

## **Settings standards for research into Rett syndrome**

In September of 2011, a workshop focused on the state of the art in animal studies of Rett syndrome (RTT) was convened by the National Institute of Neurological Disorders and Stroke (NINDS), the Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD), the International Rett Syndrome Foundation (IRSF) and the Rett Syndrome Research Trust (RSRT). A broad cross-section of basic scientists, clinicians, and representatives from the National Institutes of Health (NIH), the Food and Drug Administration (FDA), the pharmaceutical industry and private foundations attended the workshop in order to identify crucial knowledge gaps and to suggest scientific priorities and best practices for the use of animal models in preclinical evaluation of potential new RTT therapeutics. On October 31, the outcomes of this workshop were published as an Open Access review article, authored by several workshop participants, in *Disease Models & Mechanisms* (DMM). As explained in this article, the combination of an urgent need for effective treatments for RTT, coupled with the availability of good mouse models, is a driving force for studies that can identify and test new drugs.

The participants of the September 2011 workshop focused on discussing how researchers can ensure that drug candidates that show promise in laboratory tests are effective in patients at the clinical testing stage. Among topics discussed were the detailed characteristics of the many mouse models of RTT that are used to test drug candidates in the lab, and the variety of tests that different labs use to study the mice (making comparison between studies difficult). The participants also set out some important guidelines that the community of RTT researchers can implement to ensure more standardized study design and transparent reporting. Overall, it is hoped that these guidelines will ensure that decisions to initiate costly clinical trials are founded on reliable preclinical data. Several workshop participants contributed to the article, and the corresponding authors are Dr. David Katz (Case Western Reserve University School of Medicine), Dr. Laura Mamounas (National Institute of Neurological Diseases and Stroke) and Dr. Huda Zoghbi (Baylor College of Medicine).

As potential new RTT drugs continue to be developed, it is increasingly important that the recommendations made by the participants of the September 2011 workshop are incorporated into the design of preclinical studies of RTT. Rigorous and standardized measures are essential to avoid involving individuals with RTT in unnecessary clinical trials, wasting precious research dollars, and raising unwarranted expectations for the patients and their families. As we move forward, funding agencies including IRSF will look for rigorous and high-quality preclinical trial design, as well as adherence to best practices, from our investigators. Ultimately, this level of rigor should shorten the time to effective treatments.