



  
rett syndrome  
research trust

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# RETT SYNDROME RESEARCH TRUST AWARDS BY YEAR

✓ TOTAL AWARDS **\$66 MILLION** (2008-2022)

# 2022

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

## **Emerald Innovations**

*Passive monitoring of Rett patients with Emerald*

**\$1,106,237**

## **Shawn Liu, PhD**

Columbia University

*Multiplex Epigenome Editing to Reactivate and Maintain MECP2 in RTT Neurons*

**\$482,877**

## **Herophilus**

*Evaluation of MECP2 Reactivating Effects of Herophilus Lead Small Molecules*

**\$200,000**

## **David Lieberman, MD, PhD**

Boston Children's Hospital Rett Clinic

**\$67,345**

## **Samir Mitragotri, PhD**

Harvard University

*Pilot Study to Explore Novel Delivery Technology*

**\$50,000**

## **John Foxe, PhD**

University of Rochester

*From sensory-perceptual representations to cognitive processing in Rett Syndrome*

**\$36,690**

## **Coriell Institute**

*Rett Syndrome biorepository*

**\$119,461**

## **Harvard Stem Cell Institute**

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

**\$10,727**

# 2021

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

## **Antonio Bedalov / Kyle Fink**

Fred Hutchinson Cancer Research Institute / University of California Davis

*Reactivation of MECP2*

**\$1,090,919**

## **Victor Faundez, PhD**

Emory University

*Systems Biology of Rett Syndrome Gene Therapy Outcomes*

**\$584,304**

## **Ciitizen**

Digital Natural History Study

**\$444,000**

## **Joseph Anderson, PhD**

University of California Davis Medical Center

*Feasibility of a stem cell gene therapy approach for the treatment of Rett Syndrome*

**\$186,254**

## **Joni N. Saby, PhD / Eric D. Marsh, MD, PhD**

Children's Hospital of Philadelphia (CHOP)

*Electrophysiological (EEG) Outcome Measures for Rett Syndrome Clinical Trials*

**\$115,906**

## **David Lieberman, MD, PhD**

Boston Children's Hospital

*Clinical Trial Consortium*

**\$67,821**

## **Stuart Cobb, PhD**

University of Edinburgh

*Genetic Analysis of the Rett Syndrome Cerebrospinal Fluid Proteome*

**\$47,014**

## **Coriell Institute**

*Rett Syndrome biorepository*

**\$53,612**

## **Harvard Stem Cell Institute**

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

**\$36,343**

## **The Jackson Laboratory**

*Generation and phenotypic assessment of mouse models for Rett Syndrome*

**\$5,620** (additional support)

## **Bryce Reeve, PhD**

Duke University School of Medicine

*Development of the Observer-Reported Communication Ability (ORCA) for Rett Syndrome*

**\$15,294**

## **Sasha Djukic, MD, PhD**

Albert Einstein College of Medicine

*Support for continuing work at the Rett Syndrome Center*

**\$25,000**

## **The Jackson Laboratory**

*Testing of siRNA compounds from Khvorova lab for MECP2 Duplication Syndrome*

**\$362,930**

## **Davut Pehlivan, MD**

Texas Children's Hospital

*Clinical studies in MECP2 Duplication Syndrome as foundation for antisense oligonucleotide drug trials*

**\$125,000**

# 2020

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

## DSG

*Development of the Rett Syndrome Global Registry*

**\$693,000**

## James Wilson, MD, PhD

University of 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**

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

# 2019

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

## Adrian Bird, PhD / Michael Greenberg, PhD / Gail Mandel, PhD

University of Edinburgh / Harvard University /  
Oregon Health and Sciences University  
*MECP2 Consortium*

**\$3,359,054**

## James Wilson, MD, PhD

University of Pennsylvania  
*MECP2 gene therapy for Rett Syndrome*

**\$765,607**

## James Wilson, MD, PhD

University of Pennsylvania  
*MECP2 gene therapy for Rett Syndrome, vector production*

**\$37,999**

## Stuart Cobb, PhD / Chris Sibley, PhD

University of Edinburgh  
*RNA trans-splicing therapy in Rett Syndrome*

**\$235,950**

## Harvard Stem Cell Institute

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

**\$101,912**

## Michael Elowitz, PhD

California Institute of Technology  
*A system for dosage-independent control of MECP2 expression in Rett Syndrome gene therapy*

**\$212,374**

## Peter Glazer, PhD / Mark Saltzman PhD

Yale University  
*PNA nanoparticles for gene editing of Rett Syndrome*

**\$275,000**

## Alanna Schepartz, PhD

Yale University  
*Evaluating cell-permeant miniature proteins (CPMPs) as a strategy for delivering functional MECP2 into model cells and neurons*

**\$297,716**

## Joost Gribnau, PhD

Erasmus Medical Center  
*Human in vitro models for X chromosome reactivation*

**\$401,000**

## Antonio Bedalov, PhD

Fred Hutchinson Cancer Research Center  
*Mouse model maintenance*

**\$20,000**

**Thorsten Stafforst, PhD**

University of Tübingen  
*RNA editing for MECP2 mutations via RESTORE*  
**\$359,856**

**Joseph Jacobson, PhD**

Massachusetts Institute of Technology  
*Correction of MECP2 mutations with engineered ScCas 9 base editors*  
**\$50,000**

**The Jackson Laboratory**

*Generation and phenotypic assessment of mouse models for Rett Syndrome*  
**\$417,690**

**Coriell Institute**

*Rett Syndrome biorepository*  
**\$135,000**

**Emerald Innovations**

*Passive monitoring of Rett patients with Emerald*  
**\$164,670**

**Beth McCormick, PhD**

University of Massachusetts Medical School  
*Microbiome study for the advancement of novel nutritional supplements*  
**\$520,316**

**Sasha Djukic, MD, PhD**

Albert Einstein School of Medicine  
*Support for continuing work at the Rett Syndrome Center*  
**\$75,000**

**Miscellaneous Pilot Studies**

**\$135,522**

**Ronald Cohn, PhD**

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

**Anastasia Khvorova, PhD**

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

# 2018

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

**Jonathan Watts, PhD / Scot Wolfe, PhD / Eric Sontheimer, PhD / Anastasia Khvorova, PhD**

University of Massachusetts Medical School  
*RNA and genome editing for treatment of Rett Syndrome*  
**\$2,403,735**

**Guoping Feng, PhD / Feng Zhang, PhD / Robert Desimone, PhD**

Massachusetts Institute of Technology / Broad Institute / Harvard University  
*RNA-editing as a gene therapy approach for Rett Syndrome*  
**\$2,332,000**

**Beam Therapeutics**

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

**John Sinnamon, PhD**

Oregon Health and Science University  
*New editing enzymes for RNA*  
**\$345,000**

**Peter Beal, PhD**

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

**Stuart Cobb, PhD / Adrian Bird, PhD**

University of Edinburgh  
*Gene Therapy Consortium 2.0*  
**\$653,856**

**Stuart Cobb, PhD**

University of Edinburgh  
*Purchase of qPCR machine*  
**\$13,945**

**Andrea Cerase, PhD**

Queen Mary University of London  
*Reactivation of MECP2 and CDKL5 genes by functional deactivation of Xist RNA*  
**\$351,022**

**James Wilson, MD, PhD**

University of Pennsylvania  
*Gene Therapy Consortium Vector Core*  
**\$131,243**

**Allan Jacobson, PhD / Jonathan Watts, PhD**

University of Massachusetts Medical School  
*Read-through of premature termination codons for treatment of Rett Syndrome*  
**\$323,000**

### Antonio Bedalov

Fred Hutchinson Cancer Research Institute  
*Reactivation of MECP2*

**\$38,000**

### Clinical Trial Consortium

David Lieberman, MD, PhD  
Boston Children's Hospital

**\$74,792**

### Laurel Joy Gabard-Durnam, PhD

Harvard University  
*Post Doctoral Fellowship, Autism Science Foundation*

**\$17,500**

### Hassan Ghasemzadeh, PhD

Washington State University  
*Pilot study to examine gait patterns in Rett Syndrome*

**\$10,000**

### Sasha Djukic, MD, PhD

Albert Einstein College of Medicine  
*Support for continuing work at the Rett Syndrome Center*

**\$75,000**

### Huda Zoghbi, MD, PhD

Baylor College of Medicine  
*A forward genetic screen to identify druggable modulators of MECP2 levels*

**\$752,660**

# 2017

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

### James Wilson, MD, PhD

University of Pennsylvania  
*Gene therapy consortium*

**\$1,585,886**

### Katherin Meyer, PhD

Nationwide Children's Hospital  
*Optimizing gene therapy for Rett Syndrome*

**\$152,489**

### Katherin Meyer, PhD

Nationwide Children's Hospital  
*A gene therapy consortium to develop and evaluate gene therapy approaches in Rett Syndrome*

**\$68,515**

### Stuart Cobb, PhD

University of Glasgow  
*Additional support for RNA-trans splicing efforts in Rett Syndrome*

**\$290,000**

### Rudolf Jaenisch, MD

Whitehead Institute for Biomedical Research  
*Reactivation of MECP2 with epigenome editing tools to rescue Rett Syndrome*

**\$599,850**

### Benjamin Philpot, PhD

University of North Carolina Chapel Hill  
*Pilot study for reactivation of silenced MECP2 by artificial transcription factors*

**\$145,443**

### Q State Biosciences

*Development of an in-vitro cell system for discovering and evaluating the effects of therapeutic candidates on neurons produced using Rett patient iPS cells*

**\$498,141**

### Michael Greenberg, PhD

Harvard University  
*Development of an in-vitro cell system for discovering and evaluating the effects of therapeutic candidates on neurons produced using Rett patient iPS cells*

**\$55,826**

### Clinical Trial Consortium

Daniel Tarquinio, DO  
Center for Rare Neurological Diseases

**\$495,000**

### Clinical Trial Consortium

David Lieberman, MD, PhD  
Boston Children's Hospital

**\$395,000**

### Clinical Trial Consortium

Eric Marsh, MD, PhD  
Children's Hospital of Philadelphia

**\$487,715**

### Clinical Trial Consortium

Alan Percy, MD, PhD  
University of Alabama Birmingham

**\$495,000**

### Clinical Trial Consortium

Jeffrey Neul, MD, PhD  
Vanderbilt University Medical Center

**\$495,000**

### Sasha Djukic, MD, PhD

Albert Einstein College of Medicine  
*Support for continuing work at the Rett Syndrome Center*

**\$103,000**

### Huda Zoghbi, MD

Baylor College of Medicine  
*Investigating the potential of antisense oligonucleotide therapy for MECP2 Duplication Syndrome*

**\$299,897**



# 2016

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

## **Adrian Bird, PhD / Michael Greenberg, PhD / Gail Mandel, PhD**

University of Edinburgh / Harvard University / Oregon Health and Sciences University  
MECP2 Consortium  
**\$3,454,921**

## **Stuart Cobb, PhD / Steve Gray, PhD / Brian Kaspar, PhD / Gail Mandel, PhD / Alysson Muotri, PhD**

University of Glasgow / University of North Carolina Chapel Hill / Nationwide Children's Hospital / Oregon Health and Science University / University of California San Diego  
A gene therapy consortium to develop and evaluate gene therapy approaches in Rett Syndrome  
**\$1,450,275**

## **Stuart Cobb, PhD**

University of Glasgow  
Scientific support for gene therapy, splicing therapy and protein therapy programmes in Rett Syndrome  
**\$210,000**

## **Stuart Cobb, PhD**

University of Glasgow  
Optimizing MECP2 trans-splicing for human translation  
**\$330,804**

## **Alysson Muotri**

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

## **Alysson Muotri**

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

## **David Katz**

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

## **ArmaGen, Inc.**

Protein replacement for Rett Syndrome  
**\$125,000**

## **Rudolf Jaenisch, MD**

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

## **Michael Greenberg, PhD**

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

## **Q State Biosciences**

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

## **Miscellaneous Pilot Projects**

**\$33,838**

## **Sasha Djukic, MD, PhD**

Albert Einstein College of Medicine  
Support for continuing work at the Rett Syndrome Center  
**\$84,000**

# 2015

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

## **Antonio Bedalov, PhD**

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

## **Jeannie Lee, PhD**

Massachusetts General Hospital / Harvard University  
Treating Rett Syndrome by targeting the Xist interactome  
**\$766,854**

## **Joost Gribnau, PhD**

Erasmus MC  
In vivo and in vitro models for X chromosome reactivation.  
**\$177,900**

## **Neurolix, 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- $\beta$ -cyclodextrin treatment on lifespan and brain cholesterol metabolism in male mecp2 deficient mice***\$156,180****Miscellaneous Pilot Studies****\$20,000****DiamiR***microRNA biomarkers in Rett Syndrome***\$26,815****David Katz, PhD**

Case Western Reserve University

*Preclinical Studies of LM22A-4 in Mouse Models of Rett Syndrome***\$14,154****The Jackson Laboratory***Development of mouse models***\$42,052****Hermano Igo Krebs, PhD**

Massachusetts Institute of Technology

*Pilot Study***\$8,000****Tim Benke, PhD / Aleksandra Djukic, PhD / Alan Percy, PhD / Daniel Tarquinio, PhD**

Children's Hospital Colorado / Montefiore Medical Center /

University of Alabama Birmingham / Children's Healthcare of Atlanta

*Outcome measures and biomarkers development***\$4,500,000****Michele Fagiolini**

Boston Children's Hospital

*Testing NR2A and NR2B NAMs in mouse models of Rett Syndrome.***\$337,336****John Foxe, PhD / Sophie Molholm, PhD**

University of Rochester / Albert Einstein College of Medicine

*From sensory-perceptual representations to cognitive processing in Rett Syndrome***\$533,607****Sasha Djukic, MD, PhD**

Albert Einstein College of Medicine

*Support for continuing work at the Rett Syndrome Center***\$88,000****2014****TOTAL AWARDS \$5,809,107****Monica Justice, PhD**

University of Toronto

*Identifying genetic modifiers of MECP2 in the mouse***\$715,680****Jeffery Neul, MD, PhD**

Baylor College of Medicine

*Identification of genetic modifiers in Rett Syndrome***\$314,456****Jeannie Lee, PhD**

Massachusetts General Hospital / Harvard University

*Re-awakening the silenced normal MECP2 allele with small molecules to treat Rett Syndrome***\$465,000****Antonio Bedalov, PhD**

Fred Hutchinson Cancer Research Center

*Chemical genetic approach to reactivate the silenced MECP2 gene on the inactive X chromosome***\$290,000****Terry Magnuson, PhD**

University of North Carolina, Chapel Hill

*Systems genetics approach toward understanding regulation of MECP2 expression***\$200,000****David Katz, PhD**

Case Western Reserve University

*Preclinical studies of LM22A-4 in mouse models of Rett Syndrome***\$271,700**

**Adrian Bird, PhD / Michael Greenberg, PhD /  
Gail Mandel, PhD**

University of Edinburgh / Harvard University / Oregon  
Health and Science University  
MECP2 Consortium

**\$250,000**

**Ali Khoshnan, PhD / Sarkis Mazmanian, PhD**

California Institute of Technology  
Exploring the link between MECP2 and gut physiology to test  
a novel probiotic therapy for Rett Syndrome

**\$200,000**

**Lucas Pozzo-Miller, PhD**

University of Alabama Birmingham  
Testing whether LM22A-4 improves hippocampal function  
in female MECP2 heterozygous mice

**\$110,000**

**Neurolix**

NLX-101 as a treatment for breathing disorders in Rett Syndrome

**\$54,945**

**Sung-Yon Kim, PhD**

Life Science Research Foundation  
Post doctoral fellowship

**\$91,500**

**Steven Gray, PhD**

University of Texas Southwestern Medical Center  
Supplement for gene therapy consortium

**\$67,401**

**Tom Frazier, PhD / David Katz, PhD /  
Daniel Sessler, MD, PhD**

Case Western Reserve University / Cleveland Clinic  
Low-dose ketamine for the treatment of Rett Syndrome

**\$1,295,131**

**Sasha Djukic, MD, PhD**

Albert Einstein College of Medicine  
Pharmacological treatment of Rett Syndrome with Lovastatin

**\$403,000**

**Sasha Djukic, MD, PhD**

Albert Einstein College of Medicine  
Supplement for copaxone clinical trial

**\$47,000**

**Debra Weese-Mayer, MD / Michael Carroll, PhD**

Lurie Children's Hospital of Chicago  
Outlining the automatic signature of Rett Syndrome

**\$157,300**

**Nurit Ballas, PhD**

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

**\$20,000**

**DiamiR**

microRNA biomarkers in Rett Syndrome

**\$6,768**

**Sasha Djukic, MD, PhD**

Albert Einstein College of Medicine  
Support for continuing work at the Rett Syndrome Center

**\$140,161**

**Stephen Turley, PhD**

University of Texas Southwestern Medical Center  
Exploration of the impact of 2-hydroxypropyl- $\beta$ -cyclodextrin treatment on  
lifespan and brain cholesterol metabolism in male mecp2 deficient mice

**\$20,000**

**Recursion Pharmaceuticals**

High content phenotypic screening of existing drugs for the  
treatment of Rett Syndrome

**\$25,000**

**Daniela Tropea, PhD**

Trinity College Dublin  
Expression of nuclear MeCP2 is dependent on neuronal  
stimulation and application of IGF1

**\$13,000**

**Miscellaneous Pilot Projects**

**\$7,000**

**Huda Zoghbi, MD, PhD**

Baylor College of Medicine  
A forward genetic screen to identify druggable modulators  
of MECP2 levels

**\$414,065**

**Huda Zoghbi, MD, PhD**

Baylor College of Medicine  
Antisense oligonucleotide therapy for the treatment of MECP2  
Duplication Syndrome

**\$230,000**



# 2013

TOTAL AWARDS \$7,167,097

## Adrian Bird, PhD / Michael Greenberg, PhD / Gail Mandel, PhD

University of Edinburgh / Harvard University / Oregon Health and Sciences University  
MECP2 Consortium

**\$3,417,575**

## Stuart Cobb, PhD / Steven Gray, PhD / Brian Kaspar, PhD / Gail Mandel, PhD

University of Glasgow / University of North Carolina Chapel Hill /  
Nationwide Children's Hospital / Oregon Health and Sciences University  
Gene Therapy Consortium

**\$1,535,942**

## Michael Green, PhD

University of Massachusetts Medical School  
Testing drugs that modulate X chromosome inactivation to reactivate  
the silent MECP2

**\$750,000**

## David Katz, PhD

Case Western Reserve University  
Preclinical evaluation of therapeutics that modulate the NMDA pathway

**\$150,000**

## Jeannie Lee, PhD

Massachusetts General Hospital / Harvard University  
An oligotherapeutics approach to treat Rett Syndrome

**\$100,000**

## Michela Fagiolini, PhD

Boston Children's Hospital  
Preclinical testing of selective novel NMDA receptor modulators

**\$126,741**

## Mark Bear

Massachusetts Institute of Technology  
*mGluR5 dependent synaptic protein synthesis in Rett Syndrome*

**\$45,943**

## Bruria Ben Zeev, MD

Sheba Medical Center  
*Copaxone clinical trial*

**\$197,962**

## Sasha Djukic, MD, PhD

Albert Einstein College of Medicine  
*Copaxone clinical trial*

**\$412,370**

## Sasha Djukic, MD, PhD

Albert Einstein College of Medicine  
*Support for ongoing work at Rett Syndrome Center*

**\$72,000**

## Huda Zoghbi, MD, PhD

Baylor College of Medicine  
*A forward genetic screen to identify druggable modulators of MeCP2 levels*

**\$319,224**

## Kevin Foust, PhD

Nationwide Children's Hospital  
*RNA interference for the treatment of MECP2 Duplication Syndrome*

**\$39,340**

# 2012

TOTAL AWARDS \$4,235,266

## Benjamin Philpot, PhD

University of North Carolina Chapel Hill  
*A chemical genetic approach for activating the dormant gene  
associated with Rett Syndrome*

**\$2,204,800**

## Jonathan Kipnis, PhD

University of Virginia  
*Immune modulation as a new therapeutic approach for Rett Syndrome*

**\$720,000**

## John Bissonnette, PhD

Oregon Health and Sciences University  
*Respiration in MECP2 deficient mice*

**\$59,642**

## Antonio Bedalov, PhD

Fred Hutchinson Cancer Research Center  
*Chemical genetic approach to reactivate the silenced MECP2  
gene on the inactive X chromosome*

**\$55,688**

## Andrew Pieper MD, PhD

University of Texas Southwestern Medical Center  
*In vivo identification of pharmacological agents for the treatment  
of Rett Syndrome*

**\$69,000**

## Monica Justice, PhD

Baylor College of Medicine  
*Identification of gene modifiers that ameliorate Rett Syndrome*

**\$757,165**

**Jay Shapiro, MD, PhD**

Kennedy Krieger Institute

*Treatment of osteoporosis in murine Rett Syndrome models***\$20,000****Sasha Djukic, MD, PhD**

Albert Einstein College of Medicine

*Support for ongoing work 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

TOTAL AWARDS **\$3,609,479****Adrian Bird, PhD / Michael Greenberg, PhD / Gail Mandel, PhD**

University of Edinburgh / Harvard University / Oregon Health and Sciences University

*MECP2 Consortium***\$1,840,441****Huda Zoghbi, MD, PhD**

Baylor College of Medicine

*Investigating novel therapeutic approaches for Rett Syndrome***\$517,054****Monica Justice, PhD**

Baylor College of Medicine

*Identification of gene modifiers that ameliorate Rett Syndrome***\$298,879****Jonthan Kipnis, PhD**

University of Virginia

*Immune modulation as a new therapeutic approach for Rett Syndrome***\$440,000****Jeannie Lee, PhD**

Massachusetts General Hospital / Harvard University

*A high-throughput screen to identify compounds that reactivate the functional MECP2 allele in Rett Syndrome***\$300,000****Mark Bear, PhD**

Massachusetts Institute of Technology

*mGluR5 dependent synaptic protein synthesis in Rett Syndrome***\$85,896****Jeffrey Macklis, PhD**

Harvard University

*Vitamin D therapy for MECP2 target Irak1/NFkB dysregulation***\$35,352****Sasha Djukic, MD, PhD**

Albert Einstein College of Medicine

*Support for ongoing work at Rett Syndrome Center***\$66,710****Benjamin Philpot, PhD**

University of North Carolina Chapel Hill

**\$10,000****John Bissonnette, PhD**

Oregon Health and Sciences University

*Respiration in MECP2 deficient mice***\$15,147**

# 2010

TOTAL AWARDS **\$1,322,052****Ronald Crystal, MD, PhD**

Weill Medical College of Cornell University

*AAV mediated gene transfer for the treatment of Rett Syndrome***\$605,121****Brian Kaspar, PhD / Gail Mandel, PhD**

Nationwide Children's 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****Jonthan Kipnis, PhD**

University of Virginia

*Immune modulation as a new therapeutic approach for Rett Syndrome***\$187,000**

### Huda Zoghbi, MD, PhD

Baylor College of Medicine

*Interventional trials in mice models of Rett Syndrome and MECP2 disorders*

**\$100,000**

### Marisa Bartolomei, PhD

University of Pennsylvania

*Analysis of epigenetic modifications of the MECP2 locus*

**\$41,255**

### Sasha Djukic, MD, PhD

Albert Einstein College of Medicine

*Support for ongoing work at Rett Syndrome Center*

**\$36,654**

### Rett Syndrome Clinic

University of Southern California

*Support for Rett Syndrome Clinic*

**\$22,022**

## 2009

TOTAL AWARDS **\$552,683**

### Monica Justice, PhD

Baylor College of Medicine

*Identification of gene modifiers that ameliorate Rett Syndrome*

**\$236,038**

### Stavros Lomvardas

University of California San Francisco

*Insight into MECP2 function raises therapeutic possibilities for Rett Syndrome*

**\$140,000**

### Huda Zoghbi, MD, PhD

Baylor College of Medicine

*Interventional trials in mice models of Rett Syndrome and MECP2 disorders*

**\$100,000**

### Marisa Bartolomei, PhD

University of Pennsylvania

*Analysis of epigenetic modifications of the MECP2 locus*

**\$40,000**

### Sasha Djukic, MD, PhD

Albert Einstein College of Medicine

*Support for continuing work at the Rett Syndrome Center*

**\$36,645**

## 2008

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

### Adrian Bird, PhD

Baylor College of Medicine

*Identification of gene modifiers that ameliorate Rett Syndrome*

**\$1,380,000**

### Andrew Pieper, MD, PhD

University of Texas Southwestern Medical Center

*In vivo identification of pharmacological agents for the treatment of Rett Syndrome*

**\$505,000**

### Monica Justice, PhD

Baylor College of Medicine

*Identification of gene modifiers that ameliorate Rett Syndrome*

**\$253,000**

### Antonio Bedalov, PhD

Fred Hutchinson Cancer Research Center

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

**\$140,000**