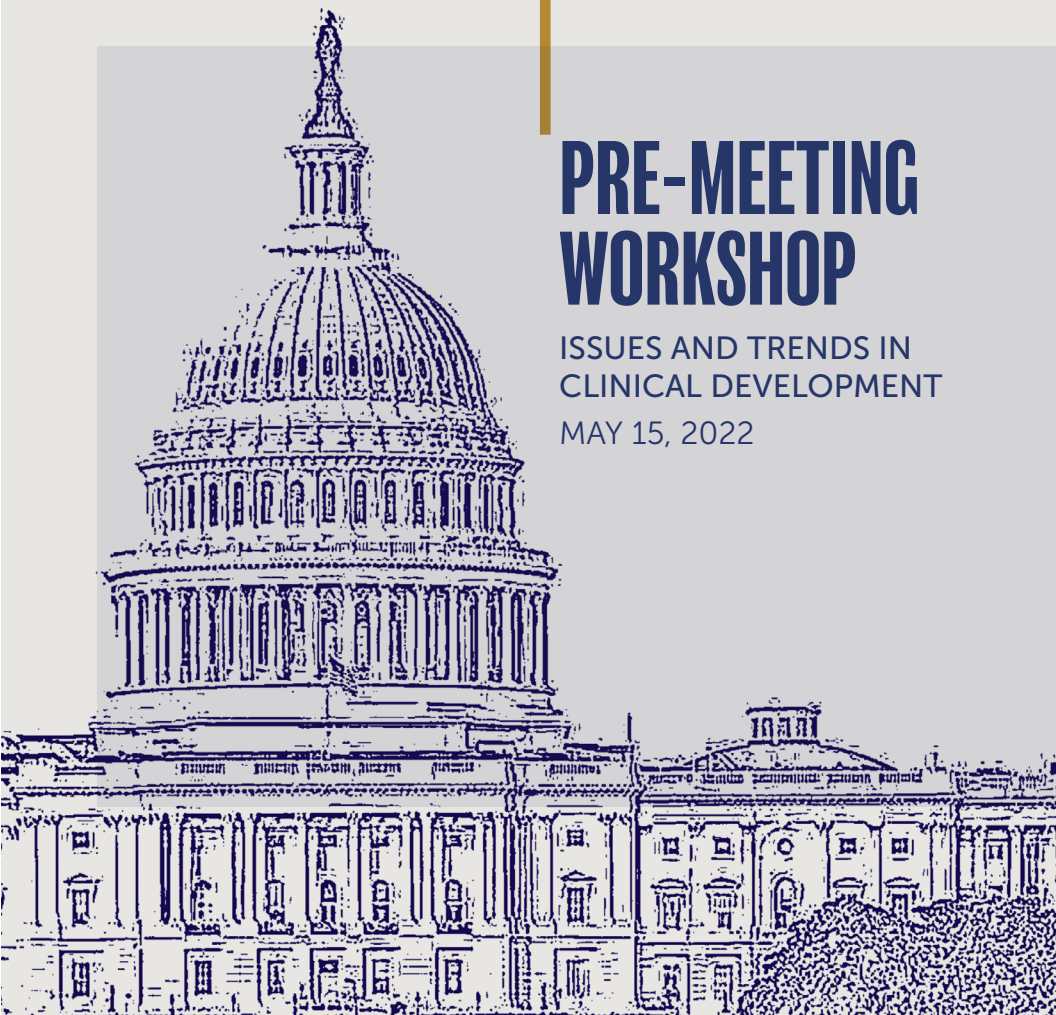
American Society of Gene + Cell Therapy

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

PRE-MEETING WORKSHOP

ISSUES AND TRENDS IN CLINICAL DEVELOPMENT

MAY 15, 2022



ISSUES AND TRENDS IN CLINICAL DEVELOPMENT

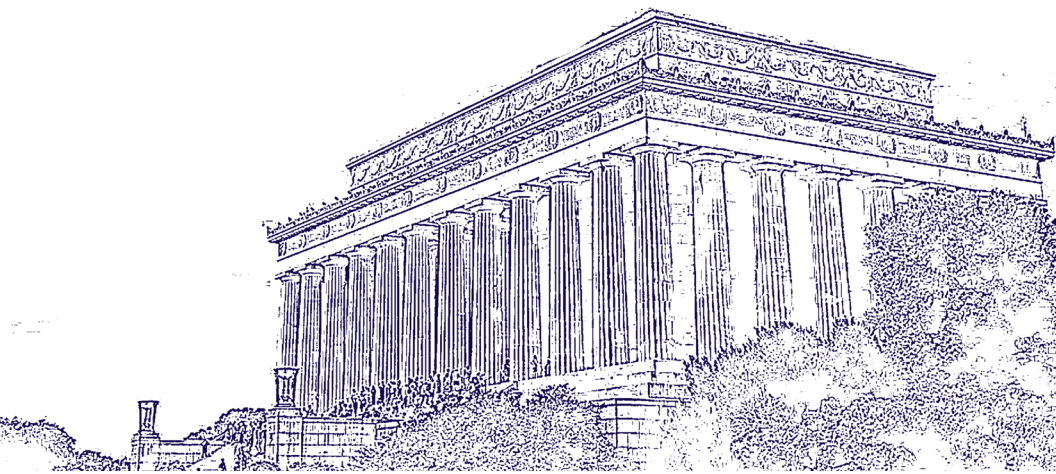
SUNDAY, MAY 15, 2022

All times listed below ET

Co-chairs: Daniela Drago, PhD, Aurion Biotech
Kaye Spratt, PhD, Unaffiliated

Description

This workshop will discuss strategies for optimizing gene and cell therapy clinical development. In particular, the program will explore the planning and execution of pediatric studies, best practices for managing unexpected adverse events, and opportunities to utilize innovative study designs. Speakers will share both conceptual ideas, as well as case studies that illustrate how to address these areas of clinical development.



ISSUES AND TRENDS IN CLINICAL DEVELOPMENT

Schedule

All times listed below ET

8:00–9:00 AM

Session 1 Baby Steps: Cell and Gene Therapy Clinical Trials in Pediatric Populations

Improving Clinical Pediatric R&D For Cell and Gene Therapies
Brian Tseng, MD, Unaffiliated

Regulatory Perspective on Pediatric Studies
Elizabeth Hart, MD, FDA

Panel Q&A
Moderated by: Daniela Drago, PhD, Aurion Biotech

9:00–10:00 AM

Session 2 When Things Go Wrong - Effectively Managing Adverse Events During Product Development

Managing Unexpected Adverse Events in Gene Therapy Clinical Trials
Bernhardt Zeiher, MD, Astellas Pharma

Regulatory Considerations and Developmental Impact of Unexpected Adverse Events: DMD Experience
Natalie Schmidt, Pfizer

Panel Q&A
Larissa Lapteva, MD, FDA
Moderated by: Kaye Spratt, PhD, BridgeBio Gene Therapy

10:00–10:30 AM

Break

ISSUES AND TRENDS IN CLINICAL DEVELOPMENT

10:30 AM–12:00 PM

Session 3 Innovation in Clinical Trial Design for Cell and Gene Therapies: What's on the Horizon?

Managing the Uncertainties of Utilizing Novel Trial Designs in Hemophilia
Alex Kuta, PhD, UniQure

Developing Innovative Clinical Trials for Rare Diseases: Danon Disease Experience
Jonathan Schwartz, MD, Rocket Pharmaceuticals

Regulatory Considerations for Clinical Trials With Advanced Therapies
Larissa Lapteva, MD, FDA

Panel Q&A
Moderated by: Snehal Naik, PhD, Spark Therapeutics

THANK YOU

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