PRE-MEETING WORKSHOP
A FOCUS ON GENE AND CELL THERAPY
MAY 15, 2022
A FOCUS ON GENE AND CELL THERAPY

SUNDAY, MAY 15, 2022
All times listed below ET

Planned by the Clinician Education Workgroup
Chair: Terence R. Flotte, MD

Description
This workshop is designed for clinicians with experience caring for patients for which a gene or cell therapy is available. Speakers will review the underlying biological basis for gene therapy, the regulatory framework for oversight of clinical trials to develop safe and effective products for gene therapy, and the primary forms of toxicity encountered with various gene therapy vector types. Attendees will gain an appreciation for the nature of the process of clinical research and the challenges and opportunities presented to patients and families participating in studies.
A FOCUS ON GENE AND CELL THERAPY

Schedule
All times listed below ET

8:00–8:20 AM
A Focus on Gene and Cell Therapy for Clinicians—
Introduction: What Therapies are Available Today
Terence R. Flotte, MD, UMass Chan Medical School

8:20–8:45 AM
Types of Therapies – Lentiviral/In-vivo HSC
Matthew Porteus, MD, PhD, Stanford University

see how ART is changing gene therapy
www.affiniatx.com
A FOCUS ON GENE AND CELL THERAPY

8:45–9:10 AM
Types of Therapies – AAV
Jerry R. Mendell, MD, PhD., The Research Institute at Nationwide Children’s Hospital

9:10–9:35 AM
Types of Therapies – CAR-T Therapies
David L. Porter, MD, Perelman School of Medicine

9:35–10:00 AM
Clinical Trials and My Role—How do I Find a Clinical Trial?
Florian Eichler, MD, Massachusetts General Hospital

10:00–10:25 AM
Clinical Trials and the Role of FDA—Identifying Definitive Clinical Outcomes
Peter Marks. Food & Drug Administration

10:25–11:05 am
Clinical Trials and My Role—Running a Clinical Trial
Kathryn Wagner, MD, PhD, F. Hoffmann-LaRoche

11:05–11:30 am
Ethics of Recommending a Clinical Trial
Alison Bateman-House, MPH, MA, New York University

11:30—11:55 AM
Hospital Pharmacy Role in Managing Insurance Issues
Michael Storey, PharmD, Nationwide Children’s Hospital
THANK YOU
25TH ANNUAL MEETING SPONSORS

affinia
THERAPEUTICS

LEGEND
BIOTECH