RETT SYNDROME RESEARCH TRUST

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FUNDS AWARDED BY YEAR

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

2023

TOTAL AWARDS **$10,274,080**

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

ProQR
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 Collection
$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

Shawn Liu, PhD
Columbia University
Multiplex Epigenome Editing to Reactivate and Maintain MECP2 in RTT Neurons
$482,877

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

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

2021

TOTAL AWARDS **$3,160,017**

Antonio Bedalov / Kyle Fink
Fred Hutchinson Cancer Research Institute / University of California Davis
Reactivation of MECP2
$1,090,919

Victor Faundez, PhD
Emory University
Systems Biology of Rett Syndrome Gene Therapy Outcomes
$584,304

Ciitizen
Digital Natural History Study
$444,000

Joseph Anderson, PhD
University of California Davis Medical Center
Feasibility of a stem cell gene therapy approach for the treatment of Rett Syndrome
$186,254

Joni N. Saby, PhD / Eric D. Marsh, MD, PhD
Children's Hospital of Philadelphia (CHOP)
Electrophysiological (EEG) Outcome Measures for Rett Syndrome Clinical Trials
$115,906

David Lieberman, MD, PhD
Boston Children's Hospital
Clinical Trial Consortium
$67,821

Stuart Cobb, PhD
University of Edinburgh
Genetic Analysis of the Rett Syndrome Cerebrospinal Fluid Proteome
$47,014

Coriell Institute
Rett Syndrome biorepository
$53,612

Harvard Stem Cell Institute
Support for development of patient derived induced pluripotent stem cell lines
$36,343

The Jackson Laboratory
Generation and phenotypic assessment of mouse models for Rett Syndrome
$5,620 *(additional support)*

Bryce Reeve, PhD
Duke University School of Medicine
Development of the Observer-Reported Communication Ability (ORCA) for Rett Syndrome
$15,294

Sasha Djukic, MD, PhD
Albert Einstein College of Medicine
Support for continuing work at the Rett Syndrome Center
$25,000

The Jackson Laboratory
Testing of siRNA compounds from Khvorova lab for MECP2 Duplication Syndrome
$362,930

Davut Pehlivan, MD
Texas Children's Hospital
Clinical studies in MECP2 Duplication Syndrome as foundation for antisense oligonucleotide drug trials
$125,000

2020

TOTAL AWARDS **$1,299,972**

DSG
Development of the Rett Syndrome Global Registry
$693,000

James Wilson, MD, PhD
University of Pennsylania
MECP2 gene therapy for Rett Syndrome
$380,686

Clinical Trial Consortium
David Lieberman, MD, PhD
Boston Children's Hospital
$94,176

Bryce Reeve, PhD
Duke University School of Medicine
Development of the Observer-Reported Communication Ability (ORCA) for Rett Syndrome
$72,225

Ciitizen
Pilot Study for Digital Natural History Study
$34,885

Sasha Djukic, MD, PhD
Albert Einstein College of Medicine
Support for continuing work at the Rett Syndrome Center
$25,000

Due to the global pandemic and the ensuing fundraising uncertainties we were cautious in taking on additional commitments. Furthermore we undertook a detailed analysis of our portfolio and were able to reduce our commitments by $6 million. This reduction allows us to focus our resources on curative projects with the greatest likelihood of success in the nearer term.

2019

TOTAL AWARDS **$8,134,666**

Adrian Bird, PhD / Michael Greenberg, PhD / Gail Mandel, PhD
University of Edinburgh / Harvard University / Oregon Health and Sciences University
MECP2 Consortium
$3,359,054

James Wilson, MD, PhD
University of Pennsylvania
MECP2 gene therapy for Rett Syndrome
$765,607

James Wilson, MD, PhD
University of Pennsylvania
MECP2 gene therapy for Rett Syndrome, vector production
$37,999

Stuart Cobb, PhD / Chris Sibley, PhD
University of Edinburgh
RNA trans-splicing therapy in Rett Syndrome
$235,950

Harvard Stem Cell Institute
Support for development of patient derived induced pluripotent stem cell lines
$101,912

Michael Elowitz, PhD
California Institute of Technology
A system for dosage-independent control of MECP2 expression in Rett Syndrome gene therapy
$212,374

Peter Glazer, PhD / Mark Saltzman PhD
Yale University
PNA nanoparticles for gene editing of Rett Syndrome
$275,000

Alanna Schepartz, PhD
Yale University
Evaluating cell-permeant miniature proteins (CPMPs) as a strategy for delivering functional MECP2 into model cells and neurons
$297,716

Joost Gribnau, PhD
Erasmus Medical Center
Human in vitro models for X chromosome reactivation
$401,000

Antonio Bedalov, PhD
Fred Hutchinson Cancer Research Center
Mouse model maintenance
$20,000

Thorsten Stafforst, PhD
University of Tubingen
RNA editing for MECP2 mutations via RESTORE
$359,856

Joseph Jacobson, PhD
Massachusetts Institute of Technology
Correction of MECP2 mutations with engineered ScCas 9 base editors
$50,000

The Jackson Laboratory
Generation and phenotypic assessment of mouse models for Rett Syndrome
$417,690

Coriell Institute
Rett Syndrome biorepository
$135,000

Emerald Innovations
Passive monitoring of Rett patients with Emerald
$164,670

Beth McCormick, PhD
University of Massachusetts Medical School
Microbiome study for the advancement of novel nutritional supplements
$520,316

Sasha Djukic, MD, PhD
Albert Einstein School of Medicine
Support for continuing work at the Rett Syndrome Center
$75,000

Miscellaneous Pilot Studies
$135,522

Ronald Cohn, PhD
The Hospital for Sick Children
Interrogation of genome editing strategies as a therapeutic modality for MECP2 Duplication Syndrome
$570,000

Anastasia Khvorova, PhD
University of Massachusetts Medical School
Development of siRNA based compounds to potently silence MECP2 towards the treatment of MECP2 Duplication Syndrome
$435,515

2018

TOTAL AWARDS **$9,956,283**

Jonathan Watts, PhD / Scot Wolfe, PhD / Eric Sontheimer, PhD / Anastasia Khvorova, PhD
University of Massachusetts Medical School
RNA and genome editing for treatment of Rett Syndrome
$2,403,735

Guoping Feng, PhD / Feng Zhang, PhD / Robert Desimone, PhD
Massachusetts Institute of Technology / Broad Institute / Harvard University
RNA-editing as a gene therapy approach for Rett Syndrome
$2,332,000

Beam Therapeutics
Developing a pre-clinical DNA base editing program to precisely correct the genetic cause of Rett Syndrome in the central nervous system
$1,870,660

John Sinnamon, PhD
Oregon Health and Science University
New editing enzymes for RNA
$345,000

Peter Beal, PhD
University of California, Davis
New molecular tools for directed editing of MECP2 mutations associated with Rett Syndrome
$563,870

Stuart Cobb, PhD / Adrian Bird, PhD
University of Edinburgh
Gene Therapy Consortium 2.0
$653,856

Stuart Cobb, PhD
University of Edinburgh
Purchase of qPCR machine
$13,945

Andrea Cerase, PhD
Queen Mary University of London
Reactivation of MECP2 and CDKL5 genes by functional deactivation of Xist RNA
$351,022

James Wilson, MD, PhD
University of Pennsylvania
Gene Therapy Consortium Vector Core
$131,243

Allan Jacobson, PhD / Jonathan Watts, PhD
University of Massachusetts Medical School
Read-through of premature termination codons for treatment of Rett Syndrome
$323,000

Antonio Bedalov
Fred Hutchinson Cancer Research Institute
Reactivation of MECP2
$38,000

Clinical Trial Consortium
David Lieberman, MD, PhD
Boston Children's Hospital
$74,792

Laurel Joy Gabard-Durnam, PhD
Harvard University
Post Doctoral Fellowship, Autism Science Foundation
$17,500

Hassan Ghasemzadeh, PhD
Washington State University
Pilot study to examine gait patterns in Rett Syndrome
$10,000

Sasha Djukic, MD, PhD
Albert Einstein College of Medicine
Support for continuing work at the Rett Syndrome Center
$75,000

Huda Zoghbi, MD, PhD
Baylor College of Medicine
A forward genetic screen to identify druggable modulators of MECP2 levels
$752,660

2017

TOTAL AWARDS **$6,166,762**

James Wilson, MD, PhD
University of Pennsylvania
Gene therapy consortium
$1,585,886

Katherin Meyer, PhD
Nationwide Children's Hospital
Optimizing gene therapy for Rett Syndrome
$152,489

Katherin Meyer, PhD
Nationwide Children's Hospital
A gene therapy consortium to develop and evaluate gene therapy approaches in Rett Syndrome
$68,515

Stuart Cobb, PhD
University of Glasgow
Additional support for RNA-trans splicing efforts in Rett Syndrome
$290,000

Rudolf Jaenisch, MD
Whitehead Institute for Biomedical Research
Reactivation of MECP2 with epigenome editing tools to rescue Rett Syndrome
$599,850

Benjamin Philpot, PhD
University of North Carolina Chapel Hill
Pilot study for reactivation of silenced MECP2 by artificial transcription factors
$145,443

Q State Biosciences
Development of an in-vitro cell system for discovering and evaluating the effects of therapeutic candidates on neurons produced using Rett patient iPS cells
$498,141

Michael Greenberg, PhD
Harvard University
Development of an in-vitro cell system for discovering and evaluating the effects of therapeutic candidates on neurons produced using Rett patient iPS cells
$55,826

Clinical Trial Consortium
Daniel Tarquinio, DO
Center for Rare Neurological Diseases
$495,000

Clinical Trial Consortium
David Lieberman, MD, PhD
Boston Children's Hospital
$395,000

Clinical Trial Consortium
Eric Marsh, MD, PhD
Children's Hospital of Philadelphia
$487,715

Clinical Trial Consortium
Alan Percy, MD, PhD
University of Alabama Birmingham
$495,000

Clinical Trial Consortium
Jeffrey Neul, MD, PhD
Vanderbilt University Medical Center
$495,000

Sasha Djukic, MD, PhD
Albert Einstein College of Medicine
Support for continuing work at the Rett Syndrome Center
$103,000

Huda Zoghbi, MD
Baylor College of Medicine
Investigating the potential of antisense oligonucleotide therapy for MECP2 Duplication Syndrome
$299,897

2016

TOTAL AWARDS **$7,571,438**

Adrian Bird, PhD / Michael Greenberg, PhD / Gail Mandel, PhD
University of Edinburgh / Harvard University / Oregon Health and Sciences University
MECP2 Consortium
$3,454,921

Stuart Cobb, PhD / Steve Gray, PhD / Brian Kaspar, PhD / Gail Mandel, PhD / Alysson Muotri, PhD
University of Glasgow / University of North Carolina Chapel Hill / Nationwide Children's Hospital / Oregon Health and Science University / University of California San Diego
A gene therapy consortium to develop and evaluate gene therapy approaches in Rett Syndrome
$1,450,275

Stuart Cobb, PhD
University of Glasgow
Scientific support for gene therapy, splicing therapy and protein therapy programmes in Rett Syndrome
$210,000

Stuart Cobb, PhD
University of Glasgow
Optimizing MECP2 trans-splicing for human translation
$330,804

Alysson Muotri
University of California San Diego
A drug-screening platform using MECP2-deficient human neurons and preclinical testing
$1,001,000

Alysson Muotri
University of California San Diego
Role of an autism-related cytokine in a genetic model of ASD (Autism Science Foundation)
$12,500

David Katz
Case Western Reserve University School of Medicine
Preclinical studies of LM22A-4 in mouse models of Rett Syndrome
$250,000

ArmaGen, Inc.
Protein replacement for Rett Syndrome
$125,000

Rudolf Jaenisch, MD
Whitehead Institute for Biomedical Research
Reversal of Rett phenotype: A screen for compounds that enhance KCC2 expression
$180,000

Michael Greenberg, PhD
Harvard University
Identifying therapeutics for treating Rett Syndrome using nuclear size as a proxy for long gene mis-regulation
$110,000

Q State Biosciences
Development of an in-vitro cell system for discovering and evaluating the effects of therapeutic candidates on neurons produced using Rett patient iPS cells
$330,000

Miscellaneous Pilot Projects
$33,838

Sasha Djukic, MD, PhD
Albert Einstein College of Medicine
Support for continuing work at the Rett Syndrome Center
$84,000

2015

TOTAL AWARDS **$8,741,782**

Antonio Bedalov, PhD
Fred Hutchinson Cancer Research Center
Genetic and pharmacologic reactivation of Mecp2 on the silent X-chromosome as a therapeutic approach to Rett Syndrome
$824,575

Jeannie Lee, PhD
Massachusetts General Hospital / Harvard University
Treating Rett Syndrome by targeting the Xist interactome
$766,854

Joost Gribnau, PhD
Erasmus MC
In vivo and in vitro models for X chromosome reactivation.
$177,900

Neurolixis, PhD
Clinical development of NLX-101 in Rett Syndrome
$530,000

Mark Zylka, PhD
University of North Carolina
High Throughput screen to identify drugs that normalize long gene expression in Rett Syndrome model neurons
$400,000

Andrew Napper, PhD
Nemours duPont Pediatrics
Discovery and in vivo characterization of compounds promoting MECP2 read-through
$230,101

Stuart Cobb, PhD
University of Glasgow
Spliceosome-mediated RNA trans-splicing therapy in Rett Syndrome
$86,208

Stephen Turley, PhD / Adam Lopez, PhD
University of Texas Southwestern Medical Center
Exploration of the impact of 2-hydroxypropyl-B-cyclodextrin treatment on lifespan and brain cholesterol metabolism in male mecp2 deficient mice
$156,180

Miscellaneous Pilot Studies
$20,000

DiamiR
microRNA biomarkers in Rett Syndrome
$26,815

David Katz, PhD
Case Western Reserve University
Preclinical Studies of LM22A-4 in Mouse Models of Rett Syndrome
$14,154

The Jackson Laboratory
Development of mouse models
$42,052

Hermano Igo Krebs, PhD
Massachusetts Institute of Technology
Pilot Study
$8,000

Tim Benke, PhD / Aleksandra Djukic, PhD / Alan Percy,PhD / Daniel Tarquinio, PhD
Children's Hospital Colorado / Montefiore Medical Center / University of Alabama Birmingham / Children's Healthcare of Atlanta
Outcome measures and biomarkers development
$4,500,000

Michele Fagiolini
Boston Children's Hospital
Testing NR2A and NR2B NAMs in mouse models of Rett Syndrome.
$337,336

John Foxe, PhD / Sophie Molholm, PhD
University of Rochester / Albert Einstein College of Medicine
From sensory-perceptual representations to cognitive processing in Rett Syndrome
$533,607

Sasha Djukic, MD, PhD
Albert Einstein College of Medicine
Support for continuing work at the Rett Syndrome Center
$88,000

2014

TOTAL AWARDS **$5,809,107**

Monica Justice, PhD
University of Toronto
Identifying genetic modifiers of MECP2 in the mouse
$715,680

Jeffery Neul, MD, PhD
Baylor College of Medicine
Identification of genetic modifiers in Rett Syndrome
$314,456

Jeannie Lee, PhD
Massachusetts General Hospital / Harvard University
Re-awakening the silenced normal MECP2 allele with small molecules to treat Rett Syndrome
$465,000

Antonio Bedalov, PhD
Fred Hutchinson Cancer Research Center
Chemical genetic approach to reactivate the silenced MECP2 gene on the inactive X chromosome
$290,000

Terry Magnuson, PhD
University of North Carolina, Chapel Hill
Systems genetics approach toward understanding regulation of MECP2 expression
$200,000

David Katz, PhD
Case Western Reserve University
Preclinical studies of LM22A-4 in mouse models of Rett Syndrome
$271,700

Adrian Bird, PhD / Michael Greenberg, PhD / Gail Mandel, PhD
University of Edinburgh / Harvard University / Oregon Health and Science University
MECP2 Consortium
$250,000

Ali Khoshnan, PhD / Sarkis Mazmanian, PhD
California Institute of Technology
Exploring the link between MECP2 and gut physiology to test a novel probiotic therapy for Rett Syndrome
$200,000

Lucas Pozzo-Miller, PhD
University of Alabama Birmingham
Testing whether LM22A-4 improves hippocampal function in female MECP2 heterozygous mice
$110,000

Neurolixis
NLX-101 as a treatment for breathing disorders in Rett Syndrome
$54,945

Sung-Yon Kim, PhD
Life Science Research Foundation
Post doctoral fellowship
$91,500

Steven Gray, PhD
University of Texas Southwestern Medical Center
Supplement for gene therapy consortium
$67,401

Tom Frazier, PhD / David Katz, PhD / Daniel Sessler, MD, PhD
Case Western Reserve University / Cleveland Clinic
Low-dose ketamine for the treatment of Rett Syndrome
$1,295,131

Sasha Djukic, MD, PhD
Albert Einstein College of Medicine
Pharmacological treatment of Rett Syndrome with Lovastatin
$403,000

Sasha Djukic, MD, PhD
Albert Einstein College of Medicine
Supplement for copaxone clinical trial
$47,000

Debra Weese-Mayer, MD / Michael Carroll, PhD
Lurie Children's Hospital of Chicago
Outlining the automatic signature of Rett Syndrome
$157,300

Nurit Ballas, PhD
Stony Brook University
Determine the proteome, secretome and transcript changes in astrocytes derived from human Rett patients iPSCs and their effect on interaction with human neurons
$20,000

DiamiR
microRNA biomarkers in Rett Syndrome
$6,768

Sasha Djukic, MD, PhD
Albert Einstein College of Medicine
Support for continuing work at the Rett Syndrome Center
$140,161

Stephen Turley, PhD
University of Texas Southwestern Medical Center
Exploration of the impact of 2-hydroxypropyl-B-cyclodextrin treatment on lifespan and brain cholesterol metabolism in male mecp2 deficient mice
$20,000

Recursion Pharmaceuticals
High content phenotypic screening of existing drugs for the treatment of Rett Syndrome
$25,000

Daniela Tropea, PhD
Trinity College Dublin
Expression of nuclear MeCP2 is dependent on neuronal stimulation and application of IGF1
$13,000

Miscellaneous Pilot Projects
$7,000

Huda Zoghbi, MD, PhD
Baylor College of Medicine
A forward genetic screen to identify druggable modulators of MECP2 levels
$414,065

Huda Zoghbi, MD, PhD
Baylor College of Medicine
Antisense oligonucleotide therapy for the treatment of MECP2 Duplication Syndrome
$230,000

2013

TOTAL AWARDS **$7,167,097**

Adrian Bird, PhD / Michael Greenberg, PhD / Gail Mandel, PhD
University of Edinburgh / Harvard University / Oregon Health and Sciences University
MECP2 Consortium
$3,417,575

Stuart Cobb, PhD / Steven Gray, PhD / Brian Kaspar, PhD / Gail Mandel, PhD
University of Glasgow / University of North Carolina Chapel Hill / Nationwide Children's Hospital / Oregon Health and Sciences University
Gene Therapy Consortium
$1,535,942

Michael Green, PhD
University of Massachusetts Medical School
Testing drugs that modulate X chromosome inactivation to reactivate the silent MECP2
$750,000

David Katz, PhD
Case Western Reserve University
Preclinical evaluation of therapeutics that modulate the NMDA pathway
$150,000

Jeannie Lee, PhD
Massachusetts General Hospital / Harvard University
An oligotherapeutics approach to treat Rett Syndrome
$100,000

Michela Fagiolini, PhD
Boston Children's Hospital
Preclinical testing of selective novel NMDA receptor modulators
$126,741

Mark Bear
Massachusetts Institute of Technology
mGluR5 dependent synaptic protein synthesis in Rett Syndrome
$45,943

Bruria Ben Zeev, MD
Sheba Medical Center
Copaxone clinical trial
$197,962

Sasha Djukic, MD, PhD
Albert Einstein College of Medicine
Copaxone clinical trial
$412,370

Sasha Djukic, MD, PhD
Albert Einstein College of Medicine
Support for ongoing work at Rett Syndrome Center
$72,000

Huda Zoghbi, MD, PhD
Baylor College of Medicine
A forward genetic screen to identify druggable modulators of MeCP2 levels
$319,224

Kevin Foust, PhD
Nationwide Children's Hospital
RNA interference for the treatment of MECP2 Duplication Syndrome
$39,340

2012

TOTAL AWARDS **$4,235,266**

Benjamin Philpot, PhD
University of North Carolina Chapel Hill
A chemical genetic approach for activating the dormant gene associated with Rett Syndrome
$2,204,800

Jonathan Kipnis, PhD
University of Virginia
Immune modulation as a new therapeutic approach for Rett Syndrome
$720,000

John Bissonnette, PhD
Oregon Health and Sciences University
Respiration in MECP2 deficient mice
$59,642

Antonio Bedalov, PhD
Fred Hutchinson Cancer Research Center
Chemical genetic approach to reactivate the silenced MECP2 gene on the inactive X chromosome
$55,688

Andrew Pieper MD, PhD
University of Texas Southwestern Medical Center
In vivo identification of pharmacological agents for the treatment of Rett Syndrome
$69,000

Monica Justice, PhD
Baylor College of Medicine
Identification of gene modifiers that ameliorate Rett Syndrome
$757,165

Jay Shapiro, MD, PhD
Kennedy Krieger Institute
Treatment of osteoporosis in murine Rett Syndrome models
$20,000

Sasha Djukic, MD, PhD
Albert Einstein College of Medicine
Support for ongoing work a the Rett Syndrome Center
$109,771

Greenwood Genetic Center
MECP2 testing
$3,000

Huda Zoghbi, MD, PhD
Baylor College of Medicine
Is MECP2 Duplication/Triplication Syndrome reversible?
$236,200

2011

TOTAL AWARDS **$3,609,479**

Adrian Bird, PhD / Michael Greenberg, PhD / Gail Mandel, PhD
University of Edinburgh / Harvard University / Oregon Health and Sciences University
MECP2 Consortium
$1,840,441

Huda Zoghbi, MD, PhD
Baylor College of Medicine
Investigating novel therapeutic approaches for Rett Syndrome
$517,054

Monica Justice, PhD
Baylor College of Medicine
Identification of gene modifiers that ameliorate Rett Syndrome
$298,879

Jonthan Kipnis, PhD
University of Virginia
Immune modulation as a new therapeutic approach for Rett Syndrome
$440,000

Jeannie Lee, PhD
Massachusetts General Hospital / Harvard University
A high-throughput screen to identify compounds that reactivate the functional MECP2 allele in Rett Syndrome
$300,000

Mark Bear, PhD
Massachusetts Institute of Technology
mGluR5 dependent synaptic protein synthesis in Rett Syndrome
$85,896

Jeffrey Macklis, PhD
Harvard University
Vitamin D therapy for MECP2 target Irak1/NFkB dysregulation
$35,352

Sasha Djukic, MD, PhD
Albert Einstein College of Medicine
Support for ongoing work at Rett Syndrome Center
$66,710

Benjamin Philpot, PhD
University of North Carolina Chapel Hill
$10,000

John Bissonnette, PhD
Oregon Health and Sciences University
Respiration in MECP2 deficient mice
$15,147

2010

TOTAL AWARDS **$1,322,052**

Ronald Crystal, MD, PhD
Weill Medical College of Cornell University
AAV mediated gene transfer for the treatment of Rett Syndrome
$605,121

Brian Kaspar, PhD / Gail Mandel, PhD
Nationwide Children's Hospita / Oregon Health and Sciences University
AAV9 gene therapy for Rett Syndrome
$80,000

Antonio Bedalov, PhD
Fred Hutchinson Cancer Research Center
Chemical genetic approach to reactivate the silenced MECP2 gene on the inactive X chromosome
$250,000

Jonthan Kipnis, PhD
University of Virginia
Immune modulation as a new therapeutic approach for Rett Syndrome
$187,000

Huda Zoghbi, MD, PhD
Baylor College of Medicine
Interventional trials in mice models of Rett Syndrome and MECP2 disorders
$100,000

Marisa Bartolomei, PhD
University of Pennsylvania
Analysis of epigenetic modifications of the MECP2 locus
$41,255

Sasha Djukic, MD, PhD
Albert Einstein College of Medicine
Support for ongoing work at Rett Syndrome Center
$36,654

Rett Syndrome Clinic
University of Southern California
Support for Rett Syndrome Clinic
$22,022

2009

TOTAL AWARDS **$552,683**

Monica Justice, PhD
Baylor College of Medicine
Identification of gene modifiers that ameliorate Rett Syndrome
$236,038

Stavros Lomvardas
University of California San Francisco
Insight into MECP2 function raises therapeutic possibilities for Rett Syndrome
$140,000

Huda Zoghbi, MD, PhD
Baylor College of Medicine
Interventional trials in mice models of Rett Syndrome and MECP2 disorders
$100,000

Marisa Bartolomei, PhD
University of Pennsylvania
Analysis of epigenetic modifications of the MECP2 locus
$40,000

Sasha Djukic, MD, PhD
Albert Einstein College of Medicine
Support for continuing work at the Rett Syndrome Center
$36,645

2008

TOTAL AWARDS **$2,278,000**

Adrian Bird, PhD
Baylor College of Medicine
Identification of gene modifiers that ameliorate Rett Syndrome
$1,380,000

Andrew Pieper, MD,PhD
University of Texas Southwestern Medical Center
In vivo identification of pharmacological agents for the treatment of Rett Syndrome
$505,000

Monica Justice, PhD
Baylor College of Medicine
Identification of gene modifiers that ameliorate Rett Syndrome
$253,000

Antonio Bedalov, PhD
Fred Hutchinson Cancer Research Center
Chemical genetic approach to reactivate the silenced MECP2 gene on the inactive X chromosome
$140,000