Accurate as of 4/6/25

Annual Meeting 2025 Program

Tuesday, May 13

Workshop (separate purchase required)

Navigating The Nonclinical Pathway To Clinical Trials For AAV Based Therapeutics

Location: NOLA Theater 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

- Basel Assaf, BVSc, PhD, DACVP, DABT, FIATP, Sanofi
  The design of nonclinical toxicity studies supporting AAV-based gene therapy products

- Joy Cavagnaro, PhD, Access BIO
  Translating data from efficacy and safety to clinical plan/FIH dose selection

- Danielle Brooks, US Food and Drug Administration
  Best practice in pre-IND and IND regulatory writing and interactions (Pre-recorded Presentation)

- 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

- Reena Patel, PhD, Janssen R&D
  Challenges with varying vector quality throughout development

- Steven Gray, PhD, University of Texas Southwestern Medical Center
  Experiences with developing streamlined regulatory paths for rare disease gene therapy.

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, PhD, Georgia Institute of Technology & Emory University
  Sonothermogenetic control of CAR T cells for brain tumor immunotherapy

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

- Thomas Finn, Center for Biologics Evaluation & Research
  High-Level Regulatory Overview: Human- and Animal-Derived Materials; Safety Testing Human Allogenic Cells; Genome Editing; Platform Technology Designation Program; Advanced Manufacturing Technologies Designation Program, Potency Assurance; and Manufacturing Changes and Comparability (Pre-recorded Presentation)

- 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

- Ann Lee, Prime Medicine
  Primed for Regulatory Success: Translating FDA Genome Editing Guidance into Next-Generation Gene Therapies

- 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

- Houman Dehghani, Cabaletta Bio
  Unpacking FDA's Guidance on Manufacturing Changes and Comparability – Case Study on Cabaletta's Approach

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, MBA, 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, MBBS PhD, 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 Zuckerburg 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, PhD, Patient-Centered Outcomes Research Institute
  Learning about PCORI: Funding Opportunities for Rare Disease Researc

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, The Broad Institute of MIT and Harvard
  Systematic Optimization of Prime Editing for the efficient correction of CFTR F508del

- 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

- 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, Takeda
  Lentivirus - CMC Activities and Assessments

- Stephen Russell, MD, PhD, Vyriad
  In vivo cell engineering using targeted lentiviral vectors

Sponsored Symposium

10x Genomics: Behind the Breakthroughs: How Single Cell Omics Are Advancing Cell and Gene Therapies

Location: Room 383-385
12:15 PM - 1:15 PM

- Lindsay Reagan, 10x Genomics
  Sponsor

Oral Abstract Session

AAV Biology and Mechanism

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

Oral Abstract Session

AAV Vector Manufacturing: Process Development

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

Oral Abstract Session

Advances in Gene Therapy of Neurological Diseases in Small Animal Models

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

Oral Abstract Session

Gene and Cell Therapy for Ophthalmic and Auditory Diseases

Location: Room 265-268

1:30 PM - 3:15 PM

Oral Abstract Session
Gene and Cell Therapy for Metabolic Diseases
Location: Room 278-282
1:30 PM - 3:15 PM

Oral Abstract Session
Physical Delivery Methods and DNA/RNA Drug Development
Location: Room 288-290
1:30 PM - 3:15 PM

Oral Abstract Session
Novel CAR-T Engineering Strategies for Hematological Malignancies
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
  Framework for assessing genomic off-targets of CRISPR gene editing therapies in a clinical context

- 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

Sponsored Symposium
Parexel: When Safety Becomes a Value Proposition For CAR-T Trials
Location: Room 383-385
2:00 PM - 2:30 PM

- Nancy Lunney, Parexel
  Sponsor

Sponsored Symposium
OXB: Accelerate Time to Clinic: A Process and Analytics Platform Approach
Location: Room 391-392
2:00 PM - 2:30 PM

- Erin Cangiano
  Sponsor

General Session
Founders, Mendell, and Catalyst Award Symposium
Location: Hall F
3:45 PM - 5:30 PM

- Donald Kohn, Unversity of California, Los Angeles
  Founder's Keynote

- Anne-Virginie Eggimann, Tessera Therapeutics
  Catalyst Keynote

- Harry Malech, MD, National Institute of Health, National Institute of Allergy and Infectious Diseases
  Mendell Keynote

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

Sponsored Symposium
MilliporeSigma: Optimizing AAV Gene Therapies: NGS applications to enhance development, manufacturing, and testing
Location: Room 271-273
8:00 AM - 8:30 AM

- McKenzie Landgraf, MS, MilliporeSigma
  Sponsor

Oral Abstract Session
Insights from AAV Engineering
Location: NOLA Theater 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

- Gregoire Cullot, ETH Zurich
  HDR mechanisms

- Gabriel Cohn, MD, MBA, iECURE
  ARCUS in vivo OTC, large gene insertion (clinical stage program)

Scientific Symposium

Hot Topics in Molecular Therapy I: Gene Therapy Approaches for Immunotherapy

Location: NOLA Theater C
8:00 AM - 9:45 AM

- Maksim Mamonkin, PhD, Baylor College of Medicine
  Feasibility and preclinical efficacy of CD7-unedited CD7 CAR T cells for T cell malignancies

- Emily Daley, University of Pennsylvania
  Tailoring the adjuvanticity of lipid nanoparticles by PEG lipid ratio and phospholipid modifications

- Miranda Meeuwsen, LUMC: Leids Universitair Medisch Centrum
  A library of cancer testis specific T cell receptors for T cell receptor gene

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

- Cherie Fathy, Food and Drug Administration
  What are Biomarkers? A regulator's guide to using biomarkers in an approved therapy (Pre-recorded Presentation)

- 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, MD, University of Minnesota, Blood and Marrow Transplant & Cellular Therapy
  HSCs targeting the CNS for ALD

- Bryan Simpson, PhD, Latus Bio
  AAV delivery to brain, peptide insertions, optimizing capsid delivery

- Olivier Danos, PhD, REGENXBIO Inc.
  RGX-121 (clemidsogene lanparvovec) : an investigational AAV Gene Therapy for the Treatment of Neuronopathic Mucopolysaccharidosis Type II

Scientific Symposium

Clinical Applications of NK Effectors (Organized by the American Society for Transplantation and Cellular Therapy (ASTCT))

Location: Room 288-290
8:00 AM - 9:45 AM

- Jeffrey Miller, MD, University of Minnesota
  Developing NK Cell Therapeutics: Off-The-Shelf NK-CARs and Trispecific Killer Engagers (TriKEs) for Cancer and Autoimmune Disease

- Dan Kaufman, MD, PhD, University of California - San Diego
  Engineered iPSC-derived NK cells with improved anti-tumor activity

- Amir Horowitz, Icahn School of Medicine at Mount Sinai
  Targeting the NKG2A and HLA-E axis to overcome treatment resistance in patients with solid tumors

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, PhD, University of California, Berkeley
  CRISPR Cures from N=1 to N-all: Building a vertically integrated nonclinical platform in pharm/tox, CMC, and regulatory

- 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
Cell Therapy Product Engineering I
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

- Britt Johnson, GeneDx
  Broader Applications of Genetic Testing: Present, Future, and Data sharing

Fireside Chat
Fireside Chat: Global Regulatory Perspectives
Location: Room 393-396
8:00 AM - 9:45 AM

- Ian Alexander, PhD, MD, Sydney Children's Hospitals Network
  Australasian Perspective on Global Regulatory Convergence

- Yoshiaki Maruyama, PMDA
  PMDA Perspective on Global Regulatory Convergence

Sponsored Symposium
Bio-Techne: Protein Quantitation Applications to Advance Gene Therapy Development – From Discovery Through Analytical Development
Location: Room 383-385
8:30 AM - 9:30 AM

- Jaime Jacobson, Bio-Techne
  Sponsor

Sponsored Symposium
Lonza & Quell Therapeutics: Large-Scale T-Cell Engineering with Non-Viral Delivery

of Complex Cargos (Session 1) | Development of CAR-Treg Therapies for Transplantation and Autoimmunity (Session 2)
Location: Room 391-392
8:30 AM - 9:30 AM

- Erin Brooks, Lonza Bioscience
  Sponsor

Networking
Exhibit Hall
Location: Exhibit Hall
9:00 AM - 5:30 PM

General Session
Presidential Symposium
Location: Hall F
10:15 AM - 12:00 PM

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

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

Networking
Post-General Session Networking
Location: ASGCT Central, Booth #837
12:00 PM - 1:00 PM

Poster Talk Session
Wednesday Poster Talk
Location: Exhibit Theater
12:15 PM - 1:00 PM

Sponsored Symposium
Cytiva: Could cell line technologies bring disruptive changes to gene therapy manufacturing?
Location: Room 271-273
12:15 PM - 1:15 PM

- Alice Giraud, Cytiva
  Sponsor

Sponsored Symposium
BioAgilytix: Overcoming Challenges for Advanced Therapeutics: Case Studies on Navigating the Unique Bioanalytical and CMC Characterization Landscape of Cell and Gene Therapies
Location: Room 383-385
12:15 PM - 1:15 PM

- Jessica Weaver, BioAgilytix
  Sponsor

Sponsored Symposium

Sarepta Therapeutics, Inc.: Targeting the Root Cause: Evaluating Protein Expression in Limb-Girdle Muscular Dystrophies

Location: Room 388-390
12:15 PM - 1:15 PM

- Dwipi Patel, Sarepta Therapeutics, Inc.
  Sponsor

Sponsored Symposium

Dyno Therapeutics: Leveling Up Genetic Medicine with Frontier AI and AAV Vectors for CNS, Eye, and Muscle

Location: Room 391-392
12:15 PM - 1:15 PM

- Alice Tirard, Dyno Therapeutics
  Sponsor

Oral Abstract Session

Neurologic Diseases - Vectorology and Gene Therapy

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

Oral Abstract Session

Gene Therapy Clinical Trials

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

Oral Abstract Session

AAV Gene Transfer (A): Crossing the Blood-Brain Barrier

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

Oral Abstract Session

Translational Applications of Base and Prime Editors

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
  AptaDiR: A New Frontier in Epigenetic Precision Medicine

- Ethan Lippmann, Vanderbilt University
  Lipid-siRNA conjugates for targeted delivery to CNS

- Chuong Hoang, National Cancer Institute (NIH)
  Localized miRNA delivery for mesothelioma therapy

- Oxana Beskrovnaya, PhD, Dyne Therapeutics
  TfR1-mediated delivery of oligonucleotides for the treatment of neuromuscular diseases: translating research into clinic

Oral Abstract Session

CAR T-Cells for Solid Tumors

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

Education Session

Preclinical Models for Cell and Gene 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

- Melanie Graham, University of Minnesota
  Non-human primates (Stem cell therapies, diabetes, transplantation, regulatory guidance for pre-clinical models)

Fireside Chat

Fireside Chat: The Regulatory Landscape with Dr. Nicole Verdun (Virtual)

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

Tools & Technology Session

Tools and Technology Forum 1

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

- Abhilasha Gupta, Vector BioLabs
  Vector Biolabs: Novel Applications of Viral-Mediated Gene Delivery: Case Studies on Cost-Effective Strategies to Accelerate Discovery to Clinic

- Adam Brooks, Wyatt Technology
  Waters | Wyatt Technology: Rapid, Automated Zeta Potential and Multi-Attribute Intact Analysis: Advanced Characterization with Wyatt Light Scattering Tools

- Megan Del Greco, MilliporeSigma
  MilliporeSigma: Harnessing Innovative Technologies for Enhance AAV Production

- Pouria Motevalian, Thermo Fisher Scientific
  Thermo Fisher Scientific: Transforming Lentiviral Production: Enhancing Titers, Infectivity and Yield for Breakthrough Gene Therapies

- Brian Tomkowicz, PhD, SK Pharmteco - US
  QIAGEN & SK Pharmteco: Leveraging the Qiagen Qiacuity dPCR Platform for Enhanced Viral Quantification, Genome Integrity Analysis, and In-Process Analytics in Gene Therapy and Biomanufacturing

- Vasileios Georgakakos, Clean Cells
  Clean Cells: Karyotyping and FISH for the characterization of cell & gene therapy products. A decade of GMP experience

- Natalia Elizalde, PhD, VIVEbiotech
  VIVEbiotech: Pioneering Excellence in Lentiviral Vector Development and Manufacturing for In Vivo and Ex Vivo Administration

Sponsored Symposium

Terumo Blood and Cell Technologies: It Takes Two: How Industry Collaboration Can Unlock CAR-T at Scale

Location: Room 383-385
2:00 PM - 2:30 PM

- Alexa Bryant
  Sponsor

Sponsored Symposium

Bio-Rad Laboratories: Beyond Genomic Titer: Leveraging the VeriCheck Empty/Full AAV ddPCR Kit by BioRad for rAAV Genomic Titer, Capsid Concentration and Empty/Full Ratio in a Single Reaction

Location: Room 391-392
2:30 PM - 3:00 PM

- Marisol Gabriel, CMP, DES, Bio-Rad Laboratories
  Sponsor

Sponsored Symposium

Forge Biologics: Great Science Needs Innovative Manufacturing Technology

Location: Room 271-273
3:45 PM - 4:15 PM

- Hannah Munizza, BS, Forge Biologics
  Sponsor

Tools & Technology Session

Tools and Technology Forum 2

Location: Exhibit Theater
3:45 PM - 5:15 PM

- Norbert Makori, Altasciences
  Altasciences: Nonhuman Primate Research Models in Gene and Cell Therapy: Fetal, Infant to Mature Animal Utility

- Oleg Shinkazh, Chromatan
  ChromaTan: ChromaTan's Kascade BioRMB system - Introducing process intensification of rAAV purification through a continuous column-free chromatography platform.

- Daozhan Yu, PhD, AAVnergene Inc
  AAVnerGene Inc.: AAVone®-A Single Plasmid Solution for Efficient AAV Production and ATHENA-AAV Capsid Engineered Platform

- Ram Shankar, PlasmidFactory GmbH
  PlasmidFactory GmbH: New transfer vectors for your rAAV production

- Stanley Prince, PathoQuest
  PathoQuest: NGS Applications for Gene Therapies Quality Control: Regulatory Considerations

Oral Abstract Session

Gene Editing: New Tools and Technology Advances

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

Oral Abstract Session

Viral Vectors in Large Animal Models

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

Oral Abstract Session

Novel Neurological Disease Models and Therapeutic Approaches

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

Oral Abstract Session

Pharmacology/Toxicology Studies and Analytics/Assay Development Session I

Location: Room 265-268
3:45 PM - 5:30 PM

Oral Abstract Session

Molecular and Cellular Methodology

Location: Room 278-282
3:45 PM - 5:30 PM

Oral Abstract Session

Innovations in in vivo Targeting of HSPCs and Immune Cells

Location: Room 288-290
3:45 PM - 5:30 PM

Oral Abstract Session

Oligonucleotide Therapeutics I

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, Versiti Blood Research Institute
  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, MD, 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 388-390
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, 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 Cell and Gene Therapy Committee)

Location: Room 391-392
3:45 PM - 5:30 PM

- Christopher Evans, PhD, DSc, 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, Unversity of California, Los Angeles
  Academic Researcher Perspective on Dropped Gene Therapies: Challenges and Pathways Forward

- Claire Booth, MBBS PhD, UCL Great Ormond Street Institute of Child Health
  Picking Up Dropped Gene Therapies for Further Development: Challenges and Pathways Forward

- Rachel McMinn, PhD, Neurogene
  Industry Perspective on Dropped Gene Therapies: Challenges and Pathways Forward

Sponsored Symposium

Sartorius: Optimizing AAV Production: A Holistic Approach to Upstream,

Downstream, and Cost Per Dose Enhancement
Location: Room 271-273
4:45 PM - 5:15 PM
- Ales Strancar, Sartorius BIAA Separations
  Sponsor

Poster Abstract Session
Wednesday Poster Reception
Location: Poster Hall Hall I2
5:30 PM - 7:00 PM

Thursday, May 15

Sponsored Symposium
GenScript USA Inc: Multiplex Base-Edited CAR-T Cells Overcome Glioblastoma's Multifaceted Suppression using GenScript's sgRNA Solutions
Location: Room 271-273
8:00 AM - 8:30 AM
- Joyce Tung, GenScript USA Inc.
  Sponsor

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-Driven Development of Lipid Nanoparticles for mRNA an Gene Editor Delivery

- Richard Price, PhD, University of Virginia
  Focused Ultrasound Delivery of Non-Viral Gene Therapies to the Blood-Tumor Barrier.....and Beyond

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, PhD, Yale University
  AAV-transposon combination for CAR T and CAR NK development

- Zheng-Yi Chen, D.Phil., 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
HSC Transplantation and Gene Therapy
Location: NOLA Theater 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

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

Scientific Symposium
Publishing 101
Location: Room 278-282
8:00 AM - 9:45 AM
- Daniel Stone, PhD, Fred Hutchinson Cancer Center
  Title To Come

- Timothy Cripe, MD, PhD, Nationwide Children's Hospital
  Title To Come

- Paloma Giangrande, Eleven Therapeutics, Inc.
  Title To Come

Member-Submitted Proposal
Harnessing Cell and Gene Therapies: New Frontiers in Kidney Treatment
Location: Room 288-290
8:00 AM - 9:45 AM
- Poulami Chaudhuri, Helex
  Non-Viral Gene Editing for Autosomal Dominant Polycystic Kidney Disease1

- Alice Brown, Purespring
  Gene Therapies targeting the podocyte

- Leif Oxburgh
  Regenerative Kidneys: Harnessing Nephron Progenitors and Developmental Pathways for Next-Generation Cell & Gene Therapies

Oral Abstract Session

Disease Models and Pre-Clinical Applications for Lysosomal Storage Diseases

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 Opthalmic Committee)

Location: Room 293-296
8:00 AM - 9:45 AM

- Fyodor Urnov, PhD, University of California, Berkeley
  Leveraging the Platform Nature of CRISPR Gene Editing to Enable Neurologic Disease Therapies

- Holly Brothers, Biogen
  BIIB080: the development of a tau-targeting antisense oligonucleotide in early AD

- Rajeev Sivasankaran, Voyager Therapeutics, Inc.
  IV-delivered AAV gene therapy targeting tau for the treatment of Alzheimer's disease: preclinical safety and pharmacology with translational considerations

- Bruno Godhino, Atalanta Tx
  IT di-siRNA for the treatment of Huntington's disease: preclinical safety and pharmacology with clinical dose projections

Scientific Symposium

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

Location: Room 388-390
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
  Clinical & Translational Findings Following Resecabtagene Autoleucel Anti-CD19 CAR T Cell Therapy in Autoimmune Disease

- Laurie Kenney, Moderna Therapeutics
  in vivo mRNA delivery of IDO1 for autoimmunity and aGVHD

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

- Olivia Zetter, ARCH Venture Partners
  Beyond Traditional Metrics: A Venture Perspective on Valuing and Funding Next-Generation CGTs

Sponsored Symposium

FUJIFILM Irvine Scientific: Scalable Solutions for Gene Therapy and Cell Therapy Workflows

Location: Room 383-385
8:30 AM - 9:30 AM

- Pinar Boyar, FUJIFILM Irvine Scientific
  Sponsor

Sponsored Symposium

Danaher Life Sciences: A Platform Approach to Designing, De-risking, and Manufacturing a Gene Editing-Based Therapeutic: the IGI-Danaher "CRISPR Cures Cookbook"

Location: Room 391-392
8:30 AM - 9:30 AM

- Jeanine DuBois-Bracey, Danaher Life Sciences
  Sponsor

Networking

Exhibit Hall

Location: Exhibit Hall
9:00 AM - 5:30 PM

General Session

George Stamatoyannopoulos Memorial Lecture

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

- Kiran Musunuru, MD, PhD, MPH, ML, MRA, University of Pennsylvania
  Therapeutic Gene Editing for Cardiovascular and Metabolic Diseases: From the Leading Cause of Death to N-of-1 Disorders

- Tippi MacKenzie, MD, University of California San Francisco
  Prenatal Gene Therapy

Networking

Post-General Session Networking

Location: ASGCT Central, Booth #837
12:00 PM - 1:00 PM

Poster Talk Session

Thursday Poster Talk

Location: Exhibit Theater
12:15 PM - 1:00 PM

Sponsored Symposium

MaxCyte: How to Mitigate Gene Editing Program Risk Through Comprehensive Off-target Safety Profiling and Characterization

Location: Room 271-273
12:15 PM - 1:15 PM

- Marissa Johnson
  Sponsor

Sponsored Symposium

Advanced Cell Diagnostics - A Bio-Techne Brand: NextGen RNAscope Multiomics Solutions for Spatial Precision: AAV, small RNA, CAR-T, and Beyond

Location: Room 383-385
12:15 PM - 1:15 PM

- Sunita Gopalan, Bio-Techne
  Sponsor

Sponsored Symposium

Biogen: Biogen Gene Therapy

Location: Room 388-390
12:15 PM - 1:15 PM

- Catherine Zheng
  Sponsor

Sponsored Symposium

Thermo Fisher Scientific: Advancements in AAV Manufacturing: Scale up from Pre-clinical to GMP Readiness

Location: Room 391-392
12:15 PM - 1:15 PM

- Darwin Asa
  Sponsor

Oral Abstract Session

Epigenetic Editing and RNA Editing
Location: NOLA Theater A
1:30 PM - 3:15 PM

Oral Abstract Session
Novel Approaches to Gene Targeting and Gene Correction
Location: NOLA Theater B
1:30 PM - 3:15 PM

Oral Abstract Session
Downstream Manufacturing for AAV Vectors
Location: NOLA Theater C
1:30 PM - 3:15 PM

Oral Abstract Session
Late-Breaking Abstracts I
Location: Room 265-268
1:30 PM - 3:15 PM

Oral Abstract Session
Viral Vector and Transgene Biology
Location: Room 278-282
1:30 PM - 3:15 PM

Oral Abstract Session
Novel Genetic Approaches for Muscle and Skeletal Diseases
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, Independent
  Venture Studio/Incubator

- Hyo Min Park, PhD, GenEdit
  Young Founder, How they pitched their ideas and built their team

Oral Abstract Session
Lipid Nanoparticles I
Location: Room 293-296
1:30 PM - 3:15 PM

Tools & Technology Session

Tools and Technology Forum 3

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

- Hannah Munizza, BS, Forge Biologics
  Forge Biologics: Great Science Needs Innovative Manufacturing Technology

- Kendra Alley, Andelyn Biosciences
  Andelyn Biosciences: A Client-CDMO Partnership Pathway to Successful Gene Therapy Process Performance Qualification and BLA filing: An Ultragenyx and Andelyn Biosciences Journey to a Commercial-Ready Platform

- Jana Merx, C-LEcta GmbH
  c-LEcta GmbH: Advancing AAV Production: Engineered Endonucleases for Highly Efficient DNA Removal Across Broad Salt Concentrations

- Jing Zhu, ReciBioPharm
  ReciBioPharm: Advancing the future of gene therapies: ReciBioPharm's new cost-effective AAV solutions

- Jill Makin, Touchlight DNA Services - Hampton
  Touchlight: Simply Scaling DNA from R&D to GMP with dbDNA™ (doggybone DNA) technology

- Amy Lamperti, Minaris Advanced Therapies
  Minaris Advanced Therapies: Meet Minaris Advanced Therapies: A Game-Changing Force in Cell Therapy

- Eugenia Jones, FUJIFILM Cellular Dynamics Inc
  FUJIFILM Cellular Dynamics, Inc.: Advancing Gene Therapy: Harnessing Human iPSC-Derived Models for Discovery and Potency Assays

Sponsored Symposium

Catalent: Partnerships to Enhance Development and Intensification of Viral Vector Production

Location: Room 383-385
2:00 PM - 2:30 PM

- Swati Roy
  Sponsor

Sponsored Symposium

Sartorius BIA Separations: Manufacturing Platform - From pDNA and mRNA to LNP With Multiple Nucleic Acids Loads

Location: Room 391-392
2:30 PM - 3:00 PM

- David Ede
  Sponsor

Networking

Patient Advocate Meet-Up

Location: TBD
3:15 PM - 4:15 PM

Networking

Trainee and New Investigator Meet-up

Location: ASGCT Central, Booth #837
3:15 PM - 4:15 PM

Sponsored Symposium

STEMCELL Technologies Inc: Cutting-Edge Tools for Hematopoietic Stem & Progenitor Cell Research

Location: Room 271-273
3:45 PM - 4:15 PM

- Christen McDonald, STEMCELL Technologies, Inc.
  Sponsor

Tools & Technology Session

Tools and Technology Forum 4

Location: Exhibit Theater
3:45 PM - 5:15 PM

- Emilie Claire Schneider, Takara Bio
  Takara Bio USA: Streamlined Ex vivo Engineering of Human T cells with a Single-Step Approach to Activation and Lentiviral Transduction

- Sarah Rains, KACTUS
  KACTUS: Revolutionize Viral Vector Manufacturing with Cost-Effective S. marcescens Nuclease: MaxNuclease™

- Jennifer Hamilton, ArcticZymes Technologies
  ArcticZymes Technologies: Clearing DNA from Viral Vectors with Salt Active Nucleases: Why Salt is the Hidden Catalyst to Bioprocessing Optimization

- Randy Dyer, Elegen
  Elegen: Accelerating mRNA Vaccine Development with Rapid, Cell-Free Synthesis of High-Accuracy Template DNA

- Shawn Sternisha, PhD, Beckman Coulter Life Sciences
  Beckman Coulter Life Sciences: Streamlining AAV Purification: Automated and Precise Density Gradients with the OptiMATE Gradient Maker

- Vanessa Kelchner, Akadeum Life Sciences
  Akadeum Life Sciences: Next Generation Cell Separation for CAR-T Manufacturing: Unlocking More Doses in a Smaller Footprint with Seamless Integration of Microbubbles

Oral Abstract Session

Novel Therapeutic Gene Editing Applications

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

Oral Abstract Session
Manufacturing and Transduction for Viral Vectors
Location: NOLA Theater B
3:45 PM - 5:30 PM

Oral Abstract Session
AAV Vector Manufacturing: Experimental Design & Analytics
Location: NOLA Theater 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

- Michela Milani, PhD, SR-TIGET
  In Vivo Lentiviral Vector Gene Transfer Into Hematopoietic Stem And Progenitor Cells

- Muhammed Burak Demircan, Postdoc, DKFZ
  In vivo CAR T Cell applications

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

- Wei Liang, PhD, FDA
  FDA introduction into the CBER START Pilot Program (Pre-recorded Presentation)

Oral Abstract Session
Vaccines and Immunotherapy for Cancer
Location: Room 288-290
3:45 PM - 5:30 PM

Oral Abstract Session
Organoids and iPSC Disease Modeling for Drug Discovery
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 on Platelet-Targeted Gene Therapy for Hemophilia A

- Yuman Fong, MD, City of Hope
  Oncolytic Viruses: Balancing Between Safety and Efficacy (Clinical Perspective)

- Shyam Nyati, Associate Scientist, Henry Ford Health System
  Utilizing Inhouse Developed Adenoviral Vectors in Clinical Trials

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

Member-Submitted Proposal
Defining the Neurological Outcome Limits in Gene Therapy Trials
Location: Room 388-390
3:45 PM - 5:30 PM

- Jerry Mendell, MD, Sarepta Therapeutics
  Clinical conditions for which clinical trials are underway with non-neurologic neurodegenerative components and the likelihood of treating both components with current strategies

- Kyle Brothers
  Neuro ethic decisions in patient care

- Alison Bateman-House, NYU Grossman School of Medicine
  Title To Come

Scientific Symposium

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

Location: Room 391-392
3:45 PM - 5:30 PM

- Christopher Porada, PhD, Wake Forest Institute for Regenerative Medicine
  Introduction talk regarding successes in the field and clinical trials

- Agnieszka Czechowicz, MD, PhD, Stanford University, School of Medicine
  Cell Therapy - Learnings from SCID/Alpha Thal towards Fanconi Anemia

- Panicos Shangaris, Department of Women and Children's Health
  Non-Viral and Lipid Nanoparticle (LNP)-Based Delivery Systems for Prenatal Applications and Ethical Considerations.

- R. Alta Charo, University of Wisconsin Law School
  Short presentation regarding ethics (~10 minutes)

Oral Abstract Session

CAR T Innovations in Autoimmune and Infectious Disease and Allergy

Location: Room 393-396
3:45 PM - 5:30 PM

Sponsored Symposium

Catalent Cell and Gene Therapy: From GMP-grade iPSCs to Scalable NK Cell Production: A Feeder-Free Approach

Location: Room 271-273
4:45 PM - 5:15 PM

- Swati Roy
  Sponsor

Reception

Women in Cell and Gene Therapy Reception

Location: Rusty Nail + Sidecar
6:00 PM - 7:00 PM

Friday, May 16

Sponsored Symposium

Cytiva: Enhancing non-viral delivery: Advancing LNP technology for vaccine and cell therapies

Location: Room 271-273
8:00 AM - 8:30 AM

- Alice Giraud, Cytiva
  Sponsor

Scientific Symposium

Tissue-Specific Insights in AAV Vector Immunogenicity (Organized by the Immune Responses to Cell and Gene 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
  Advanced T cell engineering using non-viral CRISPR screens

- Luca Gattinoni, Leibniz Institute for Immunotherapy
  Stem t cells improving responses, CD8, clinical trial

- Michael Klichinsky, PharmD, Carisma Therapeutics
  CAR Macrophages

- Angel Corria Osorio, LICR University of Lausanne
  Next generation TIL therapies

Scientific Symposium

Hot Topics in Molecular Therapy II: Gene Therapy and Gene Editing Approaches for Human Disease

Location: NOLA Theater C
8:00 AM - 9:45 AM

- Toni Cathomen, PhD, Medical Center - University of Freiburg
  On- and off-target effects of paired CRISPR-Cas nickase in primary human cells

- Miffy Hok Yan Cheng, The University of British Columbia
  Lipid nanoparticle mRNA systems containing high levels of sphingomyelin engender higher protein expression in hepatic and extra-hepatic tissues

- Megan Keiser, PhD, Ohio State University
  APOE2 gene therapy reduces amyloid deposition and improves markers of neuroinflammation and neurodegeneration in a mouse model of Alzheimer disease

Oral Abstract Session

Immune Cell Therapies

Location: Room 265-268
8:00 AM - 9:45 AM

Oral Abstract Session

Emerging Delivery Platforms for In Vivo Gene Editing

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

Member-Submitted Proposal

Translational Strategies for Cell and Gene Therapies in Inherited Neurotransmitter Disorders

Location: Room 291-292
8:00 AM - 9:45 AM

- Steven Gray, PhD, University of Texas Southwestern Medical Center
  Transgene regulation strategies for dose-sensitive genes involved with neurodevelopmental disorders

- Brad Elder, MD, The Ohio State University College of Medicine
  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

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, MD, PhD, Center of Medical Innovation & Translational Research, University of Osaka
  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

- Michelle Lorentzos, Sydney Children's Hospitals Network
  Australia based clinical trials

- Gloria Gonzalez-Aseguinolaza, PhD, Center for Applied Medical Research (CIMA) of the University of Navarra
  GATEWAY clinical trial for Wilson Disease

Scientific Symposium

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

Location: Room 388-390
8:00 AM - 9:45 AM

- Kimberly Haugstad, MBA, 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

- 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 from bridging gene therapies from bench to bedside-An academic perspective

- Leslie Meltzer, PhD, Orchard Therapeutics
  Developing and delivering hematopoietic stem cell gene therapies to patients with rare neurometabolic diseases

- 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

Sponsored Symposium

MilliporeSigma: To 1000L and Beyond: Introducing the first-and-only transfection complex stabilizer for simple AAV manufacturing scale-up

Location: Room 383-385
8:30 AM - 9:00 AM

- Laura Juckem, PhD, Mirus Bio
  Sponsor

Sponsored Symposium

Waters Corporation & Lexeo Therapeutics: "Breakthroughs in Gene Therapy Analytics: Potency and Purity-Indicating Assays Using Advanced Size Exclusion Chromatography (SEC) and Charge Detection Mass Spectrometry (CDMS)"

Location: Room 391-392
8:30 AM - 9:00 AM

- Matthew Lauber, PhD, Waters Corporation
  Sponsor

- Noah Miller-Medzon, LEXEO Therapeutics
  Sponsor

Networking

Exhibit Hall

Location: Exhibit Hall
9:00 AM - 5:30 PM

General Session

Outstanding New Investigator Symposium

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

- Rebecca Ahrens-Nicklas, PhD, MD, Children's Hospital of Philadelphia
  Transforming the Care of Patients with Rare Metabolic Diseases Through Gene Therapy

- Eric Smith, MD, PhD, Dana Farber Cancer Institute
  Pushing the Boundaries of CAR T-Cell Therapy for Immunotherapy of Cancer

- Xavier Anguela, Nava Therapeutics
  Talk Title Forthcoming

- Benjamin Deverman, Broad Institute of MIT and Harvard
  Talk Title Forthcoming

Scientific Symposium
Citeline Presentation
Location: Exhibit Theater
12:15 PM - 12:45 PM

Sponsored Symposium
Pacira Biosciences: High-Capacity Adenoviral Vectors: Advancing Gene Therapy Beyond AAV to Deliver Cost-effective Therapies for Common Diseases
Location: Room 383-385
12:15 PM - 1:15 PM
- Derek Jackson
  Sponsor

Oral Abstract Session
AAV Gene Transfer (B): Ocular, Neurological & Immune Cell Systems
Location: NOLA Theater 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)

- Mitchell Tai, Ph.D., Bristol Myers Squibb
  Navigating the Curves: Global Lifecycle Management Experiences in a Commercial Cell Therapy Product

Oral Abstract Session
Clinical Trial Spotlight Symposium
Location: NOLA Theater 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, Sana Biotechnology
  Hypo-Immune cells

- Deepta Bhattacharya, University of Arizona
  Engineering pluripotent stem cells to evade and promote immunity

- Andras Nagy, Lunenfeld-Tanenbaum Research Institute
  Immune privileged/immune cloaking

- Xiaokoui Zhang, PhD, Aspen Neuroscience, Inc.
  Autologous iPSC-Derived Neuron Replacement for Parkinson's Disease

Oral Abstract Session

Novel Models and Advances for Heart, Lung, and Kidney Gene Therapy

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

Oral Abstract Session

Modulation of Humoral Immune Responses in AAV Gene Transfer

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

- Michelle Lee, PhD, Medra
  Embodied AI for Biology Labs

Oral Abstract Session

Lipid Nanoparticles II

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

- Maria Gonzalez, Viralgen Vector Core - San Sebastian
  Viralgen Vector Core: A Rock in a Storm: Using Data as Your Foundation to Speed and De-risk AAV Manufacturing

- Laura Griffin, Taconic Biosciences
  Taconic Biosciences: Strategies for Improved Engraftment and Data Reproducibility in Humanized Immune System (HIS) Mice

- Irene Song, Packgene Biotech
  PackGene Biotech: Driving Down Costs: Advancing AAV Manufacturing for Affordable Gene Therapy

- Roy Liu, Shenzhen Eureka Biotechnology Co., Limited
  EurekaBio: Next Generation Lentiviral Vector Production System - Intergrated Packaging System and Stable Producer System

- Glenda Dickson, ViroCell Biologics, 12-18 Theobalds Road, London, WC1X 8SL, UK
  ViroCell Biologics: Rethinking lentiviral stable producer cells lines: VSVg or not VSVg. That is the question.

- Hiroki Hasegawa, Mitsubishi Gas Chemical Company, Inc.
  Mitsubishi Gas Chemical: OXYCAPT™ Multilayer Plastic Vial contributes to optimal risk managements on deep-cold supply chain for cell gene therapy products

- Kerri McWeeny, Stilla Technologies
  Stilla Technologies: Maximizing Throughput, Minimizing Complexity: The Nio® dPCR Advantage in CGT

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

- Carmen Sivakumaren, IQVIA
  BGTC Coordination Center Rep

- James Noll, IQVIA
  BGTC Coordination Center Rep

- Sharon King, Aldevron
  BGTC/FNIH Partner

- Sarah Cortell Vandersypen, United MSD Foundation - Biloxi, MS
  Patient Advocacy Rep for BGTC disease

- Jill Chertow, Propionic Acidemia Foundation
  Patient Advocacy Rep for BGTC disease

- Leah Byrne, UC Berkeley
  BGTC AAV Biology Awardee

- Fred Bunz, Johns Hopkins University School of Medicine
  BGTC AAV Biology Awardee

- Courtney Coates, Hope in Focus
  Hope in Focus

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, PhD, Sail Biomedicines
  Pioneering the design and deployment of fully programmable RNA medicines

- Priya Karmali, Capstan Therapeutics
  In vivo immune cell engineering using targeted nanoparticles

- David Oupicky, PhD, University of Nebraska Medical Center
  Targeted Renal Delivery: Polysaccharide RNA Carriers to Treat Acute Kidney Injury

- Yizhou Dong, Icahn School of Medicine at Mount Sinai
  Lipid nanoparticles enabled mRNA therapeutics

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 in rhesus macaques

Scientific Symposium

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

Location: Room 388-390
3:45 PM - 5:30 PM

- 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

AAV Preclinical and Proof-of-Concept Studies for Neurological Diseases

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

Oral Abstract Session

AAV Vector Manufacturing: Plasmids & Cell Line Development

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

Oral Abstract Session

Challenges in Immunological Responses to Therapeutic Interventions

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

Oral Abstract Session

CMC for AAV Vectors

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

Oral Abstract Session

Oligonucleotide Therapeutics II

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

Oral Abstract Session

Cell Therapy Product Engineering II

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

Oral Abstract Session

Across Platforms for Hematopoietic Disorders

Location: Room 391-392
3:45 PM - 5:40 PM

Oral Abstract Session

Gene Therapy Trials - In-Vivo Gene Therapy Modification
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
AAV Preclinical and Proof-of-Concept Studies
Location: NOLA Theater A
8:00 AM - 9:45 AM

Oral Abstract Session
Translational Approaches: Gene Therapy of Neurological Diseases in Large Animal Models
Location: NOLA Theater B
8:00 AM - 9:45 AM

Oral Abstract Session
Upstream Manufacturing for AAV Vectors 1
Location: NOLA Theater C
8:00 AM - 9:45 AM

Oral Abstract Session
On and Off-Target Method Development
Location: Room 278-282
8:00 AM - 9:45 AM

Oral Abstract Session
Pharmacology/Toxicology Studies and Analytics/Assay Development Session II
Location: Room 288-290
8:00 AM - 9:45 AM

Oral Abstract Session
Targeted Gene and Cell Therapy for Cancer
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 erythematous, myositis, systemic sclerosis and generalized myasthenia gravis

- Jeff Bluestone, PhD, Sonoma Biotherapeutics
  Engineered Treg therapies to treat Rheumatoid Arthritis - early Phase 1b results

- 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
  Base and prime editing mutant CFTR in patient-derived cell model to treat cystic fibrosis

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

- Stephen Hyde, PhD, University of Oxford
  Lentiviral gene transfer for CF and SP-B deficiency

Oral Abstract Session

Chemistry, Manufacturing, and Controls

Location: Room 388-390
8:00 AM - 9:45 AM

Oral Abstract Session

Late-Breaking Abstracts II

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, PhD, Johns Hopkins University
  Phase I clinical trial results for DNA vaccine for HPV16-associated cervical intraepithelial neoplasia (CIN-2/3)

- Ye Zhang, MD, PhD, Arcturus Therapeutics
  Self-Amplifying mRNA Vaccines: Pioneering a New Era in Infectious Disease Prevention

- Matthias Schnell, Thomas Jefferson University
  Rabies virus (RABV) based vaccines for emerging infectious diseases

Oral Abstract Session

AAV Gene Transfer (C): Antibody Evasion, Cardiac & Neuromuscular Targets

Location: NOLA Theater A
10:15 AM - 12:00 PM

Oral Abstract Session

Molecular and Cellular Methods - Applications

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

Oral Abstract Session

Upstream Manufacturing for AAV Vectors 2

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

Oral Abstract Session

B-cell and Solid Organ Therapies

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

Oral Abstract Session

Gene Therapy for Muscle Diseases

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

Oral Abstract Session

Engineered Immune Effector Cells for Solid Tumors

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

Oral Abstract Session

Lipid Nanoparticles III

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

Oral Abstract Session

Vector Product Engineering, Development and Manufacturing (excluding AAV)

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

Oral Abstract Session

Oncolytic Virus Therapies

Location: Room 388-390
10:15 AM - 12:00 PM

Oral Abstract Session

Innovation in Alternative Cell Therapy Sources

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

Member-Submitted Proposal

Crosstalk Between DNA Repair Mechanisms and Gene Therapy

Location: Room 393-396
10:15 AM - 12:00 PM

- Toni Cathomen, PhD, Medical Center - University of Freiburg
  Pharmacological Strategies to Shift DNA Repair Pathway Choice in Gene Editing

- Ayal Hendel, PhD, Bar Ilan University
  Fine-Tuning DNA Repair in Stem Cells for Safer CRISPR Therapeutics

- Alberto Ciccia, Columbia University
  Guiding DNA Repair to Reduce Unwanted Mutations in Gene Editing