

# **Advancing Gene-Targeted Therapies for Central Nervous System Disorders**

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**A Workshop**

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

# Session V: Moving Forward

## *Objectives*

- Discuss new technologies on the horizon, for example, non-viral approaches, small molecules targeting RNA, chaperones, targeted protein degradation, and cell penetrant stapled peptide therapeutics.
- How can these approaches be used for psychiatric disorders and other circuit disorders?
- What else do we need to know that we don't know? For example, precision medicine for low incidence disorders, developing a strategic pipeline for treatments, Timothy syndrome, neuregulins.
- Briefly discuss issues related to cost, access, and health equity, as well as AAV manufacturing capacity.

