

# Welcome to The Gene Therapy Summit

## THANK YOU TO OUR SPONSORS

### PLATINUM



### GOLD



### BRONZE



## 2024 Gene Therapy Agenda

All sessions will be in the Amphitheater, except for Concurrent 2 and 4, which will be in the Bray Room. Wi-Fi instructions can be found at the registration desk.

### DAY 1: TUESDAY, NOVEMBER 5

TIME	TOPIC
5:30 - 7:30 PM	Welcome Reception
6:30 - 7:00 PM	<b>Keynote Speaker: The Promise of Hematopoietic Gene Therapy</b> George Daley, MD, PhD; Dean of the Faculty of Medicine; Harvard University

### DAY 2: WEDNESDAY, NOVEMBER 6

9:00 - 9:10 AM	<b>Welcome and Summit Goals</b> Christy Duncan, MD; Dana-Farber Cancer Institute	
9:10 - 10:10 AM	<b>Not-So-Secret Societies: Understanding Gene Therapy and the ASTCT, ASGCT and AABB</b> <i>Moderator: Corey Cutler, MD, MPH; American Society of Transplantation and Cellular Therapy President; Dana-Farber Cancer Institute</i>  <b>SPEAKERS</b> <ul style="list-style-type: none"> <li>Corey Cutler, MD, MPH; American Society of Transplantation and Cellular Therapy President; Dana-Farber Cancer Institute</li> <li>Matthew Porteus, MD, PhD; American Society of Gene and Cellular Therapy Vice-President; Stanford University</li> <li>Jose Cancelas, MD, PhD; Association for the Advancement of Blood &amp; Biotherapies President-Elect; Dana-Farber Cancer Institute; Harvard Medical School</li> </ul>	
10:10 - 10:30 AM	<b>BREAK</b>	
10:30 AM - 12:00 PM	<b>Concurrent 1: The ABC's of Transfusion Medicine in Gene Therapy: The Critical Role of Apheresis, Blood Banking and Cellular Therapy Laboratories</b> <i>Moderator: Suzanne Thibodeaux, MD, PhD; Washington University</i>	<b>SPEAKERS</b> <ul style="list-style-type: none"> <li>John Manis, MD, PhD; Boston Children's Hospital</li> <li>Stella Chou, MD; University of Pennsylvania</li> <li>Jose Cancelas, MD, PhD; Dana-Farber Cancer Institute; Harvard Medical School</li> </ul> <b>TOPICS</b> <ul style="list-style-type: none"> <li>Apheresis in <i>Ex Vivo</i> Hematopoietic Gene Therapy: Complex Patients and Processes</li> <li>Transfusion Support of the Complex Sickle Cell Disease Patient</li> <li>Cellular Therapy Laboratory Considerations for Hematopoietic Gene Therapies</li> </ul>

# 2024 Gene Therapy Agenda

## DAY 2: WEDNESDAY, NOVEMBER 6

<p>10:30 AM - 12:00 PM</p>	<p><b>Concurrent 2: Equity + Engagement = Access in Gene Therapy</b></p> <p><b>Moderators:</b></p> <ul style="list-style-type: none"> <li>• Jeffery Auletta, MD; Senior Vice President, Health Equity, NMDP; Chief Scientific Director, CIBMTR-NMDP</li> <li>• David Barrett, JD; American Society of Gene and Cellular Therapy</li> <li>• Rayne Rouce, MD; Baylor College of Medicine</li> </ul>	<p><b>SPEAKERS</b></p> <ul style="list-style-type: none"> <li>• Amar Kelkar, MD; Dana-Farber Cancer Institute</li> <li>• Rayne Rouce, MD; Baylor College of Medicine</li> <li>• Sarah Rayel, MPP; PhRMA</li> <li>• Adrienne Shapiro; Axis Advocacy</li> </ul> <p><b>TOPICS</b></p> <ul style="list-style-type: none"> <li>• Complexities of Access Related to Gene Therapy</li> <li>• Patient Engagement: How To Make It Happen</li> <li>• Practical Considerations for Emerging Access Models for Medicaid/Medicare Recipients: CMMI CGT Access Model</li> </ul>
<p>12:00 - 1:00 PM</p>	<p><b>LUNCH</b></p>	
<p>1:00 - 2:30 PM</p>	<p><b>Concurrent 3: Long-Term Follow Up</b></p> <p><b>Moderators:</b></p> <ul style="list-style-type: none"> <li>• Sung-Yun Pai, MD; National Institute of Health</li> <li>• Hemalatha Rangarajan, MD; Nationwide Children's Hospital</li> </ul>	<p><b>SPEAKERS</b></p> <ul style="list-style-type: none"> <li>• Sonali Chaudhury, MD; Ann &amp; Robert H. Lurie Children's Hospital of Chicago</li> <li>• Patricia Steinert, PhD, MBA; Executive Scientific Director, Policy and Governance, CIBMTR; Associate Professor, Department of Medicine, Medical College of Wisconsin</li> <li>• Kevin Flanigan, MD; Nationwide Children's Hospital</li> </ul> <p><b>TOPICS</b></p> <ul style="list-style-type: none"> <li>• Strategies for Long-Term Follow-Up: Registry Interaction With Industry</li> <li>• Data Repository Experience</li> <li>• Sample Sharing and Shared Research</li> <li>• Adverse Event Monitoring</li> </ul>
<p>1:00 - 2:30 PM</p>	<p><b>Concurrent 4: Transitioning From Investigational to Commercial Product in Ex Vivo Gene Therapy</b></p> <p><b>Moderator:</b> Colleen Dansereau, MSN, RN, CPN; Boston Children's Hospital</p>	<p><b>SPEAKERS</b></p> <ul style="list-style-type: none"> <li>• Colleen Dansereau, MSN, RN, CPN; Boston Children's Hospital</li> <li>• Keren Shani, MPH; Executive Director, Cell &amp; Gene Therapy Center of Excellence, Trinity Life Sciences</li> <li>• Lisa Eisenbud, Senior Director, Patient Financial Services; Boston Children's Hospital</li> </ul> <p><b>TOPICS</b></p> <ul style="list-style-type: none"> <li>• Becoming a Qualified Treatment Center: The Center Perspective</li> <li>• The Transition From Study to Commercial Therapy: The Industry Perspective</li> <li>• Securing Financial Approval for Commercial Gene Therapy</li> </ul>
<p>2:30 - 2:45 PM</p>	<p><b>BREAK</b></p>	
<p>2:45 - 3:45 PM</p>	<p><b>Industry/Academic Interactive Roundtable: Critical Issues in Commercial Gene Therapy: Manufacturing, Handling Post-Marketing Insertional Oncogenesis, Access and Future Directions</b></p> <p><b>Moderator:</b> Akshay Sharma, MD; St. Jude Children's Research Hospital</p> <p><b>SPEAKERS</b></p> <ul style="list-style-type: none"> <li>• Julie Kanter, MD; Professor, University of Alabama Birmingham; President, National Alliance of Sickle Cell Centers</li> <li>• Esther Shamir, MPH; President, Shamir Consulting</li> <li>• Matthew Porteus, MD, PhD; American Society of Gene and Cellular Therapy Vice-President; Stanford University</li> <li>• Melissa Bonner, PhD; Chief Scientific Officer, Nvelop Therapeutics</li> </ul>	
<p>3:45 - 4:30 PM</p>	<p><b>Concurrent Session Reports</b></p>	