

Institution	Project Name	Project Description	Patient Population	Technique	Principal Investigator (s)	Amount Funded (EBRP)
University of Colorado	EBRP Matching to the NIH U01 AR075932- 63019654	iPS Cell Consortium Patients with EB may develop immune reactions to Collagen VII. This lab will develop CAAR-T cell therapy for collagen VII antibodies within a mouse model	RDEB	Stem Cells	Dennis Roop PhD	\$264,702.45
Stanford University	Overcoming immunity to corrective EB therapies	This project is a renewal of Dr. Oro's work on iPS therapy for esophageal wounds. The lab was successful creating the manufacturing method for the therapy, called indSOCET, in Year 1. This funding would support further optimization, demonstration of disease-modifying properties in organoids and mouse models, and prototyping of methods to apply the therapy in humans - generating pre-clinical data necessary to move into clinical trials.	Dystrophic EB (DEB)	CAAR-T Cell Therapy	Peter Marinkovich	\$244,738.00
Stanford University	GMP Manufacturing of Autologous Esophageal Epithelial Cells for the Prevention of Esophageal Strictures- Year 2	Wings Therapeutics is conducting its Phase Ib/II clinical trial of QR-313 for the treatment of both Dominant Dystrophic Epidermolysis Bullosa (DDEB) and Recessive Dystrophic Epidermolysis Bullosa (RDEB). QR-313 is an investigational RNA therapy candidate for the treatment of DEB due to exon 73 mutations	Dystrophic EB (DEB) Junctional EB (JEB) Kindler Syndrome	Stem Cells	Anthony Oro	\$311,723.00
Phoenicis Therapeutics	Wings Therapeutics Exon Skipping in DEB		Dystrophic EB (DEB)	Gene Therapy	Hald Landy, MD Ravi Hiremagalore MD, Gurudatta Baraka PhD, Arun Inamadar MD, Sacchidanand MD	\$577,777.00
Centre for Human Genetics	Development of a Registry for Epidermolysis Bullosa in India Delivery of C7 encapsulated Collagen-I/ GRAS polymers microneedle array for existing ulcer healing and skin protectant for recessive dystrophic epidermolysis bullosa (RDEB) patients	Development of a Registry for Epidermolysis Bullosa in India This project aims to develop microneedle arrays for topical delivery of Collagen 7. They aim to test in patients by Q3 2022 (project end date)	All	Registry		\$31,520.00
Stanford University		This project will test a gene therapy focused on Junctional EB, which typically causes loss of life in infancy due to failure to thrive and respiratory arrest. The researchers will transplant gene edited stem cells to the oropharynx in a mouse model.	Dystrophic EB (DEB)	Protein Therapy	Jayakumar Rajadas, Peter Marinkovich	\$750,000.00
School of Medicine, Stanford University	Overcoming lethality of junctional EB through stem cell based gene therapy		Junctional EB (JEB)	Gene Therapy	Peter Marinkovich, Dawn Bravo, Jayakar Nayak	\$216,306.00

University College Dublin (UCD)	Development of gene-editing therapy to restore Type VII Collagen for the treatment of RDEB using a topical RNP CRISPR system	Year 2 of this project. In the first year, the researchers tested their topical gene therapy for RDEB on in vitro models (in cells in the lab). This funding will allow them to test the formulation in an in vivo mouse model. They have begun the Orphan Drug Designation process with the EMA (Europe's FDA) this August, which, if approved, helps accelerate the clinical trial process.	Dystrophic EB (DEB)	Gene Therapy	Wenxin Wang, Irene Lara-Saez	\$189,506.00
INSERM (Institut National de la Santé et de la Recherche Médicale)	Multi-omics of Recessive Dystrophic Epidermolysis Bullosa-associated Squamous Cell Carcinoma for targeted antitumor therapy.	This lab aims to characterize RDEB squamous cell carcinoma (SCC) tumors to understand the underlying mechanisms of their development. SCC is life-threatening to patients with RDEB. The researchers will use their learnings to identify potential drugs for anti-tumor treatments.	Dystrophic EB (DEB)	Cancer	Alain Hovnanian, Helene Ragot	\$245,963.00
University of Freiburg, Thomas Jefferson University	Targeted Therapies for Junctional and Kindler Epidermolysis Bullosa-Associated Squamous Cell Carcinoma	This study focuses on Squamous Cell Carcinoma (SCC) in both Junctional EB and Kindler Syndrome. The researchers aim to sequence tumors to compare their learnings to RDEB SCCs, looking for similarities and differences to help identify personalized therapeutic options and molecular targets for future development.	Junctional EB (JEB) Kindler Syndrome	Cancer	Cristina Has, Andrew South	\$152,038.00
Mariposa Therapeutics Ltd	Ex vivo proof of concept confirmation of NB05219 efficacy in human skin	Mariposa has identified a molecule that elevates levels of keratins 17 and 6 that can compensate for loss of function of keratins 14 and 5 caused by EB Simplex. This study aims to demonstrate that the molecule is effective in ex vivo skin (skin taken from a living patient in the lab) and in vitro cells (cells artificially created in a lab) and establish primary safety margins. After this study is complete, the team aims to complete IND-enabling studies, file, and begin a Phase 1 clinical trial in late 2023.	EB Simplex (EB)	Wound Healing	Tracy Nevitt	\$278,392.00
Phoenicis Therapeutics	A First-In-Man Clinical Trial of Human Recombinant Decorin as an Anti-Fibrotic Therapy for Dystrophic Epidermolysis Bullosa	We have funded this study through FIBRX in the past, and it is now ready to move into clinical trial. Decorin is an anti-fibrotic protein. Our past funding has allowed development of a manufacturing protocol for a topical Decorin product and completion of studies necessary for IND submission. This 2 year funding request will help manufacture the Decorin product, file an IND, and conduct a clinical trial in patients with RDEB.	Dystrophic EB (DEB)	Wound Healing	Hald Landy, MD	\$2,000,000

Thomas Jefferson University

Thomas Jefferson University - Targeting
Fibrosis for RDEB Therapy

RDEB

Cancer/Wound Healing Andrew South PhD

\$79,527.00