

# **Abstract**

## **Somatic Mosaicism in Neurological Disorders**

Jeong Ho Lee MD PhD<sup>1,2</sup>

*Professor/CSO*

*1. Graduate School of Medical Science and Engineering, Korea Advanced Institute Science and Technology (KAIST), Daejeon, Korea*

*2. Sovargen Co. LTD., Daejeon, Korea*

*E-mail address: jhlee4246@kaist.ac.kr*

Since neural stem cells or progenitor cells continue to undergo cell division throughout human life, somatic mutations (or mosaicism) in human brain can arise during development and accumulate with the aging process. Although somatic diversity is an evident feature of the brain, the extent of somatic mutations affecting the neuronal structure and function and their contribution to neurological disorders remain largely unexplored. Over the last decade, we have provided the molecular genetic evidence that brain somatic mutations arising from neural stem cells (NSCs) indeed lead to the structural and functional abnormalities of the brain observed in neurodevelopmental disorder, brain tumors, and neurodegenerative disorders. In this seminar, I will present our recent findings of brain somatic mosaicism implicated in intractable focal epilepsy and brain tumor evolution. I will also discuss antisense oligonucleotide (ASO) therapy for neurological disorders with somatic mosaicism.