

FUNDS AWARDED BYYEAR

TOTAL AWARDS: **\$76 MILLION** 2008-2023

Adrian Bird, PhD / Jacky Guy, PhD University of Edinburgh

Correcting Rett syndrome-causing C-terminal Deletions using Adenine Base Editors

\$315.502

Erik Sontheimer, PhD / Jonathan Watts, PhD / Scot Wolfe, PhD UMASS Medical School

Base and Prime Editing Approaches for Rett syndrome

\$2,343,091

Guoping Feng, PhD

MIT

Single AAV Deliverable and Transiently Inducible Base Editors for Rett syndrome

\$3,734,738

Peter Beal, PhD UC Davis

Directed RNA editing for the repair of MECP2 mutations causing Rett syndrome

\$390,506

ProOR

Correction of R270X mutations in MECP2 RNA using Axiomer® Technology \$1,120,000

Michael Elowitz, PhD / Viviana Gradinaru, PhD CalTech

Quantitative, dosage-compensated gene therapy for Rett syndrome **\$500,000**

Victor Faundez, MD, PhD Emory University

Correlating Rett Syndrome Brain CSF Proteomes with Blood Plasma Profiles \$1,150,965

Victor Faundez, MD, PhD / Stuart Cobb, PhD Emory University / Edinburgh University

Systems Biology of Rett Syndrome Gene Therapy Outcomes (Supplement)

\$103,120

RSRT Biorepository

Induced Pluripotent Stem Cell and Fibroblast Cell Collections

\$230.949

Rett Syndrome Global Registry

Parent-reported SHARE Study

\$100,750

Emerald Innovations

Digital Technologies for the Assessment of Rett Symptoms

\$20,075

Vivalink

Digital Technologies for the Assessment of Rett Symptoms

\$7.446

Boston Children's Hospital Rett Syndrome Research Team

Rett Clinic Support

\$69,088

Montefiore Rett Syndrome Research Team

Rett Clinic Support

\$50,000

Orrin Devinsky, MD

NYU Langone

Improving Diagnostic Accuracy of Seizure and Non-Seizure Events to Enhance Clinical Care and Trial Outcomes

\$50,000

Adrian Bird, PhD / Stuart Cobb, PhD University of Edinburgh

Data Analysis Equipment

\$87.850

2022 TOTAL AWARDS: **\$2,073,337**

Emerald Innovations

Passive monitoring of Rett patients with Emerald

\$1,106,237

Herophilus

Evaluation of MECP2 Reactivating Effects of Herophilus Lead Small Molecules

\$200,000

David Lieberman, MD, PhD

Boston Children's Hospital Rett Clinic

\$67,345

Samir Mitragotri, PhD

Harvard University

Pilot Study to Explore Novel Delivery Technology

\$50,000

Shawn Liu, PhD

Columbia University

Multiplex Epigenome Editing to Reactivate & Maintain MECP2 in RTT Neurons

\$482,877

John Foxe, PhD

University of Rochester

From sensory-perceptual representations to cognitive processing in Rett

Syndrome

\$36,690

Coriell Institute

Rett Syndrome biorepository

\$119,461

Harvard Stem Cell Institute

Support for development of patient derived induced pluripotent stem cell lines

\$10,727