

### **A Rett Workshop focused on “*Setting Priorities for Therapy Development in Rett Syndrome*”**

The National Institute of Neurological Disorders and Stroke (NINDS) of the National Institutes of Health (NIH) sponsored a workshop focused on “*Setting Priorities for Therapy Development in Rett Syndrome*” that occurred on September 25-27 in Bethesda, MD. IRSF and RSRT also co-sponsored this workshop that brought together basic research scientists, clinicians, representatives from the pharmaceutical and biotech industries, and officials from the FDA and the NIH.

Laura Mamounas, PhD, Program Director, Neurogenetics of the NINDS/NIH and Coryse St. Hillaire-Clarke, Program Analyst, Neurogenetics of the NINDS/NIH launched this workshop into fruition and set forth the workshop’s focused goals for Rett syndrome translational research.

1. Define ideal outcome measures for preclinical efficacy testing in Rett animal models that can inform future clinical trials in Rett patients.
2. Establish criteria and standards for a successful preclinical animal trial.
3. Set priorities and criteria for launching early-stage candidate compounds into a drug development pathway.

The workshop recommendations and outcomes will be published in *Disease Models & Mechanisms* to serve as a guide for the field when conducting translational/preclinical studies in Rett syndrome.

While encouraging progress has been made, IRSF recognized the need to have and sponsor this workshop for Rett syndrome research to keep us all on track for moving translational research forward, towards finding therapeutics for the disease and its compounding symptoms. We give thanks to all those who spearheaded the workshop, generated great discussion, and are dedicated to Rett syndrome research.