RETT SYNDROME RESEARCH TRUST

AWARDS BY YEAR

TOTAL AWARDS $66 MILLION (2008-2022)
2022

**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  
$87,345

**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  
$87,345

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

**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

**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

**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 Pehliv, MD**  
Texas Children’s Hospital  
Clinical studies in MECP2 Duplication Syndrome as foundation for antisense oligonucleotide drug trials  
$125,000

**TOTAL AWARDS $2,073,337**
**2020**

**DSG**

*Development of the Rett Syndrome Global Registry*

$693,000

**James Wilson, MD, PhD**

University of Pennsylvania

*MECP2 gene therapy for Rett Syndrome*

$380,868

**Clinical Trial Consortium**

David Lieberman, MD, PhD

Boston Children's Hospital

$94,176

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**

**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*

$765,507

**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**

Albert Einstein College of Medicine

*Support for continuing work at the Rett Syndrome Center*

$25,000

**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**

*Support for development of patient derived induced pluripotent stem cell lines*

$101,912

**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

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

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

2018

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

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

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
$131,243

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

TOTAL AWARDS $9,956,283
2017

**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

**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*  
$280,000

**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 iPSC 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 iPSC 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

**TOTAL AWARDS $6,166,762**
2016

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

O 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

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
$786,854

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
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

The Jackson Laboratory  
*Development of mouse models*  
$42,052

Herman 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 Foxx, 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

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-β-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

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

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Stephen Turley, PhD / Adam Lopez, PhD  
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$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

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 Foxx, 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
<table>
<thead>
<tr>
<th>Name(s)</th>
<th>University/Institution</th>
<th>Project Description</th>
<th>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 Science University</td>
<td>MECP2 Consortium</td>
<td>$250,000</td>
</tr>
<tr>
<td>Ali Khoshnan, PhD / Sarkis Mazmanian, PhD</td>
<td>California Institute of Technology</td>
<td>Exploring the link between MECP2 and gut physiology to test a novel probiotic therapy for Rett Syndrome</td>
<td>$200,000</td>
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<tr>
<td>Lucas Pozzo-Miller, PhD</td>
<td>University of Alabama Birmingham</td>
<td>Testing whether LM22A-4 improves hippocampal function in female MECP2 heterozygous mice</td>
<td>$110,000</td>
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<tr>
<td>Neurolixsis</td>
<td></td>
<td>NLX-101 as a treatment for breathing disorders in Rett Syndrome</td>
<td>$54,945</td>
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<tr>
<td>Sung-Yon Kim, PhD</td>
<td>Life Science Research Foundation</td>
<td>Post doctoral fellowship</td>
<td>$91,500</td>
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<tr>
<td>Steven Gray, PhD</td>
<td>University of Texas Southwestern Medical Center</td>
<td>Supplement for gene therapy consortium</td>
<td>$87,401</td>
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<tr>
<td>Tom Frazier, PhD / David Katz, PhD / Daniel Sessler, MD, PhD</td>
<td>Case Western Reserve University / Cleveland Clinic</td>
<td>Low-dose ketamine for the treatment of Rett Syndrome</td>
<td>$1,295,131</td>
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<tr>
<td>Sasha Djukic, MD, PhD</td>
<td>Albert Einstein College of Medicine</td>
<td>Pharmacological treatment of Rett Syndrome with Lovastatin</td>
<td>$403,000</td>
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<tr>
<td>Sasha Djukic, MD, PhD</td>
<td>Albert Einstein College of Medicine</td>
<td>Supplement for copaxone clinical trial</td>
<td>$47,000</td>
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<tr>
<td>Debra Weese-Mayer, MD / Michael Carroll, PhD</td>
<td>Lurie Children's Hospital of Chicago</td>
<td>Outlining the automatic signature of Rett Syndrome</td>
<td>$157,300</td>
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<tr>
<td>Nurit Ballas, PhD</td>
<td>Stony Brook University</td>
<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>$20,000</td>
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<tr>
<td>DamiR</td>
<td></td>
<td>miRNA biomarkers in Rett Syndrome</td>
<td>$6,768</td>
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<tr>
<td>Sasha Djukic, MD, PhD</td>
<td>Albert Einstein College of Medicine</td>
<td>Support for continuing work at the Rett Syndrome Center</td>
<td>$140,161</td>
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<tr>
<td>Stephen Turley, PhD</td>
<td>University of Texas Southwestern Medical Center</td>
<td>Exploration of the impact of 2-hydroxypropyl-B-cyclodextrin treatment on lifespan and brain cholesterol metabolism in male mecp2 deficient mice</td>
<td>$20,000</td>
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<tr>
<td>Recursion Pharmaceuticals</td>
<td></td>
<td>High content phenotypic screening of existing drugs for the treatment of Rett Syndrome</td>
<td>$25,000</td>
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<tr>
<td>Daniela Tropea, PhD</td>
<td>Trinity College Dublin</td>
<td>Expression of nuclear MeCP2 is dependent on neuronal stimulation and application of IGF1</td>
<td>$13,000</td>
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<tr>
<td>Miscellaneous Pilot Projects</td>
<td></td>
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<td>$7,000</td>
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<tr>
<td>Huda Zoghbi, MD, PhD</td>
<td>Baylor College of Medicine</td>
<td>A forward genetic screen to identify druggable modulators of MECP2 levels</td>
<td>$414,065</td>
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<tr>
<td>Huda Zoghbi, MD, PhD</td>
<td>Baylor College of Medicine</td>
<td>Antisense oligonucleotide therapy for the treatment of MECP2 Duplication Syndrome</td>
<td>$230,000</td>
</tr>
</tbody>
</table>
### 2013

**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 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

**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*  
$319,224

**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

**TOTAL AWARDS $7,167,097**
<table>
<thead>
<tr>
<th>Name</th>
<th>Institution(s)</th>
<th>Funding Amount</th>
<th>Description</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 MECP2 Consortium</td>
<td>$1,840,441</td>
<td>Investigating novel therapeutic approaches for Rett Syndrome</td>
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<tr>
<td>Huda Zoghbi, MD, PhD</td>
<td>Baylor College of Medicine</td>
<td>$517,054</td>
<td>Identification of gene modifiers that ameliorate Rett Syndrome</td>
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<tr>
<td>Monica Justice, PhD</td>
<td>Baylor College of Medicine</td>
<td>$298,879</td>
<td>Immune modulation as a new therapeutic approach for Rett Syndrome</td>
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<td>Jonthan Kipnis, PhD</td>
<td>University of Virginia</td>
<td>$440,000</td>
<td>A high-throughput screen to identify compounds that reactivate the functional MECP2 allele in Rett Syndrome</td>
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<tr>
<td>Jeannie Lee, PhD</td>
<td>Massachusetts General Hospital / Harvard University</td>
<td>$300,000</td>
<td>MECP2 testing</td>
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<tr>
<td>Mark Bear, PhD</td>
<td>Massachusetts Institute of Technology</td>
<td>$85,896</td>
<td>mGluR5 dependent synaptic protein synthesis in Rett Syndrome</td>
</tr>
<tr>
<td>Jeffrey Macklis, PhD</td>
<td>Harvard University</td>
<td>$35,352</td>
<td>Vitamin D therapy for MECP2 target Irak1/NFkB dysregulation</td>
</tr>
<tr>
<td>Sasha Djukic, MD, PhD</td>
<td>Albert Einstein College of Medicine</td>
<td>$66,710</td>
<td>Support for ongoing work at Rett Syndrome Center</td>
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<tr>
<td>Benjamin Philpot, PhD</td>
<td>University of North Carolina Chapel Hill</td>
<td>$10,000</td>
<td></td>
</tr>
<tr>
<td>John Bissonnette, PhD</td>
<td>Oregon Health and Sciences University</td>
<td>$187,000</td>
<td>Immune modulation as a new therapeutic approach for Rett Syndrome</td>
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</tbody>
</table>
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

**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