

Advancing Gene-Targeted Therapies for Central Nervous System Disorders

A Workshop

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

Workshop Planning Committee

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Joshua Gordon, National Institute of Mental Health

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John Krystal, Yale University School of Medicine

Maryann Redford, National Eye Institute

Todd Sherer, Michael J. Fox Foundation for Parkinson's Research

Hao Wang, Takeda

Clinton Wright, National Institute of Neurological Disorders and Stroke



Workshop Objectives

Bring together experts and key stakeholders from academia, government, industry, and non-profit organizations to explore approaches for advancing the development of gene-targeted therapies for CNS disorders, including approaches that target nucleic acids, such as AAVs, ASOs, and RNAi, as well as gene product-targeted therapies.

- Provide an overview of the current landscape
- Discuss lessons learned from recent advances in gene therapy and ASO development
- Compare features of different gene-targeted therapy approaches, and discuss approaches to matching the approach to specific diseases, addressing their respective administration, distribution, and dose challenges, and potential long-term effects
- Explore clinical development and the regulatory pathway
- Discuss what it would take to move beyond rare genetic disorders
- Explore opportunities for catalyzing development of gene-targeted therapy



Agenda Overview

April 23

Session I: Current Landscape and Lessons Learned

Session II: Selecting Gene-Targeted Therapy Approaches for CNS Disorders

Day One Closing Talk: The Vista for Developing Gene-Targeting Therapies for Psychiatric and Other Circuit Disorders

April 24

Session III: Gene-Targeting Therapy Technologies for CNS Disorders

Session IV: Clinical Trial Design and Regulatory Pathways

Session V: Moving Forward

