RETT SYNDROME RESEARCH TRUST

FUNDS AWARDED BY YEAR

TOTAL AWARDS: **$85 MILLION** 2008-2024

RETT SYNDROME RESEARCH TRUST

ProQR Therapeutics
Development of AX-2402 for the Treatment of Rett Syndrome
$2,650,000

Apertura Gene Therapy
License of Novel Blood Brain Barrier Penetrating Capsid
$2,000,000

Kai Chen, PhD
UC BERKELEY (JENNIFER DOUDNA LAB)
A New Brain Delivery Platform for Base Editing of MeCP2 Mutations and Gene Knock-in for Rett Syndrome
$1,158,523

Omar Abudayyeh, PhD and Jonathan Gootenberg, PhD
BRIGHAM AND WOMEN'S HOSPITAL & BETH ISRAEL MEDICAL DEACONESS CENTER
Programmable RNA Writing for Treatment of Rett Syndrome
$859,990

Jiangbing Zhou, PhD
YALE UNIVERSITY
Non-viral delivery of base editing therapy for Rett syndrome
$588,306

Jiangbing Zhou, PhD
YALE UNIVERSITY
Non-viral delivery of epigenome editing therapy for Rett syndrome
$548,306

Shawn Liu, PhD
COLUMBIA UNIVERSITY
Non-viral delivery of epigenome editing therapy for Rett syndrome
$476,231

RCourage, Inc.
STEP-RNP production of epigenetic and base editors
$60,000

Kyle Fink, PhD
UC DAVIS
Rett syndrome tRNA screening
$97,554

Jef Boeke, PhD
NEW YORK UNIVERSITY
Development of a Fully Humanized MECP2 Mouse Model
$161,753

Vivek Kumar, PhD
JACKSON LABORATORIES
Automated Rett Syndrome Phenotyping and Efficacy Testing
$258,979

Han Zhang, PhD
UMASS MEDICAL SCHOOL (ERIK SONTHEIMER LAB)
Base Editing Program Support
$194,732

Rett Syndrome Global Registry
$88,536

RSRT Biorepository
$162,873

Aleksandra Jacobs, MD, PhD
MONTEFIORE RETT SYNDROME CLINIC
$25,000

2024

RETT SYNDROME RESEARCH TRUST

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 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

2023

Emerald Innovations
Passive monitoring of Rett patients with Emerald
$1,106,237

Shawn Liu, PhD
COLUMBIA UNIVERSITY
Multiplex Epigenome Editing to Reactivate & 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
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

2022

RETT SYNDROME RESEARCH TRUST

Antonio Bedalov, MD, PhD / Kyle Fink, PhD
FRED HUTCHINSON CANCER INSTITUTE / UC 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
UC 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
$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

2021

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

2020

RETT SYNDROME RESEARCH TRUST

Bird / Greenberg / Mandel Labs
EDINBURGH / HARVARD / OREGON HEALTH & SCIENCES
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
CALTECH
A system for dosage-independent control of MECP2 expression in Rett 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, MD, 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

Beth McCormick, PhD
UMASS 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

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
UMASS MEDICAL SCHOOL
Development of siRNA based compounds to potently silence MECP2 towards the treatment of MECP2 Duplication Syndrome
$435,515

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

Misc. Pilot Studies
$135,522

Coriell Institute
Rett Syndrome biorepository
$135,000

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

2019

RETT SYNDROME RESEARCH TRUST

Jonathan Watts, PhD / Scot Wolfe, PhD / Eric Sontheimer, PhD / Anastasia Khvorova, PhD
UMASS MEDICAL SCHOOL
RNA and genome editing for treatment of Rett Syndrome
$2,403,735

Guoping Feng, PhD / Feng Zhang, PhD / Robert Desimone, PhD
MIT/HARVARD/BROAD INSTITUTE
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
UC DAVIS
New molecular tools for directed editing of MECP2 mutations associated with Rett
$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
UMASS MEDICAL SCHOOL
Read-through of premature termination codons for treatment of Rett Syndrome
$323,000

Antonio Bedalov, MD, PhD
FRED HUTCHINSON CANCER RESEARCH INSTITUTE
Reactivation of MECP2
$38,000

Clinical Trial Consortium: David Lieberman, MD, PhD
BOSTON CHILDREN'S HOSPITAL
Reactivation of MECP2
$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

2018

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
$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
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
$299,897

2017

Bird / Greenberg / Mandel Labs
EDINBURGH / HARVARD / OREGON HEALTH & SCIENCES
MECP2 Consortium
$3,454,951

Stuart Cobb, PhD / Steve Gray, PhD / Brian Kaspar, PhD Gail Mandel, PhD / Alysson Muotri, PhD
UNIVERSITY OF GLASGOW / UNC CHAPEL HILL / NATIONWIDE CHILDREN'S / OREGON HEALTH & SCIENCE UC SAN DIEGO
A gene therapy consortium to develop and evaluate gene therapy approaches in Rett
$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, PhD
UNIVERSITY OF CALIFORNIA SAN DIEGO
A drug-screening platform using MECP2-deficient neurons and preclinical testing
$1,001,000

Alysson Muotri, PhD
UNIVERSITY OF CALIFORNIA SAN DIEGO
Role of an autism-related cytokine in a genetic model of ASD (Autism)
$12,500

David Katz
CASE WESTERN 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

2016

RETT SYNDROME RESEARCH TRUST

Antonio Bedalov, MD, 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, MD, PhD
MASSACHUSETTS GENERAL HOSPITAL / HARVARD
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
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
MIT
Pilot Study
$8,000

Tim Benke, MD, PhD / Aleksandra Djukic, MD, PhD
Alan Percy, MD / Daniel Tarquinio, DO
CHILDREN'S HOSPITAL COLORADO / MONTEFIORE
UA BIRMINGHAM / CHILDREN'S HEALTHCARE OF ATLANTA
Outcome measures and biomarkers development
$4,500,000

Michele Fagiolini, PhD
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
From sensory-perceptual representations to cognitive processing in Rett Syndrome
$533,607

Sasha Djukic, MD, PhD
ALBERT EINSTEIN COLLEGE OF MEDICINE
Support for continuing
$88,000

2015

2014 TOTAL AWARDS: $5,809,107

RETT SYNDROME RESEARCH TRUST

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, MD, PhD
MASSACHUSETTS GENERAL HOSPITAL / HARVARD
Re-awakening the silenced normal MECP2 allele with small molecules to treat Rett
$465,000

Antonio Bedalov, MD, 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 SCIENCES UNIVERSITY
MECP2 Consortium
$250,000

Ali Khoshnan, PhD / Sarkis Mazmanian, PhD
CALTECH
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

2014

RETT SYNDROME RESEARCH TRUST

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
$25,000

Daniela Tropea, PhD
TRINITY COLLEGE DUBLIN
Expression of nuclear MeCP2 dependent on neuronal stimulation & application of IGF1
$13,000

Miscellaneous Pilot Projects
Induced Pluripotent Stem Cell and Fibroblast Cell Collections
$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

2014

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
UMASS 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, MD, PhD
MASS. 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, PhD
MIT
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

2013

Benjamin Philpot, PhD
UNIVERSITY OF NORTH CAROLINA CHAPEL HILL
A chemical genetic approach for activating the dormant gene associated with Rett
$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, MD, 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

2012

RETT SYNDROME RESEARCH TRUST

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, MD, 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
MIT

mGluR5 dependent synaptic protein synthesis in Rett Syndrome

$85,896

Jeffrey Macklis, MD, 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

2011

2010 TOTAL AWARDS: $1,322,052

RETT SYNDROME RESEARCH TRUST

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 / OREGON HEALTH & SCIENCES
AAV9 gene therapy for Rett Syndrome
$80,000

Antonio Bedalov, MD, PhD
FRED HUTCHINSON CANCER RESEARCH CENTER
Genetic approach to reactivate the silenced MECP2 gene on 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

2010

Monica Justice, PhD
BAYLOR COLLEGE OF MEDICINE
Identification of gene modifiers that ameliorate Rett Syndrome
$236,038

Stavros Lomvardas, PhD
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

2009

RETT SYNDROME RESEARCH TRUST

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, MD, PhD
FRED HUTCHINSON CANCER RESEARCH CENTER
Chemical genetic approach to reactivate the silenced MECP2 gene on the inactive X chromosome
$140,000

2008