



## PRE-MEETING WORKSHOP

A FOCUS ON  
GENE AND CELL THERAPY  
MAY 15, 2022



## A FOCUS ON GENE AND CELL THERAPY

**SUNDAY, MAY 15, 2022**

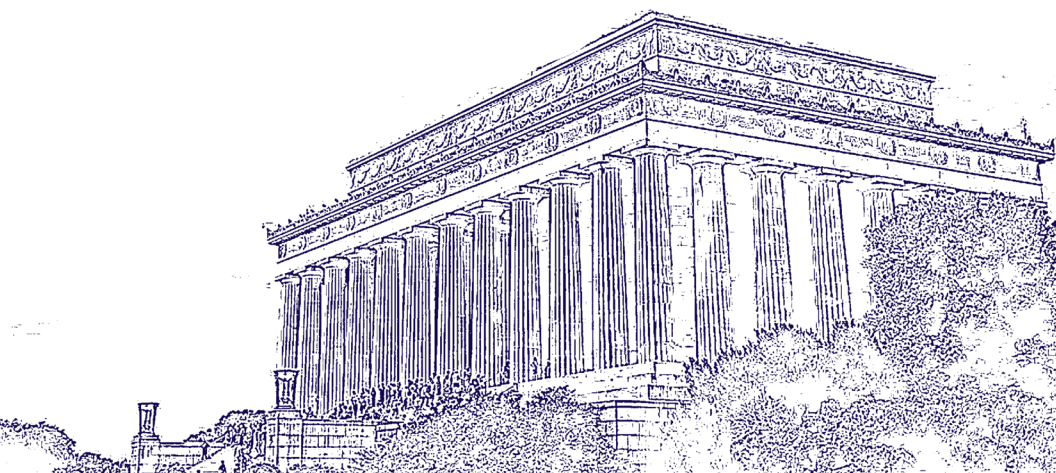
All times listed below ET

**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*

---



**affinia**<sup>TM</sup>  
THERAPEUTICS

see how ART is changing gene therapy  
[www.affiniatx.com](http://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