

FUNDS AWARDED BY YEAR

TOTAL AWARDS: **\$91.3 MILLION** 2008-2025

AAV development and Manufacturing
\$1,100,000

MIT Core Facility
Safety studies for gene replacement using BBB penetrant AAV delivery
\$1,100,000

Tacit Therapeutics
Multi-allelic correction using RNA trans-splicing
\$1,000,000

Profluent
AI Designed improvements to base editor
\$1,000,000

Shape Therapeutics
Single allele correction using small RNAs delivered by AAV
\$300,000

Resplice Therapeutics
Trans-splicing for Rett syndrome
\$130,000

Senti-Bio / Jackson Laboratories
Design and test of an inducible control circuit for base editing activity
\$250,000

Evox
Exosome delivery platform evaluation
\$240,000

Jonathan Watts, PhD
UNIVERSITY OF MASSACHUSETTS
Transvascular Delivery of mRNA and guide RNA to the CNS to Correct MECP2 Mutations that drive Rett Syndrome
\$84,791

Kyle Fink, PhD
UC DAVIS
Long term safety risk linked to constitutive expression of DNA base editing
\$205,647

Kyle Fink, PhD
UC DAVIS / EPICRISPR BIO
X-reactivation of wild type alleles in Rett syndrome
\$269,247

Jackson Laboratories
Partnership with Uncommon Therapeutics to test an ABE (adenosine base editor) design with low bystander effects
\$219,317

Aleksandra Jacobs, MD, PhD
MONTEFIORE RETT SYNDROME CLINIC
Extending Rett syndrome CSF biomarkers to blood based detection
\$40,123

Bryce Reeve, PhD
DUKE UNIVERSITY SCHOOL OF MEDICINE
Advancing Patient Centered Research
\$215,254

RSRT Biorepository
\$49,817

Rett Syndrome Global Registry
\$107,740

Couragene
\$15,000

Vibrant Study
\$8,741

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**

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

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 & 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

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

Citizen
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

2020

TOTAL AWARDS: \$1,299,972



DSG

Development of the Rett Syndrome Global Registry

\$693,000

James Wilson, MD, PhD

UNIVERSITY OF PENNSYLVANIA

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

Citizen

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

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

**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

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

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

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 interactorome

\$766,854**Joost Gribnau, PhD****ERASMUS MC**

In vivo and in vitro models for X chromosome reactivation

\$177,900**Neurolisis, 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

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

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- β -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

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
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 at 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

**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



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

2009

TOTAL AWARDS: \$552,683



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

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, MD, PhD

Fred Hutchinson Cancer Research Center

Chemical genetic approach to reactivate the silenced MECP2 gene on the inactive X chromosome

\$140,000

2008