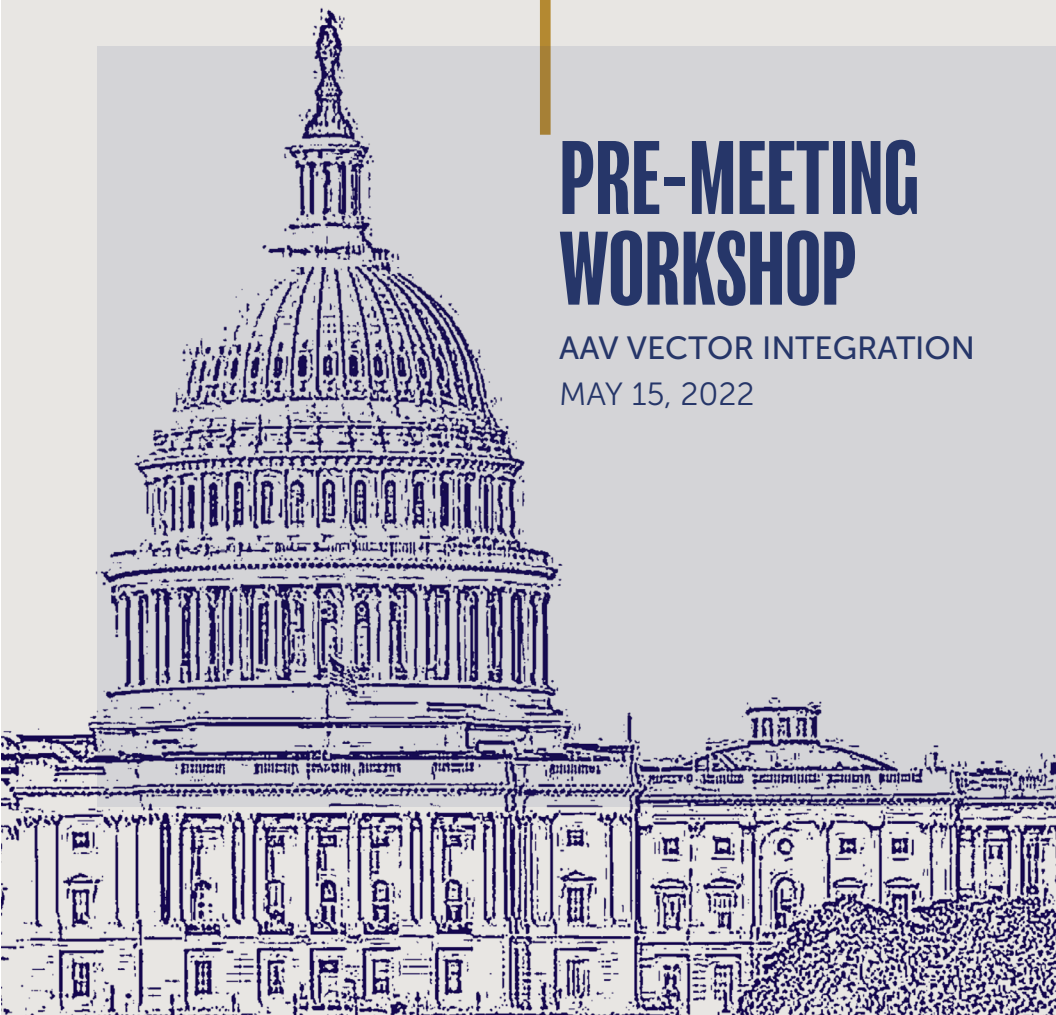
American Society of Gene + Cell Therapy

ASGCT 25TH ANNUAL MEETING MAY 16–19·2022 WASHINGTON DC

PRE-MEETING WORKSHOP

AAV VECTOR INTEGRATION

MAY 15, 2022



AAV VECTOR INTEGRATION

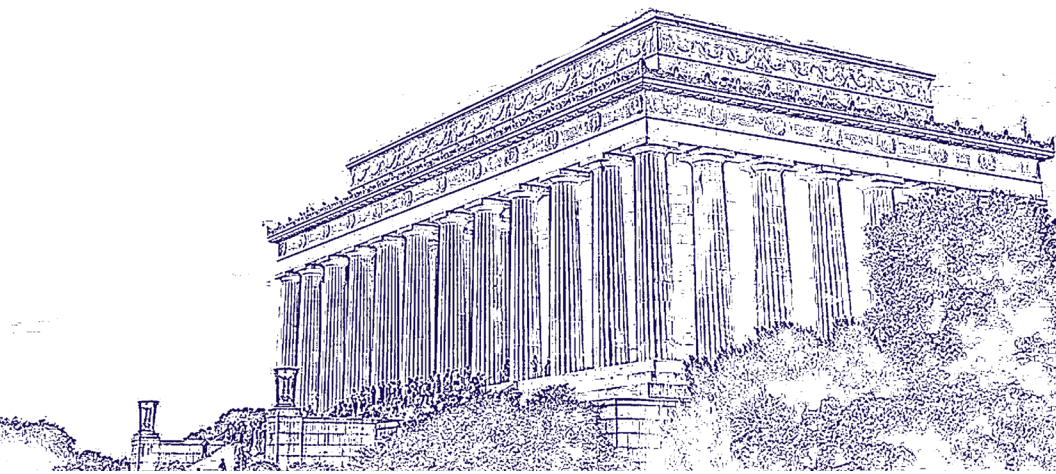
SUNDAY, MAY 15, 2022

All times listed below ET

Co-chairs: Mark Kay, MD, PhD, Stanford University
Guangping Gao, PhD, University of Massachusetts

Description

While AAV vector genomes primarily persist as episomes, low rates of vector integration into the host genome do occur. This workshop will address various facets of this topic, including animal models, and genomic and bioinformatic platforms to assess clinical risk. The program will also cover the pathogenesis of hepatocellular carcinoma, clinical monitoring, and regulatory requirements.



AAV VECTOR INTEGRATION

Schedule

All times listed below ET

1:00–1:40 PM

Session 1 Introduction and Overview

Overview: Knowns and Unknowns of AAV Integration Risk
Mark Kay, MD, PhD, Stanford University

The Impact of CRISPR Editing in Rodent CNS
Beverly Davidson, PhD, Children's Hospital of Philadelphia

1:40–2:55 PM

Session 2 Non-clinical Models for Assessing Clinical Risk

Sequence-based Methods for Quality Control and Assessing Outcomes in Gene Therapy
Frederic Bushman, PhD, University of Pennsylvania

Humanized Mouse Liver Systems
Markus Grompe, MD, Oregon Health & Science University

AAV Vector Integration Behavior in Primary Human Hepatocytes in the FRG Mouse Liver
Ian Alexander, PhD, University of Sydney

2:55–3:15 PM

Break

AAV VECTOR INTEGRATION

3:15–4:55 PM

Session 3 Clinical and Regulatory Considerations for Assessing AAV Integration Risk

Pathogenesis of Hepatocellular Carcinoma and Implications for Clinical Monitoring
Dean Felsher, MD, PhD, Stanford University

Genomics of Hepatocellular Carcinoma: Role of Viral Insertional Mutagenesis
Jessica Zucman-Rossi, MD, PhD, University of Paris Descartes

Considerations for Preclinical Development of AAV-based Gene Therapy Products: A Regulatory Perspective
Gaya Hettiarachchi, PhD, FDA

Industry Panel: Considerations for Assessing Risk in Clinical Trials
Ricardo Dolmetsch, PhD, Uniqure
Kevin Eggan, PhD, BioMarin
Sam Wadsworth, PhD, Ultragenyx
Laurence Whiteley, DVM, Pfizer
Moderated by: Federico Mingozzi, PhD, Spark Therapeutics

4:55–5:00 PM

Closing Remarks
Guangping Gao, PhD, University of Massachusetts