



**EB** RESEARCH  
PARTNERSHIP

# impact report 2018







Elodie took her first ambulance ride in July of 2016 — just hours after she was born. Her family would quickly learn the reason that skin was shearing off her body. Elodie was diagnosed with Epidermolysis Bullosa (EB). She is one of only 500,000 people in the world living with EB. With no disease-modifying treatments yet available, Elodie’s day consists of painful full-body bandage changes and difficulties with daily activities like eating and running around the playground. While EB is a diabolical opponent, Elodie’s parents remain hopeful, largely because of EB Research Partnership’s commitment to heal and cure this life-threatening disease.

*“We believe that, in the end, Elodie will be one of the lucky ones. For a disease with so many complications, the genetics of EB are relatively uncomplicated, which increases the likelihood that a cure will come. The foundation for a cure and a treatment for Elodie has already been built by EBRP. The progress made in the last ten years is nothing short of remarkable. Our job is to make sure that this progress continues and the goal of a treatment or cure by the time Elodie is in grade school remains attainable.”*

— Emily and David, Elodie’s Parents

With you on our side, EBRP has embarked on a relentless mission to accelerate treatments and cures for EB. We have constructed a culture of urgency and innovation, including launching the largest EB data project imaginable that aggregates, analyzes, and decodes complex data underlying the disease. EBRP brings together the often siloed academic, medical, and patient communities by breaking down barriers to cooperation and ensuring compounding benefits to each new discovery. EBRP’s Scientific Advisory Board carefully vets the most promising research projects so we can invest in the most promising research and stack the odds of finding a cure in our favor.

With your generous support, we are delivering hope for a future without EB. Since 2010, we have funded more than 50 research projects, united a global consortium of 20 academic medical centers of excellence, and established the leading venture philanthropy model to create a sustaining investment portfolio. Our work in under a decade has already led to more than 10 times the number of active clinical trials. As research advances into later stages, the need for larger amounts of resources sooner is a challenge we embrace for all children living with EB. We have embarked on a \$25 million capital campaign to meet this need.

If we can do this, what does the future look like for Elodie and the hundreds of thousands just like her? It means running and riding a bike just like other kids. It means that a schedule once full of doctor visits can be filled with new adventures. 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 on accelerating the path to healing 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 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 group of devastating and life-threatening genetic skin disorders that affect children from birth.

## OUR MODEL

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. This means your generous donation has the potential to grow to multiples of its original value.



progress  
to a cure



**19 Projects  
in 6  
Countries**  
funded in 2018.

**50  
Projects  
Funded**  
to date.

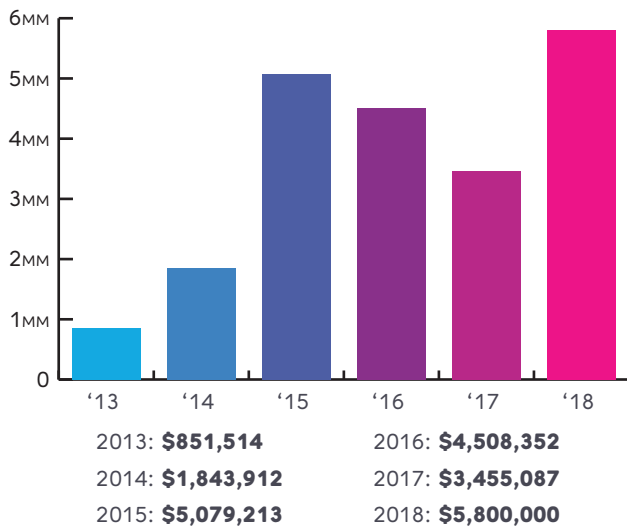
**+42%  
Fundraising  
Revenue**  
from 2017–18.

**+299%  
in Grant  
Awards**  
from 2017–18.

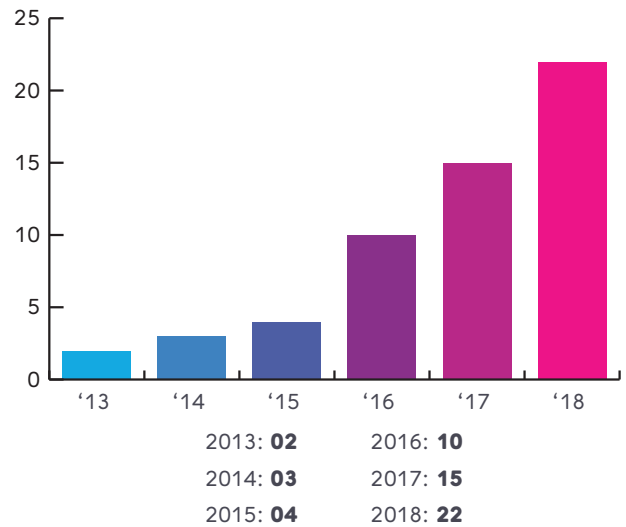
**Data from  
over 800  
Patients at  
20 Medical  
Centers**  
in our CCOD.

**14x the  
Number  
of Active  
EB Clinical  
Trials**  
since our founding.

**EBRP Revenue 2013–18**



**Clinical Trials Data 2013–18**



**\$25 Million raised to date.**



research





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 2018, the SAB recommended nearly \$11M in funding for 16 new research projects and funding for our EB Clinical Research Consortium. EBRP funded all efforts, securing matching funds from our partners EB Research Foundation of Australia, EB Medical Research Foundation, and Cure EB.

## 2018 Newly Approved Research 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 & Pontificia Universidad Catolica de Chile	A double-blind, randomized, cross-over, 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	\$179,978
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 Centre	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	Clinical development of QR-313 for treatment of DEB	David Rodman, MD	\$3,835,000
Spin Therapeutics, LLC, INSERM	Discovery of Systemically-Administered siRNAs Targeting Mutant KRT5 and KRT14 as Therapeutics for Epidermolysis Bullosa Simplex	Aaron Sato, PhD Alain Hovnanian, MD, PhD	\$749,070
Stanford University, Children's Hospital Colorado (Fibrocell)	A Phase 1/2 Study of FCX-007 (Genetically-Modified Autologous Human Dermal Fibroblasts) for Recessive Dystrophic Epidermolysis Bullosa (RDEB)	M. Peter Marinkovich, MD Anna Bruckner, MD	\$900,000
FIBRX Tissue Repair, Inc.	Development of Human Recombinant Decorin Core Protein as a Topical Anti-Scarring Therapy for Dystrophic Epidermolysis Bullosa	Professor Jean Tang, MD, PhD	\$1,750,000

# Research

## 2018 Newly Approved Research Projects (Continued)

INSTITUTION	PROJECT NAME	PRINCIPAL INVESTIGATOR(S)	AMOUNT AWARDED
University of Colorado School of Medicine Anschutz Medical Campus	Testing a “Spray on Skin” Approach as an Alternative Method for Delivering Keratinocytes and Fibroblasts derived from Gene-edited induced Pluripotent Stem Cells (iPSCs) to Recessive Dystrophic Epidermolysis Bullosa Patients	Dennis R. Roop, PhD Ganna Bilousova, PhD Igor Kogut, PhD Anna Bruckner, MD	\$290,067
University of Colorado School of Medicine Anschutz Medical Campus	Developing a Therapeutic Approach for Delivering Stem Cells Systemically to Treat Fragile Internal Epithelia in Recessive Dystrophic Epidermolysis Bullosa Patients	Dennis R. Roop, PhD Ganna Bilousova, PhD Igor Kogut, PhD Anna Bruckner, MD	\$295,563
Stanford University School of Medicine	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	\$372,043
Stanford University School of Medicine	Proposal for additional funding for Collagen 7 protein therapy intradermal injection	M. Peter Marinkovich, MD	\$330,378
Tufts	Development of a Collagen VII Eye Drop for the Treatment of Ocular Disease in RDEB	Viki Chen MD	\$277,700
University of Colorado School of Medicine Anschutz Medical Campus	Prodigy System Spray-On-Skin Machine	Dennis R. Roop, PhD Ganna Bilousova, PhD Igor Kogut, PhD Anna Bruckner, MD	\$230,079
<b>TOTAL AWARDED</b>			<b>\$10,953,460</b>

In addition to the newly funded projects, EBRP granted a total of \$83,508 for two ongoing research projects that were initially approved by the SAB in previous grant cycles.

## 2018 Ongoing Research Funding

INSTITUTION	PROJECT NAME	PRINCIPAL INVESTIGATOR(S)	AMOUNT AWARDED
Columbia University	Characterization of Skin Care Practices and Antimicrobial Resistance Patterns in EB: Paving the Way for Individualized Treatment and Identification of High Risk Patients	Kimberly D Morel, MD	\$3,981
Thomas Jefferson University	Targeting Fibrosis for RDEB Therapy	Andrew South, PhD	\$79,527
<b>TOTAL AWARDED</b>			<b>\$83,508</b>



# Research Highlights



## Title: Development of a Collagen VII Eye Drop for the Treatment of Ocular Disease in RDEB

**Institution:**  
Tufts University

**Award Amount:**  
\$277,700

**Principal Investigator:**  
Vicki Chen, MD

**Patient Population:**  
Recessive Dystrophic EB

**About:** Corneal blistering, scarring, and vision loss occur in approximately 40% of patients with Dystrophic EB. Dr. Chen aims to create an eye drop that can deliver Collagen 7, the affected protein in RDEB, to the basement membrane zone of the eye to treat EB-associated eye issues.

*“Corneal abrasions are among the most common and most painful of EB related problems. Our goal is to alleviate pain and prevent vision loss by creating therapies that target corneal blisters and scarring.”*

— Vicki Chen, MD



## Title: Testing a “Spray on Skin” Approach as an Alternative Method for Delivering Keratinocytes and Fibroblasts derived from Gene-edited induced Pluripotent Stem Cells (iPSCs) to Recessive Dystrophic Epidermolysis Bullosa Patients

**Institution:**  
University of Colorado, Anschutz Medical Campus

**Award Amount:**  
\$290,067

**Principal Investigators:**  
Dennis R. Roop, PhD  
Ganna Bilousova, PhD  
Igor Kogut, PhD  
Anna Bruckner, MD

**Patient Population:**  
Recessive Dystrophic EB

**About:** As a member of the EB iPS Cell Consortium, this team studies induced pluripotent stem cell technology as a potentially curative therapy for EB. This specific project investigates if spray-on delivery of skin cells is a feasible method to treat patients with the Consortium’s iPSC therapy rather than with skin sheets, which are time-intensive and costly to generate and more difficult to apply.

*“I believe that using the “Spray on Skin” device to deliver keratinocytes and fibroblasts derived from gene corrected iPSCs will revolutionize how we treat RDEB patients.”*

— Dennis Roop, PhD

## Research Highlights Continued



### **Title:** Discovery of Systemically-Administered siRNAs Targeting Mutant KRT5 and KRT14 as Therapeutics for Epidermolysis Bullosa Simplex

**Institution:**  
Spin Therapeutics

**Award Amount:**  
\$531,250

**Principal Investigators:**  
Aaron Sato, PhD  
Alain Hovnanian, MD, PhD

**Patient Population:**  
EB Simplex

**About:**

EB Simplex is caused by mutations in the genes for keratin 5 and keratin 14. This research aims to generate siRNAs, which can prevent gene expression, that target these mutant keratins. The scientists will also develop a novel method for delivery of the fragile siRNAs to skin cells to ensure they are able to enter cells without degrading.

*“While siRNAs are an attractive therapeutic approach for EB simplex, It has been challenging to deliver them topically. We are grateful for the support of the EBRP to conduct research to determine if we may deliver siRNAs systemically for the treatment of EBS”*

— Aaron Sato, PhD

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### **Title:** Randomized Controlled Trial of a Neurokinin-1 Receptor Antagonist for the Treatment of Pruritus in Patients with Epidermolysis Bullosa

**Institution:**  
Stanford University

**Award Amount:**  
\$372,043

**Principal Investigators:**  
Albert Chiou, MD  
Jean Y. Tang, MD, PhD

**Patient Population:**  
All subtypes

**About:**

Pruritus, or itch, is the most common complaint among EB patients and leads to scratching which causes new wounds. There is no effective treatment. Based on the promising results from a prior EBRP-funded pilot study, the investigators will initiate a trial for EB patients 13 and older to assess the efficacy of Serlopitant, the drug candidate, in reducing EB itch.

*“Itching is such a challenging issue for many patients with EB, and our hope is that this clinical trial will potentially open up a new avenue for helping to improve EB-related itching, along with laying the foundation for much-needed future research into itch-focused EB treatments.”*

— Albert Chiou, MD

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# Research

## 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 Anna Bruckner, MD, 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.



**2018 Funding: \$132,272**



## Interviewing Anna Bruckner, MD



### Q | Why is collaboration in the EB space so important?

The goal of all individuals fighting EB should be to improve quality of life for patients and their families. It is important for researchers and clinicians to collaborate and compare what we are doing so that we find the most effective and feasible ways to make our patients lives better. Working together aligns everyone on the common goal, and it will accelerate the pace of discovery, enabling better treatments and an eventual cure for EB.

### Q | How have the EBCRC and CCOD contributed to greater understanding of EB and its effects?

The EBCRC and EBCCOD have contributed to a greater understanding of the natural history of EB, which may guide clinical care. For example, we have shown that the first intervention that patients with recessive dystrophic EB are likely to need is a gastrostomy tube, often before the child is one year old. A child's first esophageal dilation often occurred at age 3–4 years. This information can help clinicians anticipate the need for testing and plan for procedures based on the likelihood of a complication of EB.

Feinstein JA, Jambal P, Peoples K, et al. Assessment of the Timing of Milestone Clinical Events in Patients With Epidermolysis Bullosa From North America. *JAMA Dermatol*. Published online December 26, 2018;155(2):196–203. doi:10.1001/jamadermatol.2018.4673

“ Working together aligns everyone on the common goal, and it will accelerate the pace of discovery, enabling better treatments and an eventual cure for EB. ”

— Anna Bruckner, MD

### Q | Why are you passionate about working with the EB community?

The patients and families that live with EB are courageous, admirable, and inspirational. I have learned so much from these individuals who find joy in life, even though they suffer with bandage changes, pain, itch and an array of other problems and procedures. Helping to shoulder and relieve the burden of EB is an honor and privilege.



# clinical landscape





## Highlighted Clinical Trials



### **Phoenix Tissue Repair: Phase 1/2 Trial of PTR-01 in Adult Patients With RDEB**

In early 2019, Phoenix Tissue Repair dosed the first patient in their Phase 1/2 clinical trial investigating PTR-01, their protein therapy candidate for treatment of Recessive Dystrophic EB. PTR-01 is a recombinant Collagen VII protein that will be delivered intravenously to patients, replacing the defective Collagen VII in their skin. Phoenix Tissue Repair has been granted Fast Track designation by the FDA and is currently enrolling new patients.



### **Krystal Biotech: Phase 2 Clinical Trial of KB103 for Treatment of DEB**

Krystal Biotech announced positive results from the GEM-2 Phase 2 trial of KB103 for treatment of Dystrophic EB. The gene therapy was well tolerated and five out of six treated wounds closed 100% during the trial. Krystal Biotech received Regenerative Medicine Advanced Therapy (RMAT) designation for KB103, allowing expedited development and approval processes with the FDA. Krystal aims to commence a pivotal Phase 3 clinical trial in the second half of 2019.



### **Fibrocell Science: Phase 3 Clinical Trial of FCX-007 for Treatment of RDEB**

In Summer 2019, Fibrocell Science initiated the pivotal Phase 3 clinical trial of FCX-007, their cell therapy for treatment of Recessive Dystrophic EB. FCX-007 is a fibroblast taken from a patient, genetically corrected to properly express Collagen VII – the defective or missing protein in RDEB skin – and intradermally injected back into the patient. Fibrocell received RMAT designation from the FDA for this therapy and projects that dosing for Phase 3 will be completed in Q3 of 2020.

A photograph of a man in a dark suit clapping on a stage. In the foreground, a young girl in a pink outfit is seen from the side, looking towards the man. The background features a large screen and stage lighting. The word "events" is overlaid in white text.

events



# In 2018 \$2,227,101 was raised

through events held throughout the country. We are grateful to the community leaders, volunteers, and supporters who made these events so successful.

## 2018 EBRP Events

### All In For A Cure

May 16  
New York City, NY

### Night of Discovery

September 26  
Long Beach, CA

### ACTion for Jackson

November 8  
New York City, NY

## 2018 Community-Led Events

### Bobby Kaps Jump for EB

January 1  
Southport, CT

### Plunge for Elodie

March 3  
Hingham, MA

### Believe in Brady

April 8  
Houston, TX

### EBelieve

August 18  
El Monte, CA

### Pursuit for Patterson

October 13  
Covington, WA

## Save the Date!

### ACTion for Jackson

November 7, 2019  
New York City, NY

We hope you will join us for a special celebration of our 10<sup>th</sup> annual ACTion for Jackson on November 7, 2019 at the Mandarin Oriental in NYC! Visit [ActionForJackson.org](http://ActionForJackson.org) to purchase tickets and learn more.

# Events

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## Believe in Brady April 8, 2018 Houston, TX

**The 2<sup>nd</sup> annual Believe in Brady raised \$112,000, increasing the event total from 2017 by nearly 60%! Friends of “Brady the Brave” and his family organized this fun-filled family day at Levy Park Conservancy. Guests enjoyed live music, great food, an awesome auction, and children’s entertainment. EBRP is grateful for communities like Brady’s that share our mission to heal EB.**



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

**Our 9<sup>th</sup> annual gala raised over \$1.48 million for life-saving EB research!** The unforgettable night featured an exclusive live and silent auction and emotional event program. EBRP Co-Founder Eddie Vedder spoke about his family's passion to find a cure and the audience was welcomed to watch a video on the life of Rowan, an adorable 3 year old who lives with RDEB. Thank you to our sponsors and supporters for making this event a success!



**financials**

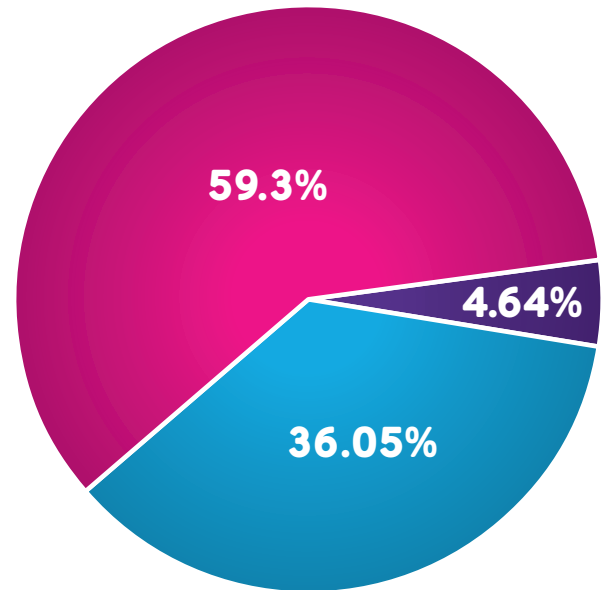


# 2018 Financial Summary

EBRP is committed to the highest financial responsibility and has received the top rating from GuideStar with the Platinum Seal of Transparency. For complete audited financials, please visit our website at [www.EBresearch.org](http://www.EBresearch.org).

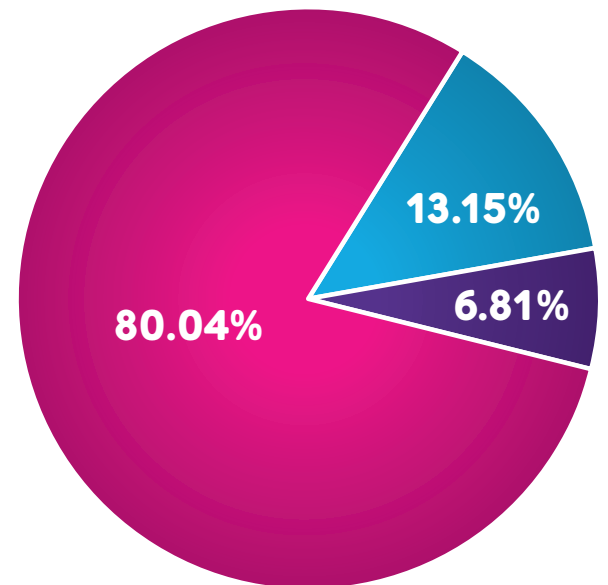
## 2018 EBRP Support & Revenue \$5,393,215

- Contributions  
\$3,198,365
- Fundraising Events  
\$1,944,478
- Other  
\$250,372



## 2018 EBRP Spending Allocation \$5,789,502

- Program & Research\*  
\$4,633,671
- Management  
\$761,392
- Fundraising  
\$394,439



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

## Ending Net Assets: \$11,185,681



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**

**Stephen Evans**

**Edward Grossmann**

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**Jeffrey Berger**

**Mark Bomback**

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**Joshua Paulson**

**Matthew Prince**

**Whitney Pollack**

**Monique Sock**

**Jared Stern**

**Rob Veres**

**Donna Whiffin**

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Controller

**Stephanie Ishoo**  
Director of Communications

**Alexandra Blumberg**  
Development Associate

**Michelle Hall**  
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