



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>