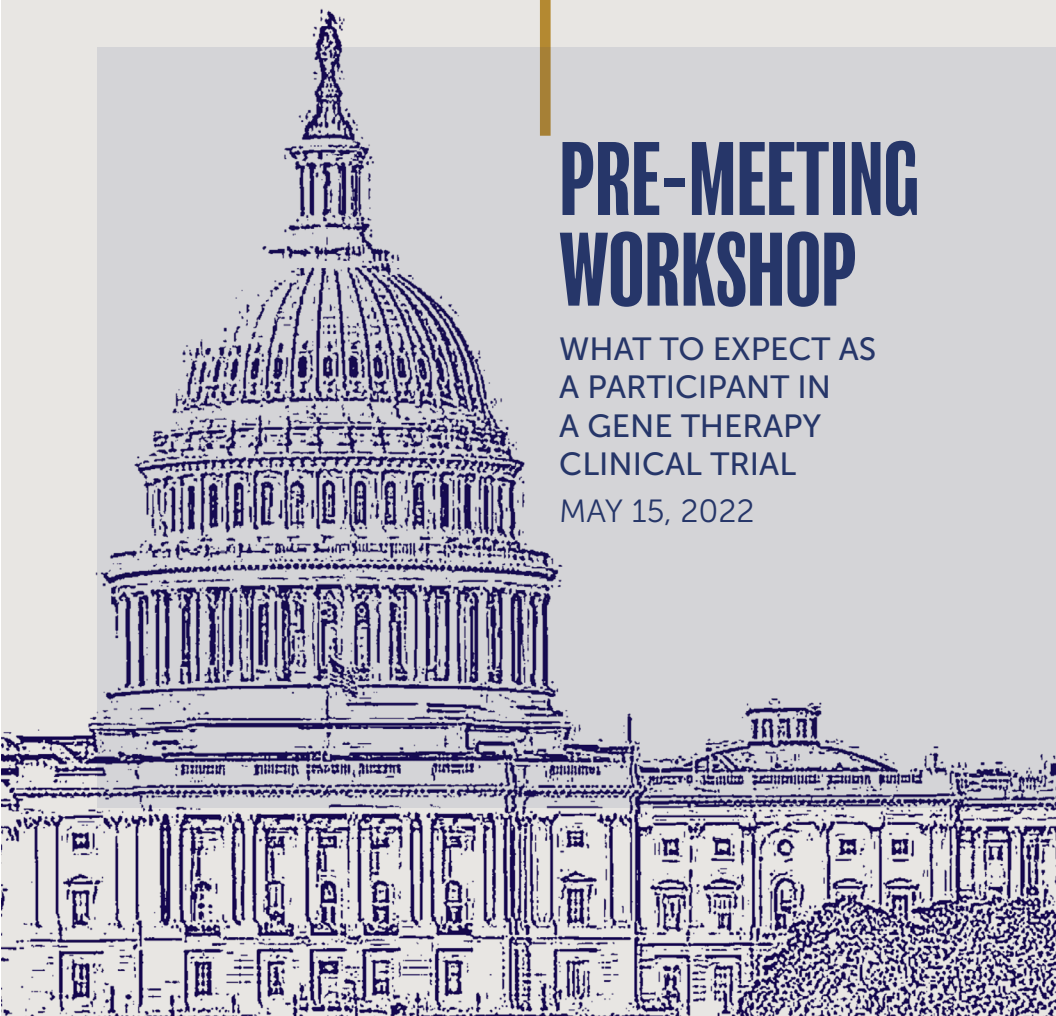




PRE-MEETING WORKSHOP

WHAT TO EXPECT AS
A PARTICIPANT IN
A GENE THERAPY
CLINICAL TRIAL

MAY 15, 2022





Scientific Potential, Unlocked

Join our teams leading the development of first-in-class cell & genetic therapies to help transform the lives of people with serious diseases — *including stem cell differentiation, novel devices for immunoprotection, CRISPR/Cas9 gene editing and adeno-associated virus (AAV) capsids.*

Learn more about joining the fast-paced and passionate teams behind our work:
<https://www.vrtx.com/working-here/>

WHAT TO EXPECT AS A PARTICIPANT IN A GENE THERAPY CLINICAL TRIAL

SUNDAY, MAY 15, 2022

All times listed below ET

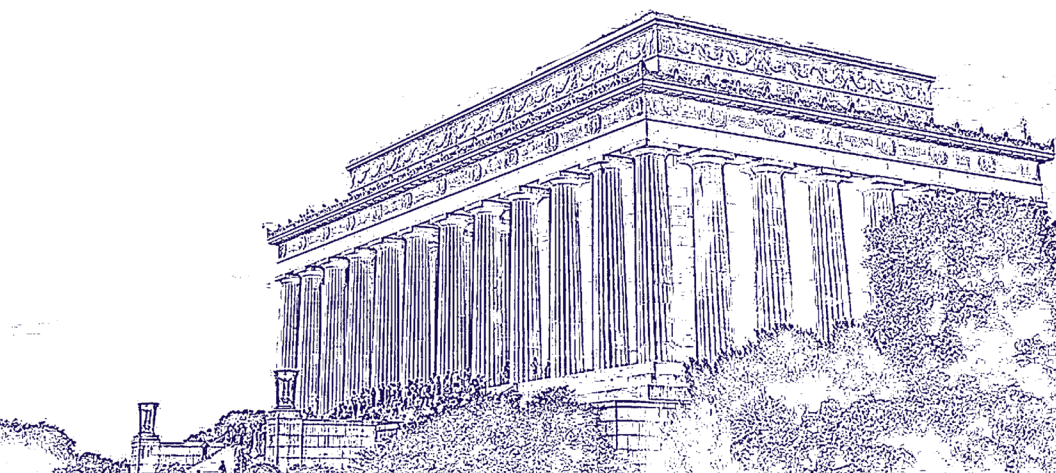
Planned by the Patient Outreach Committee

Co-chairs: Emily McGinnis

Kimberly Goodspeed, MD

Description

This workshop is intended for patients and caregivers, along with advocates and providers to better understand what to expect when participating in a clinical trial for gene therapy. It is a complex process with many important considerations from eligibility, to understanding the procedure, potential risks or outcomes, and logistical challenges an individual or family may face. The speakers will share their knowledge to address these topics and help manage expectations for those considering clinical trial participation.





WHAT TO EXPECT AS A PARTICIPANT IN A GENE THERAPY CLINICAL TRIAL

Schedule

All times listed below ET

1:00–3:00 PM

Session 1 The Process: From Setting Up a Clinical Trial to Being a Participant

Current Trends in Clinical Trials

Kimberly Goodspeed, MD, UT Southwestern Medical Center

How a Clinical Trial is Setup and Run

Megan Waldrop, MD, Nationwide Children's Hospital

Complexities of Eligibility: Why do we have to exclude patients?

*Heather Lau, MD, Yale University School of Medicine,
Ultragenyx Pharmaceutical Inc.*

What to Expect Before and During a Clinical Trial

Nicolas Abreu, MD, NYU Langone Health

What to Expect After a Clinical Trial

Perry Shieh, MD, PhD, FAAN, University of California, Los Angeles

Panel Q&A

moderated by Emily McGinnis, Taysha Gene Therapies

3:00–3:30 PM

Break



WHAT TO EXPECT AS A PARTICIPANT IN A GENE THERAPY CLINICAL TRIAL

3:30–5:00 PM

Session 2 Risks, Challenges, and Hopes

Caregiver Insights on Clinical Trial Participation

Emily McGinnis, Taysha Gene Therapies

Weighing the Risks for Participation in a Gene Therapy Clinical Trial

Diana Bharucha-Goebel, MD, National Institutes of Health

Participant Challenges

Pat Furlong, Parent Project Muscular Dystrophy

Making the Decision

Natalie Wheatley, Mother of a child who received gene therapy

Julia Vitarello, Mila's Miracle Foundation

Panel Q&A

moderated by Kimberly Goodspeed, MD, UT Southwestern Medical Center



THANK YOU

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