PBSS
PHARMACEUTICAL & BIOSOSCIENCE SOCIETY
SAN FRANCISCO BAY

RAPS SAN FRANCISCO BAY AREA CHAPTER

Friday, Sept. 26th, 2025 8:45 am - 5:10 pm Pacific Time

Gene Therapy Challenges and Best Practices:
From Discovery to IND and Beyond (jointly by PBSS and RAPS-SF)

Organizers: Toufan Parman, PhD, DABT, Alector; Yoshiko Stowell, PhD, Kenai Therapeutics

San Francisco Bay Area: Crowne Plaza Foster City; available as webcast to other PBSS chapters

Michael Templin, PhD, DABT
Director, Scientific Advisory Services, **Charles River Laboratories**

Forging a New Path: The Journey From Discovery to Clinical Trials With a Gene therapy

Aaron DeLoughery, PhD
Principal Scientist, Protein discovery, **Mammoth Biosciences**

Discovery of NanoCas, an Ultracompact CRISPR Nuclease Capable of Single-AAV Editing of Skeletal Muscle in Non-human Primates

Kathleen Meyer, PhD, DABT
VP, Development Sciences, **Sangamo Therapeutics**

Laying the Groundwork: Nonclinical Strategies in Gene Therapy Development

Toufan Parman, PhD, DABT
Head of Nonclinical Sciences, **Alector**

Genotoxicity and Carcinogenicity of Cell Therapies Utilizing Adeno-Associated Viral Vectors

Yanmei Lu, PhD
VP, Biomarker and BioAnalytical Sciences, **Sangamo Therapeutics**

Navigating Companion Diagnostic Development for AAV Gene Therapy in the US: A Drug Sponsor's View

Cori Gorman, PhD, MBA
Previously: Senior Director, Biopharmaceutical CMC and Regulatory Affairs, **Cencora-Pharmalex**

What Role Can RNA Therapeutics Play in Gene Therapies?

John Shoffner, MD, FACMG
Retired; previously at Novartis, Sangamo Therapeutics, and Astellas Pharma

rAAV Gene Therapies for Rare Neurological Diseases: Clinical Development Considerations

Sandeep Yadav, PhD
VP, Technical Development, **Sangamo Therapeutics**

Challenges and Opportunities with AAV Derived Genomic Medicines - a CMC Perspective

Michael Molony, MA
Executive Director, Analytical Development & Quality Control, **Insmed Inc.**

Analytical Development and Quality Control Overview for AAV Gene Therapies from R&D to BLA

Tiffany Lucas, PhD
Principal Consultant - CMC Cell & Gene Therapy Regulatory Affairs, **ELIQUENT Life Sciences**

Past Guidance, Future Uncertainty: Interpreting FDA Guidance Documents Amid Leadership Shifts in Gene Therapy

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Registration: https://www.pbss.org

- Regular Attendees: $295 (Registration closes one day before the event or when full)
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Time (PT)	Topic	Presenter(s)
8:45 - 9:05 am	PBSS Overview	**Shichang Miao**, PhD, **PBSS** President; **Yoshiko Stowell**, PhD, RAC, PMP, Chapter Chair, **RAPS-SF**
9:05 - 9:30 am	Forging a New Path: The Journey From Discovery to Clinical Trials With a Gene therapy	**Michael Templin**, PhD, DABT, Director, Scientific Advisory Services, **Charles River Laboratories**
9:30 - 10:00 am	Discovery of NanoCas, an Ultracompact CRISPR Nuclease Capable of Single-AAV Editing of Skeletal Muscle in Non-human Primates	**Aaron DeLoughery**, PhD, Principal Scientist, Protein discovery, **Mammoth Biosciences**
10:00 - 10:55 am	Laying the Groundwork: Nonclinical Strategies in Gene Therapy Development	**Kathleen Meyer**, PhD, DABT, VP, Development Sciences, **Sangamo Therapeutics**
10:55 - 11:05 am	*Morning Sponsor*	*Labcorp Biopharma Solutions*
11:05 - 11:25 am	*Break*	**-**
11:25 - 11:55 am	Genotoxicity and Carcinogenicity of Cell Therapies Utilizing Adeno-Associated Viral Vectors	**Toufan Parman**, PhD, DABT, Head of Nonclinical Sciences, **Alector**
11:55 am - 12:55 pm	*Lunch*	*Sponsor (TBD)*
12:55 - 1:25 pm	Navigating Companion Diagnostic Development for AAV Gene Therapy in the US: A Drug Sponsor's View	**Yanmei Lu,** PhD, VP, Biomarker and BioAnalytical Sciences, **Sangamo Therapeutics**
1:25 - 1:50 pm	What Role Can RNA Therapeutics Play in Gene Therapies?	**Cori Gorman, PhD**, Previously: Senior Director, Biopharmaceutical CMC and Regulatory Affairs, **Cencora-Pharmalex**
1:50 - 2:20 pm	rAAV Gene Therapies for Rare Neurological Diseases: Clinical Development Considerations	**John Shoffner**, MD, FACMG, Retired; previously at Novartis, Sangamo Therapeutics, and Astellas Pharma
2:20 - 2:50 pm	Challenges and Opportunities with AAV Derived Genomic Medicines - a CMC Perspective	**Sandeep Yadav**, PhD, VP, Technical Development, **Sangamo Therapeutics**
2:50 - 3:40 pm	Analytical Development and Quality Control Overview for AAV Gene Therapies from R&D to BLA	**Michael Molony,** MA, Executive Director, Analytical Development & Quality Control, **Insmed Inc.**
3:40 - 3:50 pm	*Afternoon Major Sponsor*	*TBD*
3:50 - 4:10 pm	*Break*	-
4:10 - 4:40 pm	Past Guidance, Future Uncertainty: Interpreting FDA Guidance Documents Amid Leadership Shifts in Gene Therapy	**Tiffany Lucas**, PhD, Principal Consultant - CMC Cell & Gene Therapy Regulatory Affairs, **ELIQUENT Life Sciences**
4:40 - 5:10 pm	Panel Discussion	-
5:10 - 6:10 pm	Happy Hour	Sponsor (TBD)

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