Advancing Gene-Targeted Therapies for Central Nervous System Disorders

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.