Annual Meeting 2025 Program

Tuesday, May 13

Workshop (separate purchase required)

Navigating The Nonclinical Pathway To Clinical Trials For AAV Based Therapeutics

Location: Room 271-273
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

- 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, Johnson & Johnson Innovative Medicine
  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, MD, Stanford University
  Delineating the role of microbiome for cell therapy

- Prasad Adusumilli, MD, 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, PhD, 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

- Kimberly Benton, PhD, Dark Horse Consulting Group
  High-Level Regulatory Overview: Human- and Animal-Derived Materials; Safety Testing Human Allogenic Cells; Genome Editing

- 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

- Tal Salz, Dark Horse Consulting Group
  High-Level Regulatory Overview: Platform Technology Designation Program; Advanced Manufacturing Technologies Designation Program; Potency Assurance; and Manufacturing Changes and Comparability

- 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, 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

- 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 College of Medicine
  Improving PE outcomes by overcoming DNA repair hurdles (AI-generated small binder)

- Jellert Gaublomme, PhD, Columbia University
  CRISPRmap: Sequencing-free optical pooled screens mapping multi-omic phenotypes in cells and tissue

- Randall Platt, PhD, ETH Zurich, Basel
  Multimodal scanning of genetic variants with base and prime editing

- Marcello Maresca, PhD, 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

- Lauren Gauthier, Takeda
  Lentivirus - Nonclincal Activities and Assessments

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

- Daniel Galbraith, Solvias
  Lentivirus integration and mechanisms; targeted locus amplification platform

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

Sponsored Symposium

GeneFab: GeneFab's Synthetic Biology Technologies for Smarter, Safer

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

Oral Abstract Session
AAV Biology and Mechanisms
Location: New Orleans Theater A
1:30 PM - 3:15 PM

- Eirini Vamva, Stanford University Laboratory for Cell & Gene Medicine
  Epigenetic Blueprint: Understanding how the AAV Capsid influences the Vector Epigenome

- Srinethe Saravanakumar, Askbio Gmbh
  Vector assembly factories in recombinant adeno-associated virus type 2 producing cells

- Conradin Baumgartl, Universitätsklinikum Heidelberg
  Capsid-mediated differences in epigenetic transgene modifications between AAV2 and AAV9

- Fred Bunz, Johns Hopkins University School of Medicine
  Inverted Terminal Repeat (ITR) Degeneracy Promotes AAV Genomic Integration

- Andrea Llanos-Ardaiz, CIMA Universidad de Navarra
  Genome Wide rAAV Integration Analysis in a Tyrosinemia Mouse Model Revealed Preference for R-loops in Actively Transcribed Genes and in the Absence of Homology Arms.

- Bijay Dhungel, PhD, University of Sydney
  An alternate receptor for adeno-associated viruses

- Xiujuan Zhang, KUMC
  Identification of KIAA0319L-interacting host factors during intracellular trafficking of AAV

Oral Abstract Session
AAV Vector Manufacturing: Process Development
Location: New Orleans Theater B
1:30 PM - 3:15 PM

- Ryan Sorensen, University of Minnesota
  Engineering Protein Carrier AAV to Rescue Intracellular Trafficking | Selective Purification of AAV Based on Capsid Composition

- Garima Thakur, Regeneron Pharmaceuticals
  Post-Processing Conjugation System for Manufacturing of Retargeted AAV-SpyT-SpyC-Ab Molecules

- Huimin Na, Sanofi
  DMSO Enhances Recombinant AAV Production via Downregulation of the Expression of the KAT5/TIP60 Acetyltransferase

- Don Startt, REGENXBIO Inc.
  Development of a Commercial Manufacturing Process for RGX-202, a Systemically Delivered AAV for the Treatment of Duchenne Muscular Dystrophy

- Niklas Kraemer, Sartorius Xell GmbH
  Nutritional Counselling at the Cellular Level – Combining Ambr® 15 and Orbitrap Mass Spectrometry to Analyze the Metabolome of HEK293 During AAV Production

- Igor Alves Mancilla, Revvity Gene Delivery
  Small-Scale Manufacturing of Innovative rAAV Capsid Variants for Enhanced Retinal Gene Therapy

- Serena Giannelli, PhD, Ospedale San Raffaele
  Harnessing protein trans-splicing by combining inteins and SpyTag systems

Oral Abstract Session

Advances in Gene Therapy of Neurological Diseases in Small Animal Models

Location: New Orleans Theater C
1:30 PM - 3:15 PM

- Alejandro Brao, Universitat Autonoma de Barcelona (UAB)
  Gene Therapy for Megalencephalic Leukoencephalopathy with Subcortical Cysts: Restoring Brain Homeostasis in Preclinical Models

- Swati Bijlani, PhD, City of Hope
  In Vivo Systemic Genome Editing to Correct Mecp2 Mutations Improves Symptoms and Extends Lifespan in a Severe Model of Rett Syndrome

- Laura Rodriguez-Estevez, Autonomous University of Barcelona
  Preclinical Study for the Treatment of Hereditary Spastic Paraplegia Type 52 (SPG52)

- Bryan Simpson, PhD, Latus Bio
  Targeting MSH3 for Huntington's Disease: Preclinical Validation of AAV-DB-3-miRNA to Prevent Somatic CAG Repeat Expansion

- Qinglan Ling, PhD, UMass Chan Medical School
  Achieving 'Just Right': Preclinical Development of A Gene Therapy for SURF1 Leigh Syndrome Using Two Disease Models

- Wassamon Boonying, PhD, UMass Chan Medical School
  Therapeutic and Safe GFAP Silencing in Astrocytes Rescues Alexander Disease in a Rat Model

- Brina Snyder, PhD, The University of Texas Southwestern Medical Center
  Assessing the Cognitive Benefit of a Vectorized Tau Reduction Therapy

Oral Abstract Session

Gene and Cell Therapy for Ophthalmic and Auditory Diseases

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

- Yiqun Yuan, HuidaGene Therapeutics Co., Ltd., Shanghai, China
  HG004 Gene Therapy Reduces Chorioretinal Atrophy and Demonstrates Superior Retinal Safety Over Luxturna Clinical Settings

- Dongjun Xing, Tianjin Branch of National Research Center for Ocular Disease, School of Optometry and Eye Institute, Tianjin Medical University Eye Hospital, Tianjin 300384, China
  CRISPR-Cas13 Gene Editing Therapy Targeting VEGFA Demonstrated Early Efficacy in Neovascular Age-Related Macular Degeneration

- Heyu Tang, Division of Cellular and Molecular Therapy, Department of Pediatrics, University of Florida, Gainesville, FL
  Development of an AAV-Based Gene Therapy for the Ocular Phenotype of Friedreich's Ataxia

- Samarendra Mohanty, Nanoscope Therapeutics Inc
  Safety and efficacy of Multi-characteristic opsin gene therapy in improving vision in NHP model of geographic atrophy

- Luoying Jiang, Fudan University Eye Ear Nose and Throat Hospital
  Gene Therapy vs Cochlear Implantation in Restoring Hearing Function and Speech Perception for Congenital Deafness Individuals: An Observational Cohort Study

- Ying Hsu, University of Iowa
  Prevention of Vision Loss using Subretinal Gene Therapy in a Mouse Model of Bardet-Biedl Syndrome Type 10

- Eugene Gonzalez-Lopez, Biotechnology, Neurotech Pharmaceuticals, Inc
  Sustained Drug Delivery of Ciliary Neurotrophic Factor by Encapsulated Cell Technology: NT-501 Implants:

Oral Abstract Session

Gene and Cell Therapy for Metabolic Diseases

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

- Sonam Gurung, PhD, University College London
  Systemically delivered mRNA therapy crosses blood brain barrier and shows neurological benefit in mouse model of Argininosuccinic Aciduria

- Yunhan Ma, Duke University
  Rescue of Glutaric Aciduria Type I by GalNAC conjugated siRNA against Aminoadipate Semialdehyde Synthase

- Francoise Piguet, PhD, TIDU GENOV, ICM
  Encapsulated cell therapy demonstrated therapeutic efficacy in metachromatic leukodystrophy and sustained ARSA production in NHP

- Elena Barbon, PhD, SR-TIGET
  Definition of a Minimal Therapeutic Dose of In Vivo Liver-Directed Lentiviral Gene Therapy for Methylmalonic Acidemia

- Jennifer Sloan, PhD, National Human Genome Research Institute
  Neonatal AAV gene therapy provides long term rescue in mouse models of combined methylmalonic acidemia and homocystinuria, cblC type

- Tia DiTommaso, Arbor Biotechnologies
  ABO-101, a Novel Gene Editing Therapy for Primary Hyperoxaluria Type 1, is Efficacious and Well Tolerated in NHPs and Results in High Fidelity Editing in Primary Hepatocytes

- Guocai Zhong, Ph.D., UMass Chan Medical School
  RNA Switch-Regulated Biofactory Gene Therapies for Rare and Common Metabolic Disorders

Oral Abstract Session

Physical Delivery Methods and DNA/RNA Drug Development

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

- Ben Hawley, Engage Bio
  The Tethosome Platform is a Non-Viral, Dual DNA/mRNA System That Facilitates Potent, Re-Dosable, and Durable Therapeutic Gene Expression

- Andy Thompson, RenBio, Inc.
  Next-Generation DNA-Based Delivery of Therapeutic Proteins Using MYO Technology: Preclinical Results on a Broadly Neutralizing Anti-Zika Virus Antibody

- Bernie Owusu-Yaw, Brigham and Women's Hospital & Harvard Medical School
  Optimizing Focused Ultrasound Parameters for Enhanced AAV Delivery Across the Blood-Brain Barrier for the Treatment of Neurodegenerative Diseases

- Ting-Yen Chao, Seattle Children's Research Institute
  Non-Viral Transcutaneous Fluoroscopy Guided Ultrasound-Mediated FVIII Delivery as a Promising Therapeutic Approach for Hemophilia A Treatment

- Saurav Mohanty, Nanoscope Therapeutics
  Safety, Tolerability, Biodistribution, Transgene Expression and Effectiveness of Image-

Guided Laser-based Non-Viral Targeted Delivery of Multi-Characteristic Opsin in Retinae of African Green Monkeys

- Ebony Gary, PhD, The Wistar Institute
  Single-dose electroporation delivery of plasmid-encoded incretin mimetics supports extended weight and blood glucose control in mouse models of diet-induced obesity

- Ami Patel, PhD, The Wistar Institute
  Expanded Analysis of in vivo-delivered SARS-CoV-2 Plasmid DNA-encoded Monoclonal Antibodies (DMAb) in a Phase 1 Clinical Trial in Healthy Adults

Oral Abstract Session

Novel CAR-T Engineering Strategies for Hematological Malignancies

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

- Chiara Magnani, PhD, Universitätsspital Zurich, University of Zurich
  Efficacy, safety, and biological properties of Sleeping Beauty-engineered CAR T cells in patients with B-cell acute lymphoblastic leukemia

- Caitlin Hopkins, University of Pennsylvania
  In Vivo Persistence and Function of CAR19 T-Cells for Pediatric B-Cell Acute Lymphoblastic Leukemia Are Impacted by Media, Cytokine, and Metabolite Bioavailability During Ex Vivo Expansion

- Corynn Kasap, UCSF
  Optimized anti-CD70 CAR-T cells for high-risk multiple myeloma

- Aiko Hasegawa, Shinshu University
  Mutated Ligand-Based CAR-T Cells Targeting the CD123/CD131 Complex Demonstrate Enhanced Cytotoxicity and Reduced Hematopoietic Toxicity Against $CD123^{+}$ AML

- Daniela Cesana, PhD, San Raffaele Telethon Institute for Gene Therapy (SR-Tiget), IRCCS San Raffaele Scientific Institute
  Cell-free DNA Profiling as a Non-Invasive Approach for Assessing CAR-T Therapy Outcomes and Toxicity

- Cheryl Bolinger, PhD, Precigen, Inc.
  Increased Potency, Persistence, and Stem Cell Memory/Naïve Phenotype is Achieved with PRGN-3008 UltraCAR-T, an Overnight-Manufactured CD19 CAR-T Cell Expressing membrane-bound IL-15 and miRNA-based PD-1 Blockade from a Single Non-Viral Transposon

- Nicola Maciocia, University College London
  Development of anti-CD21 Chimeric Antigen Receptor (CAR)-T Cells for T-Cell Acute Lymphoblastic Leukemia (T-ALL) - CAR engineering for a complex antigen.

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

- 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

Sponsored Symposium

OXB: Accelerate Time to Clinic: A Process and Analytics Platform Approach

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

General Session

Founders, Mendell, and Catalyst Award Symposium

Location: Hall F
3:45 PM - 5:30 PM

- Donald Kohn, MD, 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
  Curative Gene and Cell Therapies for Chronic Granulomatous Disease: Successes, Problems and Future Challenges

Reception

Welcome Reception

Location: Exhibit Hall
5:30 PM - 7:30 PM

Wednesday, May 14

Oral Abstract Session

Insights from AAV Engineering

Location: New Orleans Theater A
8:00 AM - 9:45 AM

- Robert Fusco, Duke University
  Evolving Synthetic Membrane Associated Accessory Proteins (synMAAPs) for Enhanced AAV Vector Egress

- Sebastian Aguirre Kozlouski, Carbon Biosciences
  CBN-1100: A Novel Recombinant Parvovirus Gene Delivery Platform with Enhanced Cargo Capacity, Immune Evasion, and Natural Liver Detargeting Properties

- Guocai Zhong, Ph.D., UMass Chan Medical School
  Synthetic RNA Switches for Temporal and Dose Control of In Vivo Gene Therapies

- Carson Key Taylor, Duke University
  In Vivo High-Throughput Screening of Regulatory Elements to Distinguish AAV Transgene Expression Between Cardiac and Skeletal Muscle

- Jeanette Zanker, GENETHON
  Understanding subcellular trafficking of natural and engineered AAV capsids by novel imaging techniques in skeletal muscle cells

- Arielle Gillies, University of Guelph
  Development of a Platform for Vectorized Expression of Secretory IgA

- Kenji Ohba, Jichi Medical University
  Novel adeno-associated virus (AAV) vector production system is useful for gene therapy and analyzing the molecular mechanism of viral replication

Scientific Symposium

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

Location: New Orleans 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
  OTC-HOPE: The first in vivo, liver directed, AAV-mediated, gene insertion clinical trial in infants

Scientific Symposium

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

Location: New Orleans 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, Leiden University Medical Center
  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

- 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
  Reaching alignment on the use of CSF biomarkers in neuronopathic MPS diseases

- Kevin Flanigan, MD, Center for Gene Therapy, Nationwide Children's Hospital
  Case study of how biomarkers may be limited in DMD

- Wilson Bryan, MD, Wilson W. Bryan Consulting, LLC
  What are Biomarkers? A regulator's guide to using biomarkers in an approved therapy

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
  Novel AAV Capsid Variants for Targeted Brain 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

- Felix Heider, Miltenyi Biotec B.V. & Co. KG
  Comparative Analysis of Lentiviral and CRISPR-Cas12i-Generated CAR T Cells: In Vitro and In Vivo Functional Outcomes

- Jae Hyun Jenny Lee, UCSF
  Development of a 1XX-Enhanced and Fully Non-Viral, CRISPR-edited BCMA CAR T Cell Therapy for Relapsed and Refractory Multiple Myeloma (UCCT-BCMA-1)

- Thomas Spoerer, University of Georgia
  Establishing Microporous Particle Scaffolds as a Scalable, 3D Platform for Continuous Production of Immunomodulatory Mesenchymal Stromal Cell Extracellular Vesicles

- Zhang Cheng, Sonoma Biotheraputics
  Transcriptomic and Epigenomic Analysis of CAR Treg Stability

- Daniele Canarutto, San Raffaele Telethon Institute for Gene Therapy (SR-TIGET)
  HDR gene correction of $CD4^+$ T cells approaching the clinic for the treatment of Hyper IgM1

- Sarah Nikiforow, MD, PhD, Dana-Farber Cancer Institute
  Comparative Analysis of Motixafortide versus Plerixafor for Autologous Stem Cell Mobilization and Collection in Multiple Myeloma: A Single Center Real-World Experience

- Leander Timothy, Starship Blood and Cancer Centre
  Use of IL-12, IL-15, and IL-18 Prior To Cryopreservation Confers a CIML Phenotype To CAR-NK Cells That Maintains Post Thaw Viability and CAR Functionality After Infusion

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
  After the diagnosis: early treatment options

- Britt Johnson, PhD, FACMG, 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

- Adnan Jaigirdar, Dark Horse Consulting Group
  Former FDA and general global landscape perspective

Sponsored Symposium

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

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

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

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

Networking

Exhibit Hall

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

Networking
Career Fair
Location: Level 200 Foyer
9:15 AM - 10:15 AM

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, MD, PhD, Gates Foundation
  Bringing effective and accessible curative interventions for HIV and sickle cell disease to all in need

- Alvin Luk, Huidagene Therapeutics Inc
  CRISPR-hfCas12Max Genome Editing Therapy Demonstrates Preclinical Efficacy and Early Clinical Benefit in Duchenne Muscular Dystrophy

- Bryan Zeitler, Sangamo Therapeutics, Inc.
  Sustained Brain-wide Reduction of Prion via Zinc Finger Repressors in Mice and Nonhuman Primates as a Potential One-Time Treatment for Prion Disease

- Chiara Bresesti, Postdoctoral fellow, SR-Tiget
  Comet LV: a lentiviral vector-based mRNA co-packaging technology for enhanced ex vivo and in vivo gene therapy

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

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

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

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

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

- Justin Fang, Mote
  Mobilize LNPs: A Novel Platform for In Vivo Targeted Delivery of RNA Therapeutics to T Cells

- Brett Roach, University of Minnesota, Twin Cities
  Treating Pancreatic Cancer with "Armed" Oncolytic Adenoviruses and Adoptive T-Cell Therapy

- Yizong Hu, Massachusetts Institute of Technology
  Supramolecular Assembly of Polycation/mRNA Nanoparticles and In Vivo Monocyte Programming

- Vibhuti Vyas, City of Hope National Medical Center
  CS1 CAR Binds to Multiple Myeloma-Specific Isoform to Enable Precise Tumor Targeting

- Elie Roumieh, Massachusetts General Hospital
  A human cell-based platform for testing olfactory ensheathing cells as vectors for cancer gene therapy in hiPSC-derived brain-glioma assembloids

- Xiaotong Wang, Stanford University
  Surfaceome CRISPR Activation Screens to Delineate Determinants of Natural Killer Cell Killing towards Cancer Cells

- Joanne Lee, Vanderbilt University
  Attenuating Inflammation in Midbrain Dopaminergic Neurons through TNF-Responsive Synthetic Receptors

- Ji Young Yoo, University of Texas Health Science Center at Houston
  Oncolytic Herpes Simplex Virus and Radiation Therapy Synergize with IGF1R-Targeted Therapy to Enhance Glioblastoma Treatment

Networking
New Member Meet-Up
Location: ASGCT Central, Booth 837
1:00 PM - 1:30 PM

Tools & Technology Session
Tools and Technology Forum 1
Location: Exhibit Theater
1:30 PM - 3:15 PM

Oral Abstract Session
Neurologic Diseases - Vectorology and Gene Therapy
Location: New Orleans Theater A
1:30 PM - 3:15 PM

- TJ Cradick, PhD, HuidaGene Therapeutics, Clinton, NJ, USA; Cholgene Therapeutics, Middletown, DE, USA
  CRISPR-hfCas12Max-Mediated Therapy HG303 Improves Neuromuscular Function and Survival in Amyotrophic Lateral Sclerosis Models

- Celeste Stephany, Capsida Biotherapeutics, Inc.
  Systemic AAV Gene Therapy with Next Generation Engineered Capsid Demonstrates Expression Levels Supporting Potential Therapeutic Benefit for CNS, Cardiac, and Sensory Symptoms in Friedreich's Ataxia

- Christopher Luthers, UCLA
  Hematopoietic Stem Cell Lentiviral Gene Therapy for the Treatment of Angelman Syndrome

- Anoushka Lotun, BS, UMass Chan Medical School
  ASPA Gene Therapy Increases Expression of Oligodendrocyte Transcription Factors, Correlating with Remyelination in Canavan Disease Animal Model

- Andrea Perez Iturralde, Children's Medical Research Institute
  Preclinical Advances for the Translation of an AAV-Mediated Gene Therapy for CTNNB1 Syndrome

- Richard Jude Samulski
  Treatment of Patients with Spinal Muscular Atrophy Using Covalently closed-end AAV

- Rachel Adams, UT Southwestern Medical Center
  Endogenous MicroRNA Feedback for Dosage-Regulated AAV/SLC6A1 Gene Therapy

Oral Abstract Session

Gene Therapy Clinical Trials

Location: New Orleans Theater B
1:30 PM - 3:15 PM

- Yi Chen, The First Affiliated Hospital of Zhejiang University, School of Medicine
  Revolutionizing Wilson's Disease Treatment: Clinical Safety and Efficacy of LY-M003, A Copper-Responsive AAV Gene Therapy Vector

- Hui Xu, Reforgene Medicine
  Updated Safety and Efficacy Results of RM-001, Autologous HBG1/2 Promoter-modified CD34+ Hematopoietic Stem and Progenitor Cells, in Treating Transfusion-Dependent β-Thalassemia

- Stephanie Cherqui, PhD, University of California, San Diego
  Phase 1/2 clinical trial of autologous hematopoietic stem and progenitor cell (HSPC) gene therapy for cystinosis

- Hui Xu, Reforgene Medicine
  First-in-Human Study of Autologous HBA2-Edited CD34+ Hematopoietic Stem and Progenitor Cells in Alpha-Thalassemia with Constant Spring Mutation

- Paul Song, NKGen Biotech, Inc.
  Use of Expanded Non-genetically Modified Natural Killer Cells (Troculeucel) with Enhanced Cytotoxicity in Patients with Alzheimer's Disease. Preliminary Clinical and Biomarker Results.

- Valeria Calbi, MD, San Raffaele Telethon Institute for Gene Therapy (SR-TIGET)
  Lentiviral hematopoietic stem cell gene therapy (atidarsagene autotemcel) for late juvenile Metachromatic leukodystrophy (MLD): Interim analysis of a Phase III trial

- Paul Orchard, MD, University of Minnesota, Blood and Marrow Transplant & Cellular Therapy
  Autologous human peripheral blood B cells genetically engineered to express human iduronidase: Results from a first-in-human clinical trial in subjects with mucopolysaccharidosis type I (MPS I)

Oral Abstract Session

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

Location: New Orleans Theater C
1:30 PM - 3:15 PM

- Mugdha Deshpande, Dyno Therapeutics
  Widespread CNS Delivery With Best-In-Class Liver Detargeting Following Intravenous Injection of a Novel AAV

- Zhenhua Wu, Exegenesis Bio Inc.
  Rationally Designed Receptor-targeting AAV Variants Achieved Efficient CNS Transduction in both Mice and NHPs

- Seongmin Jang, Caltech
  Cryo-EM unveils molecular mechanism of liver detargeting and neuronal tropism of CNS-enhanced AAV capsids

- Damien Maura, PhD, Voyager Therapeutics
  Discovery of AAV9-derived CNS capsids evading pre-existing neutralizing antibodies

- Yuhei Ashida, JCR Pharmaceuticals Co., Ltd.
  Incorporation of Transferrin receptor binder and surface mutations into AAV enables efficient brain delivery and reduced liver tropism

- Nicholas Goeden, Capsida Biotherapeutics, Inc.
  Identification of Multiple Novel Blood-Brain-Barrier Receptors for CNS Gene Therapy and Other Drug Modalities via an Integrated AAV Capsid Engineering Platform

- Mengying Zhang, PhD, Biogen
  Engineering Second-Generation Human Transferrin Receptor 1 (TfR1)-Targeting Capsids with Enhanced Brain Transduction via Differential Directed Evolution and Site Saturation Mutagenesis

Oral Abstract Session

Translational Applications of Base and Prime Editors

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

- Li Li, Editas Medicine, Inc.
  In Vivo Delivery of HBG1/2 Promoter Editing Cargo to HSC of Humanized Mouse and Non-Human Primate with Lipid Nanoparticle

- Alexander Sousa, The Broad Institute of MIT and Harvard
  In Vivo Prime Editing Rescues Alternating Hemiplegia of Childhood in Mice

- Andrew Mudreac, Children's Hospital of Philadelphia
  Long-term Brain Editing and Disease Mitigation After Intracerebroventricular Administration of Base Editor in a Murine Model of Mucopolysaccharidoses Type I

- Daqi Wang, Eye & ENT Hospital of Fudan University
  A base editor for the long-term restoration of auditory function in mice with recessive profound deafness

- Xin Gao, PhD, Harvard University/Broad Institute
  Rescue of Zellweger Spectrum Disorder in Mice and in Patient Cells by Base Editing

- Yan Zhang, University of Michigan
  A Type I CRISPR adenine base editor with a guide-length-tunable editing window and its application in correcting CFTR-G542X null mutation

- Hui Yang, HuidaGene Therapeutics Co., Ltd., Shanghai, China; Shanghai Institute of Meteria Medica, Chinese Academy of Sciences, Shanghai, China
  AI-Assisted Development of Compact Base Editors for Enhanced Efficiency and Specificity with Broad Targeting Range

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 and gene silencing within the 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

Liver Targeted Genetic Therapies

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

- David Waterman, Ph.D., Prime Medicine
  LNP-formulated Prime Editor enables in vivo therapeutic precise editing of the ATP7B p.H1069Q and p.R778L mutations causing Wilson disease

- Roza Ogurlu, Duke University
  Engineering an Endoplasmic Reticulum (ER) Stress Responsive RNA Structural Switch for mRNA and Gene Therapies

- Gerald Lipshutz, MD, David Geffen School of Medicine at UCLA
  A Hybrid AAV PiggyBac Transposon and mRNA-LNP Transposase System for Arginase Deficiency: Long-Term Survival and Arginine Control, with a 50-fold Dose Reduction Compared to AAV Alone

- Zhong-Dong Shi, PhD, Frontera Therapeutics
  Identifying Novel Single Amino Acid Substitutions of Human Factor VIII with Enhanced Cofactor Function for Next Generation Hemophilia A Gene Therapy

- Ivan Krivega, SonoThera
  Development of a Novel Non-Viral Genetic Therapy for Hemophilia A Utilizing Durable, Redosable, and Titratable Approach of Ultrasound-Mediated Delivery of an Oversized Episomal hFVIII DNA Vector

- Chiara Simoni, SR-TIGET
  Liver Fibrosis Negatively Impacts in Vivo Gene Transfer to Hepatocytes

- Ype De Jong, PhD, MD, Weill Cornell Medicine
  Steatosis increases human hepatocyte susceptibility to AAV vector transduction

Oral Abstract Session

CAR T-Cells for Solid Tumors

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

- Jaime Mateus-tique, Icahn School of Medicine
  Armored anti-macrophage CAR T cells remodel the tumor microenvironment and control metastatic ovarian and lung tumor growth.

- Dejah Blake, M.S., Emory University School of Medicine
  Peptide Drug-Secreting CAR T cells Targeting the VIP/VIPR Pathway Display a Novel Phenotype and Superior Antitumor Efficacy

- Edward Song, PhD, Seattle Children's Research Institute
  Engineered CXCR3-A Expression Promotes Intracerebroventricularly Delivered B7-H3-specific CAR T Cell Trafficking and Efficacy against Diffuse Intrinsic Pontine Glioma in Orthotopic Mouse Models

- Elizabeth Carstens, MD, Dana Farber Cancer Institute
  Modeling and Use of Engineering to Overcome On-Target/Off-Tumor Toxicity of Claudin 18.2-Targeted CAR-T cells in Gastric Cancer

- Elliott Brea, Dana-Farber Cancer Institute
  Engineering CAR T cellular therapy around resistance mechanisms in TROP2+ solid tumors

- Courtney Kernick, Stanford University
  Chimeric RNA-Binding Proteins Enhance CAR T Cell Function

- Paula Barbao, IDIBAPS
  CRISPR-Based In Vivo Screen Identifies Key Gene Mediators of CAR-T Cell Dysfunction in Solid Tumors

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, PhD, Recombinetics
  Gene-Edited Swine in Translational Medicine: An Emerging Paradigm for Gene Therapy Testing

- Dmitry Shayakhmetov, PhD, Emory University
  Addressing the viral vector safety in advanced preclinical models

- Melanie Graham, MPH, PhD, University of Minnesota
  Translating with Confidence: De-Risking Cell and Gene Therapies in Primate Models

Fireside Chat

Fireside Chat: European Regulation and the Future of Advanced Therapies

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

- Ilona Reischl, Austrian Agency for Health and Food Safety
  Regulatory Perspectives from the EMA CAT Chair on Advancing CGTs in the EU

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

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

Networking

Career Fair

Location: Level 200 Foyer

3:15 PM - 4:15 PM

Sponsored Symposium
Forge Biologics: Great Science Needs Innovative Manufacturing Technology
Location: Room 271-273
3:45 PM - 4:15 PM

Tools & Technology Session
Tools and Technology Forum 2
Location: Exhibit Theater
3:45 PM - 5:15 PM

Oral Abstract Session
Gene Editing: New Tools and Technology Advances
Location: New Orleans Theater A
3:45 PM - 5:30 PM

- Wendy Shoop, Precision Biosciences
  Systemic Delivery of a Mitochondria-Targeting ARCUS Gene Editing Nuclease by AAV Eliminates Mutant Mitochondrial DNA, Demonstrating Therapeutically Meaningful Heteroplasmy Shifts In Vivo

- Simon Eitzinger, Harvard University
  Programmable gene insertion in human cells using an evolved CRISPR-associated transposase

- Alison Fanton, Arc Institute
  Site-Specific DNA Insertion into the Human Genome with Engineered Recombinases

- Aidan Laird, Seattle Children's Research Institute
  Monoallelic Knockout of Mutant ELANE in Congenital Neutropenia Patient HSPCs Rescues Neutrophil Development In Vivo

- Daniele Canarutto, San Raffaele Telethon Institute for Gene Therapy (SR-TIGET)
  Highly efficient and seamless selection of long-range gene-edited HSPCs by targeting haploinsufficient genes

- Sofia Luna, Stanford University
  Engineered Hematopoietic Stem Cells Give Rise to Therapeutic Antibody Secreting B Cells

- Guocai Zhong, Ph.D., UMass Chan Medical School
  RNA Switch-Based Inducible AAV-CRISPR System for In Vivo Genome Editing

Oral Abstract Session
Viral Vectors in Large Animal Models

Location: New Orleans Theater B
3:45 PM - 5:30 PM

- Nicholas Flytzanis, Capsida Biotherapeutics, Inc.
  Systemic Gene Therapy CAP-002 Demonstrates Potential for Disease-Modifying Treatment of Seizures and Motor and Cognitive Deficits of STXBP1-DEE Using an Engineered, CNS-Targeted AAV

- Avery Hunker, PhD, Allen Institute For Brain Science
  Enhancer AAV Toolbox for Accessing and Perturbing Striatal Cell Types and Circuits

- Priya Dhole, Postdoc, Emory University, Atlanta, GA
  Therapeutic efficacy of AAV-delivered HIV-1 bNAbs to prevent SHIV rebound in rhesus macaques

- Bradford Elmer, PhD, Sanofi
  Focused Ultrasound Enhances Deep Brain Gene Delivery via Intra-CSF AAV Administration in Rodents and Non-Human Primates

- Siddaraju Boregowda, Epicrispr Biotechnologies Inc
  Non-Human Primate (NHP) Safety Study of High-Dose EPI-321: A Novel AAV-Delivered Epigenetic Editing Gene Therapy for the Treatment of FSHD

- Stephen Baine, Sarepta Therapeutics
  Cardiovascular Investigation of SRP-9005 (AAVrh74.MHCK7.hSGCG) in Non-Human Primates: A Gene Therapy for Limb-Girdle Muscular Dystrophy 2C/R5

- Nathan Yingling, UMass Chan
  Evaluating a Bicistronic AAV9 Vector for Tay-Sachs and Sandhoff Disease: Impact of Promoter-Driven Genome Truncations on RNA Expression

Oral Abstract Session

Novel Neurological Disease Models and Therapeutic Approaches

Location: New Orleans Theater C
3:45 PM - 5:30 PM

- Isaac Villegas, UC Davis
  Mouse Models of Jordan's Syndrome Provide New Insights Into Disease Pathophysiology and Therapeutic Development

- Lucas James, BS, UNC Chapel Hill
  Comparison of Angelman Syndrome Candidate Therapeutics Using a Novel Humanized Ube3a-ATS Mouse Model Highlights Major Differences in Efficacy, Tolerability, and Biodistribution

- Aarushi Gandhi, Massachusetts General Hospital
  Investigating Blood-Brain Barrier Functionality in ACTA2 Multisystemic Smooth Muscle Dysfunction Syndrome: A Murine Model Study

- Meagan Quinlan, Ph.D., Allen Institute for Brain Science
  AAV Delivery of Full-Length SYNGAP1 Rescues Epileptic and Behavioral Phenotypes in a Mouse Model of SYNGAP1-Related Disorders

- Ailing Du, PhD, University of Massachusetts Medical School
  Developing an AAV-based gene replacement therapy for leukodystrophy caused by mitochondrial alanyl-tRNA synthetase 2 (AARS2) deficiency

- Kathryn Reynolds, Tufts University
  Isoform-specific re-expression of human FMRP in mice rescues Fragile X syndrome-related translation and behavior phenotypes

- Morgan Mooney, UMass Chan Medical School
  How Knocking Out Galectin-3 In Canavan Disease Model Mice Affects Disease Progression

Oral Abstract Session

Pharmacology/Toxicology Studies and Analytics/Assay Development Session I

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

- Maryam Tarazkar, Genentech
  Enhancing TCR Off-Target Potency and Safety Through In Vitro and In Silico Approaches

- Tejashree Redij, Catalent Pharma Solutions
  A Comprehensive and Robust Analytical Platform for Clinical Grade CAR-T Cells

- SUBRATA BATABYAL, Nanoscope Technologies LLC
  Development of Orthogonal Potency Assays for Multi-Characteristic Opsin Gene Therapy: Gene Expression and Light-Stimulated Activity

- Pushpendra Singh, Ocugen
  Preclinical safety evaluation of AAV5-hNR2E3 (OCU400), a mutation agnostic gene therapy candidate for retinitis pigmentosa and leber congenital amarousis

- Lakmini Wasala, Kriya Therapeutics
  Development and Evaluation of AAV9.anti-IGF1R (Insulin-like Growth Factor-1 Receptor) Vector Potency Methods for KRIYA-586, a One-time Gene Therapy for Thyroid Eye Disease (TED).

- Fabrizio Benedicenti, SR-TIGET
  Automated and Miniaturized Sonication Linker-Mediated PCR (SLiM-PCR) for High-Throughput Integration Site Analysis in Gene Therapy Applications

- Dehui Kong, UCSF
  Evaluation and Modeling of TransAct Addition for synNotch--CAR Induction Assay in E-SYNC T Cells

Oral Abstract Session

Molecular and Cellular Methodology

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

- Joanna Szumska-Aubermann, ProtaGene CGT GmbH
  Highly Sensitive Detection and Characterization of Intended and Unintended Gene Editing Events by TES.

- Junping Zhang, PhD, Indiana University
  Bioinformatic analysis of the genetic basis of differential AAV production capability of 293 variants

- Ajeet Singh, ATCC
  ATCC® Cell Line Land: an OMICS Data Repository for ATCC® Cell Models that Drives Scientific Innovation and Improves Reproducibility

- Ali Nowrouzi, PhD, Spark Therapeutics
  Comparison of Linker mediated PCR (LM-PCR) and Target enrichment mediated (TES) methods for genome wide retrieval of rare AAV integration events in preclinical models

- Myriam Lemmens, Novartis Institutes for BioMedical Research Inc
  Evaluation of Target Enrichment Sequencing to Assess AAV Integration Patterns for the Safety Assessment of Gene Therapies

- Tim Rath, ProtaGene CGT
  Highly Sensitive Detection of Integration Sites by S-EPTS/LM-PCR and TES for Various Types of Therapeutic Vectors

- Luca Nanni, NewBiologix
  Redefining rAAV Vector Analysis and Quality Control with Orthogonal Long-read Sequencing Technologies.

Oral Abstract Session

Innovations in in vivo Targeting of HSPCs and Immune Cells

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

- Giulia Schiroli, PhD, Tessera Therapeutics
  In Vivo HSC Gene Editing for Correction of the Sickle Cell Mutation Using RNA Gene Writers

- Justin Eyquem, PhD, UCSF
  In Vivo Generation of TRAC-Targeted CAR T Cells via Site-Specific Integration Enables Cell-Specific Engineering and Potent Antitumor Activity

- Pauline Schmit, nChroma Bio
  Efficient and Liver-Detargeted In Vivo Multiplex Gene Editing of Human Hematopoietic Stem and Progenitor Cells

- Alberto De Iaco, Tessera Therapeutics
  Targeted LNP Delivery of an RNA Gene Writer In Vivo Enables Generation of functional CAR-T Cells in a Humanized Mouse Model

- Justin Thomas, Fred Hutchinson Cancer Center
  Targeted Multiplexed Virus-like Particles (MVPs) enable robust in vivo Hematopoietic Stem Cell (HSC) engineering

- Anna Anderson, University of Washington
  In Vivo Hematopoietic Stem Cell Gene Therapy for HIV by Base Editing of CCR5 Alone and in Combination with HIV Decoy Receptor Expression

- Jia Yao, PhD, Emory University
  Analysis of safety, selectivity, and efficacy of in vivo hematopoietic stem cell transduction after intravenous administration of AVID adenovirus vector in Rhesus macaques

Oral Abstract Session

Oligonucleotide Therapeutics I

Location: Room 291-292
3:45 PM - 5:30 PM

- Deeann Wallis, University of Alabama At Birmingham
  Preclinical development and in vivo delivery of antisense oligonucleotides for targeted NF1 exon 17 skipping.

- Naoki Iwamoto, Wave Life Sciences
  Silencing of Inhbe mRNA Using GalNAc-siRNAs Induces Durable Weight Loss in a Mouse Model of Diet-Induced Obesity

- Jessica Centa, University of Michigan
  Splice-Switching Antisense Oligonucleotide Drug Discovery for CLN3 Batten Disease

- Marcin Kortylewski, PhD, City of Hope Comprehensive Cancer Center
  Thiopurine-Based Oligonucleotide for Targeted Telomere Damage in Leukemia and Lymphoma Cell in Vivo

- Hector Ribeiro Benatti, UMass Chan Medical School
  Advancing HTT-lowering divalent siRNA therapy for enhanced safety and efficacy in the central nervous system

- Benjamin Spangler, Evercrisp Biosciences
  Computationally Designed Miniproteins Enable Tissue-Specific Oligonucleotide Delivery Through Cell Surface Receptor-Targeting

- Hagoon Jang, Stanford University
  NEAT1 lncRNA Structure-Mediated Functional Modulation as a Novel Therapeutic Approach for MYC-Driven Cancers.

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, Foundation for Angelman Syndrome Therapeutics
  Collaborative translational and transformative research driven by patient advocacy groups and research labs

- Michael Deininger, MD, PhD, Versiti Blood Research Institute
  Maintaining an applied and translational research lab in academics

- Mary Eapen, MRCPI, MS, MBBS, Medical College of Wisconsin
  Academia and industry collaboration to advance patient care through early clinical trials

Scientific Symposium

Career Development & DEI Awardee Presentations 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, 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 for treatment of LGMDR9 in 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, MD, 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 Inc.
  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

Thursday, May 15

Networking
Global Gathering Breakfast
Location: Room 265-268
7:00 AM - 8:00 AM

Scientific Symposium
Improved Therapeutic Delivery of Nanoparticles (Organized by the Nonviral Therapeutic Delivery Committee)
Location: New Orleans 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: New Orleans 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 dystrophyBypassing issues relative to capsid and transgene expression in AAV-mediated transfer for Muscular Dystrophies

- Mansuo Shannon, AskBio
  AI use for promotor and transgene design

Oral Abstract Session

HSC Transplantation and Gene Therapy

Location: New Orleans Theater C
8:00 AM - 9:45 AM

- Natnicha Jakramonpreeya, Chakri Naruebodindra Medical Institute (CNMI)
  Attack and Defend Against HIV-1 Infection Using HSPC-based Gene Therapy With a Safety Kill Switch

- Jennifer Okalova, Emory University School of Medicine
  Enhancing ADC-Based Non-Genotoxic Conditioning by Targeting Cycling Stem Cells and Lymphocyte Depletion for HSC-Directed Lentiviral Gene Therapy for Hemophilia A

- Francesco Mazziotta, Fred Hutchinson Cancer Center
  Dilanubicel Improves Single Cord Blood Transplantation: Updated Results with a Larger Cohort

- Yoonjeong Jang, DVM, PhD, St. Jude Children's Research Hospital
  BCL11A-Deficient Human Erythropoiesis is Impaired in In Vitro Culture and Xenotransplanted Bone Marrow

- Hyunmin Cho, Stanford University Laboratory for Cell & Gene Medicine
  Myeloid Cell Replacement Therapy Improves Function in Friedreich Ataxia Mice by Intercellular Mitochondrial Transfer

- Nicholas Petty, Student, Fred Hutchinson Cancer Center
  CSF1R Inhibitor Treatment Facilitates Engraftment of Hematopoietic Stem Cell-Derived Microglia-Like Cells in Nonhuman Primates

- Oluwaseun Babatunde, Wake Forest Institute for Regenerative Medicine
  Investigating Novel Non-Genotoxic Conditioning Approaches for In Utero Hematopoietic Stem Cell Transplantation

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

- Gregory Sowd, Caring Cross
  Lowering CAR T cell therapy cost and increasing accessibility

- John Tisdale, MD, NIH, NHLBI
  Federal government role in regulating CGT's

Scientific Symposium

Publishing 101

Location: Room 278-282
8:00 AM - 9:45 AM

- Paloma Giangrande, Eleven Therapeutics, Inc.
  Before Submission

- Daniel Stone, PhD, Fred Hutchinson Cancer Center
  Revision and Review

- Timothy Cripe, MD, PhD, Nationwide Children's Hospital
  After Publication

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
  Advancing Gene Therapy to Treat Kidney Disease

- Leif Oxburgh, DVM PhD, The Rogosin Institute
  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

- Patricia Lam, PhD, Nationwide Children's Hospital
  Persistent Expression of Liver-Directed AAV Gene Therapy Improves Long-term Outcomes in a Mouse Model of Lysosomal Acid Lipase Deficiency

- Rafael Badell-Grau, UC San Diego
  Hematopoietic stem cell gene therapy for Mucopolysaccharidosis type IIIC

- Jillian Gallagher, BS, UMass Chan Medical School
  Testing a Dual AAV Gene Therapy Vector Construct to Treat Sialidosis and Galactosialidosis Using Small and Large Animal Models

- Shih-Hsin Kan, Children's Hospital of Orange County
  Evaluating the Efficacy of iPSC-Derived Neural Stem Cell Transplantation in an MPS I Mouse Model

- Rose Sheridan, PhD, Spur Therapeutics Ltd
  Durability of FLT201: an Investigational Gene Therapy for Gaucher Disease Type 1 Encoding an Engineered Variant of the GCase Enzyme

- Samantha Howard, PhD, Alexion AstraZeneca
  Optimized Liver Targeted GBA Expression via AAV3b for Type 1 Gaucher Disease Treatment

- Ludovica Santi, SR-TIGET
  An Innovative Platform Approach for the Parallel Development of HSPC-GT for Rare/Ultra-Rare Lysosomal Storage Disorders with Severe Skeletal and Neurological Manifestations.

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

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

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

- Rajeev Sivasankaran, Voyager Therapeutics, Inc.
  Intravenous Delivery of VY1706, a CNS penetrant AAV gene therapy for Alzheimer's Disease, Provides Broad Tau Lowering in NHP

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

- Megan Hoban, Orna Tx
  In Vivo panCAR™ Therapy Using Circular RNA for the Treatment of Autoimmune Disease

Fireside Chat

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

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

Sponsored Symposium

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

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

Sponsored Symposium

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

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

Networking

Exhibit Hall

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

Networking

Career Fair

Location: Level 200 Foyer
9:15 AM - 10:15 AM

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 Therapies for Severe, Early-Onset Genetic Diseases

- Jeffrey Chamberlain, PhD, University of Washington
  Outstanding Achievement Award

Networking

Post-General Session Networking

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

Sponsored Symposium

Bio-Techne: Protein Quantitation Applications to Advance Gene Therapy Development – From Discovery Through Analytical Development

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

Sponsored Symposium
Biogen: Biogen Gene Therapy
Location: Room 388-390
12:15 PM - 1:15 PM

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

Poster Talk Session
Thursday Poster Talks
Location:
12:15 PM - 1:00 PM

- Andrew Nelson, Broad Institute of MIT and Harvard
  Adenine Base Editing Rescues Dravet Syndrome in Mice

- Sandhiya Ravi, PostDoctoral Fellow, Umass Medical School
  Enhancing Truncation Event Prediction in AAV Vector Genome Designs Through Advanced Deep Learning

- Hao Liu, PhD, UMass Chan Medical School
  A New AAV Manufacturing Platform with in Cellulo Replication of Plasmid DNA in HEK293 Cells

- Tapan Sharma, UMass Chan Medical School
  Deep learning-trained codon optimization algorithms identify novel tissue-derived codon usage patterns and enhance transgene expression in vivo

- Ryan Giovenco, Children's Hospital of Philadelphia
  Delivery of miRNA by Self-Complimentary AAV Provides ATXN1 Knockdown in SCA1 Mice and Supports Therapeutic Translation

- Anna Keegan, UCL
  AAV-mediated gene therapy for Pyruvate Dehydrogenase Complex Deficiency

- Bang Wang, GenEditBio Limited
  Safe and Efficient Editing of Novel CRISPR-Cas Ribonucleoprotein Complexes Delivered by Engineered Protein Delivery Vehicle

- Pranav Mathur, DVM, PhD, Cirsium Biosciences
  Addressing the Manufacturing Bottleneck in Gene Therapies Through AAV Production in Whole Nicotiana benthamiana Plants

- Nayra Gad, Pfizer
  AAV9 Neutralizing Antibody Seroconversion in Household Contacts Of Participants With Duchenne Muscular Dystrophy Receiving Fordadistrogene Movaparvovec

Networking
New Member Meet-Up
Location: ASGCT Central, Booth 837
1:00 PM - 1:30 PM

Tools & Technology Session
Tools and Technology Forum 3
Location: Exhibit Theater
1:30 PM - 3:15 PM

Oral Abstract Session
Epigenetic Editing and RNA Editing
Location: New Orleans Theater A
1:30 PM - 3:15 PM

- Cian Schmitt-Ulms, MIT
  Programmable RNA Writing with Cleavage Enhanced Trans-splicing

- Ornit Chiba-Falek, Duke University
  Neuronal-Type Specific SNCA-Targeted Epigenome Therapy for Precision Medicine in Synucleinopathies

- Hui Xu, Reforgene Medicine
  Efficacy and Long-term Safety of CRISPR/Cas13 RNA-targeting Medicine in Mouse and Non-human Primate Models of Neovascular Age-related Macular Degeneration

- Ian Harding, Wave Life Sciences
  Applying AIMer-Based RNA Editing Technology to Correct a Nonsense Mutation in the Lung

- Julian Halmai, PhD, MS, UC Davis
  Molecular and behavioral rescue following dual AAV targeted CRISPR epigenome editing in CDKL5 deficiency disorder patient-derived organoids and transgenic mice

- Sonia Vallabh, Broad Institute
  Investigator-initiated Development Paths for Oligonucleotide and Epigenetic Therapies in Prion Disease

- Fan Yang, City of Hope
  An epigenetic gene therapy delivered using lipid nanoparticles as a treatment for HPV-associated malignancies

Oral Abstract Session

Novel Approaches to Gene Targeting and Gene Correction

Location: New Orleans Theater B
1:30 PM - 3:15 PM

- Jamaica Siwak, St. Jude Children's Research Hospital
  Essential HDRescue: Enhancement of Genome Editing by Harnessing Cellular Vulnerabilities

- Brent Stead, PhD, MBA, Specific Biologics Inc.
  Precise Removal of a Large Pathogenic Repeat Expansion In Vitro and In Vivo Using a Dual-guided TevCas9 (Dualase®) Genome Editor Encoded in a Single AAV

- Sébastien Levesque, Boston Children's Hospital
  In Cellulo DNA Assembly for Targeted Genomic Integration in Human Cells

- Chang Li, PhD, University of Washington
  Development of Novel HDAd Vectors for Efficient Targeted Integration via Prime Editing and Site-Directed Recombination

- Christopher Wilson, Stylus Medicine
  Engineering High-Efficiency, High-Specificity Recombinases for Therapeutic In Vivo Genome Engineering

- Daisy Ayala-Gomez, Children's National Hospital
  Alemtuzumab-Resistant Virus-Specific T (VST) Cells Developed for the Prevention of Viral Infections After Hematopoietic Stem Cell Transplantation Retain Antiviral Activity In-Vivo and In-Vitro

- Swati Bijlani, PhD, City of Hope
  Nuclease-Free In Vivo Genome Editing of the CNS Following Systemic Administration of AAVHSC15 Vectors

Oral Abstract Session

Downstream Manufacturing for AAV Vectors

Location: New Orleans Theater C
1:30 PM - 3:15 PM

- Dalton Kinnard, MS, Chromatan
  Integrated AAV Capture and Polishing via BioRMB™, a Continuous, Column-free Chromatography Platform

- Kelvin Idanwekhai, University of North Carolina, Chapel Hill
  Data-Efficient and Adaptive Machine Learning Framework for Accelerated AAV Downstream Processing.

- Benjamin Graf, Sartorius Lab Instruments GmbH & Co KG
  Combining Steric Exclusion with Anion Exchange Chromatography – Development of an Innovative, Affinity-Independent, and Scalable AAV Downstream Process

- Jennifer Haley, Isolere Bio
  Neutral pH Elution of AAV using Affinity Liquid Phase Separation (ALPS) in Centrifugation and Tangential Flow Filtration (TFF) Formats for Flexible Downstream Processing

- Qiuge Zhang, Solventum
  Development of a Scalable and Cost-Effective Clarification Strategy for Adeno-Associated Virus Bioprocessing Using Single-Use Anion Exchange Fibrous Technology

- Wenning Chu, North Carolina State University
  Purification of a broad range of adeno-associated virus serotypes from HEK293 cell lysate using peptide-based affinity AAVW$_{II}$ adsorbent and quantitative proteomic analysis of residual host cell protein via LC-MS/MS technology

- Arjun Bhadouria, Sanofi
  Novel anion exchange chromatography elution approach to improve the Adeno-associated virus process robustness

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

- Yumi Sano, Heidelberg University
  Engineering of Next-Generation Bocavirus Vectors by Surface Loop Modification and Peptide Insertion

- Selene Ingusci, pHD, University of Pittsburgh
  Investigating the Influence of Viral and Cellular Insulators on Stable HSV-1 Vector-Mediated Transgene Expression in Various Tissues

- Katrin Schröer, Witten/Herdecke University
  Novel adenovirus vaccine vectors lacking binding to the thrombosis associated Platelet Factor 4 protein

- MIJEONG KIM, Pacira Biosciences Inc.
  Understanding the Clinical Immunogenicity of Locally Injected HCAd Vector Provides Insight Into Optimizing Dosing Strategy

- Megha Gupta, Fred Hutchinson Cancer Center
  Suspension Cell Platform for the Production of Cocal-pseudotyped Lentiviral Vectors

- John Mich, Allen Institute for Brain Science
  Widespread AAV chimerism and other sources of noise confound multiplexed enhancer screening in brain

- Ni Shuai, Waker Bioscience Co.
  PACS1 Gene Integration and Clonal Expansion in HIV-1 Persistence: Insights from Longitudinal Analysis of HIV Patients

Oral Abstract Session

Novel Genetic Approaches for Muscle and Skeletal Diseases

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

- Renan Sper, Mammoth Biosciences
  Non-human primate muscle gene editing via single systemic AAV delivery of ultra-compact CRISPR nuclease

- Julien Oury, Tevard Biosciences
  Rescue of Full-Length Dystrophin Protein and Motor Performance in a Mouse Model of Duchenne Muscular Dystrophy Using an AAV-tRNA Therapeutic

- Maëlle RALU, GENETHON
  CRISPR-Cas9 Mediated Endogenous Utrophin Upregulation Improves Duchenne Muscular Dystrophy

- Zelong Dou, Baylor College of Medicine
  Overexpressing Murine Prg4 Isoforms with High-Capacity Adenoviral Vectors (HCAd) by Intra-Articular Injection Improves Articular Cartilage Preservation in a Murine Model of Post-Traumatic Osteoarthritis (PTOA)

- John Sincavage, Children's Hospital of Philadelphia
  A Prenatal Gene Editing Approach to Congenital Musculoskeletal Disease with Systemically Delivered Lipid Nanoparticles

- Burcak Ozes, PhD, Nationwide Childrens Hospital
  AAVrh74.tMCK.hBAG3 Gene Therapy Improves Phenotype in a Hereditary IBM Model, VCP-A232E Mouse

- Fady Guirguis, BS, NINDS
  Identifying Adeno-Associated Virus Serotypes that Transduce Fibro-Adipogenic Progenitors in Mouse Healthy and Fibrotic Skeletal Muscles In Vivo

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: An academic perspective

- Michael Poisel, Independent
  Venture Studio/Incubator: What do they do and how can they help?

- Hyo Min Park, PhD, GenEdit
  Young Founder Perspective

Oral Abstract Session

Lipid Nanoparticles I

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

- Melissa Soto, PhD, PharmD, The University of Texas at Austin College of Pharmacy
  Discovery of peptides for targeted delivery of mRNA lipid nanoparticles to cystic fibrosis lung epithelia

- Jacek Lubelski, NanoCell
  Novel Non-Viral DNA-based Gene Therapy Vector for CAR T Engineering In Vivo

- Poulami Chaudhuri, Helex
  Lipid Nanoparticle-Mediated CRISPR-based Therapy Enables Mutation-Agnostic Gene Editing Solving for Autosomal Dominant Polycystic Kidney Disease

- Grishma Pawar, NanoVation Therapeutics
  Rational Design and Preclinical Applications of Long Circulating Lipid Nanoparticles (lcLNPs) for Extra Hepatic Delivery of Nucleic Acids

- Johannes Schwerk, Poseida Therapeutics
  Enhancing Liver DNA Delivery with a Fully Non-Viral Multifunctional LNP Approach for In Vivo Transposon-Mediated Genomic Integration of Large DNA Cargo

- Sean Semple, Acuitas Therapeutics, Inc.
  Pharmacodynamic Activity, Pharmacokinetics and Tolerability of Lipid Nanoparticle Formulations of mRNA Following Repeated Intravenous Dosing in Monkeys

- Nicholas Tursi, The Wistar Institute
  Modulation of Lipid Nanoparticle-Formulated Plasmid DNA Drives Innate Immune Activation Promoting Adaptive Immunity

Sponsored Symposium
Catalent: Partnerships to Enhance Development and Intensification of Viral Vector Production
Location: Room 383-385
2:00 PM - 2:30 PM

Sponsored Symposium
Sartorius BIA Separations - Orthogonal chromatography analytics to allow for faster AAV or LNP process development and better in-process control
Location: Room 391-392
2:30 PM - 3:00 PM

Networking
Career Fair
Location: Level 200 Foyer
3:15 PM - 4:15 PM

Networking
Patient Advocate Meet-Up
Location: Nonprofit Pavilion, Exhibit Hall (Halls G-H, First Floor)
3:15 PM - 4:15 PM

Networking
Trainee and New Investigator Meet-up
Location: ASGCT Central, Booth 837
3:15 PM - 4:15 PM

Tools & Technology Session
Tools and Technology Forum 4
Location: Exhibit Theater
3:45 PM - 5:15 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

Oral Abstract Session
Novel Therapeutic Gene Editing Applications
Location: New Orleans Theater A
3:45 PM - 5:30 PM

- Scot Wolfe, UMass Chan Medical School
  Efficient Cas9 LNP Mediated Repair of a Pathogenic TCAP Mutation in Skeletal Muscle

- Xiaona Lu, Yale University School of Medicine
  Brain-wide Genome Editing via STEP-RNPs for Treatment of Angelman Syndrome

- Chiara Simoni, SR-TIGET
  Development of in Vivo Genome Editing for the Treatment of Progressive Familial Intrahepatic Cholestasis Type 2

- Shlomo Moss, Tel Aviv University
  In vivo site-specific T cell engineering allows complete remission of human leukemia in mice

- Guoxiang Ruan, Excision BioTherapeutics
  Preclinical Development of a CRISPR-Cas9-Based Therapeutic for the Treatment of Herpes Keratitis

- Michael Martinez, Oregon Health and Sciences University
  Enhancing HDR-Mediated Pah Transgene Insertions in a Murine Model of Phenylketonuria

- Jack Castelli, HBSc, Fred Hutchinson Cancer Center
  Editing Hematopoietic Stem and Progenitor Cells by Non-Viral Gene Knock-In Produces Anti-HIV Antibody In Vivo

Oral Abstract Session

Manufacturing and Transduction for Viral Vectors

Location: New Orleans Theater B
3:45 PM - 5:30 PM

- Annika Mittelhauser, UCLA
  Suppressing APOBEC3 in Lentiviral Vector Producer Cells to Maximize Transgene Fidelity

- Devin Stranford, Syenex, Inc.
  Engineering Lentiviruses for Enhanced Transduction of Primary Human T Cells

- Michael Barry, PhD, Mayo Clinic
  Purification and Chemical Shielding of Adenoviral Vectors for Gene Therapy, Vaccines, and Oncolytic Virotherapy

- Yue Zhang, Ring Therapeutics
  AnelloBricks: Development of a Scalable, Low-Cost, In-Vitro Assembled Anellovirus-Derived Platform for Gene Therapy Applications

- Joseph Collins, PhD, Asimov
  A Massively Parallel Reporter Assay for Evaluating Lentiviral Transfer Plasmid Design

- Jonathan Gunn, UCSD
  Non-Integrative and Long-Lived Expression of Chimeric Antigen Receptor with Self- and Trans-Amplifying RNA

- Brenna Stevens, University of Guelph
  Comparison of dual AAV vector strategies for lung gene therapy

Oral Abstract Session

AAV Vector Manufacturing: Experimental Design & Analytics

Location: New Orleans Theater C
3:45 PM - 5:30 PM

- Belinda Mativenga, North Carolina State University
  Quantification of Empty and Full Adeno-associated viral Capsids using Chip-Based Molecular Diagnostics

- Anisha Haris, Waters Corporation
  Leveraging Charge Detection Mass Spectrometry to Overcome Key Characterization Challenges in Recombinant Adeno-Associated Viruses

- Susumu Uchiyama, Osaka University
  Production of adeno-associated viral vectors by a novel human derived cell line HAT and comprehensive characterization of purified vectors

- Emilie Gateau, Exothera
  Building efficient AAV testing platform: Prioritizing critical quality attributes with risk ranking-based scoring

- Thibaut Deschamps, MilliporeSigma
  Enhanced AAV Capture Using Membrane Chromatography and Optimized Mid-Stream Processing

- Ram Shankar, PlasmidFactory GmbH
  Stable maintenance of full-length ITRs in AAV transfer plasmids

- Olaide Ibiyemi, University College London
  Shedding Light on AAV Manufacturing: Application of Raman Spectroscopy for Real-Time Process Monitoring

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, PhD, 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 Inc.
  Neurogene's experience with the START Pilot Program in advancing their NGN-401 product for Rett Syndrome

- Susan Telliard, MS, MBA, 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 Gene Therapy
  Myrtelle's experience with the START Pilot Program in advancing their rAAV-Olig001-ASPA product for Canavan Disease

Oral Abstract Session

Vaccines and Immunotherapy for Cancer

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

- Timothy Cripe, MD, PhD, Nationwide Children's Hospital
  Programmable Adeno-Associated Virally Delivered Lifelong In Vivo T Cell Engagement Provides a Novel Framework for Prevention and Treatment of Metastatic Cancer

- Jian-Dong Huang, PhD, The University of Hong Kong
  Employing Pre-Existing Immunity Against Pathogens for Enhanced Cancer Immunotherapy via mRNA Vaccines

- Shoji Saito, MD, PhD, Shinshu University
  mRNA-LNP Vaccine Providing Antigen and Co-Stimulation in the Tumor Microenvironment (CART-Vac) Enhances CAR-T Cell Function

- Richard Vile, PhD, Mayo Clinic
  Cancer Immunotherapy Using AIRE Conditioning of the Tumour Epitopeome

- William Jia, Virogin Biotech Canada Ltd.
  Unlocking Durable Anti-Tumor Immunity: Oncolytic Virotherapy as a Solution to Tumor Vaccine Shortcomings

- Mansi Narula, Baylor College of Medicine
  A Dual-Stimulatory Receptor Provides T-Cell Activation-Dependent Costimulation to Augment Native and Transgenic TCR-Based T-Cell Therapies

- Pin Wang, PhD, University of Southern California
  APC-Targeted LNP Enables Systemic Delivery of Neoantigen mRNA Vaccines and Enhanced Antigen-Specific T Cell Responses

Oral Abstract Session

Organoids and iPSC Disease Modeling for Drug Discovery

Location: Room 291-292
3:45 PM - 5:30 PM

- Helen Streff, Duke University
  Engineering Functionally Mature iPSC-derived Hepatocytes through Mapping Transcription Factor Regulatory Mechanisms

- Katherine Whiteman, Children's Hospital of Philadelphia
  Phenotypic Characterization and AAV-Mediated Therapeutic Delivery in C9ORF72 ALS iPSC-Derived Motor Neurons

- Marisa Hamilton, Duke University
  Understanding Schizophrenia-Associated Loci Using iPSC-Derived Neurons and CRISPR Screening

- RAMI AQEILAN, Hebrew University of Jerusalem
  From Gene to Therapy: Unraveling WWOX and Its Role in Neurological Diseases

- Vania Broccoli, Ospedale San Raffaele
  A fully human pluripotent stem cell-derived blood-brain barrier model validates the therapeutic potential of neurotropic adeno-associated viruses

- Mariana Argenziano, Ncardia
  Advancing Cardiovascular Drug discovery with iPSC-Derived 3D Cardiac Microtissues in High-Throughput Screening

- Gaia Ruggeri, Genentech, Inc
  In Vitro Assays to Study Neuroprotection and Axon Regeneration in Human Neurons Differentiated from Neurogenin-2 Engineered Induced Pluripotent Stem Cells

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

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

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

- David Wilcox, PhD, Medical College of Wisconsin
  Follow-up on Platelet-Targeted Gene Therapy for Hemophilia A

Scientific Symposium

Career Development & DEI Awardee Presentations 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

- Jose Martinez-Navio, PhD, University of Miami
  Preventing humoral responses to AAV-delivered anti-HIV antibodies in rhesus macaques

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, MD, PhD, Norton Chlidren's Research Institute
  Neuro ethic decisions in patient care

- Alison Bateman-House, MPH, PhD, 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, JD, University of Wisconsin Law School
  Ethical Challenges in Post-Roe America

Oral Abstract Session

CAR T Innovations in Autoimmune and Infectious Disease and Allergy

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

- Nils Wellhausen, PhD, University of Pennsylvania
  Selection for an HIV-Resistant Immune System by Multiplex Base-Edited CD45 CAR-T Cell Therapy

- Federica Severi, Wistar Institute
  Multivalent CAR T cell therapy targeting membrane distal epitopes restricts HIV escape kinetics and enhances control of HIV replication in humanized mice

- Christopher Moore, Gentibio
  CAR19 Engineered Regulatory T Cells, a Novel Approach for Immune Reset in B and T Cell Mediated Autoimmune Disorders

- Tali Stauber, Tel Aviv University
  Chimeric Antigen Receptor T Cells Targeting the IgE B Cell Receptor Specifically Eliminate Human IgE Producing B cells

- Haig Aghajanian, Capstan Therapeutics
  A Two-Infusion Regimen with a Novel In Vivo Non-Viral Chimeric Antigen Receptor (CAR) Achieves up to 90% CD8+ T Cell Engineering and Tissue Depletion of Target Cells in Non-Human Primates (NHPs)

- Sarah Doherty, Fred Hutchinson Cancer Center
  Depleting the Latent HIV-1 Reservoir with Anti-CD4 CAR T-cells

- Christopher Borges, Sanofi
  CD19 CAR T Cells Generated In Vivo by T Cell Targeted Lipid Nanoparticles Demonstrate Robust and Durable B Cell Depletion in Non-Human Primates

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

Reception
Women in Cell and Gene Therapy Reception
Location: Midhouse Level Balcony
6:00 PM - 8:00 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

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

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

Scientific Symposium
Tissue-Specific Insights in AAV Vector Immunogenicity (Organized by the Immune Responses to Cell and Gene Therapy Committee)
Location: New Orleans 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
  AAV ocular toxicity: the impact of defining biological characteristics

- Anna Kajaste-Rudnitski, PhD, University of Pavia
  Innate immune sensing of AAV in CNS cells (Virtual Presentation)

Scientific Symposium

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

Location: New Orleans 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

- 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: New Orleans 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

- Liliana Thron, University of Minnesota
  Armored HIV-specific CAR NK and Activated NK Cells Are Safe and Led to Viremic Control in a Subset of HIV-Infected Humanized DRAGA Mice

- Jason Murray, PhD, Fred Hutchinson Cancer Center
  Broadly-neutralizing antibodies and CCR5-edited hematopoietic stem cell transplantation synergistically delay virus recrudescence in a nonhuman primate model of HIV

- Christian McRoberts Amador, Duke University
  Epigenetic Reprogramming of Tumor-Infiltrating Lymphocytes for Improved Anti-Tumor Killing

- Jared Pudiwitr, Deverra Therapeutics
  Producing Genetically Engineered, Cytokine-Secreting Allogeneic Monocytes from CD34+ Cord Blood Cells as Cell Therapy for Solid Tumors

- Martina Spiga, San Raffaele Scientific Institute
  TITLE: TIGIT Deletion Rescues the Antitumor Activity of Low Avidity T cells by Increasing TCR Signal Strength

- Lauren Sarko, University of Wisconsin-Madison
  CRISPR-Engineered CAR T Cells for Targeting Senescence-Associated Urokinase-Type Plasminogen Activator Receptor (uPAR)

- Joey Leal, Lyell Immunopharma
  Engineered T Cells Combining Stackable Reprogramming Technologies Enable Durable Anti-tumor Activity in Xenograft Solid Tumors

Oral Abstract Session

Emerging Delivery Platforms for In Vivo Gene Editing

Location: Room 278-282
8:00 AM - 9:45 AM

- Samantha Roudi, Karolinska Institutet
  Engineered Extracellular Vesicles Enable Efficient Gene Editing in the Mouse Brain

- Xiaofei Gao, Westlake University
  The First Coacervate-based Delivery System for Advanced Gene Therapy

- Zihua Jiang, Karolinska
  Engineered Protein Delivery Vehicles Enable Single Intrastromal CRISPR-Cas9 Therapy for TGFBI Corneal Dystrophies with High Efficacy and Safety: A Comprehensive Preclinical Assessment in Non-Human Primates

- Tomohiro Umezu, PhD, Tokyo Medical University
  CRISPR/Cas9 delivery using acerola-derived nanoparticles for targeted gene editing in the central nervous system

- Kenneth Sims, PhD, Battelle Memorial Institute
  Enhancing Nanoparticle Delivery of Therapeutic Neurofibromatosis Type 1 Epigenetic Regulatory Protein Payloads In Vivo

- Karthik Karuppusamy, PhD, University of Washington
  In vivo Hematopoietic Stem Cells transduction to Deliver Neuroprotective Extracellular Vesicles for Parkinson's Disease Treatment

- Xiaoshu Pan, University of Florida
  Enabling Extracellular Vesicles for Targeted Gene Delivery Using Deep Learning Models-Designed Ligands

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

- Olajide Williams, Columbia University
  Access to GCT Clinical Trials: local and global challenges

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

- James Elder, The Ohio State University
  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, MBBS PhD, 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, 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, PhD, 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, PhD, University of California Berkeley
  The pathway to bringing gene and cell therapies from bench to bedside

- J. Fraser Wright, PhD, Kriya Therapeutics
  The pathway to bringing gene and cell therapies from bench to bedside

Networking
Exhibit Hall
Location: Exhibit Hall
9:00 AM - 3:45 PM

Networking
Career Fair
Location: Level 200 Foyer
10:15 AM - 11:15 AM

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

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

Scientific Symposium
ASGCT and Citeline Present: The Gene, Cell, and RNA Therapy Landscape

Location: Exhibit Theater
12:15 PM - 12:45 PM

- David Barrett, American Society of Gene and Cell Therapy
  Key Highlights from the Quarterly Landscape Report

- Daniel Diguadio, Citeline
  Deep Dive into Landscape Data and Products

Networking

New Member Meet-Up

Location: ASGCT Central, Booth 837
1:00 PM - 1:30 PM

Tools & Technology Session

Tools and Technology Forum 5

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

Oral Abstract Session

AAV Gene Transfer (B): Ocular, Neurological & Immune Cell Systems

Location: New Orleans Theater A
1:30 PM - 3:15 PM

- Shengjiang Liu, Avirmax Inc.
  Introduction of a Lysine Acetylation Motif into the AAV2 VP1 to Enhance Macular Tropism and Intravitreal Administration

- Richard Sullivan, Shape Therapeutics, Inc.
  Systemically delivered AAV5-based capsid variants enable up to 88% targeted RNA editing in primate brain

- Alice Reschigna, Department of Ophthalmology, LMU University Hospital, LMU Munich
  Rational Design to Develop Novel AAV Variants for Improved Gene Delivery to Retinal Microglia

- Jianghui Wang, UMass Chan Medical School
  A novel dual affinity selection (DAS) capsid screening platform for engineering AAV capsids with enhanced photoreceptor transduction via intravitreal delivery

- Muhammed Burak Demircan, PhD, DKFZ
  Targeted DART-AAVs as In Vivo Gene Delivery Platform for the Selective Transduction of TME Cell Subsets

- Amanda Miles, Dyno Therapeutics
  Selective Improvement in Retinal Bipolar Cell Targeting with Intravitreal Injection of a Novel AAV Capsid in Mouse and NHP

- Sourav Choudhury, PhD, Sanofi
  Generative AI Discovers GMU037, a Dual-Fitness Capsid with Simultaneous Superior NHP Ophthalmic Transduction and High Production Yield

Scientific Symposium

Global Convergence: CMC Harmonization for Advanced Therapies (Organized by the Chemistry, Manufacturing, Controls (CMC) Committee)

Location: New Orleans 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

- Malou Gemeniano, Iovance Biotheraputics
  Case studies developing the same product in different regions

- Sara Mills, Artiva Biotherapeutics
  Case studies developing the same product in different regions

Oral Abstract Session

Clinical Trial Spotlight Symposium

Location: New Orleans Theater C
1:30 PM - 3:15 PM

- Valeria Calbi, MD, San Raffaele Telethon Institute for Gene Therapy (SR-TIGET)
  Treatment effect of atidarsagene autotemcel (arsa-cel) in age-matched treated vs. untreated sibling pairs with early-onset metachromatic leukodystrophy (MLD)

- Samik Basu, Cabaletta Bio
  RESET-Myositis™: Clinical Trial Evaluating Rese-cel (Resecabtagene Autoleucel), A Fully Human, Autologous 4-1BB Anti-CD19 CAR T Cell Therapy in Idiopathic Inflammatory Myopathies: Correlative Findings

- Giulia Consiglieri, San Raffaele Telethon Institute for Gene Therapy
  Extensive detoxification and favorable effects on systemic clinical outcomes after Hematopoietic Stem Cell Gene Therapy for Mucopolysaccharidosis Type I-Hurler (OTL-203)

- Stephen Kaminsky, Weill Cornell Medical College
  Phase I Clinical Trial of Safety and Efficacy of the dAd5GNE Anti-Cocaine Vaccine

- Evanthia Galanis, MD, Mayo Clinic
  Phase I/II Trial of Adipose Tissue Derived Mesenchymal Stem Cell Delivery of a Measles Virus Strain Engineered to Express the Sodium Iodine Symporter (MV-NIS) in Ovarian Cancer Patients

- Doug Kerr, Dyne Therapeutics
  Safety and Efficacy of DYNE-101 in Adults with DM1 in the Phase 1/2 ACHIEVE Trial

- Aimee Donald, MBChB, PhD, University of Manchester
  Hemophagocytic Lymphohistiocytosis (HLH)/hyperinflammatory syndrome following high dose AAV9 therapy

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

- Abigail Benkert, Duke University Medical Center
  AAV-Mediated Gene Replacement Preserves Exercise Tolerance and Prevents Cardiac Remodeling in a Murine Knockout Model of SLC25A4 Deficiency

- Isabella Hetherington, University of South Florida
  misiRNA: A Dual-Action mRNA Therapeutic to Treat Atherosclerosis

- Xiao Wang, University of Pennsylvania
  Corrective Editing to Treat Pseudoxanthoma Elasticum

- Ranjan Das, Oregon Health and Science University
  Delivery Route and Species-Specific Transduction Mechanisms Enable Robust Proximal

Tubule Transduction of AAV-KP1 and AAV9 Irrespective of Neutralizing Antibodies in Non-Human Primates

- Sarah Smith-Moore, PhD, Purespring Therapeutics
  Podocyte Gene Therapy Enables Kidney Complement Modulation for IgA Nephropathy (IgAN) Treatment

- Eric Zheng, PhD, Prime Medicine
  Developing Hotspot Prime Editors to enable therapeutic correction of multiple CFTR mutations in Cystic Fibrosis

- Douglas Brown, Entos Pharmaceuticals
  Optimized Non-Viral Fusogenix Proteolipid Vehicles for Precision Gene Therapy in the Lungs

Oral Abstract Session

Modulation of Humoral Immune Responses in AAV Gene Transfer

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

- Timothy Cripe, MD, PhD, Nationwide Children's Hospital
  Development of an Adeno-Associated Virus Expressing a Secreted T Cell Engager for Long-Term B Cell Ablation with High Transgene Expression to Minimize Vector Dose

- Nicholas Giovannone, PhD, Regeneron
  Successful AAV Vector Re-administration via Two Distinct B Cell Immunomodulation Strategies in Non-Human Primates

- Elena Campbell, University of Guelph
  Innovative Strategies to Enhance AAV Gene Transfer in the Muscle in the Presence of Pre-existing Immunity

- Jonathan Rosenberg, Weill Cornell Medical College
  Early Time Post-vector Activation of Complement by AAV Vectors in Nonhuman Primates

- Jessica Boehler, Solid Biosciences
  Potential for AAV-SLB101-mediated gene transfer treatment in the context of natural seropositivity and after an AAVrh74 treatment

- Yunxia (Erica) Xu, Bao Pharma
  KJ103: An IgG-Degrading Enzyme That Reduces Neutralizing Antibodies Against AAV2 and AAV8 to 10% with a Single Dose, with Repeat Dosing Capability Allowing Further Reductions and Potential for AAV Re-DosingKJ103: An IgG-Degrading Enzyme That Reduces Neutralizing Antibodies Against AAV2 and AAV8 to 10% with a Single Dose, with Repeat Dosing Capability Allowing Further Reductions and Potential for AAV Re-Dosing

- Michael Kuipa, Emory University
  AAV-vectored PD-L1 Co-Expression as a Strategy to Enhance AAV-Delivered bNAb Efficacy

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
  Designing wet lab experiments to leverage ML

- Michelle Lee, PhD, Medra
  Physical AI, Agents, LLMS - Path Towards Autonomous Science

Oral Abstract Session

Lipid Nanoparticles II

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

- Gopi Nath Vemuri, Poseida Therapeutics
  Novel Cyclohexane Based Ionizable Lipids (CHILs) for Non-viral Liver Delivery of Complex Nucleic Acid Genetic Medicine Payloads

- Menggui Huang, Script Biosciences
  A CNS-Tropic Lipid Nanoparticle Platform Inspired by Natural Brain Chemistry Menggui Huang1, Rebecca Haley1*, Saigopalakrishna Yerneni2*, Ursula Andreo1, Sapanna Chantarawong1, Ryshone Duncan1, Ryan Gaudlip1, Lozen Robinson1, Kathryn Whitehead2, and Ravi Iyer1 1Script Biosciences, Inc., 2929 Arch Street, Philadelphia PA 19104 2Department of Chemical Engineering, Carnegie Mellon University, Pittsburgh, PA 15213 *Joint first authors*

- Prakash Bhandari, Entos Pharmaceuticals US Inc
  Ocular Gene Therapy Using the Proteolipid Vehicle Nucleic Acid Delivery Platform

- Francesca Ferraresso, The University of British Columbia
  Applying RNA-lipid nanoparticles to modulate protein expression in swine

- Bin Wu, Cytodigm, Inc.
  Leveraging Novel PEG-free Lipid Nanoparticles for Tissue and Cell Targeting While Reducing Immunogenicity and Toxicity

- Carly Starke, Fred Hutch Cancer Center
  Retargeting Lipid Nanoparticles for Optimized mRNA Delivery in Non-Human Primates

- Ruhina Maeshima, PhD, UCL Institute of Child Health
  Anionic double-layered nanocomplexes with receptor-targeting motifs penetrate Cystic Fibrosis mucus better than cationic LNPs and retains effective transfection efficiency in vivo.

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

- Kira Gillett, Foundation for the National Institutes of Health (FNIH)
  Opening Remarks

- Carmen Sivakumaren, IQVIA
  Update on BGTC Disease Portfolio Progress

- Philip Brooks, PhD, NIH / NCATS
  Update on BGTC Disease Portfolio Progress

- Timothy Miller, MD, PPD, Part of Thermo Fisher Scientific
  Update on BGTC Disease Portfolio Progress

- James Noll, IQVIA
  BGTC Regulatory Playbook Digitization

- Sharon King, Aldevron
  Patient Voice Spotlight Panel

- Sarah Cortell Vandersypen, United MSD Foundation
  Patient Voice Spotlight Panel

- Jill Chertow, Propionic Acidemia Foundation
  Patient Voice Spotlight Panel

- Dominique Pichard, NIH - National Center for Advancing Translational Science (NCATS)
  Patient Voice Spotlight Panel

- Courtney Coates, Hope in Focus
  Patient Voice Spotlight Panel

- Fred Bunz, Johns Hopkins University School of Medicine
  Stable adeno-associated virus vectors for human gene therapy

- Leah Byrne, PhD, University of Pittsburgh
  Quantification of AAV dose-response with single cell resolution

Networking

Career Fair

Location: Level 200 Foyer
3:15 PM - 4:15 PM

Oral Abstract Session

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

Location: New Orleans Theater A
3:45 PM - 5:30 PM

- Juan Antinao Díaz, MSc, PhD, UCL
  AAV9-Mediated Gene Targeting of a Natural Antisense Transcript as a Novel Treatment for Dravet Syndrome

- Daniel Dubreuil, Sanofi
  Development of a Secretable Frataxin for Enhanced Efficacy in Treating Friedreich's Ataxia

- Jorge Santiago-Ortiz, Apertura Gene Therapy
  FDA-Aligned Strategy for AAV-Based TSC1 Gene Therapy: Advancing Preclinical Development Without Non-Human Primates

- Meghan Eller, BS, UT Southwestern Medical Center
  Benefit and Tolerability of AAV Gene Replacement in ECHS1D Mice

- Eric Morrow, Brown University
  Proof-of-Concept AAV Gene Therapy for Christianson Syndrome (CS) in CRISPR-targeted Rat Model

- Monique Otero, UMass Chan Medial School
  Development of a Self-Regulating AAV Gene Therapy for the Safe and Effective Treatment of Rett Syndrome

- Zhenhua Wu, Exegenesis Bio Inc.
  EXG202: A Next Generation AAV-based Gene Therapy for Neovascular AMD Delivered by Intravitreal Injection with Superior Retinal Transduction in NHPs and Remarkable Inhibitory Effect on Retinal Detachment in a Severe Neovascular Mouse Model

Scientific Symposium

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

Location: New Orleans 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

Oral Abstract Session

AAV Vector Manufacturing: Plasmids & Cell Line Development

Location: New Orleans Theater C
3:45 PM - 5:30 PM

- Richard Gilmore, OXB
  Enhancing Upstream Processes for High-Yield, High-Quality AAV Vector Production Using a Novel In-House Cell Line

- Yu-Hsin Chang, Chitose Laboratory Corp.
  Establishment of a Novel Human Cell Line, HAT, for High-Yield and High-Quality AAV Manufacturing

- Bhargavi Kondragunta, PhD, Catalent
  Improved Adeno-Associated Vectors for High-Yielding AAV Manufacturing

- Sonja Lochmüller, University of Applied Science Biberach
  Unveiling small non-coding RNA dynamics during rAAV production

- Ben Hudjetz, Cytiva
  Enhancing Gene Therapy Access: Standardizing AAV Production With Cell Line Development

- Celine Winckler, 4basebio
  Optimising AAV Vectors Quality and Characterisation Using Synthetic Linear DNA

- Sandhya Pande, Associate Director, Shape Tx
  Mechanistic Insights Enhance Multi-Serotype Production in the TruStable™ AAV Producer Cell Line

Oral Abstract Session

Challenges in Immunological Responses to Therapeutic Interventions

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

- Di Cao, Indiana University
  Potential for Potent Intrahepatic $CD8^+$ T Cell Responses Despite Lack of Peripheral Responses Following Systemic Muscle-directed Gene Delivery with AAV

- Catherine O'Riordan, PhD, Sanofi
  Intra-Ocular Delivery of the Bacterial Protease IdeS in Non-Human Primates: A Potential

Strategy to Circumvent Pre-existing Immunity to Enable Successful AAV Gene Transfer to the Eye

- Hannah Rinehardt, MD, Children's Hospital of Pitsburgh
  Immune Modulation Sustains Alpha Cell Reprogramming and Mitigates Immune Responses to AAV in a Diabetic Non-Human Primate Model

- Andrea Annoni, PhD, SR-TIGET
  Co-Stimulatory Blockade Regimen Prevents anti-Transgene and anti-Vector Immune Responses for an Effective and Re-Dosable in vivo Gene Therapy for Hemophilia A.

- Julie Crudele, PhD, University of Washington
  Stimulation with Self Peptides Led to Interleukin-17 Secretion by Peripheral Blood Mononuclear Cells from an AAV-Treated Patient with Duchenne Muscular Dystrophy and Unexplained Fatality

- Wei Zhan, University of Massachusetts Medical School
  The Endosomal Recognition of AAV Genome is Critical for the Development of AAV Capsid-Specific Adaptive Immunity

- Di Cao, Indiana University
  Efficient Trafficking of AAV Encoded Protein from Hepatocytes to Hepatic Antigen Presenting Cells

Oral Abstract Session

CMC for AAV Vectors

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

- Catherine Dial, Kriya Therapeutics
  Identifying AAV9 Degradation Pathways Caused by Stresses Encountered During the Product Lifecycle

- Pierre Axel Vinot, SparingVision
  The Need for an Unbiased Assay to Detect and Quantify Replication Competent AAV in Clinical Vector Products

- Nipun Goel, Senior Scientist, Genzyme, a Sanofi Company
  pCO_2 Modulated Control of Recombinant AAV Production and Capsid Quality via Regulation of Pathways Linked to Rep and Cap Protein Expression

- Robert Damitz, Kriya Therapeutics
  Stability of AAV2 Vector During Dose Preparation and Suprachoroidal Injection Using the Everads Injector

- Matt Edwards, Affinia Therapeutics
  Development of a Flexible High Yielding, High Performing Process for Manufacturing of

AFTX-201, a Novel Investigational AAV Gene Therapy for Treatment of BAG3 Dilated Cardiomyopathy

- Michaela Duffy, OXB
  Diving Deeper: Using a SYBR Gold Capsid Ejection Assay as an Orthogonal Method of Measuring Potency and VP1 Deamidation in AAV Drug Product Samples

- Kiley Coates, Kriya Therapeutics
  Simplifying AAV Supply Chains: Demonstrating Equivalence of Long-Term Stability at -20 °C and -80 °C

Oral Abstract Session

Oligonucleotide Therapeutics II

Location: Room 291-292
3:45 PM - 5:30 PM

- Norio Motohashi, National Center of Neurology and Psychiatry
  Investigating the role of inflammatory cells in improving PMO delivery and exon-skipping efficiency in Duchenne muscular dystrophy

- Seyda Acar-Broekmans, UniQure Inc.
  Alpha Synuclein Lowering by miRNA-Based AAV Gene Therapy for Synucleinopathies: Proof of Concept and Biodistribution Studies in Rodent Disease Models and Non-Human Primates

- Gerard Platenburg, PhD, ProQR Therapeutics B.V.
  ADAR-Mediated RNA Editing of SLC10A1 (NTCP) as a Therapeutic Approach to Reduce Liver Bile Acid Re-Uptake in Cholestatic Diseases

- Daniel Tardiff, CAMP4 Therapeutics
  Targeting Regulatory RNAs with Antisense Oligonucleotides for the Potential Treatment of Urea Cycle Disorders

- Yuri Maricich, MD, Camp4 Therapeutics Co
  A First-in-Human Double-Blind, Placebo-Controlled Single and Multiple Ascending Dose Study (SAD, MAD) in Healthy Volunteers to Evaluate the Safety and Tolerability of an Investigational Antisense Oligonulceotide Therapy (CMP-CPS-001) for the Treatment of Urea Cycle Diseases (UCDs): Interim Safety Readout

- Liubin Yang, Yale University
  Antisense oligonucleotides targeting toxic CGG-repeat expansion in primary ovarian insufficiency due to Fragile X premutation

- Daniel Tardiff, CAMP4 Therapeutics
  Targeting Regulatory RNAs with Antisense Oligonucleotides for the Potential Treatment of SYNGAP1-Related Disorders

Oral Abstract Session

Cell Therapy Product Engineering II

Location: Room 293-296
3:45 PM - 5:30 PM

- Sarah Nikiforow, MD, PhD, Dana-Farber Cancer Institute
  Feasibility of Manufacturing CARv3-TEAM-E T Cells for Intraventricular Injection in Recurrent Glioblastoma

- Chuan Wang, PhD, MD, Legend Biotech
  dnTGFβRII-Armored DLL3-Targeted CAR-T Cells Maintain TGFβ Resistance with Early Signals of T-Cell Exhaustion Modulation After Expansion in SCLC

- Rachel Kyeyune, Fred Hutchinson Cancer Center
  A Novel Hybrid Lentiviral Envelope Pseudotype for the Efficient Transduction of Hematopoietic Stem and Progenitor Cells with Minimal Manipulation

- Madelyn VanBlunk, University of North Carolina, Chapel Hill
  Biomaterials Manufacture and Deliver CAR T Cells In Vivo for Potent and Affordable Glioblastoma Treatment

- Steven Howe, Resolution Therapeutics
  Development of a manufacturing process for a Regenerative Macrophage Therapy to treat end-stage liver disease

- Jason Skowronski, Kamau Therapeutics
  Continued Clinical Scale Process Development to Enable Safe and Effective Homology Directed Repair Gene Edited Hematopoietic Stem and Progenitor Cells for Sickle Cell Disease

- Mariana Argenziano, Ncardia
  Scalable Production of Hematopoietic Stem Cells and Microglia from iPSCs Using Stirred Tank Bioreactors for Consistent Cell Therapy Manufacturing

Scientific Symposium

Career Development & DEI Awardee Presentations 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

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

- Ashley Hume, Emerging Therapy Solutions
  Introduction

- Kelly Maynard, Little Hercules Foundation
  Centering the Patient Voice in CGT Coverage Decisions

- Luke Prettol, AT&T
  How Employers Consider CGT Coverage in Private Plans

Oral Abstract Session

Across Platforms for Hematopoietic Disorders

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

- MICHAELA SEMERARO, Hôpital Necker Enfants Malades
  Long-term Inflammatory manifestations post lentiviral hematopoietic stem/progenitor cell gene Therapy for Wiskott Aldrich syndrome

- Eva Segura Gensler, BS, UCLA
  Lentiviral Vectors for Hematopoietic Stem Cell Gene Therapy for Alpha Thalassemia

- Alex Cho, NIH - National Institute of Allergy and Infectious Diseases
  Lentivector Gene Therapy Mediates Reduction of Induced Inflammation in a Mouse Model of Deficiency of Interleukin-1 Receptor Antagonist

- Robert Torrance, University College London
  Functional Restoration of Immune Defects in STAT1 Gain-of-Function Immunodeficiency Following Gene Editing

- Ralph Valentine Crisostomo, University of California, Los Angeles
  Site-Specific Gene Integration Strategies for IL7Rα-SCID Hematopoietic Stem Cell Gene Therapy

- Ngoc Tung Tran, PhD, Indiana University School of Medicine
  Restore in vivo functions of mouse Fancc$^{-/-}$ hematopoietic stem cells using the CRISPR/Cas9 system.

- Vrishti Sinha, University of California Los Angeles
  Split Intein Lentiviral Vector Hematopoietic Stem Cell Gene Therapy for the Treatment of DOCK8 Deficiency

Oral Abstract Session

Gene Therapy Trials - In-Vivo Gene Therapy Modification

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

- Mark Sulkowski, MD, Division of Infectious Diseases Johns Hopkins Bayview Medical Center, John Hopkins University & Medicine
  Initial safety data from ELIMINATE-B, the first clinical trial of a gene editing treatment for chronic hepatitis B

- David Cooper, AviadoBio
  Direct image-guided convective perfusion of the bilateral thalami offers a consistent approach to CNS dosing: first-in-human experience with gene therapy for frontotemporal dementia.

- Olivier Danos, PhD, REGENXBIO Inc.
  RGX-202, an Investigational Gene Therapy for the Treatment of Duchenne Muscular Dystrophy: Interim Clinical Data

- Aravindhan Veerapandiyan, Arkansas Children's Hospital
  Long-Term Functional Outcomes and Safety Following Delandistrogene Moxeparvovec Treatment in DMD: EMBARK 2-Year Results

- Xinting Liu, Dr., PLA General Hospital
  Safety and Efficacy of AAV9-Mediated Gene Therapy GC301 in ERT-Stabilized Infantile-Onset Pompe Disease: An Open-Label, Single-Arm Study with Over 12 Months of Follow-Up

- Yongzhong Wang, PhD, Accuredit Therapeutics US
  ART001: Development and Interim Clinical Outcomes of a CRISPR-Based In Vivo Gene-Editing Therapy for Hereditary ATTR

- Shannon Boye, PhD, Division of Cellular and Molecular Therapy, Department of Pediatrics, University of Florida, Gainesville, FL
  Safey and Efficacy of ATSN-201 Dose Escalation in Patients with X-linked Retinoschisis (XLRS)

Reception

Closing Night Reception (Separate Purchase Required)

Location: Mardi Gras World
7:00 PM - 10:00 PM

Saturday, May 17

Business Meeting
Society Business Meeting
Location: Room 383-385
7:00 AM - 8:00 AM

Oral Abstract Session
AAV Preclinical and Proof-of-Concept Studies
Location: New Orleans Theater A
8:00 AM - 9:45 AM

- Chris Towne, PhD, Gordian Biotechnology
  In Vivo Mosaic Screens Uncover Novel Gene Therapies for Age-Related Diseases

- Sonia Albini, GENETHON
  Optidys: a Dual-AAV Gene Therapy Strategy for Duchenne Muscular Dystrophy

- Wenjun Zhang, Indiana University School of Medicine
  Hepatocyte Tropism of a Bioengineered AAV3B Capsid Variant in a Machine-perfused Normal and Steatotic Human Liver

- Nicolas Wein, PhD, Center for Gene Therapy - Nationwide Children's Hospital
  Interfering with CUG toxic repeats using AAV.U7snRNA rescue myotonia and splicing defects in myotonic dystrophy type 1

- Aleksandra Maciejczuk, University of Virginia
  Insulin Replacement Gene Therapy Using a Novel Glucose- and Drug-Inducible Adeno-Associated Virus

- Timothy Kieffer, PhD, Fractyl Health, Inc.
  Endoscopic Ultrasound-Guided Delivery of Human Glucagon-like Peptide-1 Pancreatic Gene Therapy: Safety and Feasibility in a Porcine Model

- Jordan Stokes, University of Florida
  Gene Therapy of Computationally Designed IL-10 in DSS-induced Colitis Mouse Models

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

- Allison Bradbury, PhD, Nationwide Children's Hospital
  Four year follow up of AAV-mediated gene replacement therapy in a large animal model of Krabbe disease.

- Yvette LOPEZ, UMass Chan Medical School
  Evaluating delivery methods for treatment of neurodegenerative disease in large animal models.

- Swathi Ayloo, Sanofi
  AAV gene therapy for GBA-PD and Gaucher Disease

- Mohammad Samie, Sangamo Therapeutics
  AAV-mediated Delivery of an Engineered Zinc Finger Lead to Selective and Potent Repression of Nav1.7 in Human Sensory Neurons and Nonhuman Primates DRG Nociceptors Following Intrathecal Injection

- Francoise Piguet, PhD, TIDU GENOV, ICM
  Development and validation of an intravenous AAV gene therapy for mucopolysaccharidosis type IIIB in mouse and dog model of the pathology

- Nanda Regmi, UTSW
  Vagus Nerve Delivery of AAV9/GAN Is Required After Intrathecal Administration for a Full Rescue of Autonomic Nervous Dysfunction in Giant Axonal Neuropathy

- Steven Gill, FRCS, MS, Neurochase Ltd
  Sub-motor Cortex Convection-enhanced Delivery of AAV5-GDNF – a Novel Treatment Strategy for Amyotrophic Lateral Sclerosis

Oral Abstract Session

Upstream Manufacturing for AAV Vectors 1

Location: New Orleans Theater C
8:00 AM - 9:45 AM

- John Joseph, Massachusetts Institute of Technology
  Achieving Continuous Production of Recombinant Adeno-Associated Virus with Baculovirus Expression Vector System

- Weiheng Su, OXGENE
  Optimizing the TESSA® Platform for Enhanced rAAV Production

- Pouria Motevalian, Thermo Fisher Scientific
  Next-Gen Recombinant Adeno Associated Virus Processes: Boosting Productivity & Quality via Process Intensification & Optimized Triple Transfection for All Common Serotypes

- Hongyun Tai, PhD, Branca Bunús Ltd
  Revolutionizing Viral Vector Production: Biodegradable Hyperbranched Poly (β-amino ester)-Based Transfection Technology Boosts Yields and Efficiency

- Jun Li, Ultragenyx Pharmaceutical Inc.
  Late-stage Development and Upstream Process Characterization of UX701 AAV Gene Therapy for Wilson Disease

- Von Wiltman, Apertura Gene Therapy
  Machine Learning-Enhanced Design of Experiments (DoE) for Optimizing AAV Plasmid Ratios in Gene Therapy Manufacturing

- Jiantao Zhang, AAVnerGene
  Reduce ITR related Impurities in AAVone Single-plasmid System Using Secondary Structure DNAs

Oral Abstract Session

On and Off-Target Method Development

Location: Room 278-282
8:00 AM - 9:45 AM

- Danilo Pellin, Boston Childrens Hospital
  Scalable assessment of genome editing off-targets associated with genetic variants with ABSOLVE-seq

- Varun Katta, St. Jude Children's Research Hospital
  CHANGE-seq-BE Enables Simultaneously Sensitive and Unbiased In vitro Profiling of Base Editor Genome-Wide Activity

- Yichao Li, St. Jude Children's Research Hospital
  Population-scale Cellular GUIDE-seq-2 and Biochemical CHANGE-seq-R Profiles Reveal Human Genetic Variation Frequently Affects Cas9 Off-target Activity

- Azusa Matsubara, St. Jude Children's Research Hospital
  Pooled GUIDE-seq-2 enables simultaneous assessment of cellular activity and specificity for thousands of Cas9 targets

- Ayal Hendel, PhD, Bar Ilan University
  Single-Cell Profiling of Genome-Editing Alterations and Functional Outcomes in CRISPR-Engineered Cells

- Sumanprava Giri, Tessera Therapeutics
  Development of a Comprehensive Framework for Assessing the Genomic Safety Profile of RNA Gene Writer Targeting Alpha-1 Antitrypsin Deficiency

Oral Abstract Session

Pharmacology/Toxicology Studies and Analytics/Assay Development Session II

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

- Margarita Romanenko, PhD, University of Minnesota
  Preclinical Models for Oncolytic Adenoviruses: Evaluating Replication and Blood Cell Interactions

- Basel Assaf, DVM, PhD, DACVP, DABT, FIATP, Sanofi
  Safety and Biodistribution Assessment of Novel AAV Capsid Developed for the Treatment of Myotonic Dystrophy Type 1 (DM1)

- Benjamin Clarke, PhD, US Pharmacopeia
  Critical Parameters for AAV Vector Genome Titer Determination: a Multi-Laboratory Collaborative Study

- Toufan Parman, Sr. Director of Nonclinical Safety Evaluations, Sangamo Therapeutics, Inc.
  Preclinical Development of an AAV-delivered Zinc Finger Transcriptional Repressor Targeting the Prion Gene as a Novel Epigenetic Gene Therapy for Prion Disease

- Mateusz Imiolek, Gene Therapy Chromatography Consumable R&D, Waters Corporation
  Hydrophilic Interaction Chromatography for Direct Online Disruption of Lipid Nanoparticles, Intact mRNA Analysis, and Measures of Encapsulation Efficiency

- Jing Jin, Life Edit Therapeutics
  Intrastriatal AAV5.SGN.LEGB (LETI-101) administration selectively targeting mutant allele of the HTT gene resulted in broad CNS distribution and transgene expression in critical NHP brain regions associated with Huntington's disease pathology

- Xiulian Sun
  Evaluation of Quality Control Methods for Circular RNA Purity and Integrity: SEC-HPLC, CE, and RP-HPLC

Oral Abstract Session

Targeted Gene and Cell Therapy for Cancer

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

- Mansi Narula, Baylor College of Medicine
  Engineered Fas88 Receptor Provides Inducible Cytokine Signaling to Enhance Functional Persistence of CAR-Vδ2 T Cells in Xenograft Models of Leukemia and Solid Tumor

- Wen Tseng, PhD, Strand Therapeutics
  STX-003: A mRNA Cancer Immunotherapy Utilizing Cancer-Selective Programmable Genetic Circuits for Systemic Tumor Control

- Freja Ekman, Stanford University
  Allele-Specific CRISPR/AAV6 Gene Correction of Dominant JAK2-V617F Mutation in Polycythemia Vera

- Gilles Divita, Aanastra Inc.
  mRNA-Mediated Rescue Loss of p53 Tumor Suppressor Function as a Potent New Strategy for Cancer Therapy across Pan-p53 Alterations.

- Justin Thomas, Fred Hutchinson Cancer Center
  CD90 Ablation on Hematopoietic Stem Cells (HSCs) Enables Targeted Leukemia Stem Cell Immunotherapy

- Timothy Cripe, MD, PhD, Nationwide Children's Hospital
  Single Dose AAV-Mediated Immuno-Gene Therapy With a Dual Targeted T-Cell Engager is Superior to Mono Targeted Engagers for Multiple Myeloma

- Mirko Luoni, PhD, San Raffaele Scientific Institute
  Covalent Antibody Conjugation to AAV9 for Targeted Gene Therapy of Metastatic Cancer

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 Autoimmune Diseases

- 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

- Tejashree Redij, Catalent Pharma Solutions
  Comparative Potency Analysis of iPSC-derived and PBMC-derived NK cells for Immunotherapeutic Application

- Tal Raz, NanoMosaic
  Novel Nanoneedle Technology for Integrating Critical Quality Attributes: Enabling Accurate and Cost-Effective Manufacturing

- Steven Henry, Eurofins BioPharma Product Testing
  Mastering hcDNA: A Harmonized Approach for AAV Gene Therapies

- Agnieszka Lass-Napiorkowska, PhD, Eng, MilliporeSigma
  Breaking Boundaries in AAV Production: Exploiting High Salt Concentrations for increased AAV yields and infectivity.

- Grace Eppolito, ImmuneBridge
  Scalable Hematopoietic Stem Cell Expansion in Stirred-Tank Bioreactors for Advancing Cell Immunotherapies

- Ethan Deitcher, Bespoke Biotherapeutics
  Negative Impact of Leukapheresis Collection on Blood-derived B-cell Gene Expression — An Artificial Intelligence and Machine Learning Enhanced Study

- Lijun Wang, CoJourney
  Quantification of residual host-cell DNA fragments of the E1A gene from HEK293 via qPCR assay

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: New Orleans Theater A
10:15 AM - 12:00 PM

- Ezra Loeb, Duke University
  Engineering non-mammalian chimeric AAVs for evasion of pre-existing antibodies and vector-induced immunity

- Jane Hsi, University of Florida
  It's a Zoo Out There: Analyzing the Cross-Reactivity of Human Antibodies Towards Animal AAVs to Expand the AAV Vector Toolkit for Gene Therapy Applications

- Nuria Roxana Botticello-Romero, Broad Institute of MIT and Harvard
  AAV-CM1, a Human Receptor-Targeted Capsid with an Enhanced Tropism for the CNS, Skeletal Muscle, and Heart

- R. Jason Lamontagne, Ph.D., GEMMA Biotherapeutics
  Position-Specific Impact of RGD Motif in AAV Capsid Variants on Muscle Tissue Targeting and Integrin Binding Affinity

- Simon Pacouret, Broad Institute of MIT and Harvard
  Antibody-evading AAV capsids compatible with CNS and muscle-targeting modifications

- Jessica Boehler, Solid Biosciences
  Insight into the mechanism of action of AAV-SLB101, a novel muscle-tropic capsid for neuromuscular and cardiac indications

- Megan Cramer, Dyno Therapeutics
  A Novel Neuromuscular AAV Capsid Combines Efficient Systemic Muscle and CNS Delivery with Liver Detargeting in NHP

Oral Abstract Session

Molecular and Cellular Methods - Applications

Location: New Orleans Theater B
10:15 AM - 12:00 PM

- Francesco Gazzo, Department of Electronics, Information and Bioengineering, Politecnico Di Milano, Milan, Italy
  Vector Insertional Mutagenesis Drives Accelerated Hematopoietic Stem Cell Aging and Acquisition of Somatic Mutations In Vivo

- Mariska Ter Haak, IN8bio
  Decoding the Molecular Signature of Expanded Gamma Delta T Cell Products; TCR and Immune Gene Expession from Allogeneic derived Products

- Aoife Doto, University of Pennsylvania
  Methods for characterizing AAV integration and genomic rearrangements in vivo.

- Cristina Colleoni, San Raffaele Telethon Institute for Gene Therapy (SR-Tiget), IRCCS San Raffaele Scientific Institute, Milan, Italy
  Unraveling the Dynamics of Senescent Cell and Immune System Interplay in HSPC Gene Therapy

- Audrey Alderman, Seattle Children's Research Institute
  Multiplex ddPCR Platform for Precision Monitoring of Multi-Antigen CAR T Cells in Pediatric CNS Cancer Therapy

- Mark Sands, PhD, Washington University School of Medicine
  Non-Rian Integrations in AAV-Associated HCCs From a Murine Model of Krabbe Disease

- Jiahe Tian, Cornell University
  A PCR-Free Approach Combining CRISPR-Cas9 and Long-Read Sequencing Reveals Unpredictable AAV Integration in the Host Genome

Oral Abstract Session

Upstream Manufacturing for AAV Vectors 2

Location: New Orleans Theater C
10:15 AM - 12:00 PM

- Thomas Robert, PhD, Univercells Technologies
  Overcoming Barriers To Accessibility: Scalable, Cost-Efficient Biomanufacturing For Gene Therapies.

- Ruchita Selot, PhD, GROW LAB NNF
  "Improved AAV Vector Production Yields From An Optimised Manufacturing Process in Fixed-Bed Bioreactors"

- Laura Juckem, PhD, Mirus Bio
  Simplifying Large-Scale Upstream AAV Production Via a Transfection Complex Stabilizing Reagent

- Lewis Hall, University College London
  From Bench to Bioprocess: Bridging AAV Capsid Engineering and Scalable Manufacturing for Clinical and Commercial Translation

- Igor Alves Mancilla, Revvity Gene Delivery
  Enhancing AAV Production Efficiency: A Dual Approach to Design of Experiments Optimization and Economic Feasibility Analysis

- Kim Schrag, MS, MilliporeSigma
  Transfection Reagent Selection and Post-Transfection Feeding of HEK293 AAV Production Platforms Based on GOI and Serotype

- Emilie Gateau, Exothera
  The use of novel PAT and soft sensor as process monitoring tool during AAV manufacturing

Oral Abstract Session

B-cell and Solid Organ Therapies

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

- Annaiz Grimm, Seattle Children's Research Institute
  CD19 CAR EngTreg Effectively Modulate Activated Human B Cells and Antibody-Secreting Plasma Cells

- Zachary Eidman, Vanderbilt University
  Synthetic Signaling to Repurpose Inflammation-Associated with Arthropathy Toward Disease Resolution

- Hanlan Liu, Be Biopharma
  Ex Vivo Gene Editing of Autologous B Cells Produce Sustained Levels of Tissue Nonspecific Alkaline Phosphatase In Vivo for the Potential Treatment of Hypophosphatasia

- Nathan Wang, MIT
  Compact Transcription Factor Cassettes Generate Functional, Engraftable Motor Neurons by Direct Conversion

- Chan-Hua Chang, University of Southern California
  Broadly-neutralizing Anti-HIV Antibody Production in Rhesus Macaques Transplanted with B Cells Engineered at the IgH Locus.

- Sunil Kumar Mallanna, PhD, Satellite Bio
  Expandable, Functional Hepatocytes Derived from Primary Cells Enable Liver Therapeutics

- Anne Vonada, Oregon Health and Science University
  Curative Allogeneic Hepatocyte Transplantation without Immune Suppression

Oral Abstract Session

Gene Therapy for Muscle Diseases

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

- Michelle Lorentzos, MBBS PhD, Sydney Children's Hospitals Network
  Recurrent Rhabdomyolysis and Fatality Following Administration of AAV Microdystrophin Gene Therapy

- Keith Connolly, Modalis Therapeutics Inc.
  Treatment of Myotonic Dystrophy Type 1 (DM1) by GNDM-mediated Suppression of the DMPK Gene

- Chunyan He, Suzhou GenAssist Therapeutics Co., Ltd
  A clinical DMD cytosine base editing drug

- Arnaud Valent, GENETHON
  GNT0004, Genethon's AAV-based gene therapy for Duchenne muscular dystrophy: long-term follow-up of ambulatory boys enrolled in the dose-escalation phase of GNT-016-MDYF.

- Deborah Zygmunt, Abigail Wexner Research Institute at Nationwide Children's Hospital
  Determining the Effectiveness of a FKRP/FST Single AAV Dual Gene Vector for the Treatment of Limb Girdle Muscular Dystrophy type R9 (LGMDR9) at Different Ages

- Matthew Burke, BS, University of Missouri
  Cross-Species Comparison of AAV8, 9, rh74, and Six Myotropic AAV Variants in Murine and Canine Models Following Systemic Delivery

Oral Abstract Session

Engineered Immune Effector Cells for Solid Tumors

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

- Miri Horovitz-Fried, Tel Aviv University
  T cell engineering using V(D)J recombination allows tumor growth inhibition in mice

- Sophie Hanina, Memorial Sloan Kettering Cancer Center
  Sensitive HLA-independent T cell Receptors Overcome Tumor Antigen Heterogeneity in Solid Tumors

- Gabriel Barragan Bravo, Baylor College of Medicine
  IL-18 Metabolically Reprograms CAR-expressing Natural Killer T Cells and Enhancing Antitumor Activity Against Neuroblastoma

- MARIA CHIARA MAFFIA, PhD, Ospedale San Raffaele
  Adoptive cell therapy with genetically engineered T cells for Epithelial Ovarian Cancer

- Kajal Chaudhry, Children's National Medical Center: Children's National Hospital
  Multimodal T cell immunotherapy CAR-TA (B7-H3 CAR and PRAME Tumor Antigen cells) for pediatric brain and solid tumor patients

- Hui Xu, Reforgene Medicine
  Allogeneic GPC3 CAR-iNKT cells show robust anti-tumor activity in hepatocellular carcinoma mouse models

- Melinda Au, Adicet Bio
  ADI-270, an Armored Allogeneic Anti-CD70 CAR γδ T Cell Therapy, Demonstrates Robust CAR-Directed and -Independent Anti-Tumor Activity Against Hematologic and Solid Tumor Models Compared to Conventional CAR αβ T Cells

Oral Abstract Session
Lipid Nanoparticles III
Location: Room 293-296
10:15 AM - 12:00 PM

- Leonardo Cheng, Johns Hopkins University School of Medicine
  Multi-Objective Machine Learning-Guided Optimization of mRNA Lipid Nanoparticles for Cell-Selective Transfection

- Yan Tang, Brigham and Women's Hospital
  Gene Replacement Therapy for Pulmonary Lymphangioleiomyomatosis

- Gun Su Han, Tessera Therapeutics
  Ionizable Lipid Development and LNP Formulation Optimization Enable the Use of RNA Gene Writers for In Vivo Treatment of Genetic Diseases in Liver

- Rosa Choi, Children's Hospital of Philadelphia
  Targeted Gene Editing in the Fetal Brain via In Utero Intracerebroventricular LNP mRNA Delivery

- Jia Nong, University of Pennsylvania
  Multi-stage-mixing to create a core-then-shell structure improves DNA-loaded lipid nanoparticles' transfection by orders of magnitude

- Anna Potenski, Novartis
  Using in vitro screening and in vivo biodistribution studies to determine endogenous tissue preference of LNP formulations to predict therapeutic potential

Oral Abstract Session
Vector Product Engineering, Development and Manufacturing (excluding AAV)
Location: Room 383-385
10:15 AM - 12:00 PM

- Marta Arrizabalaga Cascallana, University College London
  Optimization of Cell Expansion Phase Parameters for a Cost-Effective Lentiviral Vector Perfusion Process

- Bojiao Yin, ElevateBio
  Advancing Lentiviral Vector Manufacturing: a Platform for Commercial Cell Therapy Success

- Glenda Dickson, ViroCell Biologics, 12-18 Theobalds Road, London, WC1X 8SL, UK
  Serum Free Suspension HEK293 Cells Stably Expressing Gag/Pol /Rev /VSVG for Efficient Production of Lentiviral Vectors

- Hanna Lesch, Exothera Jumet, Beligium
  A to Z of replication-competent free adenovirus production from gene to manufacturing process intensification

- Chris Brown, ReciBioPharm
  Establishing a Versatile Platform for Intensified Lentiviral Vector Manufacturing

- Alicia Powers, PhD, St. Jude Children's Research Hospital
  Efficient and Scalable Chromatographic Purification of Clinically Relevant Lentiviral Vectors

- Maliha Zahid, Mayo Clinic
  Novel Lung Cell Penetrating Peptides Target Airway Epithelial Type 2 Cells

Oral Abstract Session

Oncolytic Virus Therapies

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

- Richard Vile, PhD, Mayo Clinic
  CRAd657-CD40L as a Potent Oncolytic and Immune-stimulatory Treatment for Hepatocellular Carcinoma

- Steve Thorne, Kalivir
  Mechanisms of synergy between TGF-beta inhibitor and IL12 expression from the systemically deliverable clinical oncolytic immunotherapy VET3-TGI

- Motomu Nakatake, PhD, Division of Genomic Medicine, Tottori University, Japan
  Fusogenic onclytic vaccinia virus armed with immunostimulatory cytokines improves tumor immune microenvironments systemically and induces complete response in mice bearing bilateral tumors and orthotopic tumors.

- Khandoker Usran Ferdous, University of Arkansas for Medical Sciences
  Stroma Modulating Recombinant Oncolytic Vesiculovirus Shrinks Pancreatic Tumor and Increases Immune Response

- Richard Vile, PhD, Mayo Clinic
  Redressing the Balance Between Immunodominant Antiviral and Immunosubdominant Antitumor T Cell Responses to Enhance the Efficacy of Oncolytic Viroimmunotherapy

- David Bartlett, MD, Allegheny Health Network
  Oncolytic Vaccinia Virus Expressing Non-Secreted Decoy-Resistant IL-18 Mutein Elicits Potent Antitumor Effects with Enhanced Safety

- Natalie Elliott, Mayo Clinic
  Enhanced Tumor Microenvironment (TME) Immunomodulation Through Repeat Dosing with a Synthetic Vesiculovirus Library

Oral Abstract Session

Innovation in Alternative Cell Therapy Sources

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

- Clara Soulard, CHU Sainte Justine
  CAR-Engineered HSCs for Treating B-cell Acute Lymphoblastic Leukemia

- Ruby Freeman, Emory University
  Revitalizing aged CAR T cells with anti-inflammatory cytokine IL-37

- Cedric Louvet, Dana-Farber Cancer Institute
  In Vivo Self-Renewing HSC-Based CAR-NK Cell Factory

- Trever Greene, Fate Therapeutics, Inc.
  Phase 1 Translational Assessment of an Off-The-Shelf CAR NK Cell Armed with Alloimmune Defense Technology for Conditioning-free Therapy

- Dar Heinze, MD, Century Therapeutics
  Generation of iPSC-derived $CD4^+$ and $CD8^+$ CD19 CAR αβ T cells with in vivo tumor control and cell expansion comparable to healthy donor T cells.

- Christos Georgiadis, PhD, UCL, GOS Institute of Child Health, London, United Kingdom
  Combinational "Off-the-Shelf' CAR T Cells: A Unified Front Against AML Heterogeneity

- Mame Diop, Columbia Initiative in Cell Engineering and Therapy, Columbia University Irving Medical Center; Cancer Center Amsterdam, Amsterdam UMC, Amsterdam, NL
  Development of a Feeder-Free Maturation Method for $TCR^{+/-}$ CAR iT Cells

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
  Chromosomal Instability from Pharmacological DNA Repair Modulation 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