University and Corium International	C7 Protein Therapy for Microneedles	Jean Tang, MD	$167,525
University of Colorado	R01 Match for Induced Pluripotent Stem Cell Research	Dennis Roop, PhD	$142,970
Stanford University	EB Therapeutic Reprogramming of iPS Cells	Anthony E. Oro MD/PhD	$40,000
Thomas Jefferson University	Targeting Fibrosis for RDEB Therapy	Andrew South, PhD	$147,848
Stanford University	Phase 2 Trial of a Neurokinin-1 Receptor Antagonist for the Treatment of Pruritus in Patients with Epidermolysis Bullosa	Jean Tang, MD	$52,318
Stanford University	Suction Blister Device and Tissue Analytics	Jean Tang, MD	$42,275
Stanford University	Laminin-332 Protein Therapy For Junctional Epidermolysis Bullosa	Jean Tang, MD	$36,686
University of Minnesota	Gene Editing Robot	Jakub Tolar, MD, PhD	$247,191
		TOTAL FUNDED	**1,659,124**

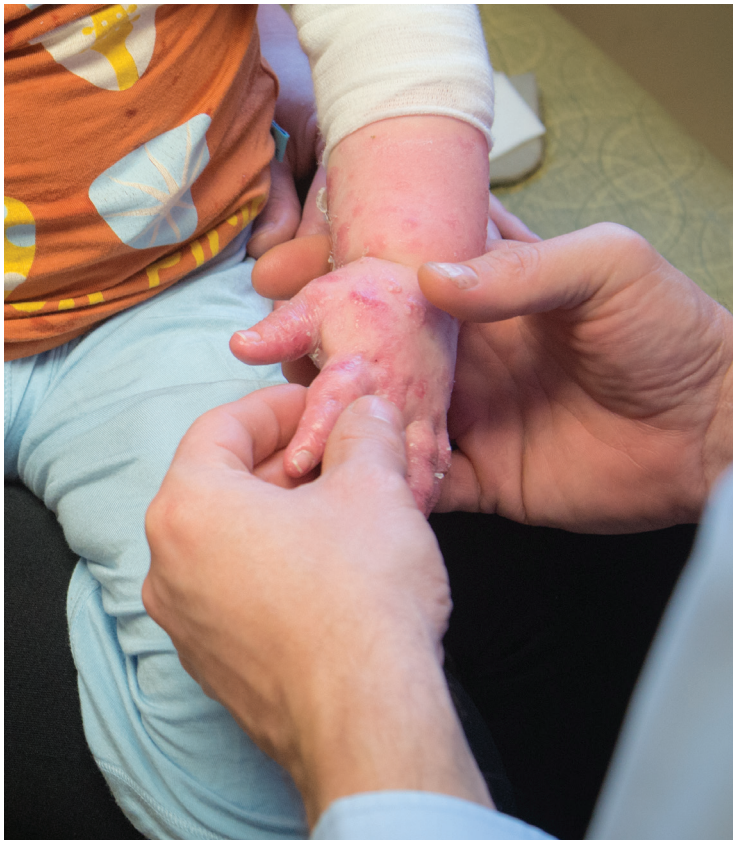
In the first grant cycle of 2018, EBRP awarded $5,758,529.86 for innovative research, more than double the amount awarded in the previous year. EBRP anticipates that this number will increase further as applications for the second grants cycle are reviewed. EBRP continues to develop the research landscape, producing an upward trend in the number of applications received and projects funded.

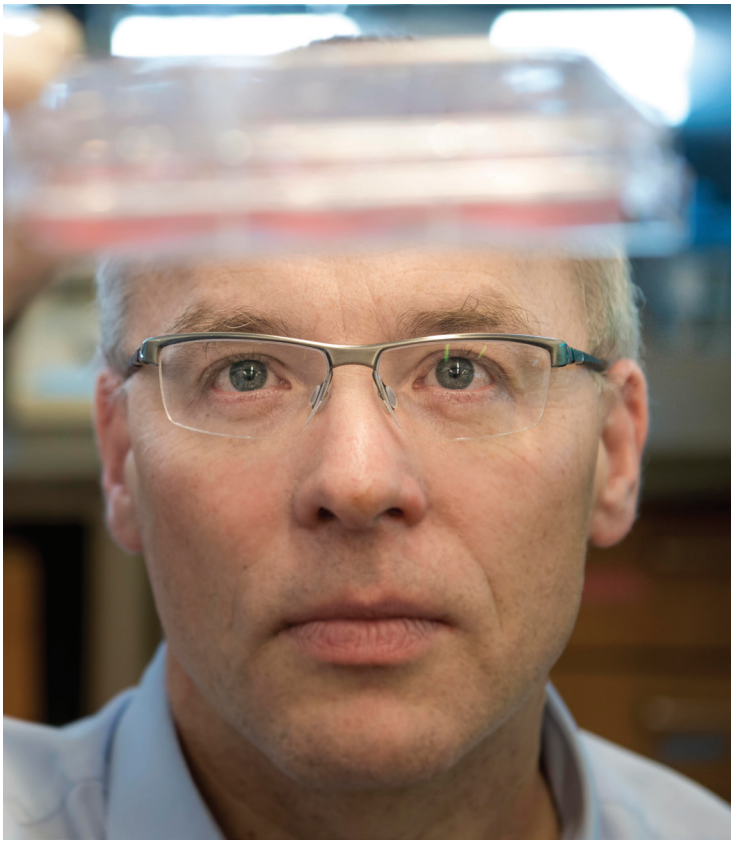
Grant Awards are up 183% Over Last Year!



2018 First Half Newly Funded Projects

INSTITUTION	PROJECT NAME	PRINCIPAL INVESTIGATOR(S)	AMOUNT AWARDED
Stanford University	Bridge Funding for R01 Application to Optimize the Manufacturing of Genetically Corrected, Induced Pluripotent Cell-Derived Epithelial Sheets for Definitive Treatment of Dystrophic Epidermolysis Bullosa	Anthony Oro, MD, PhD	$258,447
University of Southern California	A Pilot Study of the Restoration of Functional Laminin 332 in JEB Patients with Nonsense Mutations After Topical and Intravenous Gentamicin Treatment	Mei Chen, PhD David Woodley, MD	$254,100
The Hospital for Sick Children	A Double-Blind, Randomized, Crossover, Multi-Center, Feasibility Trial of Pregabalin for the Treatment of RDEB-Associated Neuropathic Pain and Itch.	Elena Pope, MD, MSc Margarita Calvo, MD, MSc, PhD Irene Lara-Corrales, MD, MSc	$179,977
Columbia University Medical Center	Conform-a-Care is a Tubular, Elasticated, Multilayered Wound Dressing that Contours to the Body, Providing Optimal Wound Care.	Laura Levin, MD	$33,500
Universite Laval Research Center	Feasibility Study on the Production of Skin Substitutes from Revertant Gene Corrected Cells from DEB Patients.	Lucie Germain, PhD Elena Pope, MD, MSc Manuel Caruso, PhD	$197,505
University of Minnesota	Next Generation Genome Editing for RDEB.	Jakub Tolar, MD, PhD	$1,000,000
ProQR Therapeutics	Clinical Development of QR-313 for Treatment of DEB	David Rodman, MD	$5,000,000
		TOTAL AWARDED	**$7,758,529**





Highlighted Projects: 2018 First Half

ProQR Therapeutics: Clinical Development of QR-313 for Treatment of DEB

This grant marks the largest EBRP-funded clinical trial with human participants. ProQR is conducting a clinical trial of QR-313, a drug candidate that causes skipping of exon 73 in collagen VII protein synthesis. The exon-skipping technique excludes the problematic exon from the final protein product, allowing the protein to function properly. A subset of RDEB patients' disease-causing mutation lies within exon 73 in collagen VII.

University of Southern California: An Evaluation of the Feasibility of Aminoglycosides to Induce Premature Termination Codon Read-through and Restore Functional Laminin 332 in Nonsense Mutations Associated with H-JEB

EBRP granted funds to Dr. Mei Chen and Dr. David Woodley to study whether aminoglycosides, a class of antibiotics, can induce the read-through of nonsense mutations in Laminin 332 that cause Junctional EB. Nonsense mutations direct cells to prematurely stop protein synthesis, leading to the production of a shortened and nonfunctional protein. Aminoglycosides can direct the cell to skip over that stop signal, restoring the production of the full-length protein. EBRP also funded a similar study conducted by these doctors on the aminoglycoside Gentamicin and its effect on nonsense mutations in collagen VII, the protein affected in Recessive Dystrophic EB.

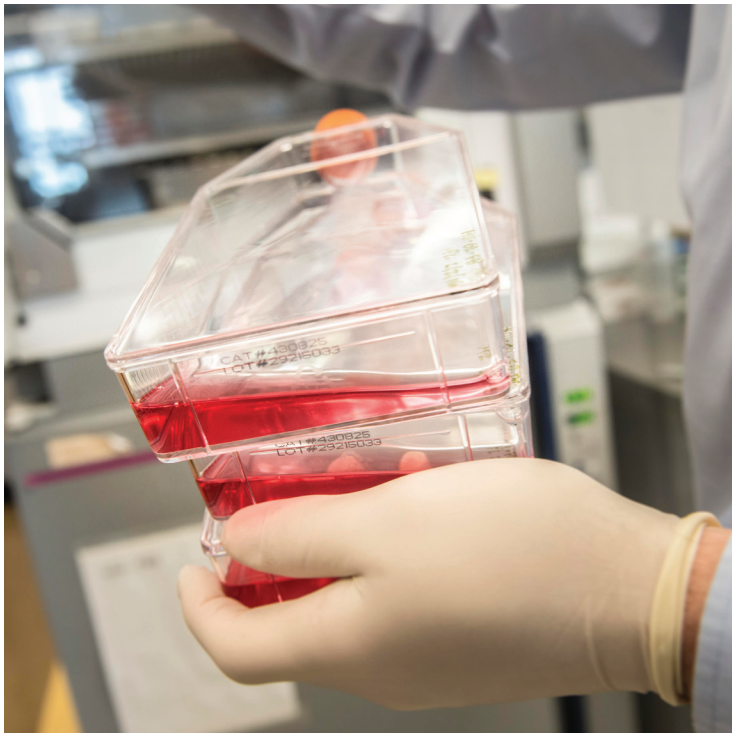
EB Clinical Research Consortium

Along with leading North American pediatric dermatologists, EBRP founded the Epidermolysis Bullosa Clinical Research Consortium (EBCRC), a collaborative research group that conducts high-quality clinical and translational research aimed at improving and advancing care for EB patients. The EBCRC, led by Dr. Anna Bruckner at Children's Hospital Colorado, has grown to include 21 prominent medical centers. Each EBCRC site contributes patient data to the EB Clinical Characterization and Outcomes Database (CCOD), which has more than 700 patients enrolled. EBRP aims to accumulate the largest data set in EB to uncover a deeper understanding of the biology of the disease and to reveal greater insights into how it can be treated. EBRP has provided over $600,000 in funds to EBCRC sites to date.

2017 Funding: $91,983

EB iPS Cell Consortium

In 2016, EBRP founded the EB iPS Cell Consortium, consisting of research teams led by Dr. Angela Christiano from Columbia University Medical Center, Dr. Anthony Oro from Stanford University School of Medicine, and Dr. Dennis Roop from the University of Colorado Anschutz Medical Campus, to foster collaboration among leading scientists in hopes of accelerating the path to treatments and cures. The consortium studies cutting-edge induced pluripotent stem (iPS) cell technology and its application as a potentially curative therapy for EB. These iPS cells are normal adult cells that are reprogrammed to act as stem cells, allowing the possibility for patients to produce their own unlimited supply of stem cells for use in life-saving therapies. The EB iPS Cell Consortium has already established a protocol for manufacturing autologous CRISPR-corrected, iPS-derived keratinocyte sheets for grafting. This technology is at the forefront of medical research, and the consortium has received additional multi-million dollar grants from highly regarded institutions, such as the California Institute of Regenerative Medicine (CIRM).



Interviewing Tony Oro, MD

Q | What is the potential impact of the iPS Cell Consortium for patients with EB and also other rare diseases?

The iPS Cell Consortium represents a talented group of EB researchers with complementary skills to transform a promising new technology into a therapeutic reality for patients with EB. As we know, iPS cells are derived from an individual's own cells and possess the ability to be genetically corrected and manufactured into tissue stem cells to replace a patient's defective ones. Previously performed experiments have shown that one dose of corrected skin stem cells can close wounds for many years, raising the possibility of "definitive" therapy. Also, iPS cells allow large numbers of corrected skin stem cells to be produced. While the goal of the consortium is to produce a safe and robust manufacturing method for EB patients, the same method might also be used in the future to manufacture other tissue stem cells to help patients with other rare or common diseases.

Q | Why is collaboration so important in medical research?

Medical researchers are like blind men/women touching different parts of the elephant. Most of the time, we only get part of the picture, or, as individuals, have the resources to develop only a portion of a therapy very slowly. A collaborative and complementary research team can "touch" many parts of the elephant simultaneously, grasping a larger part of the whole picture. Team members bring resources and talents that will help researchers overcome stumbling blocks and accelerate therapy development.

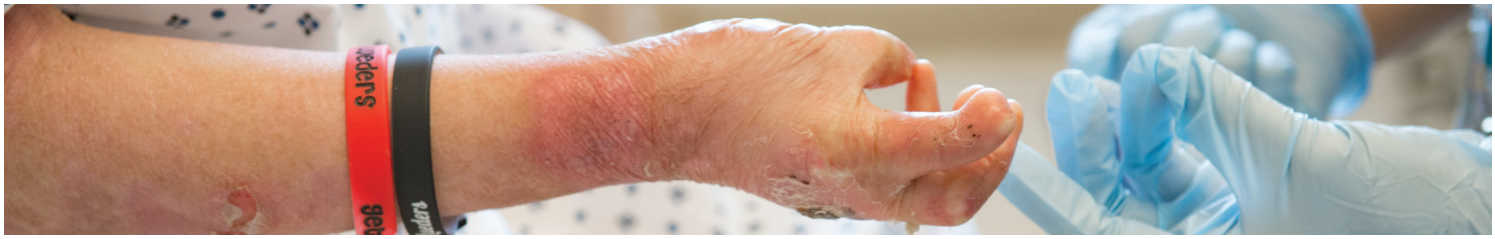
> **"Our work in skin and stem cell biology has opened up therapeutic avenues we never thought possible."**
>
> **— Tony Oro, MD**

Q | What inspired you to focus on research for patients with EB?

I completed my dermatology clinical training at Stanford University, where we have a clinic focused on patients and their families suffering from EB. Watching heroic RDEB patients navigate life despite the suffering inspires a researcher to find ways to help them. Our work in skin and stem cell biology has opened up therapeutic avenues we never thought possible. Our patients and their families have given so much to us; ultimately, we hope our work will give back to the EB community.

clinical landscape

When EBRP was founded in 2010, only two clinical trials were underway for potential EB therapies. Today, more than 20 such trials are ongoing. EBRP's funding of innovative research over the last four years has directly impacted the EB clinical landscape.



Highlighted Clinical Trials

Stanford University and Abeona Therapeutics, Inc.: Gene Transfer for Recessive Dystrophic Epidermolysis Bullosa

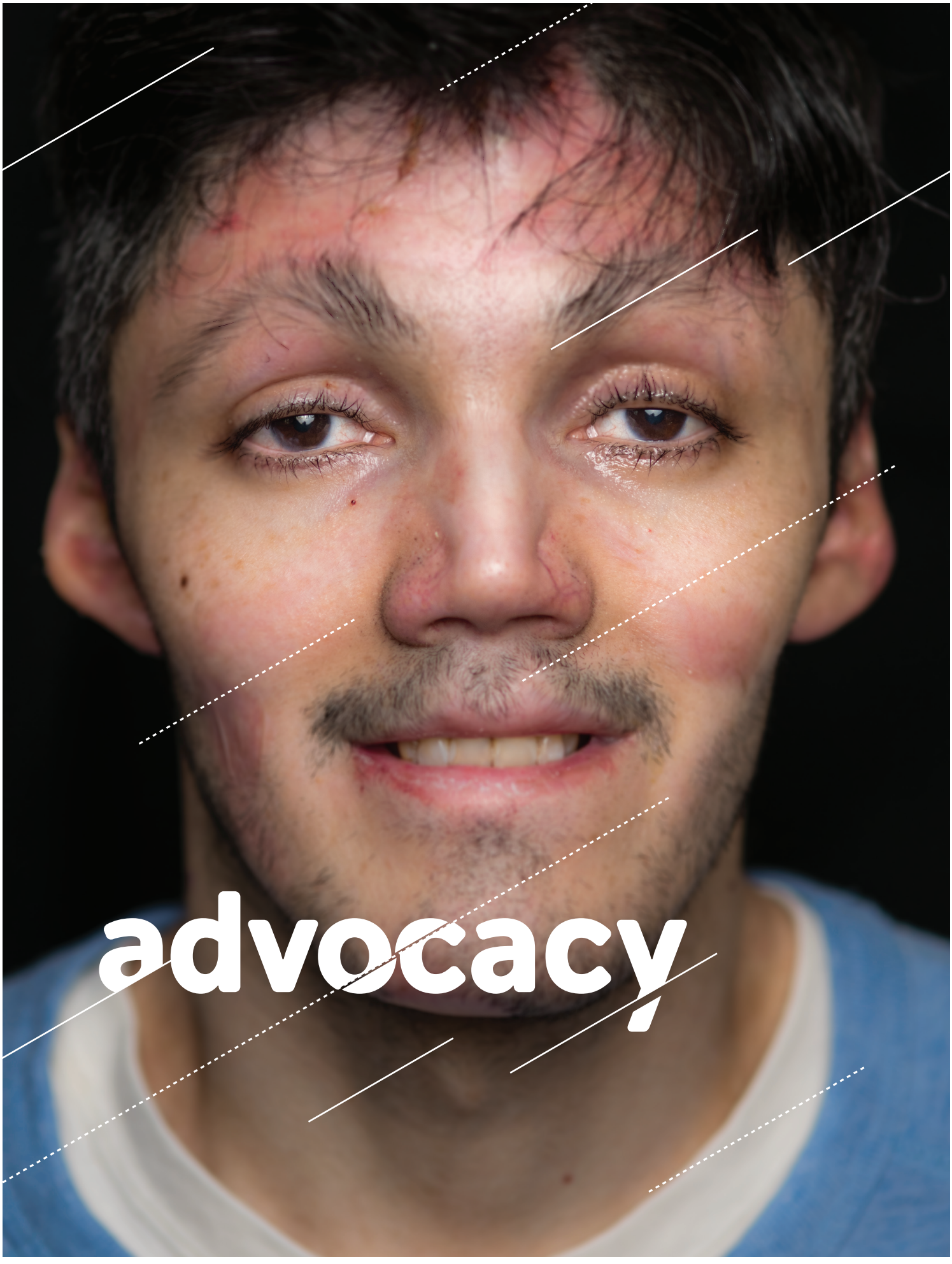
Abeona Therapeutics and Stanford University are working with the FDA to commence a pivotal Phase 3 clinical trial of EB-101, their cell-therapy product for the treatment of RDEB, as of late 2018. EB-101 is a skin graft made from a patient's own cells that have been genetically corrected to produce collagen VII protein. In May, results from the completed Phase 1/2 clinical trial confirmed that EB-101 is safe and well-tolerated, established anchoring fibrils, and significantly healed treated wounds, with >50% closure two years post administration. This trial will be the first to reach Phase 3 for an EB therapy, marking significant progress in the EB research community.

Castle Creek Pharmaceuticals, LLC: Safety and Efficacy of Diacerein 1% Ointment Topical Formulation Compared to Placebo for Subjects with Epidermolysis Bullosa Simplex (EBS)

In the Phase 2/3 DELIVERS study, researchers are testing the safety and efficacy of CCP-020, Castle Creek's topical treatment for EBS. CCP-020 is a diacerein 1% ointment with the potential to block an inflammatory pathway, which may promote healing in EBS patients. EBS is the most prevalent subtype of EB, so progress in this field will impact a significant number of those living with EB.

Fibrocell Science, Inc.: A Study of FCX-007 for Recessive Dystrophic Epidermolysis Bullosa (RDEB)

In May, Fibrocell Science announced positive interim results on their Phase 1/2 clinical trial of FCX-007, a gene therapy candidate for the treatment of RDEB. The therapy involves injections of patients' cells that have been genetically modified to produce collagen VII into wounded areas. FCX-007 was well-tolerated and promoted wound healing in patients, marked by the presence of collagen VII and anchoring fibrils, up to 52 weeks post administration. Fibrocell is currently enrolling patients for Phase 2 of this trial.


advocacy

Food & Drug Administration

In April, EBRP Senior Accountant and EB Advocate, Michelle Hall, sat on the panel of an Externally-Led Patient-Focused Drug Development (EL-PFDD) meeting with the Food and Drug Administration (FDA) to provide her unique perspective. Along with others affected by EB, Michelle helped lead a discussion on living with the disorder and what constitutes meaningful treatment. The information shared by the panel and the data collected at the meeting will be considered in the FDA's risk-benefit analyses for potential EB therapies. In addition, academic researchers and the regulated drug industry may consider this data when designing clinical trials for their products. Collaboration between the FDA and the EB research and patient communities is critical to furthering progress in our mission to find treatments and cures. In response to the data collected at the meeting, the FDA released guidance for the industry on drug development in EB.

Department of Defense

The United States Department of Defense (DoD) granted $9.3 million to EB researchers for the next five years through their Peer Reviewed Medical Research Program (PRMRP). Despite making up less than 0.5% of the program applicants, EB researchers comprised 5% of the grant winners. Additionaly, 67% of EB research applicants received awards, gaining a higher acceptance level than in any other disease group. EBRP-backed scientists Dr. Dennis Roop of the University of Colorado Anschutz and Dr. Andrew South of Thomas Jefferson University were awarded more than $5 million of this funding. Dr. Roop will receive $3.8 million to continue studies on potential stem cell treatments for chronic skin wounds, and Dr. South will receive $1.7 million for his work on squamous cell carcinoma in EB patients. The DoD's funding of these projects proves the merit of EBRP's SAB in determining the most innovative research to fund.


The largest nonprofit dedicated to funding research to heal and cure Epidermolysis Bullosa
#healEB
EB RESEARCH PARTNERSHIP

events

In 2017 $1,902,288 was raised

through EBRP's annual events and by community-led events held throughout the country by driven supporters of EBRP and the EB community.

2017–18 EBRP Events

All In For A Cure
May 11, 2017
New York, NY

Night of Discovery
Sept. 23, 2017
Long Beach, CA

ACTion for Jackson
Nov. 8, 2017
New York, NY

All In For A Cure
May 16, 2018
New York, NY

2017–18 Community-Led Events

Change for Charley
Nov. 11, 2017
Chicago, IL

Pursuit for Patterson
Nov. 11, 2017
Seattle, WA

Evening at Malibu Farm
Dec. 14, 2017
Miami Beach, FL

Bobby Kaps Jump for EB
Jan. 1, 2018
Southport, CT

Plunge for Elodie
March 3, 2018
Hingham, MA

Believe in Brady
April 18, 2018
Houston, TX

Upcoming Events | Save the Date!

Night of Discovery
Oct. 6, 2018
Long Beach, CA

ACTion for Jackson
Nov. 8, 2018
New York, NY

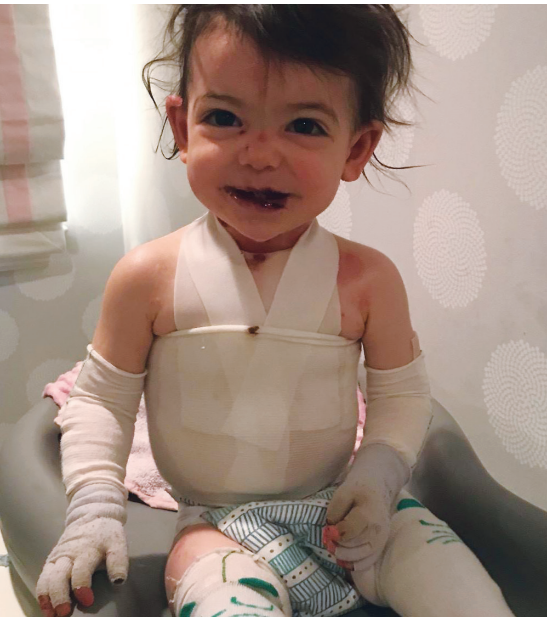
Plunge for Elodie
March 2, 2019
Wellesley, MA

Change for Charley
March 9, 2019
Chicago, IL

Believe in Brady
April 7, 2019
Houston, TX

ACTion for Jackson
Nov. 8, 2017
New York, NY

The eigth annual ACTion for Jackson gala was a record-breaking success! More than 700 supporters gathered at 583 Park Avenue and raised nearly $1.3 million to accelerate EB research. Michael and Nell Valentine graciously shared a video of their son Gabe, a young boy with EB who passed away in June 2017, and Michael gave an inspiring speech to pass on his son's final wish — to find a cure for EB. Within minutes, the room rallied to fully fund the evening's special project, a gene-editing robot in honor of Gabe for Dr. Jakub Tolar's lab at the University of Minnesota. We greatly appreciate all the generous sponsors, donors, and friends who came together to make the event such a success.



Plunge for Elodie

March 3, 2018

Hingham, MA

The first annual Plunge for Elodie was organized by a group of women in support of their lifelong friend, EBRP Board Member Emily Kubik, whose two-year-old daughter Elodie lives with RDEB. Despite harsh weather conditions, supporters plunged into frigid winter waters and raised $150,000 for EBRP. Actress Jessica Biel joined the action by taking the plunge remotely and posting a video on her social media channels, sparking viewers to virtually join the fun and plunge into pools, tubs, and even the snow!

financials

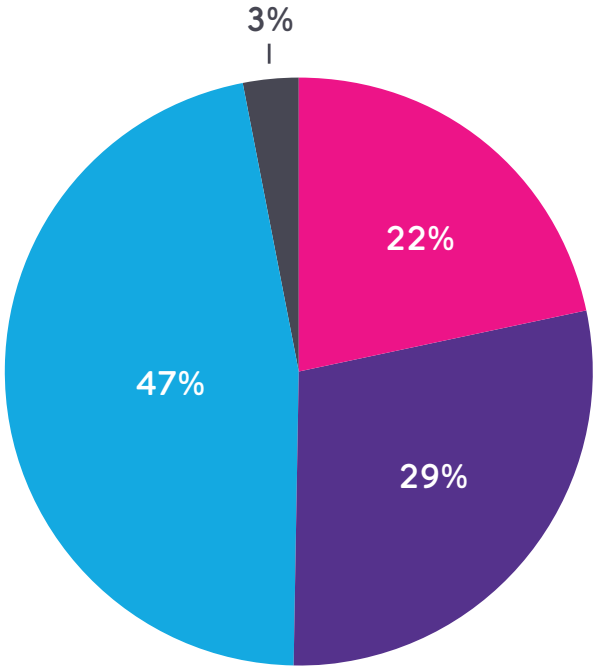
2017 Financial Summary

EBRP is committed to the highest financial responsibility, directing 90% of revenue to research and related programming. For complete audited financials, please visit our website at **www.ebresearch.org**.

2017 EBRP Support & Revenue

$6,604,913

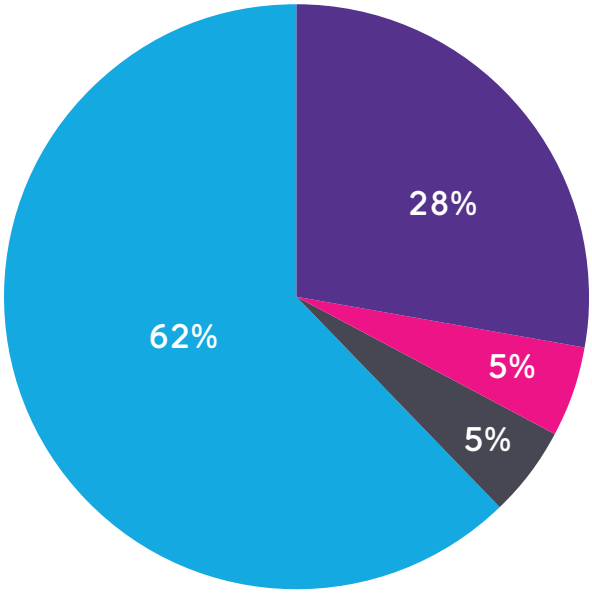
- Contributions **$1,428,927**
- Fundraising Events **$1,902,288**
- Venture Investments **$3,075,590**
- In-kind Contibutions **$198,108**



2017 EBRP Spending Allocation

$6,604,913

- Program & Research **$1,866,523**
- Management **$330,004**
- Fundraising **$313,625**
- Designated Research Funding **$4,094,761**



Ending Net Assets: $12,018,774


statistics

500,000 People
are estimated to have EB worldwide.

1 in 30,000 People
are estimated to be affected by EB.

50 Research Projects
funded since inception.

25 Concurrent Research Projects
funded today.

10x the Clinical Trials
In 2010, there were two. Today there are 20.

$25 Million
raised since EBRP's inception.

7,000 Rare Diseases
impacting 10% of the global population that our model can impact.


leadership

Board of Directors

Alexander Silver
Chairman & Co-Founder

Jill Vedder
Vice Chair & Co-Founder

Ed Vedder
Co-Founder

Jamie Silver
Co-Founder

Heather Fullmer
Co-Founder

Eileen Attar

Tracy Baldwin

Jeffrey Berger

Mark Bomback

Chad Ceretto

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Daniel Deshe

Faye Dilgen

Stephen Evans

Edward Grossmann

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Abbie Levine

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Joshua Paulson

Matthew Prince

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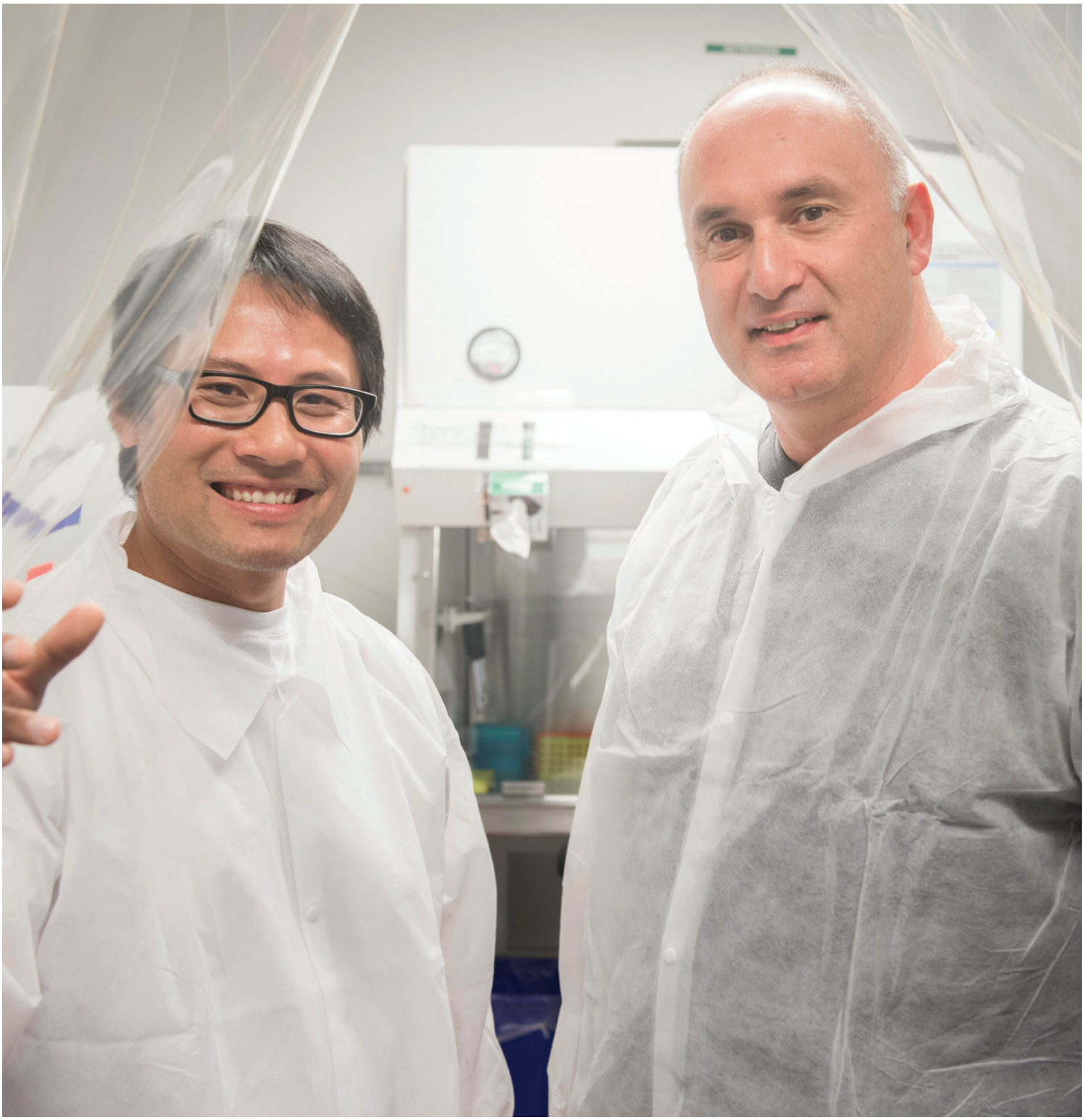

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