Molecular and Neurobiological Studies in Rett Syndrome and Other MECP2 Disorder

Rett syndrome is a delayed-onset childhood disorder, typically found in girls, that causes a broad range of severe neurological disabilities, including loss of the ability to speak and socialize, and the development of tremors, ataxia, seizures, autonomic dysfunction, and stereotypic hand-wrapping movements. Dr. Zoghbi and her team discovered that mutations in the gene MECP2 cause Rett syndrome, and before long it became clear that mutations in MECP2 can also cause other neuropsychiatric phenotypes ranging from autism to bipolar disorders. The lecture will highlight neurobiological and molecular studies that are providing insight into the pathogenesis of Rett syndrome and MECP2 disorders, and will highlight discoveries that chart the path for therapeutic opportunities.