

# 10<sup>TH</sup> ANNUAL IRSF SCIENTIFIC SYMPOSIUM

## SUNDAY, JUNE 28, 2009

5:00 – 6:00pm	<b>WELCOME RECEPTION</b> – Our Presenting Sponsor: Optronics
6:00 – 7:15pm	<b>DINNER &amp; OPENING REMARKS</b> - Antony Horton, PhD, Chief Scientific Officer, International Rett Syndrome Foundation
7:25 – 7:30pm	<b>Meeting &amp; Session Overview</b> – David M. Katz, PhD, Case Western School of Medicine & Monica J. Justice, PhD, Baylor College of Medicine
<b>I. KEYNOTE ADDRESS: NATURE, NURTURE AND TUNING UP BRAIN CIRCUITS</b> <b>Keynote Speaker:</b> Carla Shatz, PhD, Stanford University	
7:30 – 8:30pm	
8:30 – 8:40pm	<b>Close Sunday Program</b> - David M. Katz, PhD, Case Western School of Medicine & Monica J. Justice, PhD, Baylor College of Medicine
8:40 – 9:40pm	<b>Cocktail Reception</b>

## MONDAY, JUNE 29, 2009

8:00 – 9:00am	<b>BREAKFAST – BURNHAM DINING ROOM</b>
<b>I. IDENTIFYING MeCP2 FUNCTION(S)</b> <b>Co-Chairs:</b> Huda Y. Zoghbi, MD, Baylor College of Medicine & Yi Sun, PhD, University of California – Los Angeles Our Presenting Sponsor: Optronics	
9:00 – 9:05pm	<b>Session Overview</b> – Huda Y. Zoghbi, MD, Baylor College of Medicine & Yi Sun, PhD, University of California – Los Angeles
9:05 – 9:30am	<b>Gene Regulation by MeCP2</b> Yi Sun, PhD, University of California – Los Angeles
9:30 – 9:55am	<b>Investigating the Role of MeCP2 in Neurons</b> Pete Skene, PhD, University of Edinburgh
9:55 – 10:10am	<b>MORNING BREAK</b>
10:10 – 10:25am	<b>The Interaction Between MeCP2 with the Nucleosome</b> Karolin Luger, PhD, Colorado State University
10:25 – 10:50am	<b>Cell Type-Specific Dysregulation of Cell Adhesion Genes in a Mouse Model of Rett Syndrome</b> Ken Sugino, PhD, Brandeis University
10:50 – 11:30am	<b>Discussion - Co-Chairs:</b> Huda Y. Zoghbi, MD, Baylor College of Medicine & Yi Sun, PhD, University of California – Los Angeles
11:30am – 12:30pm	<b>LUNCH</b>
<b>II. PATHOPHYSIOLOGY OF RETT SYNDROME</b> <b>Chair:</b> David M. Katz, PhD, Case Western School of Medicine	
12:30 – 12:35pm	<b>Session Overview</b> – David M. Katz, PhD, Case Western School of Medicine
12:35 – 1:00pm	<b>Using Embryonic Stem Cells to Study Neuronal Function and Dysfunction</b> Yves-Alain Barde, PhD, University of Basel
1:00 – 1:25pm	<b>Identification of new MecP2 Target Genes / Candidates in Developing Cerebral Cortex Interhemispheric Projection Neurons</b> Jeffrey D. Macklis, PhD, Massachusetts General Hospital
1:25 – 1:50pm	<b>Norepinephrine and Respiratory Dysfunction in Mouse Models of RTT</b> Jan-Marino Ramirez, PhD, Seattle Children's Hospital

1:50 – 2:15pm	<b>The Role of MeCP2 and HDACs in Regulating Synapse Function</b> Lisa Monteggia, PhD, University of Texas Southwestern Medical Center - Dallas
2:15 – 2:30pm	<b>AFTERNOON BREAK</b>
<b>III. PHENOTYPIC VARIABILITY AND GENETIC MODIFIERS OF DISEASE</b> <b>Chair:</b> Monica J. Justice, PhD, Baylor College of Medicine <b>Our Presenting Sponsor:</b> PsychoGenics, Inc.	
2:30 – 2:35pm	<b>Session Overview</b> – Monica J. Justice, PhD, Baylor College of Medicine
2:35 – 3:00pm	<b>A Screen for Genetic Suppressors of Rett Syndrome in the Mouse</b> Monica J. Justice, PhD, Baylor College of Medicine
3:00 – 3:25pm	<b>Transgenerational Epistasis: Interacting Genes in Different Generations in Mouse Models of Human Disease</b> Joseph Nadeau, PhD, Case Western School of Medicine
3:25 – 3:50pm	<b>Using Behavioral Phenotyping to Analyze Mouse Models of Human Disease</b> Richard Paylor, PhD, Baylor College of Medicine
3:50 – 4:20pm	<b>Discussion - Panelists:</b> Monica J. Justice, PhD, Baylor College of Medicine, Joseph Nadeau, PhD, Case Western School of Medicine & Richard Paylor, PhD, Baylor College of Medicine
5:00pm	<b>Close Monday Program</b>
5:00 – 6:00pm	<b>Cocktail Reception &amp; Poster Session</b>
6:00pm – 7:30pm	<b>Banquet Dinner /Family Address</b> – Chris Kolendrianos
7:30pm –	<b>Cocktail Reception</b>

TUESDAY, JUNE 30, 2009	
8:00 – 9:00am	<b>BREAKFAST – BURNHAM DINING ROOM</b>
<b>IV. TREATMENT STRATEGIES</b> <b>Co-Chairs:</b> John McCall, PhD, PharMac, LLC & Alan P. Kozikowski, PhD, University of Illinois, Chicago <b>Our Presenting Sponsor:</b> Optronics	
9:00 – 9:05am	<b>Session Overview</b> – John McCall, PhD, PharMac, LLC
9:05 – 9:30am	<b>Deficits in Synapse Maturation and their Therapeutic Reversal in a Mouse Model of Rett Syndrome</b> Mriganka Sur, PhD, Massachusetts Institute of Technology
9:30 – 10:00am	<b>The Role of Brain-Derived Neurotrophic Factor in HD</b> Chiara Zuccato, PhD, University of Milan
10:00 – 10:25am	<b>Suppression of Nonsense Mutations in Rett Syndrome by Aminoglycoside Antibiotics in an In Vitro System</b> Peter Huppke, MD, University of Göttingen
10:25 – 10:40am	<b>MORNING BREAK</b>
10:40am – 11:05pm	<b>EOS Lentivector Selection of Mouse and Human Rett Syndrome iPS Cells</b> James Ellis, PhD, University of Toronto
11:05 – 11:30pm	<b>Global Delivery of Adeno-Associated Virus (AAV) to the CNS: Implications for Rett Syndrome Gene Therapy</b> Steven Gray, PhD, University of North Carolina – Chapel Hill
11:30 – 12:00pm	<b>Discussion - Co-Chairs:</b> John McCall, PhD, PharMac, LLC & Alan P. Kozikowski, PhD, University of Illinois – Chicago
12:00 – 12:05pm	<b>Closing Remarks:</b> Antony Horton, PhD, Chief Scientific Officer, International Rett Syndrome Foundation
12:05pm	<b>End of Tuesday Program</b>