Chen Institute Symposium 2025

Keynote Address

Speaker: Huda Y. Zoghbi

Talk title: Genetic and Neurobiological Studies in Rett syndrome

and other MECP2 disorders

Abstract: 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-wringing movements. We 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.