

Phase 2 Clinical Trial Study of IGF-1

IRSF Announces Funding to Boston Children's Hospital for the Phase 2 Clinical Trial Study of IGF-1 for Rett Syndrome

The International Rett Syndrome Foundation (IRSF) is pleased to announce the approval of funding to Dr. Walter Kaufmann and the Boston Children's Hospital (CHB) for the second phase of the IGF-1 clinical trial for treatment of Rett syndrome.

The project entitled "A Phase 2b placebo-controlled crossover study of rhIGF1 (mecasermin [DNA] injection) for treatment of Rett syndrome and development of Rett-specific novel biomarkers of cortical and autonomic function" was submitted under IRSF's Translational Research ANGEL (Advanced Neurotherapeutic Grant of Excellence) grant award mechanism. The application had undergone an extensive, rigorous review by a distinguished scientific review committee, and a programmatic review by Dr. Steve Kaminsky, IRSF Chief Science Officer, who moved to recommend the application for funding. The IRSF Board of Directors (comprised mainly of parents of children with Rett syndrome) unanimously voted to approve funds in the amount of \$600,000.

Prior support from IRSF and Autism Speaks allowed CHB and then Principal Investigator (PI), Dr. Omar Khwaja to successfully complete the Phase 1 study that involved the safety and tolerability of IGF-1 use in patients diagnosed with Rett syndrome. The Phase 2 study will continue to investigate the efficacy of IGF-1 in a larger cohort of Rett patients (30) in a double-blind placebo-controlled cross-over trial.

IRSF is excited to work with Dr. Kaufmann who is now the PI heading this study and is also the Director of the Rett Syndrome Program at CHB. By supporting this project, IRSF continues to accelerate and develop treatments for Rett syndrome.

For questions about this Phase 2 Trial, please contact Theresa Trapilo of CHB for more information. Email Theresa.Trapilo@childrens.harvard.edu.