

Advancing Gene-Targeted Therapies for Central Nervous System Disorders

A Workshop

**April 23rd - 24th
Washington, DC**

Session III: Gene-Targeting Therapy Technologies for CNS Disorders

Session Objectives

- For different therapy modalities, and with a focus on general issues rather than specific disease indications:
 - Discuss approaches to addressing their respective administration challenges,
 - Explore CNS fluid dynamics and barriers, as well as delivery routes, distribution, and dose,
 - Examine what is known about clinical and nonclinical safety, as well as potential long-term effects.
- Consider how previously successful approaches for spinal muscular atrophy and retinal dystrophy would need to be adapted for monogenetic disorders that have more variable onset and slower progression, and discuss timing of interventions.
- Discuss what it takes to move beyond monogenetic disorders to develop gene therapy approaches for common, heterogeneous disorders such as Alzheimer's and Parkinson's diseases.
- Examine key challenges such as CNS cell type-specific transduction; regulation of viral gene expression to optimize safety and efficacy; capsid engineering to improve tissue-specific targeting and BBB penetration.

