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**International Rett Syndrome Foundation**

### **IRSF Awards over \$650,000 for Translational Rett Syndrome Research**

Cincinnati, (OH) - The International Rett Syndrome Foundation (IRSF) announced today that it is awarding over \$650,000 to support eight cutting-edge projects that aim to accelerate translational research to develop treatments for Rett syndrome. IRSF is the world's largest private source of funding for biomedical and clinical Rett syndrome research. Since 1998, IRSF has cumulatively funded over \$24M in high quality, peer-reviewed basic and translational research grants that have significantly advanced Rett syndrome research towards finding a cure.

IRSF's Translational Research grant program includes two types of awards, which are the Help Accelerate Rett Therapeutics (HeART) and the Advanced Neurotherapeutic Grant of Excellence (ANGEL) awards. The funding from these grant awards will provide for early and late stage translational research to treat and reverse Rett syndrome (RTT).

The awarded projects from the first round of applications to the Translational Research grant program are geared towards drug discovery and development efforts as well as preparing for later stages of translational research. These funded projects fall into four main categories: (1) the development and testing of potential therapeutic compounds in animal models of Rett syndrome, (2) the development of outcome measures in humans that will be used in future clinical studies, (3) the development of a method for treatment, and (4) the development of a stem cell-based experimental system to be used in high-throughput drug screens. Together, they are in alignment with the objectives of IRSF's Translational Research grant program.

Stephen Bajardi, the executive director of IRSF, commented, "IRSF is pleased with the quality of these research projects and the significant steps they represent in moving Rett syndrome translational research forward."

### **New Translational Research Awards**

- **John M. Bissonnette, MD**, Oregon Health Sciences University  
*Serotonin and small molecule treatment of respiratory disorders in a mouse model of Rett syndrome*
- **Qiang Chang, PhD**, of University of Wisconsin-Madison  
*Establishing Neurons Differentiated from an Isogenic Pair of Rett Syndrome iPSC lines as Cell-Based Assay for Future Drug Screens*

- **Jenny Downs, PhD**, Curtin University  
*Daily physical activity in girls and women with Rett syndrome: An important outcome for clinical trials*
- **Steven J. Gray, PhD**, University of North Carolina at Chapel Hill  
*Development of Optimized AAV Vectors for Intra-CSF Administration in Rett mice*
- **Walter Kaufmann, MD**, Hugo W. Moser Research Institute at Kennedy Krieger, Inc. *Development of a Behavioral Outcome Measure for Rett Syndrome*
- **Lucas Pozzo-Miller, PhD**, University of Alabama-Birmingham  
*IGF-1 and TrkB Agonists as BDNF Mimetics for the Reversal of Dendritic Spine Pathologies and Network Hyperexcitability in the Hippocampus of MeCP2 Mutant Mice*
- **Jay R. Shapiro, MD**, Hugo W. Moser Research Institute at Kennedy Krieger, Inc. *Treatment of Osteoporosis in Murine Rett Syndrome Models: A Comparison of Zoledronic Acid vs. Teriparatide on Osteoblast Function, Gene Expression and Bone Mass*
- **Huda Y. Zoghbi, MD**, Baylor College of Medicine  
*Therapeutic intervention to modulate the GABAergic and cholinergic systems in animal models of Rett syndrome*

## About Rett Syndrome

Rett syndrome (RTT), a developmental neurological disorder, occurs almost exclusively in females. RTT results in severe movement and communication problems following apparently normal development for the first six to 18 months of life. Characteristic features of the disease include loss of speech and purposeful hand use, repetitive hand movements, abnormal walking, abnormal breathing, slowing in the rate of head growth and increased risk of seizures. Current treatment for girls with RTT includes physical and occupational therapy, speech therapy, and medication for seizures. There is no known cure for RTT. In 2007, researchers heralded a major breakthrough by reversing RTT symptoms in mouse models. RTT is considered a "Rosetta Stone" that is helping scientists understand multiple developmental neurological disorders, and shares genetic links with other conditions such as autism and schizophrenia.

## About the International Rett Syndrome Foundation.

IRSF is the world's leading private funder of basic, translational and clinical Rett syndrome research, funding over \$24M in high-quality, peer-reviewed research grants and programs to date. Annually, IRSF hosts the world's largest gathering of global Rett researchers and clinicians to establish research direction and priorities while exchanging ideas and the most recent information. IRSF is the most comprehensive non-profit organization dedicated to providing thorough and accurate information about Rett syndrome, offering informational and emotional family support, and stimulating research aimed at accelerating treatments and a cure for Rett syndrome and related disorders. IRSF has earned Charity Navigator's most prestigious 4 star rating. To learn more about IRSF and Rett syndrome, visit [www.rettsyndrome.org](http://www.rettsyndrome.org) or call IRSF at 1-800-818-RETT (7388).