

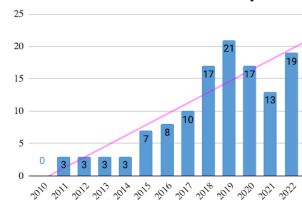
# 2023 Update



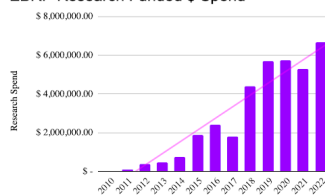
**EB** RESEARCH PARTNERSHIP



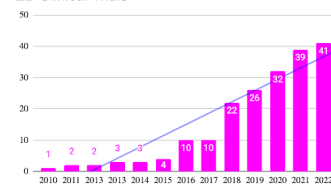
EBRP Research Funded # of Projects



EBRP Research Funded \$ Spend



EB Clinical Trials



## Key 2023 Accomplishments Thus Far:

- **May 2023: First Ever FDA Approved Treatment for EB-** Landmark victory for individuals living with EB and their families, the FDA announced their approval of Krystal Biotech's VYJUVEK™ for the treatment of Dystrophic Epidermolysis Bullosa (DEB).
  - EBRP invested in Krystal Biotech by way of a private placement after Krystal's Biotech's initial public offering in 2017 using EBRP's Venture Philanthropy model. This is not only the first-ever FDA approved treatment for those battling DEB, but also the first FDA approved topical, redosable gene therapy.
  - EBRP's investment was made via a private placement of Krystal Biotech's common stock, and as part of the stock purchase agreement, Krystal Biotech committed to commence a Phase I clinical trial of VYJUVEK. The funding followed the highly competitive application and screening process overseen by EBRP's Scientific Advisory Board (SAB), which is composed of leading scientists and physicians. EBRP was able to generate a more than double return on its investment and reinvest that capital back into more EB projects.
  - [PRESS RELEASE](#)
- **EBRP Achieves Record Research Funding Year in 2022, On Track to Hit New Record in 2023:**
  - In 2022, EBRP achieved a record year for research funding: we funded 19 innovative research projects in 6 countries, accelerating cutting-edge science with potential to heal all forms of EB, totaling over \$6.6 million in grant awards.
  - In 2023, our goal is to exceed 2022 and fund \$7 million in EB research projects, the first round of the year we funded \$1.49 million for 6 global research grants including curative gene editing, immunotherapies targeted at squamous cell carcinoma cancer, one of the leading causes of death for EB, and drug repurposing projects. The next research round has a deadline of September 12, 2023.
  - [2022 REPORT](#)
- **EBRP Wins Award from MIT for having the Most Innovative Rare Disease Technology:**
  - The Prize focused on improving quality of life for those diagnosed with rare diseases. Nearly 180 innovators, founders and inventors from around the world applied to answer the question "How can we improve the quality of life of people who have been diagnosed with a rare disease?"
  - Horizon Therapeutics in collaboration with MIT Solve, a marketplace for social impact innovation, announced the winning solution of this year's Horizon Prize: Patient-Driven Data Platform for Rare Disease by EB Research Partnership.
  - [PRESS RELEASE](#)

2023 Projects Funded for First Half of the Year with Help from Brothers Trust Cure Innovation Award:

Institution	Project Name	Principal Investigator(s)	Patient Population	Technique	Amount Approved (Total)
INSERM - Imagine Institute for genetic disease	Multi-omics of Recessive Dystrophic Epidermolysis Bullosa-associated Squamous Cell Carcinoma for targeted anti-tumor therapy.	Alain Hovnanian, Helene Ragot	Dystrophic EB (DEB)	Anti-tumor Therapy	\$295,718.53
Northwestern University	Suppressing the Itch of Dystrophic Epidermolysis Bullosa	Amy Paller and Ziyou Ren	EB Simplex (EBS) Dystrophic EB (DEB) Junctional EB (JEB)	Immunotherapy Drug Repurposing	\$236,423
Thomas Jefferson University	Targeting Innate Signaling Pathways in Treatment of Recessive Dystrophic Epidermolysis Bullosa-Associated Squamous Cell Carcinoma	Neda Nikbakht and Andrew South	Dystrophic EB (DEB)	Cancer Research Immunotherapy	\$200,000
INSERM U1163-Imagine Institute for genetic disease	Developing ex-vivo and in-vivo Base and prime editing strategies to treat Recessive Dystrophic Epidermolysis Bullosa	Araksya Izmiryan, Matthias Titeux, and Alain Hovnanian	Dystrophic EB (DEB)	Gene Therapy Stem Cell Therapy	\$498,431
INSERM - Institut Necker	Strategies for efficient and long-term engraftment of Mesenchymal Stromal Cells for the treatment of Recessive Dystrophic Epidermolysis Bullosa	Alain Hovnanian	Dystrophic EB (DEB)	Stem Cell Therapy	\$242,694.32
UMass Chan Medical School	Ataluren Treatment in Patients with Epidermolysis Bullosa	Karen Wiss, Sarah Servattalab, and Carolyn Foley	Dystrophic EB (DEB) Junctional EB (JEB)	Gene Therapy Drug Repurposing	\$20,100.00
				TOTAL	\$1,493,366.85