

Advancing Gene-Targeted Therapies for Central Nervous System Disorders

A Workshop

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

Session I: Current Landscape and Lessons Learned

Session Objectives

- Provide an overview of the current landscape of gene-targeted therapies approaches for central nervous system disorders.
- Explore lessons learned from gene and ASO therapies that have achieved FDA approval—including translation plans and which animal models were used in preclinical studies, use of dog model for RPE65, role of natural history studies for spinal muscular atrophy (SMA) therapy, and other lessons learned in translation to clinical development.
- Examine lessons learned from gene therapy efforts that were not successful, including neurotrophins for neurodegenerative diseases.

