

# Annual Meeting 2025 Program

## Tuesday, May 13

Workshop (separate purchase required)

### **Navigating The Nonclinical Pathway To Clinical Trials For AAV Based Therapeutics**

Location: NOLA A

8:00 AM - 12:00 PM

- Nicholas Buss, PhD, Eli Lilly  
*Biodistribution assessment not one size fits all/managing unique routes of administration and injection devices*
  
- Joy Cavagnaro, PhD, Access BIO  
*Translating data from efficacy and safety to clinical plan/FIH dose selection*
  
- Steven Gray, PhD, University of Texas Southwestern  
*Unique considerations with rare disease*
  
- SunJung Kim, PhD, DABT, Ultragenyx Pharmaceutical Inc.  
*Nonclinical considerations for pregnancy and children*
  
- Ali Nowrouzi, PhD, Spark Therapeutics  
*Genomic integration – when to assess and what happens when tumor occur in clinical trials*
  
- Basel Assaf, Sanofi  
*Design of tox studies (length, power, endpoints)/species selection*
  
- Beverly Davidson, PhD, Children's Hospital of Philadelphia  
*Proof of direct benefit through nonclinical pharmacology*

- Tristan Marshall, Regenxbio  
*Challenges with varying vector quality throughout development*

Workshop (separate purchase required)

### **Current Advancements In The Development Of Cell Therapy For Cancer**

Location: Room 278-282

8:00 AM - 12:00 PM

- Teresa Manzo, University of Turin  
*Understanding how T cell metabolism affects cell therapy*
  
- Melody Smith, Stanford University  
*Delineating the role of microbiome for cell therapy*
  
- Prasad Adusumilli, MSKCC  
*Current advancements in combination therapy with immune checkpoint inhibitors and T cell therapy*
  
- H. Trent Spencer, PhD, Emory University School of Medicine  
*Cancer immunotherapy utilizing gamma delta t cells*
  
- Ryan Larson, Umoja BioPharma  
*Current advancements in in vivo CAR T-cell delivery*
  
- Gabriel Kwong, Port Therapeutics  
*Utilizing nanotechnology to improve cell therapies*

Workshop (separate purchase required)

### **Regulation Ready: Key Compliance Updates and What They Mean for CGT Development**

Location: Room 288-290

8:00 AM - 12:00 PM

- Ann Lee, Prime Medicine  
*Unpacking FDA's Guidance on Human Gene Therapy Products Incorporating Human Genome Editing – Case Study on Prime's Approach*

- George Buchman, Catalent Pharma Solutions  
*Unpacking FDA's Guidance on the Platform Technology Designation Program – CDMO Perspective on Considerations for Sponsors*
- John Tomtishen, PhD, Cellares  
*Unpacking FDA's Guidance on the Advanced Manufacturing Technologies Designation Program – Considerations for Sponsors*
- Mandy Xie, BMS  
*Unpacking FDA's Guidance on Potency Assurance for CGT Products – Case Study on BMS' Approach*
- Houman Dehghani, Cabaletta Bio  
*Unpacking FDA's Guidance on Manufacturing Changes and Comparability – Case Study on Cabaletta's Approach*
- Ezequiel Zylberberg, Akron Biotech  
*Unpacking FDA's Guidance on Considerations for the Use of Human- and Animal-Derived Materials + Safety Testing of Human Allogenic Cells - Considerations for Sponsors*

Workshop (separate purchase required)

### **The Business of Advocates Advancing CGTs**

Location: Room 291-292

8:00 AM - 12:00 PM

- Kim Nye, BA, TESS Research Foundation  
*Introducing the business of advocacy*
- Nasha Fitter, FOXG1 Research Foundation  
*A model for an ultra-rare non-profit*
- Allyson Berent, DVM, DACVIM, Foundation for Angelman Syndrome Therapeutics  
*A model for a mature non-profit*

- Michael Hund, EB Research Partnership  
*A model for a larger organization doing venture philanthropy*
- Terry Pirovolakis, Elpida Therapeutics SPC  
*A model for an ultra-rare venture philanthropy and how to reinvest to advance the field*
- Claire Booth, UCL Great Ormond Street Institute of Child Health  
*A model for a non-profit institution to license and deliver gene therapy products*
- Heidi Wallis, Association for Creatine Deficiencies  
*Diversifying an organization's revenue streams*
- Heidi Bjornson-Pennell, The Chan Zuckerberg Initiative  
*CZI/Rare is One grant funding and support for capacity building*
- Tiina Urv, National Institutes of Health  
*Review of NIH grant funding and new ways to involve patient groups*
- Carly Paterson, Patient-Centered Outcomes Research Institute  
*Learning about PCORI: Funding Opportunities for Rare Disease Research*

Workshop (separate purchase required)

### **Implementing Prime Editing For In Vivo Therapeutic Development And Towards Better Analysis Of High Throughput Functional Screens**

Location: Room 293-296

8:00 AM - 12:00 PM

- Alexander Sousa, Broad Institute  
*Guide sequence optimization to hit CFTR*
- Sangsu Bae, PhD, Seoul National University  
*Improving PE outcomes by overcoming DNA repair hurdles (AI-generated small binder)*

- Jellert Gaublomme, Columbia University  
*CRISPRmap: Sequencing-free optical pooled screens mapping multi-omic phenotypes in cells and tissue*
- Hyongbum (Henry) Kim, Yonsei University College of Medicine  
*DeepPrime for machine learning prediction of Prime-editor*
- Randall Platt, ETH Zurich, Basel  
*Multimodal scanning of genetic variants with base and prime editing*
- Marcello Maresca, AstraZeneca-Gothenburg  
*Improving Prime Editing Repair Outcomes by DNA Repair Modulation and pegRNA Engineering*

Workshop (separate purchase required)

### **Moving Lentiviral Vectors Through The Investigational New Drug (IND) Process**

Location: Room 265-268

8:00 AM - 12:00 PM

- Joseph Lee, PhD, Bristol Myers Squibb  
*AAV and Lentiviral vectors: overview of integration, safety, and immunity*
- Jose Cancelas, PhD, MD, Dana-Farber Cancer Institute  
*Lentivirus production for ex vivo vs in vivo applications (emphasis on safety package differences)*
- David Williams, MD, Boston Children's Hospital  
*Translating data from efficacy and safety to clinical plan/first in-human dose dose selection*
- Erik Splinter, Cergentis  
*Lentivirus integration and mechanisms; targeted locus amplification platform*
- Lauren Gauthier  
*Lentivirus - CMC Activities and Assessments*

- Stephen Russell, MD, PhD, Vyriad  
*Lentivirus - adverse outcomes of lentivirus modified cells*

Oral Abstract Session

**Oral Abstract Session**

Location: NOLA A  
1:30 PM - 3:15 PM

Oral Abstract Session

**Oral Abstract Session**

Location: NOLA B  
1:30 PM - 3:15 PM

Oral Abstract Session

**Oral Abstract Session**

Location: NOLA C  
1:30 PM - 3:15 PM

Oral Abstract Session

**Oral Abstract Session**

Location: Room 265-268  
1:30 PM - 3:15 PM

Oral Abstract Session

**Oral Abstract Session**

Location: Room 278-282  
1:30 PM - 3:15 PM

Oral Abstract Session

**Oral Abstract Session**

Location: Room 288-290  
1:30 PM - 3:15 PM

Oral Abstract Session

**Oral Abstract Session**

Location: Room 291-292  
1:30 PM - 3:15 PM

Scientific Symposium

**Who's Afraid Of Off-Target Editing? A Discussion Of Hypothetical Risks In The Context Of Known Genotoxic Medical Interventions**

Location: Room 293-296  
1:30 PM - 3:15 PM

- Claire Clelland, UCSF  
*Considering off-target editing in the context of other genotoxic medical interventions*

- Petros Giannikopoulos  
*Case example: Ionizing radiation for childhood medulloblastoma*
- Krishanu Saha, PhD, University of Wisconsin-Madison  
*Patient perspectives on gene editing and off-target editing*
- Kiran Musunuru, MD, PhD, MPH, ML, MRA, University of Pennsylvania  
*Framework for considering which off-target edits are of concern*

General Session

**Founders, Mendell, and Catalyst Award Symposium**

Location: Hall F

3:45 PM - 5:30 PM

Reception

**Welcome Reception**

Location: Exhibit Hall

5:30 PM - 7:30 PM

Poster Abstract Session

**Tuesday Poster Reception**

Location: Poster Hall (Hall I2)

6:00 PM - 7:30 PM

## Wednesday, May 14

Oral Abstract Session

**Oral Abstract Session**

Location: NOLA A

8:00 AM - 9:45 AM

Scientific Symposium

**Advances In Genome Editing: Novel Large DNA Insertion Technologies And Their Potential Towards Curative Therapies (Organized by the Genome Editing Committee)**

Location: NOLA Theater B

8:00 AM - 9:45 AM

- Frank Buchholz, PhD, TU Dresden  
*Zinc finger recombinases*

- Matthew Durrant, Arc Institute  
*Bridge recombinases*
- Grégoire Cullot, ETH Zurich  
*HDR mechanisms*
- Gabriel Cohn, iECURE  
*ARCUS in vivo OTC, large gene insertion (clinical stage program)*

Scientific Symposium

**Hot Topics in Molecular Therapy I**

Location: NOLA Theater C

8:00 AM - 9:45 AM

Scientific Symposium

**To Biomarker Or Not To Biomarker? Use Considerations For CGT Drug Development (Organized by the Patient Outreach Committee)**

Location: Room 265-268

8:00 AM - 9:45 AM

- Rebecca Ahrens-Nicklas, PhD, MD, Children's Hospital of Philadelphia  
*Investigators guide to identifying and validating biomarkers for clinical trials*
- Cara O'Neill, MD, Cure Sanfilippo Foundation  
*Case study of how CSF biomarkers were approved by the FDA in MPS*
- Kevin Flanigan, MD, Center for Gene Therapy, Nationwide Children's Hospital  
*Case study of how biomarkers may be limited in DMD*

Scientific Symposium

**CNS Gene Delivery for Metabolic Diseases: History and Challenges (Organized by the Genetic and Metabolic Diseases Committee)**

Location: Room 278-282

8:00 AM - 9:45 AM

- Ronald Crystal, Weill Cornell Medical College  
*History of access to the CNS for genetic therapies for metabolic disorders*



- Paul Orchard, University of Minnesota, Blood and Marrow Transplant & Cellular Therapy  
*HSCs targeting the CNS for ALD*
- Bryan Simpson, Latus Bio  
*AAV delivery to brain, peptide insertions, optimizing capsid delivery*
- Olivier Danos, PhD, REGENXBIO  
*Dosing into the CNS based on brain weight vs body weight*

#### Scientific Symposium

### **Interdisciplinary Approaches: Combining Gene Therapy with Other Fields (Organized by the New Investigator Committee)**

Location: Room 291-292

8:00 AM - 9:45 AM

- James Dahlman, PhD, Georgia Tech  
*Nanotechnology: Discussing how nanoparticles are being used for more efficient gene delivery systems*
- Fyodor Urnov, University of California, Berkeley  
*General Overview of Combining Tech*
- Le Cong, PhD, Stanford University  
*Artificial Intelligence: Exploring how machine learning algorithms are helping to predict off-target effects in gene editing or optimize vector designs*
- Shivani Srivastava, Fred Hutch  
*Immunology: Investigating the integration of gene therapy with immunotherapy approaches for cancer treatment*

#### Oral Abstract Session

### **Oral Abstract Session**

Location: Room 293-296

8:00 AM - 9:45 AM

Scientific Symposium

**Past, Present and Future of Genetic Testing (Organized by the Ethics Committee)**

Location: Room 388-390

8:00 AM - 9:45 AM

- Terry Pirovolakis, Elpida Therapeutics SPC  
*The diagnostic journey in rare disease*
  
- Sharon Terry, MA, Genetic Alliance  
*Genetic Testing in 202 – Rare Disease and preventative routine screening*
  
- Tippi MacKenzie, MD, University of California San Francisco  
*Interpretation of genetic test results*
  
- Lindsey Byrne, OSUMC  
*Broader applications of genetic testing: Cancer and beyond*

Fireside Chat

**Fireside Chat: Global Regulatory Convergence**

Location: Room 393-396

8:00 AM - 9:45 AM

- Ian Alexander, Sydney Children's Hospitals Network  
*Australasian Perspective on Global Regulatory Convergence*
  
- Sol Ruiz, PhD, AEMPS (Spanish Medicines Agency)  
*Europe Perspective Global Regulatory Convergence*

General Session

**Presidential Symposium**

Location: Hall F

10:15 AM - 12:00 PM

- Drew Weissman, University of Pennsylvania  
*Nucleoside-modified mRNA-LNP Therapeutics*

- MIKE Mccune, Bill & Melinda Gates Foundation  
*Presidential Symposium Keynote*

Networking

**Post-General Session Networking**

Location: TBD

12:00 PM - 1:00 PM

Poster Talk Session

**Wednesday Poster Talk**

Location: Exhibit Theater

12:15 PM - 1:00 PM

Oral Abstract Session

**Oral Abstract Session**

Location: NOLA A

1:30 PM - 3:15 PM

Oral Abstract Session

**Oral Abstract Session**

Location: NOLA B

1:30 PM - 3:15 PM

Oral Abstract Session

**Oral Abstract Session**

Location: NOLA C

1:30 PM - 3:15 PM

Oral Abstract Session

**Oral Abstract Session**

Location: Room 265-268

1:30 PM - 3:15 PM

Scientific Symposium

**Targeted Delivery Of Oligonucleotides (Organized by the Oligonucleotide and RNAi Therapeutics Committee)**

Location: Room 278-282

1:30 PM - 3:15 PM

- Marcin Kortylewski, PhD, City of Hope Comprehensive Cancer Center  
*Challenges of different types of oligonucleotide therapeutics/approaches and how to address*
  
- Annalisa Di Ruscio, Beth Israel Deaconess Medical Center - Harvard Medical School  
*Aptamer conjugates, new intracellular targets*

- Ethan Lippmann, Vanderbilt University  
*Lipid-siRNA conjugates for targeted delivery to CNS*
- Chuong Hoang, National Cancer Institute (NIH)  
*Localized miRNA delivery for mesothelioma delivery*
- Oxana Beskrovnaya, PhD, Dyne Therapeutics  
*Transferring ASO for myotonic dystrophy (DM1)*

Oral Abstract Session

**Oral Abstract Session**

Location: Room 288-290

1:30 PM - 3:15 PM

Oral Abstract Session

**Oral Abstract Session**

Location: Room 291-292

1:30 PM - 3:15 PM

Education Session

**Preclinical Models for Gene and Cell Therapies: From Rodents to Pigs and Non-Human Primates (Organized by the Education Committee)**

Location: Room 293-296

1:30 PM - 3:15 PM

- Daniel Carlson, Recombinetics  
*Gene-edited pigs for human diseases*
- Dmitry Shayakhmetov, PhD, Emory University  
*Addressing the viral vector safety in advanced preclinical models (e.g., mice, human blood, non-human primates)*
- Melanie Graham, University of Minnesota  
*Non-human primates (Stem cell therapies, diabetes, transplantation, regulatory guidance for pre-clinical models)*

Tools & Technology Session

**Tools and Technology Forum 1**

Location: Exhibit Theater

1:30 PM - 3:15 PM

Tools & Technology Session

**Tools and Technology Forum 2**

Location: Exhibit Theater

3:45 PM - 5:15 PM

Oral Abstract Session

**Oral Abstract Session**

Location: NOLA A

3:45 PM - 5:30 PM

Oral Abstract Session

**Oral Abstract Session**

Location: NOLA B

3:45 PM - 5:30 PM

Oral Abstract Session

**Oral Abstract Session**

Location: NOLA C

3:45 PM - 5:30 PM

Oral Abstract Session

**Oral Abstract Session**

Location: Room 265-268

3:45 PM - 5:30 PM

Oral Abstract Session

**Oral Abstract Session**

Location: Room 278-282

3:45 PM - 5:30 PM

Oral Abstract Session

**Oral Abstract Session**

Location: Room 288-290

3:45 PM - 5:30 PM

Oral Abstract Session

**Oral Abstract Session**

Location: Room 291-292

3:45 PM - 5:30 PM

Education Session

**Bridging Research and Medicine: The Path to Becoming a Translational-Scientist  
(Organized by the Education Committee)**

Location: Room 293-296

3:45 PM - 5:30 PM

- Allyson Berent, DVM, DACVIM, Foundation for Angelman Syndrome Therapeutics  
*Understanding patients' and caregivers' lives and needs*

- Michael Deininger  
*Maintaining an applied and translational research lab in academics*
- Mary Eapen, Medical College of Wisconsin  
*Academia and industry collaboration to advance patient care through early clinical trials*

Scientific Symposium

**CDA/DEI Awards 1**

Location: Room 383-385

3:45 PM - 5:30 PM

- Leonid Cherkassky, Roswell Park Comprehensive Cancer Center  
*Inducible negative feedback expression circuits to generate self-tuning, exhaustion resistant CAR T cells*
- Ashley Cooney, PhD, University of Iowa  
*Increasing saline tonicity enhances viral gene transfer in airway epithelia*
- Lukas Landegger, MD, PhD, MBA, Stanford University  
*AAV-mediated gene therapy to target vestibular schwannoma*

Scientific Symposium

**Targeting Myocardium: To The Heart Of The Matter (Organized by the Cardiovascular CGT Committee)**

Location: Room 389-390

3:45 PM - 5:30 PM

- Tamir Mohammed, Baylor  
*Human heart tissue slices, utilizing as model system to evaluate efficacy of viral vectors for diff cardiac diseases*
- Camila Hochman-Mendez, MSc, PhD, Texas Heart Institute  
*Reengineering the heart using iPSCs*

- Daniel Blessing, HAYA Therapeutics  
*Targeting Fibrosis with ASO in CV disease and Heart Failure, large and small animal models*
- Dirk Grimm, PhD, Heidelberg University Hospital  
*Cardiac targeting by viral vectors*

#### Scientific Symposium

### **Overcoming Challenges in Efficient Delivery of Gene Therapy to Muscle and Bone (Organized by the Musculoskeletal Gene and Cell Therapy Committee)**

Location: Room 391-392

3:45 PM - 5:30 PM

- Christopher Evans, Mayo Clinic  
*Gene delivery to chondrocytes (including systemic aspect)*
- Hichem Tasfaout, PhD, University of Washington  
*Split intein-mediated protein trans-splicing to express large dystrophins*
- Patricia Lam, PhD, Nationwide Children's Hospital  
*Dual FKRP/FST gene therapy LGMDR9 mice*
- Mahasweta Girgenrath, Entrada Therapeutics Inc  
*Clinical Trial of ENTR-601-44, an Endosomal Escape Vehicle (EEV)-conjugated PMO, for the Treatment of Duchenne Muscular Dystrophy*

#### Fireside Chat

### **Fireside Chat: Reviving Hope in Deprioritized Cell and Gene Therapy Programs**

Location: Room 393-396

3:45 PM - 5:30 PM

- Oralea Marquardt, National Tay-Sachs & Allied Diseases Association  
*Patient Advocate Perspective on Dropped Gene Therapies: Challenges and Pathways Forward*
- Donald Kohn, University of California, Los Angeles  
*Academic Researcher Perspective on Dropped Gene Therapies: Challenges and Pathways Forward*

- Sukumar Nagendran, MD, Taysha Gene Therapies  
*Industry Perspective on Dropped Gene Therapies: Challenges and Pathways Forward*
- Claire Booth, UCL Great Ormond Street Institute of Child Health  
*Picking Up Dropped Gene Therapies for Further Development: Challenges and Pathways Forward*

Poster Abstract Session

### **Wednesday Poster Reception**

Location: Poster Hall (Hall I2)

5:30 PM - 7:00 PM

## Thursday, May 15

Scientific Symposium

### **Improved Therapeutic Delivery of Nanoparticles (Organized by the Nonviral Therapeutic Delivery Committee)**

Location: NOLA Theater A

8:00 AM - 9:45 AM

- Anna Blakney, University of British Columbia - Vancouver  
*Self amplifying RNA and polymeric NPs; strategies to reduce immunogenicity of LNPs*
- Bowen Li, University of Toronto  
*AI-Guided Ionizable Lipid Engineering (AGILE)*
- Richard Price, PhD, University of Virginia  
*FUS-mediated Brain Delivery of NPs and LNPs, Tumor Genome Editing and ECs Transfection*
- Avi Schroeder  
*Advanced approaches to the design of brain-targeted liposomes*

Scientific Symposium

### **Novel Approaches To Overcome Limits Of Therapeutic Transgene Delivery And**



## **Durability (Organized by the Viral Gene Transfer Vectors Committee)**

Location: NOLA Theater B

8:00 AM - 9:45 AM

- Sidi Chen, Yale University  
*AAV-transposon combination for CAR T and CAR NK development*
- Zheng-Yi Chen, Massachusetts Eye & Ear Infirmary  
*Dual AAV vectors, clinical trial data for hearing loss*
- Isabelle Richard, PhD, GENETHON  
*Dual AAV for muscular dystrophy*
- Mansuo Shannon, AskBio  
*AI use for promotor and transgene design*

Oral Abstract Session

### **Oral Abstract Session**

Location: NOLA C

8:00 AM - 9:45 AM

Scientific Symposium

### **Challenges and Opportunities for Developing Cell and Gene Therapies in LMICs (Organized by the Global Outreach Committee)**

Location: Room 265-268

8:00 AM - 9:45 AM

- Carlos Javier Alméciga-Díaz, BPharm, Ph.D., Pontificia Universidad Javeriana  
*LMIC manufacturing considerations*
- Martín Bonamino, PhD, INCA  
*Academic collaboration for rapid deployment of genetic therapy*
- Francielli Melo, ANVISA  
*Regulatory considerations for the advancement of cell and gene therapies.*

- John Tisdale, MD, NIH, NHLBI  
*Running clinical trials in LMICs*

Scientific Symposium

**Publishing 101**

Location: Room 278-282

8:00 AM - 9:45 AM

Scientific Symposium

**Translational Strategies for Gene and Cell Therapies in Inherited Neurotransmitter Disorders**

Location: Room 288-290

8:00 AM - 9:45 AM

- Steven Gray, PhD, University of Texas Southwestern  
*Transgene regulation strategies for dose-sensitive genes involved with neurodevelopmental disorders*
  
- Brad Elder  
*Gene Therapy for AADC*
  
- Dimitri Kullmann, University College London  
*Self-regulated closed-loop gene therapy for disorders of circuit excitability*
  
- Cory Nicholas, Neurona Therapeutics  
*First-in-human clinical investigation of GABAergic inhibitory interneuron cell therapy for drug-resistant epilepsy*

Oral Abstract Session

**Oral Abstract Session**

Location: Room 291-292

8:00 AM - 9:45 AM

Scientific Symposium

**Comparing Treatment Modalities for Neurologic Diseases: Insights and Preliminary Clinical Observations (Organized by the Neurologic and Ophthalmic Committee)**

Location: Room 293-296

8:00 AM - 9:45 AM

- Fyodor Urnov, University of California, Berkeley  
*Overview of therapeutic genetic modalities for CNS disease treatment*

- Aimee Jackson, Atalanta Therapeutics  
*IT di-siRNA for the treatment of Huntington's disease: preclinical safety and pharmacology with clinical dose projections*
- Holly Brothers, Biogen  
*BIIB080: the development of a tau-targeting antisense oligonucleotide in early AD*
- Rajeev Sivasankaran  
*IV-delivered AAV gene therapy targeting tau for the treatment of Alzheimer's disease: preclinical safety and pharmacology with translational considerations*

Scientific Symposium

**Cellular and Gene Therapies for Autoimmune Disease (Organized by the Hematologic and Immunologic Gene and Cell Therapy Committee)**

Location: Room 389-390

8:00 AM - 9:45 AM

- Lili Yang, PhD, University of California Los Angeles  
*Overview of different gene and cell therapy approaches to auto-immunity, considerations around for immune reset*
- Jenell Volkov, PhD, Cabaletta Bio  
*CAR-T in AID clinical experience with an understanding of potential safety issues and considerations for the autoimmune patient population (Focus on pathway to broader AID indications)*
- Laurie Kenney, Moderna Therapeutics  
*in vivo mRNA delivery of IDO1 for autoimmunity and aGVHD*
- Simön Volkl, Friedrich-Alexander University Erlangen-Nuremberg  
*Comparison of the Immune and Safety profile of CD19 CAR T-cell therapy in patients with SLE and B-cell lymphoma*

Fireside Chat

## **Funding the Future of Cell and Gene Therapy Development**

Location: Room 393-396

8:00 AM - 9:45 AM

- Mimi Lee, ARPA-H  
*Reimagining CGT Development in ARPA-H's Novel Funding Models for High-Impact Solutions*
- Philip Brooks, PhD, NIH / NCATS  
*How NIH and Federally-funded Research Support Innovative CGT Approaches*
- Deborah Palestrant, 5AM Ventures  
*Beyond Traditional Metrics: A Venture Perspective on Valuing and Funding Next-Generation CGTs*

General Session

### **George Stamatoyannopoulos Memorial Lecture**

Location: Exhibit Theater

10:15 AM - 12:00 PM

- Kiran Musunuru, MD, PhD, MPH, ML, MRA, University of Pennsylvania  
*Gene Therapy for N1 Cardiovascular Disease*
- Tippi MacKenzie, MD, University of California San Francisco  
*Prenatal Gene Therapy*

Networking

### **Post-General Session Networking**

Location: TBD

12:00 PM - 1:00 PM

Poster Talk Session

### **Thursday Poster Talk**

Location: Exhibit Theater

12:15 PM - 1:00 PM

Oral Abstract Session

### **Oral Abstract Session**

Location: NOLA A

1:30 PM - 3:15 PM

Oral Abstract Session

**Oral Abstract Session**

Location: NOLA B  
1:30 PM - 3:15 PM

Oral Abstract Session

**Oral Abstract Session**

Location: NOLA C  
1:30 PM - 3:15 PM

Oral Abstract Session

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1:30 PM - 3:15 PM

Oral Abstract Session

**Oral Abstract Session**

Location: Room 288-290  
1:30 PM - 3:15 PM

Scientific Symposium

**The Basics of Building Your Own Biotech Company (Organized by the Trainee Committee)**

Location: Room 291-292  
1:30 PM - 3:15 PM

- Margaret Barkett, PhD, Nationwide Children's Hospital  
*Intellectual property from an academic perspective, define what IP is, what you need in taking your tech out of Academia, technology transfer*
  
- Michael Poisel, PCI Ventures  
*Venture Studio/Incubator*
  
- Jennifer Hamilton, Azalea Therapeutics  
*Young Founder, How they pitched their ideas and built their team*

Oral Abstract Session

**Oral Abstract Session**

Location: Room 293-296  
1:30 PM - 3:15 PM

Tools & Technology Session

**Tools and Technology Forum 3**

Location: Exhibit Theater

1:30 PM - 3:45 PM

Networking

**Patient Advocate Meet-Up**

Location: TBD

3:15 PM - 4:15 PM

Networking

**Trainee and New Investigator Meet-up**

Location: TBD

3:15 PM - 4:15 PM

Tools & Technology Session

**Tools and Technology Forum 4**

Location: Exhibit Theater

3:45 PM - 5:15 PM

Oral Abstract Session

**Oral Abstract Session**

Location: NOLA A

3:45 PM - 5:30 PM

Oral Abstract Session

**Oral Abstract Session**

Location: NOLA B

3:45 PM - 5:30 PM

Oral Abstract Session

**Oral Abstract Session**

Location: NOLA C

3:45 PM - 5:30 PM

Scientific Symposium

**The Coalition of International Gene Therapy Societies Showcases: Moving from ex vivo Cell Therapies to in vivo**

Location: Room 265-268

3:45 PM - 5:30 PM

- Adi Barzel, Tel Aviv University  
*In vivo targeting, include work on B cells*
  
- Philip Johnson, MD, Interius Biotherapeutics, Inc  
*Investigational in vivo CAR-T therapy designed to treat B-cell malignancies*

- Christian Buchholz, Paul-Ehrlich-Institut  
*In vivo generation of CAR T cells selectively in human CD4+ lymphocytes*
- Michela Milani, PhD, SR-TIGET  
*In vivo HSCs*

Scientific Symposium

**FDA's START Pilot Program in Action: Insights from Year One (Organized by the Regulatory Affairs Committee)**

Location: Room 278-282

3:45 PM - 5:30 PM

- Rachel McMinn, PhD, Neurogene  
*Neurogene's experience with the START Pilot Program in advancing their NGN-401 product for Rett Syndrome*
- Susan Telliard, Moderna Therapeutics  
*Moderna's experience with the START Pilot Program in advancing their mRNA-3705 product for Isolated methylmalonic acidemia due to complete or partial methylmalonyl-coenzyme A mutase deficiency*
- Adrian Stecyk  
*Myrtelle's experience with the START Pilot Program in advancing their rAAV-Olig001-ASPA product for Canavan Disease*

Oral Abstract Session

**Oral Abstract Session**

Location: Room 288-290

3:45 PM - 5:30 PM

Oral Abstract Session

**Oral Abstract Session**

Location: Room 291-292

3:45 PM - 5:30 PM

Education Session

**Advanced Clinical Trials and Long-Term Follow-Up: Striking the Balance Between Safety and Efficacy (Organized by the Education Committee)**

Location: Room 293-296

3:45 PM - 5:30 PM

- David Wilcox, PhD, Medical College of Wisconsin  
*Follow-up study of gene therapy in hemophilia A*
- Yuman Fong, MD, City of Hope  
*Oncolytic Viruses: Balancing Between Safety and Efficacy (Clinical Perspective)*
- Ramon Alemany, IDIBELL - Institut Catala D'Oncologia  
*Updates on European clinical trials with Oncolytic Adenoviruses*

Scientific Symposium

**CDA/DEI Awards 2**

Location: Room 383-385

3:45 PM - 5:30 PM

- Tomas Gonzalez Fernandez, Lehigh University  
*Novel Cell Penetrating Peptide for Multimodal CRISPR Gene Editing of Primary Mesenchymal Stromal Cells*
- Pradip Bajgain, PhD, National Cancer Institute (NIH)  
*Engineering chimeric antigen receptors to alleviate tonic signaling*
- Ngoc Tam Tran, PhD, University of Massachusetts Medical School  
*Analyzing the Integration of AAV Vectors Carrying Truncated Inverted Terminal Repeats in the Genomes of Transduced Cells*

Scientific Symposium

**Unique Biologic Opportunities to Treat Monogenic Blood Disorders Prenatally  
(Organized by the Prenatal Gene and Cell Therapy Committee)**

Location: Room 391-392

3:45 PM - 5:30 PM

- Christopher Porada, Wake Forest Institute for Regenerative Medicine  
*Introduction talk regarding successes in the field and clinical trials*
- Agnieszka Czechowicz, Stanford School of Medicine  
*Cell Therapy - Learnings from SCID/Alpha Thal towards Fanconi Anemia*



- Panicos Shangaris, Department of Women and Children's Health  
*LNPs/non viral deliveries for prenatal*
- R. Alta Charo, University of Wisconsin Law School  
*Short presentation regarding ethics (~10 minutes)*

Oral Abstract Session

**Oral Abstract Session**

Location: Room 393-396

3:45 PM - 5:30 PM

Poster Abstract Session

**Thursday Poster Reception**

Location: Poster Hall (Hall 12)

5:30 PM - 7:00 PM

Reception

**Women in Gene and Cell Therapy Reception**

Location: TBD

6:30 PM - 8:00 PM

## Friday, May 16

Scientific Symposium

**Tissue-Specific Insights in AAV Vector Immunogenicity (Organized by the Immune Responses to Gene and Cell Therapy Committee)**

Location: NOLA Theater A

8:00 AM - 9:45 AM

- Mark Brimble, PhD, St. Jude Children's Research Hospital  
*Introduction to immunogenicity of AAV vectors*
- Carsten Bonnemann, MD, NINDS/NIH  
*Liver toxicities in AAV trials in particular MTM1, focusing on immune responses*
- Alison Clare, PhD, University of Bristol  
*Age and sex impact on ocular toxicity, intravitreal*

- Anna Kajaste-Rudnitski, PhD, University of Pavia  
*Innate immune sensing of AAV in CNS cells*

Scientific Symposium

### **Reprogramming Immune Cells To Improve Therapeutic Responses And Indications For Cancer (Organized by the Cancer CGT Committee)**

Location: NOLA Theater B

8:00 AM - 9:45 AM

- Franziska Blaeschke, German Cancer Research Center  
*New trends exploring non-viral gene editing for T cell engineering*
  
- Luca Gattinoni, Leibniz Institute for Immunotherapy  
*Stem t cells improving responses, CD8, clinical trial*
  
- Michael Klichinsky, Carisma Therapeutics  
*CAR Macrophages*
  
- Jesus Corria  
*Next generation TIL therapies*

Scientific Symposium

### **Hot Topics in Molecular Therapy II**

Location: NOLA Theater C

8:00 AM - 9:45 AM

Scientific Symposium

### **The Coalition of International Gene Therapy Societies Showcases: Clinical Trials Around the Globe**

Location: Room 293-296

8:00 AM - 9:45 AM

- Ryuichi Morishita, Center of Medical Innovation & Translational Research  
*HGF gene therapy- Phase II data in USA, and FDA approved Breakthrough therapy*
  
- Alberto Auricchio, PhD, MD, TIGEM, Telethon Institute of Genetics and Medicine  
*Clinical Trials of AAV gene therapy directed to liver and retina*

- Leszek Lisowski, Children's Medical Research Institute  
*Clinical trial for OTC Deficiency*
- Rahul Purwar, Indian Institute of Technology Bombay  
*CAR T trials*

Oral Abstract Session

**Oral Abstract Session**

Location: Room 278-282

8:00 AM - 9:45 AM

Scientific Symposium

**Ensuring Diversity and Inclusion in Later Stage Cell and Gene Therapy Development  
(Organized by the Diversity, Equity, and Inclusion Committee)**

Location: Room 288-290

8:00 AM - 9:45 AM

- Pat Furlong, Parent Project Muscular Dystrophy (PPMD)  
*The Patient's Role in Expanding the Reach of Advanced Therapies in the Post-Approval Period*
- Elizabeth Cohn, Northwell Health  
*Bridging Gaps: Ensuring/advancing/fostering Equity and Inclusion in the Post-Approval Landscape of Cell and Gene Therapies*
- Olajide Williams, Columbia University  
*Access and globalization of GCTs; how approved therapies are reaching patients in leading markets {the US, Europe} (evaluated through a DEI lens); The promise of one and done therapies in developing countries and the significant challenges to bring them there*

Scientific Symposium

**Harnessing Cell and Gene Therapies: New Frontiers in Kidney Treatment**

Location: Room 291-292

8:00 AM - 9:45 AM

- Rajiv Patni, Judo Bio  
*siRNA mediated therapies for kidney and systemic diseases*

- Poulami Chaudhuri, Helex  
*Non-Viral Gene Editing for Autosomal Dominant Polycystic Kidney Disease1*
- Moin Saleem, University of Bristol Medical School  
*Gene Therapies targeting the podocyte*
- Sarah Thompson, Vertex  
*School Overcoming Challenges in Clinical Translation of Kidney-Targeted Cell and Gene Therapies*

Scientific Symposium

**Bringing Scientific Communications from the Bench to the Bedside (Organized by the Communications Committee)**

Location: Room 389-390

8:00 AM - 9:45 AM

- Kimberly Haugstad, RareRising  
*Engaging in Patient-Centered Scientific Communication*
- Allyson Berent, DVM, DACVIM, Foundation for Angelman Syndrome Therapeutics  
*Translating Clinical Developments for Families and Patients with Rare Diseases*
- Gwendolyn Wu, BioPharma Dive  
*Learning to Communicate Complex Science to a Broad Lay Audience*
- Benjamin McLeod, Convey Bio  
*Communicating Complex Science on Social Media*

Scientific Symposium

**Perspectives on Successful Translation from Bench to Bedside (Organized by the Bio-Industry Committee)**

Location: Room 393-396

8:00 AM - 9:45 AM

- Uta Griesenbach, Imperial College Faculty of Medicine  
*The pathway to bringing gene and cell therapies from bench to bedside*

- Leslie Meltzer, PhD, Orchard Therapeutics  
*The pathway to bringing gene and cell therapies from bench to bedside*
- David Schaffer, University of California Berkeley  
*The pathway to bringing gene and cell therapies from bench to bedside*
- J. Fraser Wright, Kriya Therapeutics  
*The pathway to bringing gene and cell therapies from bench to bedside*

General Session

**Outstanding New Investigator Symposium**

Location: Hall F  
10:15 AM - 12:00 PM

Oral Abstract Session

**Oral Abstract Session**

Location: NOLA A  
1:30 PM - 3:15 PM

Scientific Symposium

**CMC Harmonization for Advanced Therapies (Organized by the Chemistry, Manufacturing, Controls (CMC) Committee)**

Location: NOLA Theater B  
1:30 PM - 3:15 PM

- Yoko Momonoi, Takeda  
*Introductory overview of some of the key differences across global jurisdictions (e.g. raw materials, donor eligibility, facility design)*
- Jim Sestic, Atara Biotherapeutics, Inc.  
*Case studies developing the same product in different regions*
- Varija Verma, Vertex  
*Case studies developing the same product in different regions*

- Lynelle Hoch, Bristol Myers Squibb  
*Case studies developing the same product in different regions*

Oral Abstract Session

**Oral Abstract Session**

Location: NOLA C  
1:30 PM - 3:15 PM

Scientific Symposium

**Next Generation Strategies For Evading Immunity In Stem Cell Therapies (Organized by the Stem Cell Committee)**

Location: Room 278-282  
1:30 PM - 3:15 PM

- Sonja Schrepfer, PhD, Sana  
*Hypo-Immune cells*
  
- Deepta Bhattacharya, University of Arizona  
*Engineering pluripotent stem cells to evade and promote immunity*
  
- Andras Nagy, PhD, Lunenfeld-Tanenbaum Research Institute at Mount Sinai Hospital  
*Immune privileged/immune cloaking*
  
- Xiaokou Zhang  
*Autologous iPSc derived dopaminergic neurons*

Oral Abstract Session

**Oral Abstract Session**

Location: Room 288-290  
1:30 PM - 3:15 PM

Oral Abstract Session

**Oral Abstract Session**

Location: Room 291-292  
1:30 PM - 3:15 PM

Education Session

**Development of AI Technologies for Cell and Gene Therapies (Organized by the Education Committee)**

Location: Room 293-296  
1:30 PM - 3:15 PM

- Benjamin Deverman, PhD, Broad Institute  
*Utilizing AI in Academic Labs*
- Victor Dzau, Duke University Hospital  
*AI application in gene therapy and gene editing*
- Michelle Lee, Medra  
*Embodied AI for Biology Labs*

Oral Abstract Session

**Oral Abstract Session**

Location: Room 391-392  
1:30 PM - 3:15 PM

Oral Abstract Session

**Oral Abstract Session**

Location: Room 393-396  
1:30 PM - 3:15 PM

Tools & Technology Session

**Tools and Technology Forum 5**

Location: Exhibit Theater  
1:30 PM - 3:15 PM

Scientific Symposium

**Annual meeting of the Accelerating Medicines Partnership® Bespoke Gene Therapy Consortium (AMP® BGTC)**

Location: Room 265-268  
1:30 PM - 5:30 PM

Scientific Symposium

**Targeted Nanosystems For Gene Transfer And Editing: Beyond Delivery To The Liver (Organized by the Nanoagents and Synthetic Formulations Committee)**

Location: NOLA Theater B  
3:45 PM - 5:30 PM

- Kerry Benenato, Moderna Therapeutics  
*Targeted nonviral delivery of genome editors in vivo*
- Priya Karmali, Capstan Therapeutics  
*In vivo immune cell engineering using targeted nanoparticles*

- David Oupicky, University of Nebraska Medical Center  
*siRNA delivery to kidney with nanoparticles*
- Connor Tsuchida, Stealth Startup  
*Targeted nonviral delivery of genome editors in vivo*

Scientific Symposium

**CDA/DEI Awards 3**

Location: Room 383-385

3:45 PM - 5:30 PM

- Denise Klatt, PhD  
*Engineering alpha-retroviral-like particles for safe and efficient in vivo hematopoietic stem cell gene therapy*
- Gabriele Casirati, MD, PhD, Boston Children's Hospital  
*Base Editors with Single-Base Selectivity to Minimize Bystander and Off-target Effects*
- Minsun Song, PhD, City of Hope  
*Transferrin receptor-targeted RNA aptamer enhanced blood-brain barrier penetration in brain metastases occurring from Triple-negative breast cancer*
- Sandhiya Ravi, PostDoctoral Fellow, Umass Medical School  
*Enhancing Truncation Event Prediction in AAV Vector Genome Designs through Advanced Deep Learning Techniques*
- Jose Martinez-Navio, PhD, University of Miami  
*Preventing humoral responses to AAV-delivered anti-HIV antibodies by transient blockage of CD4 in rhesus macaques.*

Scientific Symposium

**U.S. Private Payment Challenges for CGTs (Organized by the Government Relations Committee)**

Location: Room 389-390

3:45 PM - 5:30 PM



- Claire White  
*The Real-World Impacts of Private Payer Policies on Access: A Hospital System Perspective*
  
- Luke Prettol, AT&T  
*How Employers Consider CGT Coverage in Private Plans*
  
- Kelly Maynard, Little Hercules Foundation  
*Centering the Patient Voice in CGT Coverage Decisions*
  
- Kevin Faber, Sanford Health Plan  
*Payer Coverage Challenges with Emerging CGT Products*

Oral Abstract Session

**Oral Abstract Session**

Location: NOLA A  
3:45 PM - 5:40 PM

Oral Abstract Session

**Oral Abstract Session**

Location: NOLA C  
3:45 PM - 5:40 PM

Oral Abstract Session

**Oral Abstract Session**

Location: Room 278-282  
3:45 PM - 5:40 PM

Oral Abstract Session

**Oral Abstract Session**

Location: Room 288-290  
3:45 PM - 5:40 PM

Oral Abstract Session

**Oral Abstract Session**

Location: Room 291-292  
3:45 PM - 5:40 PM

Oral Abstract Session

**Oral Abstract Session**

Location: Room 293-296  
3:45 PM - 5:40 PM

Oral Abstract Session

**Oral Abstract Session**

Location: Room 391-392

3:45 PM - 5:40 PM

Oral Abstract Session

**Oral Abstract Session**

Location: Room 393-396

3:45 PM - 5:40 PM

Reception

**Closing Night Reception (Separate Purchase Required)**

Location: Mardi Gras World

7:00 PM - 10:00 PM

## Saturday, May 17

Business Meeting

**ASGCT Business Meeting**

Location: Exhibit Hall

7:00 AM - 8:00 AM

Oral Abstract Session

**Oral Abstract Session**

Location: NOLA A

8:00 AM - 9:45 AM

Oral Abstract Session

**Oral Abstract Session**

Location: NOLA B

8:00 AM - 9:45 AM

Oral Abstract Session

**Oral Abstract Session**

Location: NOLA C

8:00 AM - 9:45 AM

Oral Abstract Session

**Oral Abstract Session**

Location: Room 278-282

8:00 AM - 9:45 AM

Oral Abstract Session

**Oral Abstract Session**

Location: Room 288-290

8:00 AM - 9:45 AM

Oral Abstract Session

**Oral Abstract Session**

Location: Room 291-292

8:00 AM - 9:45 AM

Scientific Symposium

**Expanding Modalities Beyond Their Initial Use: Discussions on Translating Immune Cell Therapies into Non-Oncology Indications and AAV Gene Therapies into Oncology Indications (Organized by the Translational Science Committee)**

Location: Room 293-296

8:00 AM - 9:45 AM

- Gwendolyn Binder, Cabaletta Bio  
*CD19 CAR-T for several non-oncology indications: systemic lupus erythematosus, myositis, systemic sclerosis and generalized myasthenia gravis*
- Jeff Bluestone, PhD, Sonoma Biotherapeutics  
*Phase 1/1b clinical trial ongoing with their Treg program in rheumatoid arthritis so can talk about both IND process and trial*
- Vijay Bhoj, MD PhD, University of Pennsylvania  
*CAR-T for alloantibodies prior to organ transplant*
- Nicole Paulk, PhD, Siren Biotechnology  
*SRN-101 universal AAV gene therapy for solid tumors*

Scientific Symposium

**Overcoming Barriers in the Lung and GI Tract: Advances in Gene Delivery, Stem Cells, and Therapeutic Targeting (Organized by the Respiratory and GI Tract Committee)**

Location: Room 383-385

8:00 AM - 9:45 AM

- Alexandra Piotrowski-Daspit, PhD, University of Michigan  
*Multi-organ nucleic acid delivery/ overcoming macrophage phagocytosis*
- Marianne Carlon, KU Leuven  
*Prime editing and novel assessment approaches for gene editing in the lung and GI tract*
- Deborah Gill, University of Oxford  
*Lentiviral gene transfer for CF and SP-B deficiency*

- Trevor Parry, PhD, Krystal Biotech  
*HSV-1 as a vector system for targeting respiratory tissues in genetic pulmonary disease*

Oral Abstract Session

**Oral Abstract Session**

Location: Room 388-390

8:00 AM - 9:45 AM

Oral Abstract Session

**Oral Abstract Session**

Location: Room 391-392

8:00 AM - 9:45 AM

Scientific Symposium

**Advances in RNA and DNA Vaccines for Infectious Diseases (Organized by the Infectious Diseases and Vaccines Committee)**

Location: Room 393-396

8:00 AM - 9:45 AM

- Richard Roden, Johns Hopkins University  
*Phase I clinical trial results for DNA vaccine for HPV16-associated cervical intraepithelial neoplasia (CIN-2/3)*
- Ye Zhang, Arcturus Therapeutics  
*Self-amplifying RNA vaccines (saRNA) for infectious diseases - first approval of saRNA vaccine (Japan) for COVID-19; longer-lasting immunity compared to mRNA vaccines*
- Matthias Schell  
*Rabies virus (RABV) based vaccines for emerging infectious diseases*
- Kizzmekia Corbett, Harvard University  
*mRNA vaccines for infectious diseases — lessons learned from COVID-19 vaccines*

Oral Abstract Session

**Oral Abstract Session**

Location: NOLA A

10:15 AM - 12:00 PM

Oral Abstract Session

**Oral Abstract Session**

Location: NOLA B  
10:15 AM - 12:00 PM

Oral Abstract Session

**Oral Abstract Session**

Location: NOLA C  
10:15 AM - 12:00 PM

Oral Abstract Session

**Oral Abstract Session**

Location: Room 278-282  
10:15 AM - 12:00 PM

Oral Abstract Session

**Oral Abstract Session**

Location: Room 288-290  
10:15 AM - 12:00 PM

Oral Abstract Session

**Oral Abstract Session**

Location: Room 291-292  
10:15 AM - 12:00 PM

Oral Abstract Session

**Oral Abstract Session**

Location: Room 293-296  
10:15 AM - 12:00 PM

Oral Abstract Session

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Location: Room 383-385  
10:15 AM - 12:00 PM

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10:15 AM - 12:00 PM

Oral Abstract Session

**Oral Abstract Session**

Location: Room 391-392  
10:15 AM - 12:00 PM