Institution	Project Name	Project Description	Patient Population	Technique	Principal Investigator	Amount Awarded
University of Colorado	A “Spray-on-Skin” Device to Deliver iPSC-Derived Skin Cells	The Colorado team is using cutting-edge and innovative approaches to treat RDEB in which patient-derived cells are simultaneously gene-edited with CRISPR-Cas9 technology and reprogrammed using iPS cells to eventually yield keratinocytes and fibroblasts that can be grafted through an FDA approved "spray on skin" device (the FDA approved the use of the RECELL® Device for treating severe burns in 2018) to affected patients. This treatment strategy would not only greatly increase patient access to this permanent corrective therapy, but also decrease the time of patient application and the cost compared to growing epidermal sheets which are transplanted in an inpatient setting.	EB Simplex (EBS) Dystrophic EB (DEB) Junctional EB (JEB) Kindler Syndrome	Stem Cell Therapy	Dennis Roop, Ganna Bilousova, and Igor Kogut	$535,268
UMass Chan Medical School	Ataluren Treatment in Patients with Epidermolysis Bullosa (Additional 2 patients)	This research explores the use of Ataluren, an oral medication that promotes readthrough of premature termination codons (PTCs), as a for EB caused by nonsense mutations. In a five-year study, a patient with junctional EB due to a PTC in the LAMB3 gene showed significant improvement in chronic wounds, overall health stabilization, and subtle restoration of laminin-332 expression at the basement membrane. Encouraged by these results, the team has secured FDA INDs, manufacturer support, and IRB approval to treat additional EB patients on a compassionate use basis. They will collect clinical and patient-reported outcomes and conduct biopsy analyses to further evaluate ataluren’s potential for treating EB with PTC-related mutations.	EB Simplex (EBS) Dystrophic EB (DEB) Junctional EB (JEB)	Drug Research	Diana Reusch	$46,340.00
Northwestern University	Augmented Intelligence in EB: Using deep learning for early detection of squamous cell Carcinoma in EB	This project proposes a sophisticated AI and bioinformatic approach to develop a model to make accurate predictions of SCC emergence in photos of RDEB-affected skin. In the second year of their project, they are validating this tool by selecting machine learning algorithms involving image classification and analysis to achieve the most accurate predictions, and collaborating with a software company to create an app for patients. This app will integrate the model and help users identify potential SCC lesions. In the third year, they plan to launch the app and add features, such as identifying the best biopsy sites. The long-term goal is to provide a tool for patients and physicians to interpret RDEB skin images, enabling earlier detection of SCC and improving outcomes.	Dystrophic EB (DEB)	Cancer Research Data	Amy Paller	$530,132.00
Stanford University	CRISPR SWAP-mediated induced Skin Composite Delivery Through Electrospray-on-Skin Technology	This gene-editing treatment has shown promise in pre-clinical tests, and now the team faces challenges in transitioning to the clinic and delivering the therapy to difficult-to-reach wounds. To address these issues, the team developed CRISPR-SWAP DEBCT, which can correct up to one-third of all DEB-related genetic mutations. They also developed a new method to spray the corrected cells onto the skin using an electrospray device. They plan to test the safety and effectiveness of this spray-on treatment in preparation for a Phase I proof-of-concept CRISPR-SWAP DEBCT electrospray-on skin trial.	Dystrophic EB (DEB)	Stem Cell Therapy	Anthony Oro	$333,900
EBRP x AWS x Slalom	Curator	Investment to build the next phase of Curator to create a market-ready product for launch.	EB Simplex (EBS) Dystrophic EB (DEB) Junctional EB (JEB) Kindler Syndrome	Data Platform	EBRP	$846,095.00
Centre for Human Genetics	Development of a Registry for Epidermolysis Bullosa in India	India lacks a structured registry for EB patients, so this project is establishing a registry including clinical and genotype information to help in building a database for future clinical trials when it becomes feasible.	EB Simplex (EBS) Dystrophic EB (DEB) Junctional EB (JEB) Kindler Syndrome	Data	Ravi Hiremagalore MD, Gurudatta Baraka PhD, Arun Inamadar MD, Sacchidanand MD	$27,620.00
Centre for Human Genetics	Development of a Registry for Epidermolysis Bullosa in India	Above	EB Simplex (EBS) Dystrophic EB (DEB) Junctional EB (JEB) Kindler Syndrome	Data	Ravi Hiremagalore MD, Gurudatta Baraka PhD, Arun Inamadar MD, Sacchidanand MD	$27,620.00
University of Southern California	Dual Therapeutic Strategy: Utilizing SRI-41315 Alone and in Combination with Gentamicin to Promote Readthrough in Patients with RDEB and JEB	To reduce gentamicin's toxicity, this research team is testing a new agent called SRI-41315, which enhances the effectiveness of lower doses of gentamicin. They will also evaluate whether SRI-41315 alone or combined with gentamicin can correct the abnormal cellular behavior in EB patients and integrate properly into the skin's structure. If successful, this study will pave the way for clinical trials using SRI-41315 and gentamicin as a treatment for EB patients with nonsense mutations	Dystrophic EB (DEB) Junctional EB (JEB)	Drug Research	Mei Chen	$265,650.00
The Children's Hospital of Philadelphia	Local and systemic therapeutic base editing strategy for recessive dystrophic epidermolysis bullosa	This team is working on a CRISPR-mediated exon-skipping approach to remove exons 73 and 80 using lipid nanoparticle (LNP) delivery and a slow-release thermogel for localized, postnatal skin application. Additionally, they aim to develop a prenatal therapy by delivering the treatment into the amniotic cavity, targeting the fetus's skin, oral cavity, and gastrointestinal tract before birth. This dual strategy could yield postnatal therapies ready for clinical trials and pave the way for prenatal interventions.	Dystrophic EB (DEB)	Stem Cell Therapy Gene Therapy	William Peranteau	$327,781.00
Children's Hospital Corporation	Modeling and Targeting RDEB with Human Skin Organoids-on-Chip	Using a cutting-edge human pluripotent stem cell (hPSC)-derived skin organoid-on-chip platform, these researchers aim to model RDEB pathogenesis, test gene therapy approaches, and study immune responses. They will create RDEB-specific skin organoids to replicate disease characteristics, validate the model, and analyze wound healing mechanisms. Gene therapy strategies will be evaluated for their ability to restore the damaged dermal-epidermal junction (DEJ), and immune cells will be incorporated to investigate inflammation and identify therapeutic targets.	Dystrophic EB (DEB)	Stem Cell Therapy Gene Therapy	Karl Koehler	$329,672.00
Imagine Institute	Nonsense readthrough using gentamicin or ELX-02 in combination with potentiator compounds for the treatment of Recessive Dystrophic Epidermolysis Bullosa and Junctional Epidermolysis Bullosa	Gentamicin has been found to help re-express important skin proteins in EB patients with nonsense mutations, but has been found to cause kidney and ear damage when used long-term or at high doses. To enhance its effectiveness and reduce toxicity, this team of researchers are exploring the use of additional compounds like amlexanox, CC-90009, SRI-41315, and CDX5-288, that can increase the effectiveness of gentamicin and its derivative ELX-02 (NB124), allowing for lower, safer doses. If successful, this combination therapy could provide a long-term treatment for EB patients with nonsense mutations.	Dystrophic EB (DEB) Junctional EB (JEB)	Drug Research	Matthias Titeux	$381,973.00
Thomas Jefferson University	Pharmacological inhibition of CXCL8-driven chemotaxis to improve wound healing and mitigate RDEB comorbidities	Expressing some concern about the long-term effects of viral/gene therapy this group is taking another approach to treatment of RDEB. They propose pre-clinical studies of a compound that would enhance wound healing, especially to prevent the formation and accelerate healing of chronic painful wounds. They have identified some of the factors that prevent wound healing and found that CXCL8, a pro-inflammatory cytokine is greatly elevated in chronic non-healing wounds and hope to find an effective inhibitor of it. They anticipate clinical trials in 1-2 years.	Dystrophic EB (DEB)	Drug Research	Olga Igoucheva	$134,927.00
Onconova Therapeutics, Inc.	Rigosertib Formulation Enhancements & Development for RDEB-SCC	Onconova Therapeutics is working on improving the formulation of Rigosertib, a drug that has shown promise in modulating PLK-1 activity in RDEB-SCC. Initial clinical trials have demonstrated the potential of both oral and intravenous (IV) Rigosertib formulations in treating this aggressive cancer. Given the unique needs of RDEB patients, such as those with upper gastrointestinal obstructions or extreme skin fragility, Onconova is exploring additional formulations. These include a liquid oral formulation for patients with G-tubes and a palatable solution for those who cannot take capsules or IV treatment.	Dystrophic EB (DEB)	Drug Research Cancer Research	Sunil Shah Ph.D. RAC	$200,000.00
Thomas Jefferson University	Targeted Therapies for Junctional and Kindler Epidermolysis Bullosa-Associated Squamous Cell Carcinoma	This project aims to identify the genetic alerations of JEB and DEB SCCs through next-generation sequencing and advanced spatial profiling technologies. By analyzing the genetic alterations and tumor microenvironments, the study seeks to identify commonalities and distinctions that can inform therapeutic strategies. Despite delays in sample quality, progress has been made by expanding collaborations and sample availability. This research has the potential to uncover molecular markers, prevention strategies, and personalized therapies for EB-associated SCCs.	Dystrophic EB (DEB) Junctional EB (JEB) Kindler Syndrome	Cancer Research	Andrew South	$163,522.00

Northwestern University	Treating Pain and Itch in Dystrophic Epidermolysis Bullosa	This research focuses on addressing the significant unmet need for effective, long-term pain and itch management. Building on a promising first year, the study will explore novel non-opioid therapies using an RDEB mouse model, including MNK1/2 inhibitors and endocannabinoid augmentation via MAGL inhibition, both of which have shown potential for pain relief in early human trials. The goal is to validate these approaches as viable candidates for clinical trials in EB. Additionally, the project will investigate biomarkers to predict response to dupilumab, an interleukin-4 receptor inhibitor currently being studied for EB-associated itch, enabling better-targeted treatments.	EB Simplex (EBS) Dystrophic EB (DEB) Junctional EB (JEB) Kindler Syndrome	Drug Research	Amy Paller	$379,877.00
King's College London	Validating new anti-cancer targets for dystrophic epidermolysis bullosa using patients derived skin organoids	This research project aims to advance understanding and therapeutic development for RDEB SCC. Building on prior work, the study leverages induced pluripotent cancer cell (iPSC) models and innovative techniques such as immunofluorescence, spatial transcriptomics, and flow cytometry to investigate the immune-tumor microenvironment (TME) interactions. Key objectives include characterizing monocytes and macrophages, developing 3D in vitro tumor models, and testing novel therapeutic targets, including IgE antibodies against CSPG4, a cSCC-associated antigen. This project not only seeks to uncover the mechanisms driving RDEB-cSCC progression but also to identify and validate therapeutic strategies that address the urgent unmet clinical needs of these patients. Additionally, it includes funding for a PhD studentship to support future advancements in the field.	Dystrophic EB (DEB)	Cancer Research	Joanna Jackow	$354,434.28
Eliksa Therapeutics	Year Two: Advancing ELK-003 Eye Drop Development for Treating Ocular Manifestations of Epidermolysis Bullosa	This project focuses on the development of ELK-003, a novel ophthalmic solution designed to treat severe ocular complications associated DEB and JEB. Eliksa has made significant strides, including initiating the first-ever clinical trial for EB-related ocular issues, enrolling 8 patients, and demonstrating ELK-003's efficacy in preclinical models. The team has also optimized trial design, secured regulatory approvals, and prepared for FDA compliance by meeting critical CMC requirements and filing patents. With renewed funding, the project will continue its clinical trial in Chile, advance regulatory preparations, and progress toward a US trial, and ultimately FDA approval	Dystrophic EB (DEB) Junctional EB (JEB)	Protein Therapy	Armen Karamanian	$250,000.00
EBCRC Funding						$501,196.22
					TOTAL	**$5,636,007.50**