RETTE SYNDROME RESEARCH TRUST

AWARDS BY YEAR

/ TOTAL AWARDS $64 MILLION (2008-2021)
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.

### 2021

**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

**Citizen**
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

### 2020

**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

**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

*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.*
<table>
<thead>
<tr>
<th>Name</th>
<th>Institution</th>
<th>Project Description</th>
<th>Award Amount</th>
</tr>
</thead>
<tbody>
<tr>
<td>Adrian Bird, PhD / Michael Greenberg, PhD / Gail Mandel, PhD</td>
<td>University of Edinburgh / Harvard University / Oregon Health and Sciences University</td>
<td>MECP2 Consortium</td>
<td>$3,359,054</td>
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<tr>
<td>James Wilson, MD, PhD</td>
<td>University of Pennsylvania</td>
<td>MECP2 gene therapy for Rett Syndrome</td>
<td>$765,607</td>
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<td>Stuart Cobb, PhD / Chris Sibley, PhD</td>
<td>University of Edinburgh</td>
<td>RNA trans-splicing therapy in Rett Syndrome</td>
<td>$37,999</td>
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<tr>
<td>Harvard Stem Cell Institute</td>
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<td>Support for development of patient derived induced pluripotent stem cell lines</td>
<td>$101,912</td>
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<tr>
<td>Michael Elowitz, PhD</td>
<td>California Institute of Technology</td>
<td>A system for dosage-independent control of MECP2 expression in Rett Syndrome gene therapy</td>
<td>$212,374</td>
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<td>Peter Glazer, PhD / Mark Saltzman PhD</td>
<td>Yale University</td>
<td>PNA nanoparticles for gene editing of Rett Syndrome</td>
<td>$275,000</td>
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<tr>
<td>Alanna Schepartz, PhD</td>
<td>Yale University</td>
<td>Evaluating cell-permeant miniature proteins (CPMPs) as a strategy for delivering functional MECP2 into model cells and neurons</td>
<td>$297,716</td>
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<td>Joost Gribnau, PhD</td>
<td>Erasmus Medical Center</td>
<td>Human in vitro models for X chromosome reactivation</td>
<td>$401,000</td>
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<td>Antonio Bedalov, PhD</td>
<td>Fred Hutchinson Cancer Research Center</td>
<td>Mouse model maintenance</td>
<td>$20,000</td>
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<tr>
<td>Thorsten Stafforst, PhD</td>
<td>University of Tubingen</td>
<td>RNA editing for MECP2 mutations via RESTORE</td>
<td>$359,856</td>
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<tr>
<td>Joseph Jacobson, PhD</td>
<td>Massachusetts Institute of Technology</td>
<td>Correction of MECP2 mutations with engineered ScCas 9 base editors</td>
<td>$50,000</td>
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<tr>
<td>The Jackson Laboratory</td>
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<td>Generation and phenotypic assessment of mouse models for Rett Syndrome</td>
<td>$471,690</td>
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<tr>
<td>Coriell Institute</td>
<td></td>
<td>Rett Syndrome biorepository</td>
<td>$135,000</td>
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<tr>
<td>Emerald Innovations</td>
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<td>Passive monitoring of Rett patients with Emerald</td>
<td>$164,670</td>
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<tr>
<td>Beth McCormick, PhD</td>
<td>University of Massachusetts Medical School</td>
<td>Microbiome study for the advancement of novel nutritional supplements</td>
<td>$520,316</td>
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<tr>
<td>Sasha Djukic, MD, PhD</td>
<td>Albert Einstein School of Medicine</td>
<td>Support for continuing work at the Rett Syndrome Center</td>
<td>$75,000</td>
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<tr>
<td>Miscellaneous Pilot Studies</td>
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<td>$135,522</td>
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<tr>
<td>Ronald Cohn, PhD</td>
<td>The Hospital for Sick Children</td>
<td>Interrogation of genome editing strategies as a therapeutic modality for MECP2 Duplication Syndrome</td>
<td>$570,000</td>
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<tr>
<td>Anastasia Khvorova, PhD</td>
<td>University of Massachusetts Medical School</td>
<td>Development of siRNA based compounds to potently silence MECP2 towards the treatment of MECP2 Duplication Syndrome</td>
<td>$435,515</td>
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### 2018

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

**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*  
$351,022

**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

**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

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

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### 2017

**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

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**TOTAL AWARDS $6,166,762**
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<tr>
<th>Name</th>
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<th>Funding</th>
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<tbody>
<tr>
<td>Katherin Meyer, PhD</td>
<td>Nationwide Children's Hospital</td>
<td>A gene therapy consortium to develop and evaluate gene therapy approaches in Rett Syndrome</td>
<td>$68,515</td>
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<tr>
<td>Stuart Cobb, PhD</td>
<td>University of Glasgow</td>
<td>Additional support for RNA-trans splicing efforts in Rett Syndrome</td>
<td>$280,000</td>
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<tr>
<td>Rudolf Jaenisch, MD</td>
<td>Whitehead Institute for Biomedical Research</td>
<td>Reactivation of MECP2 with epigenome editing tools to rescue Rett Syndrome</td>
<td>$599,850</td>
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<tr>
<td>Benjamin Philpot, PhD</td>
<td>University of North Carolina Chapel Hill</td>
<td>Pilot study for reactivation of silenced MECP2 by artificial transcription factors</td>
<td>$145,443</td>
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<td>Q State Biosciences</td>
<td>Development of an in-vitro cell system for discovering and evaluating the effects of therapeutic candidates on neurons produced using Rett patient IPS cells</td>
<td>$498,141</td>
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<tr>
<td>Michael Greenberg, PhD</td>
<td>Harvard University</td>
<td>Development of an in-vitro cell system for discovering and evaluating the effects of therapeutic candidates on neurons produced using Rett patient IPS cells</td>
<td>$55,826</td>
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<td>Clinical Trial Consortium</td>
<td>Daniel Tarquinio, DO</td>
<td>Center for Rare Neurological Diseases</td>
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2016

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

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<td>Adrian Bird, PhD / Michael Greenberg, PhD / Gail Mandel, PhD</td>
<td>University of Edinburgh / Harvard University / Oregon Health and Sciences University</td>
<td>MECP2 Consortium</td>
<td>$3,454,921</td>
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<tr>
<td>Stuart Cobb, PhD / Steve Gray, PhD / Brian Kaspar, PhD / Gail Mandel, PhD / Alysson Muotri, PhD</td>
<td>University of Glasgow / University of North Carolina Chapel Hill / Nationwide Children's Hospital / Oregon Health and Science University / University of California San Diego</td>
<td>A gene therapy consortium to develop and evaluate gene therapy approaches in Rett Syndrome</td>
<td>$1,450,275</td>
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<tr>
<td>Stuart Cobb, PhD</td>
<td>University of Glasgow</td>
<td>Scientific support for gene therapy, splicing therapy and protein therapy programmes in Rett Syndrome</td>
<td>$210,000</td>
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<tr>
<td>Stuart Cobb, PhD</td>
<td>University of Glasgow</td>
<td>Optimizing MECP2 trans-splicing for human translation</td>
<td>$330,804</td>
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<tr>
<td>Alysson Muotri</td>
<td>University of California San Diego</td>
<td>A drug-screening platform using MECP2-deficient human neurons and preclinical testing</td>
<td>$1,001,000</td>
</tr>
</tbody>
</table>

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
Antonio Bedalov, PhD  
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

2015

Total Awards $8,731,782

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

Alysson Muotri  
University of California San Diego  
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$12,500

David Katz  
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$250,000

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

Alysson Muotri  
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*Role of an autism-related cytokine in a genetic model of ASD*  
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Joost Gribnau, PhD  
Erasmus MC  
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*Clinical development of NLX-101 in Rett Syndrome*  
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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  
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*Exploration of the impact of 2-hydroxypropyl-B-cyclodextrin treatment on lifespan and brain cholesterol metabolism in male mecp2 deficient mice*  
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David Katz, PhD  
Case Western Reserve University  
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$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

2014

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
$280,000

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

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

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

TOTAL AWARDS $5,809,107
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<thead>
<tr>
<th>Project Title</th>
<th>Principal Investigator(s)</th>
<th>Award Amount</th>
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<tr>
<td>Outlining the automatic signature of Rett Syndrome</td>
<td>Debra Weese-Mayer, MD / Michael Carroll, PhD Lurie Children’s Hospital of Chicago</td>
<td>$157,300</td>
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<tr>
<td>Determine the proteome, secretome and transcript changes in astrocytes derived from human Rett patients iPSCs and their effect on interaction with human neurons</td>
<td>Nurit Ballas, PhD Stony Brook University</td>
<td>$20,000</td>
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<tr>
<td>MicroRNA biomarkers in Rett Syndrome</td>
<td>DiamiR</td>
<td>$6,768</td>
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<tr>
<td>Support for continuing work at the Rett Syndrome Center</td>
<td>Sasha Djukic, MD, PhD Albert Einstein College of Medicine</td>
<td>$140,161</td>
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<tr>
<td>Exploration of the impact of 2-hydroxypropyl-β-cyclodextrin treatment on lifespan and brain cholesterol metabolism in male mecp2 deficient mice</td>
<td>Stephen Turley, PhD University of Texas Southwestern Medical Center</td>
<td>$20,000</td>
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<tr>
<td>High content phenotypic screening of existing drugs for the treatment of Rett Syndrome</td>
<td>Recursion Pharmaceuticals</td>
<td>$25,000</td>
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<tr>
<td>Expression of nuclear MeCP2 is dependent on neuronal stimulation and application of IGF1</td>
<td>Daniela Tropea, PhD Trinity College Dublin</td>
<td>$13,000</td>
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<tr>
<td>Antisense oligonucleotide therapy for the treatment of MECP2 Duplication Syndrome</td>
<td>Huda Zoghbi, MD, PhD Baylor College of Medicine</td>
<td>$230,000</td>
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<tr>
<td>A forward genetic screen to identify druggable modulators of MECP2 levels</td>
<td>Huda Zoghbi, MD, PhD Baylor College of Medicine</td>
<td>$414,065</td>
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<tr>
<td>Testing drugs that modulate X chromosome inactivation to reactivate the silent MECP2</td>
<td>Adrian Bird, PhD / Michael Greenberg, PhD / Gail Mandel, PhD University of Edinburgh / Harvard University / Oregon Health and Sciences University MECP2 Consortium</td>
<td>$3,417,575</td>
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<tr>
<td>Preclinical testing of selective novel NMDA receptor modulators</td>
<td>Michela Fagiolini, PhD Boston Children’s Hospital</td>
<td>$126,741</td>
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<td>mGluR5 dependent synaptic protein synthesis in Rett Syndrome</td>
<td>Mark Bear Massachusetts Institute of Technology</td>
<td>$45,943</td>
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<td>Copaxone clinical trial</td>
<td>Bruria Ben Zeev, MD Sheba Medical Center</td>
<td>$197,962</td>
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<tr>
<td>Copaxone clinical trial</td>
<td>Sasha Djukic, MD, PhD Albert Einstein College of Medicine</td>
<td>$412,370</td>
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<tr>
<td>Preclinical evaluation of therapeutics that modulate the NMDA pathway</td>
<td>Michael Green, PhD University of Massachusetts Medical School</td>
<td>$150,000</td>
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<tr>
<td>Preclinical evaluation of therapeutics that modulate the NMDA pathway</td>
<td>Jeannie Lee, PhD Massachusetts General Hospital / Harvard University An oligotherapeutics approach to treat Rett Syndrome</td>
<td>$100,000</td>
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<tr>
<td>Gene Therapy Consortium</td>
<td>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</td>
<td>$1,535,942</td>
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<tr>
<td>Preclinical testing of selective novel NMDA receptor modulators</td>
<td>$750,000</td>
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<td>mGluR5 dependent synaptic protein synthesis in Rett Syndrome</td>
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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

**2012**

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  
$59,842

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 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

**2011**

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

TOTAL AWARDS $4,235,266

TOTAL AWARDS $3,609,479
**Monica Justice, PhD**
Baylor College of Medicine  
*Identification of gene modifiers that ameliorate Rett Syndrome*  
$298,879

**Jonathan 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

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

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**Ronald Crystal, MD, PhD**
Weill Medical College of Cornell University  
*AAV mediated gene transfer for the treatment of Rett Syndrome*  
$805,121

**Brian Kaspar, PhD / Gail Mandel, PhD**
Nationwide Children’s Hospital / 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

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

**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

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**2010**

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**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

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

2008

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

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

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

TOTAL AWARDS $552,683

TOTAL AWARDS $2,278,000