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

- Ezequiel Zylberberg, Akron Biotech
  Unpacking FDA's Guidance on Considerations for the Use of Human- and Animal-Derived Materials + Safety Testing of Human Allogenic Cells - Considerations for Sponsors

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

- George Buchman, Catalent Pharma Solutions
  Unpacking FDA's Guidance on the Platform Technology Designation Program – CDMO Perspective on Considerations for Sponsors

- John Tomtishen, PhD, Cellares
  Unpacking FDA's Guidance on the Advanced Manufacturing Technologies Designation Program – Considerations for Sponsors

- Mandy Xie, BMS
  Unpacking FDA's Guidance on Potency Assurance for CGT Products

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

Workshop (separate purchase required)

The Business of Advocates Advancing CGTs

Location: Room 291-292
8:00 AM - 12:00 PM

- Kim Nye, BA, TESS Research Foundation
  Introducing the business of advocacy

- Nasha Fitter, FOXG1 Research Foundation
  A model for an ultra-rare non-profit

- Allyson Berent, DVM, DACVIM, Foundation for Angelman Syndrome Therapeutics
  A model for a mature non-profit

- Michael Hund, MBA, EB Research Partnership
  A model for a larger organization doing venture philanthropy

- Terry PIrovolakis, Elpida Therapeutics SPC
  A model for an ultra-rare venture philanthropy and how to reinvest to advance the field

- Claire Booth, MBBS PhD, UCL Great Ormond Street Institute of Child Health
  A model for a non-profit institution to license and deliver gene therapy products

- Heidi Wallis, Association for Creatine Deficiencies
  Diversifying an organization's revenue streams

- Heidi Bjornson-Pennell, The Chan Zuckerburg Initiative
  CZI/Rare is One grant funding and support for capacity building

- 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 - CMC Activities and Assessments

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

- Daniel galbraith, Solvias NL
  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

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

Sponsored Symposium

GeneFab: GeneFab’s Synthetic Biology Technologies for Smarter, Safer Therapies

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

- Duncan Liew, Genefab
  GeneFab: GeneFab’s Synthetic Biology Technologies for Smarter, Safer Therapies

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.

- Lingyan Wang, Cargo Therapeutics
  CRG-023, A Tri-specific CAR T-cell Product Candidate, Demonstrates Strong and Durable Anti-Tumor Activity In Vivo Across Lymphoma Models

Scientific Symposium

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

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

- Claire Clelland, UCSF
  Framework for assessing genomic off-targets of CRISPR gene editing therapies in a clinical context

- Petros Giannikopoulos
  Case example: Ionizing radiation for childhood medulloblastoma

- Krishanu Saha, PhD, University of Wisconsin-Madison
  Patient perspectives on gene editing and off-target editing

- Kiran Musunuru, MD, PhD, MPH, ML, MRA, University of Pennsylvania
  Framework for Considering Which Off-target Edits are of Concern

Sponsored Symposium

Parexel: When Safety Becomes a Value Proposition For CAR-T Trials

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

- Nancy Lunney, Parexel
  Parexel: When Safety Becomes a Value Proposition For CAR-T Trials

Sponsored Symposium

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

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

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

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

Poster Abstract Session

Tuesday Poster Reception

Location: Poster Hall Hall I2
6:00 PM - 7:30 PM

- Zeinab Asgarian, University College London
  Novel Liver-Targeted Gene Therapy Platform vs. Brain-Targeted Approaches for

Inherited Metabolic Liver Diseases

- Fanny Collaud, Genethon
  Long-term correction of metabolic impairment in a mouse model of Glycogen Storage Disease type III after rAAV gene transfer reveals disease-specific host-vector interactions.

- LUCIA DE STEFANO, PhD, Telethon Institute of Genetics and Medicine
  mRNA therapy for Glycogen Storage Disease type 1b.

- Matthew Remy, Dimension Inx
  3D-printed Highly Porous PLG-based Scaffolds as a Platform for Therapeutic Cell Delivery

- Brandon Burch, Kriya Therapeutics
  Characterization of KRIYA-839, an AAV-based Gene Therapy Providing Insulin and Glucokinase, in Streptozotocin-induced Diabetic Mice

- Randy Chandler, PhD, National Institutes of Health
  Towards the Development of Adeno-Associated Viral Gene Therapy for the Treatment of Patients with Propionic Acidemia Resulting from a Deficiency of PCCB

- Linda Chio, Gordian Biotechnology
  In Vivo Pooled Mosaic Screening for the Discovery of Drug Targets in Adipose Tissue

- Josefa Sullivan, MeiraGTx
  An Ultra-Low Dose of a Localized CNS Gene Therapy for Severe Pediatric Obesity

- Minh-Ha Do, Rampart Bioscience
  A Novel Non-Viral DNA Gene Therapy Ameliorates Disease in a Mouse Model of Hypophosphatasia

- Chun-Yu Chen, PhD, Seattle Children's Research Institute
  Homology-Independent Targeted Integration-Based Strategy Restores Canine FVIII Activity from FVIII Intron 22 Inversion Mutation

- Yuezhe Li, Metrum Research Group
  A Novel Agent-based Computational Model for Liver-targeting, AAV-based Gene Therapies Could Predict Response Durability in Hemophilia B Patients Treated with Etranacogene Dezaparvovec

- Jonathan Lindgren, Children's Hospital of Philadelphia
  Identification of bioengineered AAV capsids with enhanced targeting of liver sinusoidal endothelial cells (LSECs)

- Kentaro Yamada, PhD, Indiana University School of Medicine
  Superior Efficacy of AAV Gene Therapy for Hemophilia A Using Engineered Factor VIII Variants and Transient B Cell Depletion for Effective Vector Readministration

- Paula Sureda Horrach, Tigem
  Homology-Independent Targeted Integration in a Humanised Mouse Model of Haemophilia A

- Cesare Canepari, SR-TIGET
  Enhancing the Potency of In Vivo Lentiviral Gene Therapy to Hepatocytes

- Devo Goldman, PhD, Yecuris
  Liver-Humanized Mice Repopulated with Hepatocytes From a Hemophilia B Donor Exhibit Delayed Blood Clotting and Have a Physiologic Response to Gene Therapy

Correction of Human FIX

- Jakob Shoti, University of Florida
  GENE THERAPY OF HEMOPHILIA A: IDENTIFICATION OF THE MOST EFFICIENT AAV SEROTYPE VECTOR FOR TRANSDUCTION OF HUMAN LIVER SINUSOIDAL ENDOTHELIAL CELLS

- Arun Srivastava, Professor, University of Florida
  GENE THERAPY OF HEMOPHILIA B: IDENTIFICATION OF THE MOST EFFICIENT AAV SEROTYPE VECTOR FOR TRANSDUCTION OF HUMAN HEPATIC CELLS

- Francoise Piguet, PhD, TIDU GENOV, ICM
  Novel intravenous AAV gene therapy for symptomatic mucopolysaccharidosis type IIIA in canine model of the pathology

- Amanda Gross, MS, PhD, Auburn University
  Dual site administration of AAV gene therapy for treatment of feline GM1 gangliosidosis

- Shuangqing Yu, Genecradle Therapeutics Inc
  Dosage Effects on Circulating GAA as a Pharmacodynamic Biomarker for systemically delivered rAAV9-coGAA Gene Therapy in Pompe Disease

- David Leib, PhD, Latus Bio, Inc
  A Non-GLP Dose Range Finding Study in Cynomolgus Macaques Evaluating the Biodistribution, Expression, and Safety of LTS-101, a Novel Preclinical Gene Therapy Candidate for the Treatment of CLN2 Batten Disease

- Sheridan Rose, PhD, Spur Therapeutics Ltd
  SPR301: Dose Dependent Reduction of Inflammation and Substrates in a Surrogate Model for GBA1-linked Neuroinflammation

- Wenjing Liang, Tenaya Therapeutics
  A Humanized RBM20 Mouse Model Exhibits Dilated Cardiomyopathy Phenotypes and Enables Development of In Vivo Prime Editing for Treating Human RBM20 Cardiomyopathy Patients

- Simone Chiola, Stanford - IMA
  Development of a Novel AAV Gene Therapy for the Treatment of Dilated Cardiomyopathy

- Brandon Chan, Alexion Pharmaceuticals Inc.
  Preclinical Efficacy and Safety of ALXN2350, a rAAV Gene Therapy for BAG3-Associated Dilated Cardiomyopathy

- Rahul Mallick, University of Eastern Finland
  Ad-VEGF-B186R127S gene therapy induces angiogenesis in mouse heart by recruiting endothelial progenitor cells

- Krish Dewan, Duke University Medical Center
  Novel Combination Gene Therapy to Prevent Rejection after Solid Organ Transplantation

- Zhong-Dong Shi, PhD, Frontera Therapeutics
  Preclinical Efficacy and Safety Evaluation of FT-017: a Novel AAV-Based Gene Therapy for MYBPC3-associated Hypertrophic Cardiomyopathy

- Nihay Laham-Karam, University of Eastern Finland
  An Effective Viral Vector for Treatment of Myocardial Infarction.

- Iain Black, University of Glasgow
  Investigating the Mechanisms of Acute Preservation of Cardiac Function Post-

Myocardial Infarction Following Angiotensin-(1-9) Gene Therapy

- Shuang Li, Indiana University School of Medicine
  Gene Therapy for Cardiac Conduction Disorders and Muscular Dystrophy in POPDC2 Null Mice by Systemic MyoAAV4A.POPDC2 Delivery

- Hong Duan, Frontera Therapeutics
  FT-018, a Novel AAV Gene Therapy for ARVC, Improves Heart Function and Rescues Survival of Cardiac Pkp2-Deficient Mice

- Sofia De La Serna Buzon, PhD, BS, Boston Children's Hospital
  Gene Therapy Targeting Atrial Fibrillation Using Mouse Models: A Novel Approach to Cardiac Rhythm Regulation

- Gabriella Kabboul, Georgia Institute of Technology & Emory University
  Engineered Epicardial Hydrogels for Cardiac-Specific Gene Therapy

- Dong Yang, PhD, HuidaGene Therapeutics Co., Ltd., Shanghai, China
  HG204 CRISPR-Cas13 RNA-Editing Therapy for MECP2 Duplication Syndrome: Preclinical Success to First-in-Human Study

- Ellie Chilcott, University College London
  RNA Editing Rescues SUDEP and Seizure Phenotype in a Mouse Model of Dravet Syndrome

- Julien Ratelade, EG427
  Long-term expression of transgene in targeted cortical neurons following inoculation in the mouse striatum of a non-replicative HSV-1 vector.

- Carl Ernst, PhD, McGill University

Successful RNA Therapy for Schinzel-Giedion Syndrome Demonstrated in Patient-Derived Stem Cell Models and a Clinically Relevant Mouse Disease Model

- Steven Gill, FRCS, MS, Neurochase Ltd
  Convection-enhanced delivery of gene therapies to the pons – a novel method of achieving transgene expression throughout the Central Nervous System

- Kalpita Karan, Weill Cornell Medical College
  AAV mediated APOE4 Silence and Replace Therapy to Treat Homozygous APOE4-associated Alzheimer's Disease

- Cassandra Retzlaff, Voyager Therapeutics, Inc.
  One-Time Delivery of a Vectorized Anti-Amyloid Antibody for Increased and Sustained CNS Expression and Target Engagement

- LAITH HADAYA, Regeneron Pharmaceuticals
  Retargeting of AAV to CNS Using Anti-AAV X TFR1 Bispecific Antibodies

- Nicholas Slimmon, UT Southwestern Medical Center
  Investigating Focused Ultrasound Enhancement of CSF-Delivered AAV to the Brain

- Roderick Slavcev, University of Waterloo
  Targeted Bacteriophage Gene Delivery to Astrocytes Using Intelligent Phagemid-Assembled Gene Expression (iPhAGE) Technology for Neuronal Regeneration

- Henry Lee, Senior Scientist, Boston Children's Hospital
  Gene replacement therapy reverses behavioral and molecular phenotypes in succinic semialdehyde dehydrogenase deficiency (SSADHD) mice

- Maria Porto Cruz, NeuroOne
  Translational Devices for Simultaneous Neural Recordings and Drug Delivery in the Brain: From Small to Large Animals

- Sirika Pillay, Encoded Therapeutics
  ETX201: An AAV9-based Vectorized miRNA Therapeutic Candidate for Angelman Syndrome

- Yuanye Yan, University of Toronto
  AAV-delivered GRIN1 gene improves synaptic NMDA receptor function in mouse models of GRIN disorder

- Karin Kojima, Jichi Medical University
  Recovery in a Mouse model of Tyrosine Hydroxylase Deficiency by Triple Transduction of Striatal Cells with AAV Vectors Expressing Dopamine-Synthesizing Enzymes

- Bryan Gore, Allen Institute
  GABAergic-Selective AAV Gene Therapy for SLC6A1-Related Disorder

- Paul Carlson, Sanofi
  A Platform for Engineered Cross-Correction of AAV-Expressed Transgenes

- Xin Chen, UT Southwestern Medical Center
  Gene Replacement Therapy for TECPR2-Related Hereditary Sensory and Autonomic Neuropathy

- Xin Chen, UT Southwestern Medical Center
  Gene Replacement Therapy for MPS IIIC with scAAV9/HGSNAT Vector

- Rika Zen, Shiga University of Medical Science
  Novel Peptide Therapy Targeting Pro-Inflammatory Microglia for Neonatal Hypoxic-Ischemic Encephalopathy in Mice

- Sophia Liu, UMass Chan Medical School
  Extracellular Vesicle Trafficking of Gangliosides Boosts rAAV-Based Correction for GM3 Synthase Deficiency

- Jessica Hogestyn, Sanofi
  Harnessing AAV-RNAi Technology for the Treatment of Neurodegenerative Diseases

- Paul Ranum, Latus Bio
  A Computational Model Predicts Treatable Huntington's Disease Patient Population and Minimum Therapeutic Efficacy Requirements for Gene Therapies Targeting Somatic Instability

- Riccardo Privolizzi, MS, PhD, University College London
  Long term safety and efficacy of BGT-DTDS in the DAT-knockout mouse model of Dopamine Transporter Deficiency Syndrome

- Hechen Bao, Voyager Therapeutics, Inc.
  Cross-Species BBB-Penetrant IV-Delivered AAV Gene Therapy Provides Broad and Robust CNS Tau Lowering in Tauopathy Mouse Models and Non-Human Primate

- Rebecca Sebastian, AbbVie
  Selective targeting of trans-synaptic tau using chimeric antibodies receptors tethered to the synaptic membrane

- Zin-Juan Klaft, Tufts University - Dept of Neurosci
  Tuberin Replacement Therapy in Cultured, Patient-Derived Neural Progenitor

Cells and a Cerebral in vivo Mouse Model of Tuberous Sclerosis Complex Type 2

- Noriyuki Watanabe, University of California, San Diego
  AAV-SynCav1 Administration to the Hippocampus of Cuprizone Mouse Model of Multiple Sclerosis Preserves Hippocampal Myelination

- Geoffrey Keeler, PhD, University of Florida
  Adeno-Associated Virus Based Therapy to Treat Disease and Reduce Demyelination in an Animal Model of Multiple Sclerosis

- Mele Avilla, Labcorp Drug Development
  A 4-Week Study of Rodent Multiple Sclerosis (MS) Model

- Wei Wang, OcuCell, Inc.
  Endo-Tek™: A Tissue-Engineered Alternative to DMEK/DSAEK for Corneal Endothelial Dysfunction

- Cecilia Marinova, Medasol
  SELECTION OF QUALIFIED TREATMENT CENTRUM FOR CELL THERAPY

- Adnan Dibas, PhD, Nanoscope Technologies
  Screening of Matrix Gla promoters to drive efficient expression of AAV-delivered Engineered Mechanosensitive Channel in trabecular meshwork for Enhancing Outflow Facility and lowering IOP in TGFβ2 Animal Model of Glaucoma

- Dave Copland, University of Bristol
  Local delivery of inflammation-inducible vectorized anti-TNF biologics to treat non-infectious uveitis

- Dongjoon Kim, Louisiana State University

AAV-based Gene Replacement Therapy Improves Visual Behavior in a Murine Model of Usher Syndrome Type 1C (USH1C)

- David Corey, PhD, Harvard Medical School
  Mini-PCDH15 Gene Therapy for Blindness in Usher Syndrome Type 1F

- Claire Williams, B.S., Salk Institute for Biological Studies
  AAV Compatible Delivery of Full-Length PCDH15 for Treatment of USH1F via RNA End Joining

- Qiang Zheng, Chengdu Origen Biotechnology
  A novel dual-target gene therapy strategy design with balanced gene expressions

- Katie Binley, Ikarovec Limited
  A Bicistronic AAV-Based Gene Therapy for Wet Age-Related Macular Degeneration Combining VEGF and CTGF Inhibition to Target Angiogenesis and Fibrosis

- Emily Warner, Ikarovec Limited
  IKC159V: A bicistronic AAV gene therapy for Geographic Atrophy delivering dual protein expression and multifaceted protection against retinal damage

- Rachel Eclov, PhD, Kriya Therapeutics
  KRIYA-825 (AAV2.CR2-CR1) for Geographic Atrophy: Characterization of Biological Activity and Biodistribution

- Pavitra Ramachandran, Genzyme, a Sanofi Company - Cambridge, MA
  Generation of a non-human primate model of Geographic atrophy, Dry Age-related macular degeneration

- Qiang Zheng, Chengdu Origen Biotechnology
  A novel AAV vector delivering sCD59 for the treatment of dry AMD in mice model

- Menglin Li, University of California, Los Angeles
  Identification of cell-type-specific cis-regulatiory modules (CRMs) in the primate retina

- Chengda Ren, West China Hospital
  AAV-delivered engineered suppressor tRNA rescues visual function in mice with an inherited retinal disease

- Cerys Easton, VisionGift
  Molecular Stability of Human Donor Retinal Gene Expression and Protein Abundance Post-Mortem: Implications for Cell and Gene Therapy Applications

- Ahmed Salman, University of Oxford
  A novel spontaneously immortalised Non-Human Primate Müller glia cell line reveals a population of Müller glia cells with stem cell-like qualities, a potential source for an in situ retinal regeneration

- Lucie Guo, MD PhD, Stanford
  Injury-responsive gene therapy for neuroprotection of retinal ganglion cells

- Xing Wang, HuidaGene Therapeutics Co., Ltd., Shanghai, China
  Intein-based Dual AAV Gene Therapy Restores ABCA4 Function in Stargardt Disease

- Vinit Mahajan, Stanford University Laboratory for Cell & Gene Medicine
  Human Retinal Cell Delivery of a Bioengineered, Synthetic Protein Sensitive to Multiple Wavelengths of Light in Patients with Stargardt Disease

- Qingyun Zheng, Ph.D., University of Oxford
  AAV-Mirtron Treatment Preserves Retinal Structure and Function in the Human P23H Knock-In Autosomal Dominant Retinitis Pigmentosa Mouse Model

- Xinyan Li, Frontera Therapeutics
  12-Month Safety and Efficacy Evaluation of Subretinal Gene Therapy (FT-002) for the Treatment of RPGR Gene Mutation Associated X-linked Retinitis Pigmentosa

- Kai Wu, UgeneX
  Optogenetic Therapy for the Treatment of Photoreceptor Degeneration Diseases

- Chang Li, PhD, University of Washington
  Therapeutic Evaluation of a Gamma-Globin Gene Addition Vector System With a Large LCR in Sickle Cell Disease Mice by In Vivo HSC Transduction

- Nikoleta Psatha, PhD, Aristotle University of Thessaloniki
  Novel Lentiviral Vectors Enable Controlled Expansion of Transduced Hematopoietic Stem Cells: Beta-Hemoglobinopathies as disease models

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

- Martin Rodriguez, PhD, Wake Forest Institute for Regenerative Medicine
  Comparing Efficacy and Safety of Prenatal versus Neonatal AAV5 Gene Therapy for Hemophilia A in a Sheep Model

- Mara Gilardi, PhD, Champions Oncology
  Development of a Humanized Immune system mouse supporting primary AML PDX engraftment for drug efficacy studies

- Sarah Wachtman, Wake Forest School of Medicine
  Human Liver Tissue Equivalent (hLTE) Models for Studying the Safety and Efficacy of AAV-based Gene Therapy for Patients with Hemophilia A

- Wenkai Jia, 4D Molecular Therapeutics
  An In Vitro Air-Liquid Interface Lung Model of Alpha1 Antitrypsin Deficiency for Assessing an Adeno Associated Virus Based Genetic Medicine

- A-Rum Yoon, Hanyang University
  A Novel Strategy for Treating Gefitinib-Resistant Cancer: Correction of Multiple pathogenic single-nucleotide polymorphism (SNP) Using Adenine Base Editor-expressing adenovirus

- Stephen Sherman, PhD, Editas Medicine, Inc.
  Design and Development of Improved LNP Targeting Ligands for In Vivo Hematopoietic Stem Cell Editing

- Yusang Jung, Yonsei University College of Medicine
  Comprehensive Analysis of ABL1 Variant Resistance to Multiple Kinase Inhibitors via Prime Editing

- Mathieu von Joest, Institut Imagine
  Fixing the Balance: A Dual-Targeted Base Editing approach to reduce α-globin and reactivate Fetal Hemoglobin to treat β-hemoglobinopathies

- Andrew Vanden Heuvel, Children's Hospital of Philadelphia
  eePASSIGE Knock-In of F8 Exons 23-26 cDNA for Intron 22 Inversion Mutations

- Jenny Gao, UMass Chan
  Base Editing Rescue of SOD1 associated ALS

- Marcel Chuecos, BS, Baylor College of Medicine
  Base Editing for Precise and Specific Elimination of Lipoprotein(a)

- Caner Günaydin, Weill Cornell Medical College
  High Efficiency Prime Editing of Alzheimer's Disease High Risk APOE4 Allele by Brain-directed AAV Vectors

- Meirui An, Harvard University
  In vivo base editing extends lifespan of a humanized mouse model of prion disease

- Claire Fong, Massachusetts General Hospital
  Targeted Gene Correction Using AAV-Delivered Base Editing Improves Smooth Muscle Cell Function in the Eye of an ACTA2^{R179H} MSMDS Mouse Model: A Promising Surrogate Endpoint For Future Clinical Trials

- Zhiquan Liu, Stanford University
  Precise Correction of the Pde6b-L659P Mutation Causing Retinal Degeneration via Base and Prime Editing

- Hongjie Wang, PhD, University of Washington
  Improving the Safety and Efficacy of In Vivo Genome Editing in HSCs by Using Strong HSC-Specific Promoters and a Pomalidomide-Induction-System to Regulate the Level and Duration of Editor Expression after HDAd Gene Transfer.

- Nemekhbayar Baatartsogt, Jichi Medical University
  Base Editing for Generating a Gain-of-Function Variant in Hemophilia B

- Hui Xu, Reforgene Medicine
  A Novel RNA Splicing Therapy for USH2A-Associated Retinitis Pigmentosa

- David Fiflis, BS, Duke University Pratt School of Engineering
  An RNA Trans-Splicing Strategy to Rewrite mRNAs Implicated in Cardiomyopathy

- Phillip Price, PhD, Spark Therapeutics
  Improving mRNA Trans-Splicing Strategies to Repair Extensive Genetic Mutations

- Samuel DeMario, Amber Bio
  Intronization of Splice Editors Expands Trans-Splicing Toolkit

- Mark Miller, Kriya Therapeutics
  Machine Learning Guided Design of Drug-Inducible Splicing Elements for Gene Therapy

- Harshitha Venugopal Lanvanya, MS, Duke
  CRISPR assisted RNA trans-splicing for controlled and transient activation of T cells

- David Fiflis, BS, Duke University Pratt School of Engineering
  Massively Parallel RNA Barcode Screens for CRISPR-Assisted Trans-Splicing

- Simon Wentworth, University of Massachusetts Medical School
  Exploration of Trans-Splicing Methodologies in AAV Therapeutic Development Targeted at Treating Neurofibromatosis Type-I and Associated Tumors

- Gabriel Butterfield, Duke University
  Discovery and Characterization of Novel Epigenome Editing Domains

- Courtney Klappenbach, Epicrispr Biotechnologies
  Directed Evolution and Characterization of Cas Effectors in Mammalian Cells for

Expanded Epigenome Editing Space

- Ashish Chougule, Revvity Inc
  Beyond the Dot Blot: A Novel Method for dsRNA Detection for Gene Therapies

- Vidya Chivukula, Medicines and Healthcare Products Regulatory Agency
  Safety Assessment of on-/off-target aberrations in the generation of Universal CAR-T cells

- Putianqi Wang, Calico Life Sciences
  Leveraging gene delivery approaches for in vivo target validation and safety assessment

- Xiulian Sun
  T-Cell Gene Editing via Integration-Free LNP-Mediated Delivery of gRNA and CRISPR Enzyme

- Matthew Nitzahn, Poseida Therapeutics
  High-Fidelity Genome Editing with Cas-CLOVER: A Platform for Advancing Liver-Targeted Therapeutics

- Bryant Chica, PhD, Editas Medicine, Inc.
  Design of Chemically Modified AsCas12a Guide RNAs for Increased Potency of LNP-Delivered Gene Editing Cargos

- Beatriz Aranda-Orgilles, PhD, Cellectis Inc
  TALEN®- Mediated non-viral Transgene Insertion for the Advancement of Cellular and Gene Therapies.

- Colin Juett, HS, University of California Los Angeles

Investigation of Double-Stranded DNA Donors and CRISPR-Cas9 RNP for Universal Correction of Cystic Fibrosis Causing Mutations in Human Airway Cells

- Ting-Yen Chao, Seattle Children's Research Institute
  Achieving Therapeutic Endogenous FVIII Levels in Hemophilia A Mice Using Ultrasound-Mediated Gene Delivery of CRISPR/Cas9 Plasmids

- Yawen Fu, Karolinska Institutet
  Highly Efficient and Easy-to-Operate HDR Gene Knock-in Editing Is Achieved by 'All-in-One' Delivery Vehicles (AiDVs)

- Christopher Brown, Metagenomi
  In Vivo Genome Editing with an Ultra-Compact Type V Nuclease for All-In-One AAV Delivery

- Arjun Padmanabhan, TIGEM
  AAV HITI for therapy of dominant Retinitis Pigmentosa

- Brandon Pekarek, Metagenomi
  A Novel Type II CRISPR System Small Enough to Fit in a Single AAV Enables Targeted Gene Knockdown in the CNS

- Rolf Turk, Integrated DNA Technologies
  Improved Prediction and Activation of Homology-Directed Repair in Translational Ex-vivo Systems

- Javier Gomez Vargas, Integrated DNA Technologies
  CRISPR Library Design Solutions for Efficient Gene Knockout Screening

- Spencer McKinstry, Precision Biosciences

Excision of the C9orf72 Hexanucleotide Repeat Expansion Using a Dual-ARCUS Gene Editing Approach Reduces Neurotoxic RNA Foci and Dipeptides in an In Vivo Model of ALS

- POUIRE YAMEOGO, University of Texas Southwestern Medical Center
  Excision of the Expanded GAA Repeats from Fxn^{null}::YG8s$(GAA)_{>800}$ Increases Frataxin Expression

- Mingming Cao, Rice University
  Single-Cell Genotyping and Transcriptomic Analysis of CRISPR/Cas9 edited hematopoietic stem and progenitor cells for Treating Sickle Cell Disease

- Seunghee Lee, Sanofi
  CasX gRNA Screening and Selection Enables Ex Vivo CD38 Knockout NK Cell Therapy

- Tomas Gonzalez Fernandez, Lehigh University
  Machine Learning-Informed CRISPR Screening for Enhanced MSC Chondrogenesis

- Leiore Ajuria, PhD, Countagen AB
  Rapid and Accurate Genotyping of 100 Gene-Edited Cells Using GeneAbacus

- Erin Cross, Kromatid Inc
  Advances in Cytogenetic Detection of Small Genomic Targets and Transgenes for Cell and Gene Therapy

- Eric Josephs, Stony Brook University
  Personalized Molecular Engineering of CRISPR Systems to Improve Activity and Specificity

- Vijetha Vemulapalli, SeQure DX
  A Robust Approach for CRISPR-Cas Therapeutic Guide Design and Selection Leveraging Variant-Aware Computational and Biochemical Approaches

- Roberto Nitsch, PhD, AstraZeneca
  Dual-Color Optical Genome Mapping for Enhanced GUIDE-seq Analysis in CRISPR-Cas9 Editing

- Laura Blaha, Sanofi
  Genome-Wide In Silico Analysis of Greater than Five Million CRISPR Guide RNAs Reveals Limited Predictive Power of Non-Human Primates for CRISPR Guide RNA Off-Target Activity in Humans

- Sarah Fong, University of California San Francisco
  Leveraging DNA Missing from the Human Genome to Generate CRISPR Targetable Sequences Without Off-Target Effects

- Ashley Herring-Nicholas, UNC Greensboro
  TOP-SECRETS Enables Cas9 Nucleases to Differentiate SNVs Outside of PAMs

- Felix Lansing, Seamless Therapeutics GmbH
  Reprogramming Large Serine Recombinases for Site-Specific Integration into Mammalian Genomes

- Jacqueline Frost, AskBio
  CRISPR Engineered Humanized Albumin Mouse (“hAlb-CR”) Achieves Robust Transgene Expression following Liver-Targeted Gene Insertion

- Jeffrey Miller, PhD, Sangamo BioSciences, Inc.
  A Protein-Guided Modular Integrase (MINT) Platform Enables Compact Therapeutic Payloads and Efficient Targeted Integration in T Cells

- Jonas Kath, Charité - University Medicine Berlin
  PASTA: CRISPR Knock-In Paired with Integrase for Simple, Precise, and Efficient Large Transgene Insertion in T Cells

- Jesse Owens, PhD, University of Hawaii at Manoa - Institute for Biogenesis Research
  Large DNA insertion at genomic safe harbors in human cells using a site-directed transposase

- Andres Valderrama, Poseida Therapeutics
  Programmable Site-Specific Super piggyBac Transposase for Integration of Large DNA Payloads into Endogenous Genomic Sites

- Christopher Brown, Metagenomi
  Site-Specific Integration of Therapeutic Transgenes with a Type V-K CAST System Engineered for Efficient and Targeted Human Genome Editing

- Hanna Lesch, Exothera Jumet, Beligium
  Proof of Concept for Automated Ntensify Manufacturing platform of mRNA: Productivity and Quality Assessment

- Amanda Haupt, Revvity
  Development of a SMARTvector™ Multiplex shRNA Platform for Complex Cell Therapy Engineering with a Single Vector

- Thomas Hansen, Circio AB
  CircVec: a powerful circular RNA expression platform to enhance viral and non-viral gene and cell therapies

- Minju Park, ST Pharm

Next-Gen Circular RNA: A Promising mRNA Alternative with Enhanced Stability and Therapeutic Applications

- Tyler Davis, SCIEX
  Advanced Capillary Electrophoresis Techniques for Monitoring mRNA-based Vaccine and Therapeutic Development: From Plasmid DNA to mRNA Encapsulation

- Alexis Bennett, Johns Hopkins University School of Medicine
  Repurposing CRISPR Knock Out Screens to Identify Cancer-Killing sgRNAs.

- Maryse Proulx, PhD, Charles River Laboratories
  Gene Expression Assays for PD Readout for siRNA Therapeutics: Validation Strategy to Support Clinical Studies

- Rudi Micheletti, HAYA Tx.
  Harnessing Functional Genomics to Advance the Discovery and Development of Oligonucleotide-Based Therapies Targeting the Regulatory Genome

- Jean Huang, Biocytogen
  Humanized INHBE Mice Provide a Robust In Vivo Platform for Evaluating RNAi Therapies.

- Jean Huang, Biocytogen
  Developing Humanized APOC3 Mice As a Robust In Vivo Model for RNAi Therapy Assessment

- Songjun Xiao, LGC Axolabs
  Optimizing Purification Strategies for sgRNA and Therapeutic Oligonucleotides

- Xia Sheng, GenScript USA Inc.
  Validated Impurity Test for Chemical Synthesized sgRNAs using NGS Detection Method

- Kazuki Hashiba, Nitto Denko Corporation
  Optimizing VHH Ligand Density in T Cell-Targeted LNPs: An Inverted U-Shaped Relationship for Effective In Vivo CAR T Cell Generation

- Cafer Ozdemir, PhD, Kernal Biologics
  T cell specific CAR-T mRNA Design for in situ T Cell Engineering

- Ying Tam, Acuitas Therapeutics Inc
  Improved T Cell Delivery and Expression of mRNA Using CD8-Targeted Lipid Nanoparticles with Athebody® Designed Ankyrin Repeat Proteins (DARPins)

- Michael Monte, Tessera Therapeutics
  In Vivo RNA Delivery to T Cells and Hematopoietic Stem Cells in Humanized Mice and Non-Human Primates using Targeting Lipid Nanoparticles

- Melissa Moy, Imanis Life Sciences
  In vivo Targeting of Hematopoietic Stem Cell Receptor cKit by Lentivirus Particles Pseudotyped with chimeric VSV-G proteins

- Esther Chen, Capstan Therapeutics
  Efficient In Vivo Gene Editing of T Cells Utilizing Novel Targeted Lipid Nanoparticles

- Xiaoya (Julia) Lu, Johns Hopkins University
  Compositional Screening of Lipid Nanoparticles for Intracellular Delivery of Gene-Editing Proteins to T cells

- Chantal PICHON, PhD, INSERM
  Lipid nanoparticles- a game changer for primary B cell engineering mediated by mRNA-based Crispr/Cas9.

- Yun Gong, PhD, MBA, BS, Thermo Fisher Scientific
  INVITROGEN™ VIVOFECTAMINE™ LIPID NANOPARTICLES (LNPS) FOR EFFICIENT NUCLEIC ACID DELIVERY INTO IMMUNE CELLS EX VIVO AND IN VIVO

- Grishma Pawar, NanoVation Therapeutics
  Rational Design and Preclinical Applications of Novel Unshielded Lipid Nanoparticles (uLNPs)

- Amit Singh, Mote Therapeutics
  Mobilize: A modular and versatile targeted LNP delivery platform for extra-hepatic RNA therapeutics

- Francesca Ferraresso, The University of British Columbia
  Self-amplifying RNA-lipid nanoparticles for long-term protein expression in liver

- Meng-Ni Fan, BS, Seattle Children's Research Institute
  Lipid Nanoparticle-Based Non-Viral Delivery of FVIII for Hemophilia A Gene Therapy

- Khalid Hajj, PhD, Poseida Therapeutics
  P-FVIII-101 is a Non-viral Genetic Medicine with the Potential for Controlled Modulation of FVIII Transgene Expression to Match Patient Needs

- Carles Bayod, Pompeu Fabra University
  Lipid Nanoparticle Delivery of Minicircle DNA for LAMA2 Gene Replacement in Muscle

- Markus Grompe, MD, Oregon Health & Science University
  Protease enhanced gene therapy of squamous epithelial stem cells in the oral cavity and skin

- Jinzhen Hu, University of Delaware
  Peptide-Assisted Lipid Nanoparticles for DNA Delivery in Pulmonary Gene Therapy

- Joseph Pisano, Factor Bioscience Inc
  A Novel Polyvalent Ionizable Lipid Library for Nebulized Delivery of mRNA to Lung Cells

- Anna Bulysheva, Ph.D., University of South Florida
  Novel Physical Gene Delivery Method Enhances Expression in Skeletal Muscle

- Michael Stehnach, CyteQuest
  Continuous-flow electroporation platform for rapid optimization and seamless scalability for cellular therapy manufacturing

- Aaron Hoover, ElevateBio
  Non-Viral Delivery of DNA Template and Gene Editing Components for Targeted Gene Insertion in Human Primary T-Cells: Electroporation vs. Lipid Nanoparticles

- Maxwell Van Buskirk, BS, MaxCyte
  Optimizing transposon-based gene delivery for cell therapy applications with the MaxCyte® ExPERT™ scalable electroporation platform

- Ranganatha Somasagara, Ph D, Thermo Fisher Scientific
  Cell Engineering Solutions for T Cell-Based Immunotherapy Manufacturing: From Resting to Activated T Cell Editing

- Amin Ahmad, Ghent University
  Transfecting iPSCs Robustly and Safely Using Photothermal Electrospun Nanofibers

- Wenjia Xie, Ghent University
  Photoporation-Enhanced Drug Loading and Genetic Engineering of Mesenchymal Stem Cells for Glioblastoma Treatment

- Deepak Kumar, Scientist, Thermo Fisher Scientific
  Scalable Non-Viral Gene Editing and Feeder-Free Production of CAR-NK Cells

- Nadezhda Korostyleva, University of South Florida
  Enhanced Gene Delivery with High-Frequency Asymmetric Biphasic Pulses: Balancing Efficiency and Reduced Muscle Contractions

- Ebony Gary, PhD, The Wistar Institute
  Electroporation and LNP-mediated delivery of HPAI HA antigens supports robust anti-influenza immunity in vivo

- David Weiner, PhD, The Wistar Institute
  A FIH dose-escalation trial of the safety and pharmacokinetics of anti-SARS-CoV-2 DNA-encoded monoclonal antibodies (DMAb) formulated for delivery by CELLECTRA in healthy adults

- Carlos Javier Alméciga-Díaz, BPharm, Ph.D., Pontificia Universidad Javeriana
  Enhanced Gene Therapy for Tay-Sachs Disease Using the PP6D5 Polymer and CRISPR/nCas9

- Cherry Gupta, MS, PhD, Battelle Memorial Institute
  High-Throughput Screening and Data-Driven Evolution of Polymeric Nanoparticles for Efficient Gene Delivery

- Michael Triebwasser, University of Michigan
  Improving Hematologic Gene Therapy by Mapping the Structure/Activity Relationship of Poly(Beta-amino ester) mRNA Nanoparticles

- Jianlei Wu, Villanova University
  Optimizing Poly (Amine-Co-Ester) Polymeric Nanoparticles for Enhanced Nucleic Acid Delivery to Endothelial Cells

- Huiyi Liang, Columbia University
  Polymeric Nanoparticles Targeting Gut-Associated Lymphoid Tissue for Oral CRISPR/Cas9 and dCas9 Delivery

- Claudia Lee, PhD, Velvet Therapeutics
  In Vivo CAR Delivery as Supercoiled DNA with Lymphatic Tropism Polyasparagine Nanoparticles

- Brian Ginn, Secant Group, LLC
  In Vitro and In Vivo Delivery of Nucleic Acids using Poly(glycerol ester)

- Shashi Murthy, PhD, Nanite, Inc.
  High Level Persistent In Vivo Production of an anti-HIV Antibody via the Delivery of Plasmid DNA by a Polymer Nanoparticle

- Andrea McCue, Battelle Memorial Institute
  Branched Polymers with a Crosslinked Core Offer Nanoparticle Size Retention and a Novel Method of Safe and Effective Non-Viral Gene Delivery

- Rakshitha Miskin, Nanite Inc
  Programmable Polymer Nanoparticles Co-Package and Deliver Dual mRNA and pDNA Cargo to Cells

- Miranda Lyons-Cohen, Seattle Children's Research Institute
  Engineering Fusion Proteins to Enhance Efficacy of Adoptive Cell Therapy for Solid Tumors

- ALBA RODRIGUEZ-GARCIA, FCRB-IDIBAPS
  DNA delivery by lipid nanoparticles induces $CD8^+$ T cell-dependent antitumor responses and synergizes with anti-PDL1 antibodies in mouse models of hepatocellular carcinoma

- Elisabeth Gillespie, Inovio Pharmaceuticals, Inc. - Plymouth Meeting
  Successful Treatment of Recurrent Respiratory Papillomatosis with INO-3107 is Irrespective of Papilloma Microenvironment and Molecular Subtype

- Xiao Han, Earle A. Chiles Research Institute
  Platelet-hitchhiking Delivery of MSA-2 and Mn^{2+}-loaded Nanoparticles for Preventing Post-surgical Tumor Recurrence

- Klaudia Kiel, Mossakowski Medical Research Institute Polish Academy of Sciences
  Ex Vivo Modified Extracellular Vesicles as a Novel Immunotherapeutic Strategy in Glioblastoma

- Robert Johnson, PhD, MD, GenVivo Inc
  Safety and efficacy evaluation of retroviral vectors GEN-1018 and GEN-1013 toward clinical translation for in vivo immuno-gene therapy and combined suicide gene therapy

- Yanruide Li, University of California, Los Angeles
  Generation of allogeneic CAR-NKT cells from hematopoietic stem and progenitor cells using a clinically guided culture method

- Ying Fang, University of California Los Angeles
  Allogeneic CD33-directed CAR-NKT Cells for the Treatment of Bone Marrow-resident Myeloid Malignancies

- Brandon Simone, University of Pennsylvania
  Reinvigoration of Exhausted T Cells Towards Improved Immunotherapy

- Giovanna Giacca, SR-TIGET
  Hemato-Chimeric Mice Hosting Human Liver-Resident Macrophages for Translational Studies Using In Vivo Gene Therapy of Liver Metastases

- Seonhee Kim, Libentech CO.LTD
  Recombinant Newcastle Disease Virus Expressing PTEN as a Novel Therapeutic Approach for Pancreatic Ductal Adenocarcinoma

- Jia Yao, PhD, Emory University
  More is Less: Cytotoxic Activity of Oncolytic Adenovirus Influences Therapeutic Efficacy in Disseminated Lung Cancer

- Katrin Schröer, Witten/Herdecke University
  In vitro and in vivo efficacy of novel “armed” oncolytic viruses derived from adenovirus species B for breast cancer therapy

- Margarita Romanenko, PhD, University of Minnesota
  Adenovirus Type 6 Expressing GM-CSF Exhibits Oncolytic Activity and Induces Antitumor Immunity in an Immunocompetent Syrian Hamster Model of Cholangiocarcinoma

- A-Rum Yoon, Hanyang University
  Oncolytic adenovirus mediated antitumor immune response for the treatment of bladder cancer

- Svetlana Atasheva, PhD, Emory University
  Overcoming Barriers to Effective Cancer Therapy with Oncolytic Adenoviruses

- Praveensingh Hajeri, PhD, University of Minnesota
  A Novel Method For Generating Unprecedented, Unbiased, Ultra-High-Sequence Diversity Libraries Of Plasmids And Recombinant Viruses (With Validation) For High-Throughput Screening, data for machine learning (AI) And Targeted Therapy.

- Jennifer Cillis, City of Hope
  Assessment of Oncolytic Properties of the Chimeric Poxvirus HOV-2 in Cholangiocarcinoma Models

- Shyambabu Chaurasiya, City of Hope
  Using an Oncolytic Virus to Expand the use of GPC3-targeted Experimental Cancer Therapeutics

- Patrick Schmidt, PhD, National Center for Tumor Diseases
  A living CAR-T library combined with optofluidics and single cell sequencing for the rapid selection of functional CAR constructs.

- HARSH GANDHI, Ph.D. Research Scholar, PMRF (Prime Minister's Research Fellow), School of Biotechnology, Jawaharlal Nehru University
  Enhancement of the Multimodal Cancer Therapeutic Efficacy in Non-Small Cell Lung Carcinoma by Hypoxia Reversal using Hemoglobin-Loaded Biomimetic Nanoparticles as Oxygen Delivery System

- Hat Nim Jeon, Gradiant Bioconvergence
  Combining AI-Based Target Discovery with CRISPR/Cas9 Editing: Validating GBC-11004 as a Novel Target to Combat Osimertinib-Resistant NSCLC

- Amadu Tadesse, UCLA
  Lipid Nanoparticle Delivery of CRISPR/Cas9 Reagents for Disruption of Oncogenic Translocations in Alveolar Rhabdomyosarcoma

- James Eshleman, Johns Hopkins University School of Medicine
  Tumoricidal Potential of CRISPR-Cas9 against Pancreatic Cancers

- Nasim Hejazi, Royan Institute
  CRISPR/Cas9 knockout of c-Met to evaluate its effects on reducing drug resistance against gemcitabine in pancreatic cancer

- Minxin Wang, Sepax Technologies
  mRNA Aggregate Analysis using SEC-MALS

- David Spencer, PhD, Phoenix-SENOLYTIX
  Improved ApoptiCIDe™ Safety Switch Provides a Novel, Controllable Therapeutic Platform for Regulated Klotho Anti-Aging Gene Therapy

- Leili Niu, Nova Southeastern University
  Gene Studies About Development of Renal Cell Carcinoma

- Dhruv Sethi, Obsidian Therapeutics
  Drug-responsive Domains Coupled with Cognate Small-molecule Ligands Drive Synthetic Gene-ON or -OFF Circuits to Regulate Expression of Therapeutic Proteins

- Yuya Sugitatsu, Kyoto Prefectural University of Medicine
  Alectinib Enhances Anti-tumor Efficacy of Disialoganglioside 2 Chimeric Antigen Receptor T Cells in ALK-Mutated Neuroblastoma by Suppressing Programmed Death-Ligand 1

- Misty Jenkins AO, Walter and Eliza Hall Institute
  Developing Novel Chimeric Antigen Receptor Therapies for Glioma

- Izadora Furtado, University of São Paulo
  GITRL Coexpression in Anti-GD2 CAR-T Cells Results in Persistent Antitumor Activity and Exhaustion Resistance in a Glioblastoma Model

- Yingjie Zhu, JW Therapeutics
  Enhanced anti-GD2 CAR-T efficacy due to the addition of encoded armored elements of persistence, expansion and shielding modules

- Paul-Joseph Aspuria, Synthekine
  Orthogonal IL-2/IL-2Rβ Armoring is Superior to TGF-β Receptor Blockade in Overcoming TGF-β-Driven Inhibition of GPC3 CAR T Cells in Hepatocellular Carcinoma

- Yuling Zhang, PhD, JW Therapeutics
  JW004: An Armored anti-GPC3 CAR T Therapy for Hepatocellular Carcinoma Enhanced through Multiple Modules

- Raisa Uchurova, Century Therapeutics
  Armoring iPSC-derived allogeneic therapies with a TGF-b neutralizing synthetic receptor that enhances solid tumor elimination in TME-mimicking conditions in vitro.

- Babak Moghimi, Children's Hospital Los Angeles (CHLA)
  Preclinical Development of CTSR-Armored B7H3 CAR-T Cells for Pediatric Solid Tumors

- Yuna Jo, Pusan National University School of Medicine

Nrf2 Deficiency Improves CAR-T Cell Survival and Function in Breast Cancer Models

- Jesus Miguens Blanco, PhD, Stanford University
  Metabolic Reprogramming of HER2 CAR-T Cells Under Hypoxic Conditions: Insights from a multi-omics approach.

- Manaka Shinagawa, Shinshu University Obstetrics and Gynecology
  EGFR-Targeting Ligand-Based CAR-T Cells Demonstrate Favorable Antitumor Activity Against Gynecologic Malignancies.

- Hui Dong, Biopharmaceutical Development Program, FNLCR, NIH, Frederick, Maryland
  Development of GMP-Compliant Processes for Novel Mesothelin Targeting hYP218 CAR Lentiviral Vector and T Cell Production

- Álvaro Muñoz-López, Miltenyi Biotec B.V. & Co. KG
  Drug-Inducible Transcriptional Control of CAR-T Cells for Enhanced Safety and Efficacy

- Shoji Hisada, Hitachi, Ltd.
  Computationally Guided Point Mutations for Screening of Enhanced CAR T cells

- Nancy Lunney, Parexel
  ICANS and CRS Following Lymphodepletion in CAR-T Therapy: A Real-World Data Analysis

- Abdu Mohammed, Trinity Health System
  Epidemiology and Impact of Acute Gastrointestinal Hemorrhage on Outcomes of Chimeric Antigen Receptor T-Cell Therapy in Patients With Hematologic Malignancies: Insights From a Nationwide Database

- Adamsegd Gebremedhen, Marshall University School of Medicine
  A United States Population-Based Study on the Epidemiology and Impact of Hemophagocytic Lymphohistiocytosis on In-Hospital Outcomes in Patients Receiving Chimeric Antigen Receptor T-Cell Therapy

- Nancy Lunney, Parexel
  Does the Occurrence of ICANS After CAR-T Therapy Increase the Risk of Late Neurological Complications: A Real-World Data Analysis

- Jamie Pierson, Parexel
  Social Determinants Hindering CART Access in Civilian and Military Life

- Mollie Jurewicz, TScan Therapeutics
  CD45 as a Universal Target for Adjuvant TCR-T Cell Therapy Following Allogeneic Hematopoietic Cell Transplantation

- Adamsegd Gebremedhen, Marshall University School of Medicine
  Impact of Protein-Energy Malnutrition on Outcomes of Chimeric Antigen Receptor T-Cell Therapy in Patients with Hematologic Malignancies: Insights from a National Database

- Smith Kungwankiattichai, Mahidol University, Faculty of Medicine, Siriraj Hospital, Division of Hematology
  Comprehensive Immune Reconstitution Profiles Following Chimeric Antigen Receptor T-Cell Therapy

- Lindsay Lathrop, UCLA Molecular and Medical Pharmacology
  Stage-specific Expression and Signaling of a CAR in TRAC$^{-/-}$ iPSC-ATOs Rescues Positive Selection and Produces More Naïve and Cytotoxic CAR T Cells

- Wiktoria Parol, University of Cambridge (MRC Toxicology)
  Targeting CCL2 to Improve Efficacy and Reduce Toxicity of CAR T-cell Therapy

- Alan Williams, Fate Therapeutics, Inc.
  Alloimmune Defense Receptor Combined with Genetic Ablation of Adhesion Ligand CD58 is a Comprehensive Approach to Promote Functional Persistence of Allogeneic Cell Therapies without Conditioning Chemotherapy

- Todd Yoder, AskBio
  Single Cell RNA-seq Detection of AAV in Circulating Leukocytes via Antibody-Derived Tags

- Larisa Pereboeva, University of Alabama at Birmingham
  Characterization of Antigen-specific Memory B Cell Repertoire in Human Tissues by Single-Cell Tools (LIBRA-Seq)

- Joshua Baal, PROGEN Biotechnik GmbH
  Pre-Existing Immunity to AAV - A Qualitative Comparison Between Different Neutralization Assay Methods

- JunHyeong Kim, UCSF
  Characterization of the Immune Landscape of the AML Bone Marrow Microenvironment by Cellular Indexing of Transcriptomes and Epitopes (CITE-Seq)

- Ashley Harkins, UMass Chan Medical School
  Understanding and characterizing the immune response to adeno-associated virus (AAV) in the central nervous system (CNS)

- Celia Sourd, University of Oxford
  Elucidating the efficacy and immune response to retinal gene therapy in non-

human primates using spatial transcriptomic and single-cell approaches

- Dongqi Liu, Ph.D., Indiana University School of Medicine
  Transient Treg Expansion Mitigates Cas9-Specific Immune Responses to Enhance Durability of AAV Gene Editing Therapy in Duchenne Muscular Dystrophy

- Tomas Baldwin, PhD, UCL Institute of Child Health
  AAV cross reactivity elicits a serotype specific response and complement activation in type I SMA patients post Zolgensma treatment

- Robert Jimenez, UCLA
  Classical Complement Ablation Leads to Divergent Lymphocyte Activation by Antigen Presenting Cells in Response to AAV

- Susana Najera, FDA
  *AAV2 Delivery of the saCas9 Gene Results in MHC I Presentation of an HLA-A*02:01 Restricted T cell Epitope Potent to Induce T Cell Cytotoxicity.*

- Daniel Stone, PhD, Fred Hutchinson Cancer Center
  All-in-one AAV Vectors Delivering Compact Dbp-CasX, Plm-CasX, Un1Cas12f and AsCas12f Nucleases as Antiviral Gene Editing Therapeutics for the Treatment of Hepatitis B Virus

- Lukasz Swiech, Aera Therapeutics
  In Vivo Generation of CAR-T Cells for the Treatment of B-Cell Mediated Autoimmune Diseases

- Norman Drzeniek, Charité Universitätsmedizin Berlin
  Chemokine-Engineered CAR-T Cells for Autoimmunity: Multiplexed mRNA Technology for Improved Mode of Action and Safety

- Brooke Benson, Sartorius Stedim Biotech GmbH
  Innovative Lipid-Based Delivery Solution: Unlocking New Opportunities in Manufacturing Genetically Modified Immune Cell Therapies

- Masahiro Fukasaka, Nitto Denko Corporation
  Cell Targeted Lipid Nanoparticle (ctLNP)-based CAR-T Therapy: A Novel Strategy for B cell Malignancies and Autoimmune Diseases

- Michael Mingueneau, industry professional, Sail Biomedicines
  In vivo Transient Programming of T cells with SAIL's Targeted Nanoparticles Encapsulating eRNA™-encoded hCD19 CAR Achieves Deep Depletion of B cells in blood and lymphoid tissues in a B-cell Repopulating Humanized Mouse Model.

- Pamela O'Neill, SynGenSys
  Next-generation synthetic signal peptides Cell and Gene Therapies

- Ko-Wei Liu, Dana-Farber Cancer Institute
  Leveraging Immuno-STAT stimulation via TCR and IL-2 signaling to specifically control CAR T cell expansion and persistence in vivo

- Xuqing Zhang, Be Biopharma
  Exploration of Allogeneic Shielding Strategies by Precise CRISPR/Cas9 Genome Engineering of Primary Human B Cells to Enable Off-the-shelf B Cell Medicines for Sustained Delivery of in vivo Biologics

- Utsav Jetley, Intellia Therapeutics
  A Differentiated and Durable Allogeneic T cell Strategy by Leveraging Orthogonal Cas9 Enzymes and an AAV & LNP Delivery Platform

- Srishti Sahu, Innovative Genomics Institute
  Electroporation-free delivery of CRISPR enzymes for efficient multiplex genome

editing of primary human immune cells and repair of patient mutations in the rare disease hemophagocytic lymphohistiocytosis (HLH)

- Nora Winter, University Tuebingen/ Miltenyi Biotec B.V. & Co KG
  Adapter-targeted lentiviral vectors for gene transfer in vitro and in vivo

- Kshitij Sharma, Strand Therapeutics
  Long-acting Circular RNA CAR Drives Potent and Durable Tumor Elimination In-vivo

- Larissa Zanetti, HIAE
  Development and Evaluation of Two BCMA-Targeted CAR-T Cell Products for Multiple Myeloma Treatment: A 2nd vs. 4th Generation Approach with IL-15 Production in Brazil

- Katie Campbell, Saint-Gobain Life Sciences
  Development of a Method for the Gene Editing of T-cells in a Closed Single-Use System

- Patrick Derigs, German Cancer Research Center, Heidelberg, Germany
  Improved Genetic Engineering of Primary Human Hematopoietic Stem and Progenitor Cells Using SMAR DNA Nanovectors

- Seunga Choo, Fred Hutchinson Cancer Center
  Epitope Editing of CD90 Protects Hematopoietic Stem Cells from Immunotherapy and Enables Targeted Enrichment for Gene Therapy

- Rui Tostoes, Immunebridge
  NK cells derived from expanded cord blood HSCs provide an optimal source for allogeneic cell therapy

- Javed Manesia, Scientist, STEMCELL Technologies
  Enhanced Ex Vivo Expansion of Hematopoietic Stem Cells by a Novel Compound Cocktail

- Tomer Rotstein, Duke University
  Transcription Factor-Wide Screens to Identify Regulators of Tissue Resident Memory T cell States

- Sean Sherman, Fate Therapeutics, Inc.
  Targeting UPAR With Multiplexed-Engineered iPSC-Derived CAR T Cells to Reverse Age- and Insult-Related Fibrotic Disease

- Nikita Trivedi, Seattle Children's Research Institute
  Role of integrins in engineered plasma cell localization and function

- William Rust, PhD, Seraxis Inc.
  SR-02, a Novel Islet Replacement Therapy for Insulin-Requiring Diabetes

- Shuangshuang Zhang, Suzhou Immunofoco Biotechnology Co., Ltd
  MxV Glycoproteins: A Novel Fusogen for In Vivo CAR-T Generation

- Philippe Parone, PhD, Esobiotec
  ENaBL, a Novel Lentiviral Vector Platform for In Vivo Engineering of Therapeutic Anti-BCMA CAR T Cells, Shows Effective T Cell Transduction and Tumor Control in a Humanized Murine Model

- Tina Xiao, Shenzhen MagicRNA Biotech
  A Vivo CAR T Producer (HN2301) for Autoimmune Disease Enables B cell Depletion in NHP Tissues and Performs Therapeutic Effect in an SLE Mouse Model

- Travis Friesen, Umoja BioPharma
  Preclinical Development of UB-VV400, a Surface Engineered Lentiviral Vector for the In Vivo Generation of CD22-Directed CAR T Cells

- Minkyeom Kim, Sungkyunkwan University
  Development of In Vivo CAR T Cell Therapy Using Nanoparticle and RNA-Based Gene Delivery Systems

- Celeste Richardson, Stylus Medicine
  Efficient and Effective in Vivo CAR T Generation Using Recombinase-Based, Precise, High-Capacity, Off-the-Shelf, LNP-Based In Vivo Genomic Engineering

- Saet Byoul Lee, Matica Biotechnology
  Development of a Scalable HEK293/HEK293T Suspension Cell Line Platform for Efficient Viral Vector Production

- Pouria Motevalian, Thermo Fisher Scientific
  Breaking the Bottleneck in Lentiviral Production: Achieving Ultra-Low Infectivity-to-Particle Ratios and Cost Efficiency for Transformative Gene Therapies

- Cathy Wang, CTMC
  Systematic Assessment and Scalable Bioreactor Process Development of Lentiviral and Gamma-Retroviral Vectors for CAR-T and TIL Therapies

- Michela Gentile, ReiThera
  From bench to dose: building a robust and scalable cGMP manufacturing process for non-replicative Herpes Simplex Virus 1 (nrHSV-1) vector as a powerful gene therapy platform

- Julie Porter, University of Utah Salt Lake City, UT
  Implementation of an Institutional Gene Therapy Safety Committee at the

University of Utah

- Nicole Nohilly, Resilience, Inc.
  Case Study: Resilience's SNAP Program from Bench Scale to GMP (USP Process and Lessons Learned)

- David Draper, Labcorp Drug Development
  Development of Cell-Based Functional Assays to Predict CAR-T Potency and Persistence

- Ian Johnston, PhD, Miltenyi Biotec B.V. & Co. KG
  Point of Care Automated Manufacturing Enables Rapid Translation of Both Novel TCR-T Cell Therapies as well as Conventional CAR T Cell Therapies into the Clinic

- Lyne Khair, Saint-Gobain Life Sciences
  Expansion and Characterization of Natural Killer Cells in a Closed Single-Use System

- Dana Margittai, Stanford Medicine, Laboratory for Cell and Gene Medicine
  G-Rex (Gas Permeable Rapid Expansion) Technology used for T regulatory Cell Culture

- Anusha Sivakumar, Project Scientist, University of California, San Diego
  Development and Translation of PPL-001, an autologous, gene-edited $CD34^{+}$ HSPC Therapy for Friedreich's Ataxia

- Ling Wu, ChemT Biotechnology
  Expansion of human T cells with greater quantity and quality via small molecule (CMT-01)

- Aaron Rosenstein, University of Toronto
  FAST-STEM: A Synthetic Biology Landing-Pad Toolkit for Next Generation Stem Cell Engineering and Regenerative Medicine

- Daniel Terheyden-Keighley, Catalent Cell & Gene Therapy
  Feeder-Free Expansion of iPSC-derived NK Cells to Clinical Scale

- Akane Urakami, PhD, BrightPath Biotherapeutics
  Generation of Functional BCMA CAR-iNKT Cells from Clinical-Grade iPSCs via a GMP-Compliant Manufacturing Process with Capacity for Linear Scale-Up

- Chu Han Hoh, Evonik Corporation
  Formulating recombinant proteins with chemically defined ingredients to replace human-derived proteins for mesenchymal stem cell culture

- Josh DiGiacomo, Thermo Fisher Scientific
  Development of Tools to Support Coating-Free Adherent Culture of Human Mesenchymal Stromal Cells Under Reduced-Serum and Serum-Free Conditions

- Cheng-Tai Chen, Industrial Technology Research Institute
  Bioinspired Microbeads for Boosting Feeder-free Manufacturing of Immune Cells

- Tim Le Fevre, STEMCELL Technologies, Inc.
  Serum- and Feeder-Free Generation of Multipotent Hematopoietic Progenitors from Pluripotent Stem Cells in a 3D Culture System

- Anthony Lauer, Promega Corporation
  Metabolic Profiling to Predict In-Vitro T-cell Fitness

- Aleksandra Nikoniuk, PhD Candidate, University College London

Microfluidic Magnetic Bead Capture of Target Cells for In-Process Phenotyping of CAR T Cells

- Chun-Wei Chi, University at Buffalo, The State University of New York
  Recirculated Tumor Chip Platform to Study How Microenvironmental Factors Affect Kinetics of Immune Cells and Immunotherapeutics

- Michael Tovey, Svar Life Science Nces
  Development of Potency Assays for Recombinant AAV vectors Independent of both the Promoter and Transgene as well as for AAV Encoded Transgenes Regulated by Photoreceptor and Retina Specific Promoters

- Chenming Lu, Lingyi Biotech
  A Novel and Reliable In Vitro Copper-Excretion Potency Assay with Engineered Huh-7-CuEP Cells for Evaluating Gene Therapy Candidates in Wilson Disease

- Christina McKenna, Matica Biotechnology
  Functional Potency-Based Bioactivity Characterization of Viral Vectors with Supportive Physical and Genomic Titer Analyses

- SN Sarah Morris, Ray Therapeutics, Inc.
  Functional cell-based models for gene therapies that utilize cell-specific promoters are enabled through CRISPR-activation

- Hester Wing Sum Chu, University College London
  BGT-DTDS vector in vivo potency analysis in mouse model of Dopamine Transporter Deficiency Syndrome

- Karthikeyan Devaraju, Cerba Research
  Optimized Automated DNA Extraction: A Pathway to Improved Patient Monitoring in CAR-T Trials

- Luca Nanni, NewBiologix
  Assessment of an Automated, Multi-dimensional Digital PCR Workflow for the Rapid Evaluation of Recombinant AAV Genome Integrity.

- Nechama Kalter, Bar-Ilan University
  High-Coverage Whole-Genome Sequencing Reveals Adverse CRISPR Editing Events in Human HSPCs Targeted by Therapeutic gRNAs

- Eleen Shum, PhD, Countable Labs
  Simultaneous measurement of viral vector titer and genome integrity with long-read amplicons enabled by Countable PCR

- Florian Sonntag, Ascend Advanced Therapies GmbH
  The Good, the Bad, & the Chimeric: Using Nanopore Sequencing to De-Risk rAAV Genome Packaging

- Andrzej Noyszewski, R&D Scientist, Agathos Biologics
  A Novel Reference Standard for Assessing AAV Titer and Genome Integrity

- Dhruv Prasad, Modalis Therapeutics
  Nanopore Sequencing and Screening of AAV Genomes for Optimal Production and Function

- Qimin Quan, NanoMosaic LLC
  Improved Quantification of Adeno-Associated Virus (AAV) Viral Genome Using Nanoneedle Technology for Enhanced Molecular Specificity

- Matteo Franco, ProtaGene US Inc.
  Comprehensive Integrity Assessment of rAAV Batches Through an Integrated Workflow Coupling Long Read Sequencing with Multiplex dPCR

- Thipa Asvarak, Genedata Inc.
  Leveraging Next-Generation Sequencing for CMC of Gene and Cell Therapy Products

- Esko Kautto, Forge Biologics
  Advancing rAAV Manufacturing with Long-Read Sequencing

- Isabella Pajevic, Solid Biosciences
  Full Length Transgene Quantification Utilizing NanoMosaic Tessie Technology

- Amanda Zhang, REGENXBIO Inc.
  Impact of Sample Collection Container Material, Hold Time, and Storage Temperature on Adeno-Associated Virus Endotoxin Testing

- Vidya Murthy, PHD, West Pharmaceutical Services, Inc.
  Ultra-Cold Storage of Lentiviral and Adenoviral Vectors in Cyclic Olefin Polymer Containers

- Andrew Hamann, PhD, InVitria
  Recombinant Animal Origin Free Albumin in Downstream Wash and Cryopreservation for CAR-T Cell Therapy Manufacturing

- Shibi Likhite, PhD, Nationwide Children's Hospital
  Development of In Vitro Potency Assay for AAV-mediated Gene Therapy for Muscular Dystrophy

- Alan Eduardo Castillo, Richter BioLogics GmbH & Co. KG - Hamburg
  pART Platform Process for Plasmid Manufacturing: Scalable, Robust, and Built for Tomorrow's Therapies

- Matthew Stork, Novartis Gene Therapies
  Risk Assessment for Cross-Contamination of Plasmids Used for AAV Production

- Rui Tang, ProBio Inc USA
  Advanced Plasmid Linearization Using Type I and Type II Restriction Enzymes: A Scalable, High-Purity Platform for Cell and Gene Therapy Applications

- Lijun Wang, CoJourney
  Revolutionizing Plasmid DNA Production: A Chromatography-Free, High-Yield, High-Quality Platform

- Jacob Hromyak, Bachelors, Akron BioManufacturing
  Animal Origin-free Yeast and Soytone-based Fed batch Fermentation as a Versatile Platform for High-Yield Plasmid Production

- Carson White, Masters, Akron BioManufacturing
  Scalable and Efficient Production of Plasmid using Yeast and Soytone Fed batch: Optimizing Single Use Technology Fermentation and Purification Processes for cGMP production

- Vassili Valayannopoulos, MD, PhD, MBA, Regeneron Pharmaceuticals
  Advances in Gene Therapy for Profound Hearing Loss: An Update on the CHORD Phase 1/2 Trial

- Lu Fang, Sichuan University West China Hospital Department of Ophthalomology
  Initial Results from Gene Therapy for X-linked Retinoschisis via Subretinal Delivery of scAAV8-hRS1 in Pediatric Patients

- Wenhui Zhou, Zhongmou Theraputics
  Safety and Efficacy of ZM-02 Optogenetic Gene Therapy Clinical Study for

Advanced Retinitis Pigmentosa

- Niranjana Kesavamoorthy, JFK University Medical Center
  Gene Therapy Clinical Trials Targeting Optic Neuropathies and Other Ocular Diseases until 2024

- Yongzhong Wang, PhD, Accuredit Therapeutics US
  Efficacy and Safety of ART002, a Single-dose Gene Editing Therapy, in Patients with Severe Heterozygous Familial Hypercholesterolemia

- Julien Baruteau, MD, PhD, Great Ormond Street Institute of Child Health University College London
  Initial clinical results from OTC-HOPE, the first in vivo, liver directed, AAV-mediated gene insertion study in neonatal OTC deficiency: complete clinical response observed in first male infant to receive ECUR-506 and complete 24-week Phase 1/2 study

- Cary Harding, MD, Oregon Health & Science University
  Durable Efficacy and Safety of DTX301: Long-term Follow Up (LTFU) of a Phase 1/2 Trial in Adults with Ornithine Transcarbamylase Deficiency (OTCD

- Paul Gissen, UCL
  First patient treated in the first phase 1–2 trial of AAVLK03hOTC gene therapy for ornithine transcarbamylase deficiency in children

- Biao Dong, PhD, Sichuan University
  rAAV Gene Therapy for Classic Fabry Disease: ZS805 Results from An Ongoing Investigator-Initiated Clinical Trial

- Heidi Wallis, Association for Creatine Deficiencies
  Title: Patient Centered Work on Considerations for Outcomes in Cerebral Creatine

Deficiency Syndromes Trial Design

- Lawrence Hayward, MD, PhD, Umass Chan Medical School
  Initial Safety Results from the PROPEL Trial Evaluating SBT101 as a Potential Gene Therapy for Spinal Cord Disease in Adult Males with X-linked Adrenoleukodystrophy

- Umar Farooq, MD, University of Iowa
  AlloNK® Cell Therapy ± Rituximab in Patients with Relapsed or Refractory Non-Hodgkin Lymphoma

- Chikako Funasaka, National Cancer Center Hospital East
  Trial in progress: Phase I study of non-viral gene-modified CAR-T cell therapy for malignant solid tumors with expression of EPHB4 receptor（CARTiEr）

- Zhigang Guo, PhD, Nanjing Miracle
  Phase I Open-label Single-arm Study to Evaluate the Safety, Tolerability and Preliminary Efficacy of CHT102, an Allogeneic Mesothelin-targeted CAR-T Cell, in Patients with Advanced Solid Tumors (CHAPTER)

- Akshara Velmurugan, University of Washington
  Improved HDAd Vector Design for Multiplex Editing and Gene Addition

- Meagan Quinlan, Ph.D., Allen Institute for Brain Science
  Cross-Species Enhancer AAV Platform for Targeting Spinal Motor Neurons and Cardiomyocytes

- Dezhong Yin, OriGene Technologies, Inc.
  A New Generation of Inducible All-In-One Tet-On-ORF/shRNA Vectors

- Aykut BOSTANCI, Wake Forest University School of Medicine
  AAV-Based Cell Type-Specific Targeting (ACTStar) System: A Novel Method for Brain Cell Access

- Angelo Raggioli, Head of Technology Development, ReiThera
  GRAd as Vaccine Platform for COVID-19 and HIV

- Chitra Gopinath, GROW Research Laboratory
  Differential expression from reconstituted AAV hybrid dual vector systems with splicing enhancers is dependent on tissue specific SRSF protein expression

- Andrew Keezer, Ring Therapeutics
  Purified Anelloviral Capsid Proteins can Bind and Encapsidate Different Nucleic Acids Payloads into Anellovectors for Gene Delivery

- Bastien Doix, PhD, EsoBiotec
  Development of Novel T Cell-Specific Synthetic Promoters for Safer and More Effective In Vivo CAR-T Cell Therapies

- Samuel Bartlett, Ultragenyx Pharmaceutical Inc.
  Optimization of Plasmid Cloning Methodology to Support Continued Development of the Pinnacle PCL™ Gene Therapy Platform

- Daksha Patel, GenScript USA Inc.
  High-Quality Engineered Plasmid Vectors for Advancing Therapeutics Development

- Michael Karney, Genewiz From Azenta Life Sciences
  Enhancing Gene Transfer Efficiency in Viral and Non-Viral Vectors for Therapeutic Delivery

- Bo Lin, Revvity
  Gene Delivery Vector Comparison Study Using the Celigo™ Image Cytometer

- Justine Basset, EG 427
  New gene therapy vehicle for the expression of multiple transgenes with distinct kinetics of expression

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

- Shohreh Ghasemi, Augusta University
  AAV Vectors in Focus: Overcoming Challenges in Gene Delivery Systems

- Victor Wakim, Weill Cornell Medical College
  Biomechanical Assessment of the Impact of Adenoviral Delivery of Three VEGF Isoforms on Rotator Cuff Tendon-to-Bone Healing in Mice

- Roderick Slavcev, University of Waterloo
  A novel miniaturized filamentous phagemid as a gene delivery vehicle to target mammalian cells

- Roderick Slavcev, University of Waterloo
  An E. coli-based platform for the production and assembly of anellovirus vectors in vitro for gene therapy platform

- Bastien Doix, PhD, EsoBiotec
  Engineering T Cell-Specific Lentiviral Vectors for Safe and Effective In Vivo CAR-T Therapy

- Isabella Gold, Ring Therapeutics

Infectivity, Expression and Redosing in mice of an Anellovirus vector produced by in vitro assembly.

- Shefali Tripathi, Indian Institute of Technology Kanpur, India
  Detyrosinated Microtubules Limits AAV2 Trafficking and Regulates Gene Transduction Efficiency

- Yuki Yamaguchi, Ph.D., Osaka University
  The influence of VP1 N-termini on heat-induced genome release of adeno-associated virus serotype 8

- Anusha Sairavi, MS
  A Single Conserved F-to-Y Mutation at the 5-Fold Interface Is Exclusively Required for an AAP-Dependent-to-Independent Capsid Assembly Switch

- Joshua Hull, Duke University
  Functional Orthogonality of Parvoviral Phospholipase A_2 (PLA_2) Domains in Recombinant AAV Vector Transduction

- Anusha Sairavi, MS
  Identification of key amino acids near the N-terminus of AAV AAP essential for driving its degron function

- Jeanette Zanker, GENETHON
  Understanding DNA host cell factors as potential restriction factors to efficient AAV gene transfer in skeletal muscle cell

- Sophie Karolczak, The Hospital for Sick Children
  EGFR Inhibition Reduces AAV8 Capsid Entry to Hepatocytes, Posing a Potential Liver De-Targeting Strategy

- Valerie Hinsch, MS, Division of Cellular and Molecular Therapy, Department of Pediatrics, University of Florida, Gainesville, FL, USA
  Interrogating the Dependency of AAV Capsids on AAVR for Retinal Transduction following Intravitreal or Subretinal Injection

- Zinia Mohanta, Kennedy Krieger Institute
  Non-Invasive Monitoring of rAAV-Mediated Gene Expression Using Chemical Exchange Saturation Transfer (CEST) MRI

- Francesco Ragazzini, Tigem
  Engineering a gene dosage compensation circuit through competitive dimerization for gene therapy

- Aradhana Kasimsetty, University of Pennsylvania
  Small Molecule Inhibition of SUMOylation Increases Expression from AAV Vectors Both During and Long After Initial Transduction in Mice

- Yating Wang, Broad Institute of Mit And Harvard
  An Enhancer-based AAV Toolbox for Precise Neuronal Targeting

- Ann Lettko, MeiraGTx
  Novel Rationally Designed Promoters Surpass CAG in Mouse and Human Models

- Nicholas Dietrich, Kriya Therapeutics - Redwood City, CA
  KT-Splice: Regulation of AAV Transgene Expression by Manipulation of Alternative Splicing

- Amparo Cosio, Northwestern University
  Ultra-Compact Synthetic Transcription Factor Platform for Small-Molecule Regulated Control of AAV Transgene Expression

- Sara Wasserman, University of North Carolina Chapel Hill
  Controlling the Expression of Dose-Sensitive AAV Transgenes with Chemically Induced Proximity

- Camilla Belamarich, MeiraGTx
  Differential Usage of Transcription Factor Binding Sites to Boost Synthetic Promoter Activity

- Jianghui Wang, UMass Chan Medical School
  Characterization of AAV ITR-proximal regions (IPRs) from different AAV serotypes reveals tissue-specific enhancer and promoter activities

- David James, SynGenSys
  A Platform for Next-Generation Synthetic Promoter Design and Engineering

- Qiang Zheng, Chengdu Origen Biotechnology
  A novel AAV2 variant designed for targeting of retinal tissue via intravitreal injection

- Qiang Zheng, Chengdu Origen Biotechnology
  Identification of a novel AAV capsid variant with enhanced transduction efficiency in mice

- Qiang Zheng, Chengdu Origen Biotechnology
  A Novel AAV Variant for Efficient RPE Transduction via Intravitreal Injection

- Florian Schmidt, MA, ETH Zurich
  Engineered AAV capsids for transduction of the retina by intravitreal injection

- Bing Wang, AAVnerGene Inc
  Systematic Evaluation of AAVs in Two Non-human Primate Models Following IVT Injection Identifies AAV(Eye) as a Promising Candidate for Further Capsid Engineering

- Sameera Peraramelli, Avirmax, Inc.
  Development of a Novel Antibody Targeting Engineered AAV2 Capsids for Gene Therapy Applications

- Mengtian Cui, Umass Chan Medical School
  Directed Evolution of an Intravitreally Delivered Random Mutagenesis Library of the AAV2 VP1u Region Generates Novel Capsids with Enhanced Retinal Transduction by Increasing Cellular Trafficking and Transcription Efficacy

- Subin Cho, Yonsei University
  Breaking Through the ILM: An Evolved AAV Variant for Efficient Photoreceptor Targeting

- Josephine Macdonald, Hannover Medical School
  Tailoring AAV Vectors for Gene Therapy of Inner Ear Disorders by Directed Evolution

- Chunyan He, Suzhou GenAssist Therapeutics Co., Ltd
  A High throughput, high-efficient AAV evolution system for tissue targeting AAV screening

- Ajay Extross, University of Minnesota
  Nanobody Incorporation Confers Precise Infectivity to Adeno Associated Virus

- Travis Bainbridge, Genentech, Inc
  AAV capsid engineering for improved AAVR binding to enhance transduction

efficiency

- Stephen Trisno, Stanford University
  Conjugation of Peptide Ligands to AAV Towards Pancreatic Cell AAV Gene Delivery

- Zhongya Wang, PhD, RenoViron
  An Approach to Facilitate Multiple Peptide Insertions at Different Locations on the Surface of AAV Viral Particle

- Dongxin Wang, University of Missouri
  Assembly-based RNA Packaging Using Adeno-associated Virus Capsids

- Mehtap Bal, BSc, University College London
  Engineering AAV2 Capsids Using a Novel Synthetic Biology Approach to Enhance Genome Packaging Efficiency and Reduce Empty Capsid Ratios

- JIn Qiu, PackGene Biotech INC
  Advancing AAV Capsid Engineering for Targeted Gene Therapy Using PackGene's π-Icosa Platform

- Mian Umair Ahsan, Children's Hospital of Philadelphia
  Generative Transformer Models for Designing Diverse and Viable AAV Capsid Sequences

- Leszek Lisowski, MBA, PhD, Sendatu Therapeutics
  Artificial Intelligence Enables Rapid and Efficient Identification of Adeno-Associated Vectors with Improved Transduction of Target Tissues

- Jinyoung Kim, Yonsei Univ.

From Data to Delivery: AI-Driven Design of Optimized AAV Vectors for Gene Therapy

- Hohyoun Lee, R&D General Manager, AAVATAR Therapeutics, Inc.
  Machine Learning-Driven AAV Capsid Engineering for Enhanced Tissue Targeting and Optimized Production Efficiency

- Jimin Han, Labcorp Drug Development - Madison, WI
  Investigating the Effect of Age on AAV2 and AAV8 Delivery and Transgene Expression via Subretinal Injection in Mice

- Victor Hernandez, PhD, Axovia Therapeutics Inc
  A new dosing regimen to treat Bardet-Biedl Syndrome 1 (BBS1) retinal degeneration with AXV-101 (AAV9-BBS1) improves histological and functional photoreceptor survival in Bbs1 M390R mice. Finding the right balance between bleb number, dose per bleb and total dose per eye.

- Lukas Bachmann, Insmed Gene Therapy LLC
  INS1203: RNA-End Joining Technology Enables Full-length ABCA4 Expression In Mouse and NHP Retinal Photoreceptors

- Mayur Choudhary, Opus Genetics
  Evaluation of MERTK gene therapy in RCS rats following a single bilateral subretinal injection.

- Sameera Peraramelli, Avirmax, Inc.
  Inhibition of Laser Induced Choroidal Neovascularization (LCNV) by Blocking Complement Activation Cascade Using AAV Vectors Carrying Complement Inhibition proteins

- Seongjin Seo, University of Iowa

Development of Gene Therapy Vectors to Deliver MYO7A, PCDH15, CDH23, and USH2A

- Sukhvinder Kaur, OSU
  Insulin-Assisted AAV Gene Therapy for Inherited Retinal Diseases

- Rachel Eclov, PhD, Kriya Therapeutics
  Inhibition of IGF1R with KRIYA-586: A Novel AAV Gene Therapy Expressing an Anti-IGF1R Antibody Demonstrates Comparable Pharmacodynamic Activity to Teprotumumab

- Ruth Castellanos, PhD, Kriya Therapeutics
  Periorbital Delivery of AAV Therapeutics for the Treatment of Orbital Diseases

- Molly Kalker, Cilcare Inc.
  Intracochlear Administration Methods Across Species

- Lu Song, Calico Life Sciences
  AAV mediated gene delivery to the aged and young mice lung

- Matthew Rioux, University of Texas Southwestern Medical Center
  A Head-to-Head Comparison of AAV9 Biodistribution in Mice: Routes of Administration and Age Dependence

- Tyler Mola, UMass Chan Medical School
  Pre-Clinical Safety and Efficacy of AAV9 gene therapy for Mucolipidosis type IV (ML4)

- Ozge Cetin, University College London
  Neonatal Intravenous Delivery of AAV-F in a Gaucher disease mouse model

- Madison Thomas, University of Pittsburgh
  AAV-Mediated Beta-Cell-Specific Smad2 Silencing: Bridging Transgenic Mouse Evidence to Gene Therapy for Hyperglycemia

- Jordan Stokes, University of Florida
  Functional Elevation of Computationally Designed IL-10 in IL-10 KO mice

- Lauren Switala, Case Western Reserve University
  InstaGTx Amplifies AAV Tropism and Is Safe in Non-human Primates

- Stephane Baudouin, Ph.D., UniQure NV
  Broad Biodistribution of AAV5 and AAV9 Following ICV Administration Compared to ICM in Non-Human Primates.

- Qiang Zheng, Chengdu Origen Biotechnology
  Biodistribution in ocular tissues after suprachoroidal injection of KH658 in cynomolgus monkeys

- Qiang Zheng, Chengdu Origen Biotechnology
  Exploring the Transduction Efficiency of Suprachoroidal Injection of AAV in the Treatment of Neovascular Age-related Macular Degeneration through A Twice-laser Modeling

- Kris Poulsen, Adverum Biotechnologies
  Biodistribution and Expression of Ixoberogene Soroparvovec (Ixo-vec) in Non-Human Primates: A Comprehensive Study on Intraocular and Systemic Distribution

- Kalpana Rajanala, Ocugen Inc
  Preclinical Safety Evaluation of AAV5-hRORA (OCU410-ST) a Gene Therapy

Candidate for Stargardt's disease and other ABCA4 related retinopathies

- Qiang Zheng, Chengdu Origen Biotechnology
  Bilateral sequential injection of KH631 in rhesus monkeys showed good safety profile

- Qiang Zheng, Chengdu Origen Biotechnology
  KH631 consistently expressed in rhesus monkey eyes over 152 weeks

- Qiang Zheng, Chengdu Origen Biotechnology
  Evaluating the impacts of pre-existing NAb and ADA in rhesus monkeys on the efficacy of subretinal injection of KH631

- Marcela Salazar Werner, Franklin BioLabs
  Prevalence of Neutralizing Antibodies Against Adeno-Associated Virus (AAV) 8 and 9 in a Specific Pathogen-Free (SPF) Colony of Cynomolgus Macaques

- Itsasne Arangoa, Viralgen Vector Core - San Sebastian
  Integrating Raman Spectroscopy and Genome-Scale Modeling to Enhance AAV Upstream Manufacturing

- Elena Bianchetti, LEXEO Therapeutics
  Improving VP1 Ratios Impact on CQAs in rh10 AAV Manufactured Through Sf9 Platform.

- Nafiseh Nafissi, University of Waterloo/Mediphage Bioceuticals
  msDNATM: A Superior DNA Vector For Improved rAAV Manufacturing

- Chien-Ting Li, PhD, Lonza Houston Inc.
  Plasmid Engineering and Process Development to Improve Self-Complementary

AAV Productivity

- Ayano Fukuhara, U-Medico, Inc.
  Investigation of the Variations in Viral Protein 2 Stoichiometric Ratio and Deamidation of Viral Protein 1 and Their Impacts on Transduction Efficiency of Adeno-Associated Virus 8 Vectors

- Charles Feathers, Tenaya Therapeutics
  High Productivity HEK293 AAV Production Platform Enabled by Novel Transfection Reagents and Proprietary Plasmid Expression Systems

- Mikkal Blick, MilliporeSigma - Carlsbad, CA
  A Scalable, DoE-based Approach to Improving Plasmid Ratios for AAV Transfection

- Paul Freeman, Affinia Therapeutics
  High-Yield, Pan-Serotype Plasmid System for Manufacturing Adeno-Associated Virus Gene Therapies: Cost and Efficiency Benefits for R&D and Commercial Processes

- Andrew Tsao, GenScript USA Inc.
  Overcoming Challenges in AAV Plasmid Preparation: Advanced Solutions for Superior ITR Integrity and High-Yield Production

- Anna Prendeville, Asimov
  Development and Optimization of a Transient Two-Plasmid AAV Manufacturing Platform

- Alina Venereo Sanchez, VVector Bio
  Streamlining Recombinant AAV Production: A Single Plasmid Approach for Higher Yields and Reduced Costs

- Siddhartha Paul, Catalent Pharma Solutions
  Assessment of Novel RepCap Vectors for Improved AAV Titers along with Superior Product Characteristics

- Xiaojun Liu, ReciBioPharm
  One Stone, Two Birds: Engineering Rep-Cap Plasmids to Enhance AAV Manufacturability and Potency

- Ruigong Wang, Catalent Cell & Gene Therapy
  Novel RepCap Vectors with a Mutated Membrane-Associated Accessory Protein for Recombinant AAV Titer Improvement

- Irene Song, Packgene Biotech
  Enhancing AAV Production Efficiency through Plasmid Modification and Dual-Plasmid Systems

- Jenna Rodden, Capsida Biotherapeutics, Inc.
  rAAV Manufacturing Solutions: Strategic Designs of Engineered rAAV Two Plasmid Systems for Cost Effective Scaling and Product Safety

- Qiantong(Tina) Chen, University College London
  An Engineering Biology Design Approach to Improving Genome Upload into AAV Capsids

- Kenneth Thompson, PhD, Thermo Fisher Scientific
  Assessing the Impact of AAV Genome Size on Vector Productivity and Quality: A Comprehensive Analysis using Automated Small Scale AAV Production

- Jiantao Zhang, AAVnerGene
  Removal of Additional P5 and VA RNA II Elements from AAVone Enhances AAV

Productivity, Increases Full Particle Ratio, and Reduces Impurities

- Chunyan He, Suzhou GenAssist Therapeutics Co., Ltd
  Application of DoE method to improve the rAAV Packaging Titer of Large GOIs

- James Kim, Epic Bio
  Small Scale AAV Bioreactor Optimization Demonstrates Iterative Titer Gains of rAAVrh74 Serotype EPI-321, a CRISPR-mediated Epigenetic Therapy

- Derek Kichula, Passage Bio
  Scalable Production of an AAV Manufacturing Platform: Transitioning from Adherent to Suspension-Based Triple Transfection

- Brandon Hoyle, Solid Biosciences
  Capillary Isoelectric Focusing (cIEF) Platform for Characterization of Charge Variants of Adeno-Associated Virus (AAV) Capsids and Impact on Their Transduction Efficiency

- Jean-Marc Guedon, PhD, Culture Biosciences
  AAV9 Process Optimization and Robustness Evaluation in Cloud-native 250mL Bioreactors

- GARIMA SINHA, Croda Inc.
  Leveraging High-Throughput Virology and Design of Experiment Expertise for Rapid Transfection Reagent Optimization for AAV/LV Production

- Laura Juckem, PhD, Mirus Bio
  One Size Does NOT Fit All: Impact of AAV Serotype and GOI on Choosing the Optimal Transfection Reagent

- David Nolan, Bioinfoexperts LLC
  Quantifying DNA Point Mutations in Commercial AAV products

- Ziwei Li, Genedata Inc.
  Leveraging Short- and Long-Read NGS Technologies In-House for Optimal Quality Control in Manufacturing of Gene and Cell Therapies

- Aaron Hall, Duke University
  Mapping of common vector genome breakpoints and non-specific DNA fragments in single-stranded and self-complementary AAV vectors by nanopore sequencing

- Christopher Shilling, MS, Forge Biologics
  Enabling Innovation Through Transition to the FUEL™ Platform: Regulatory Feedback on the Comparability of Ad Helper Plasmid pEMBR™ 1.2 vs. pEMBR™ 2.0

- Jondavid De Jong, Virica Biotech
  Optimizing AAV Production: Accelerating Discovery of Small Molecule Enhancers using High Throughput Virology and Design of Experiments

- Margherita Neri, AGC Biologics
  Optimizing AAV Production: Advances in Transfection Agents, Additives, and Clonal Cell Line Development

- Sonal Patel, MS, MilliporeSigma
  Increasing rAAV productivity through selection of a high producing HEK293 transient cell line and addition of an enhancer

- Junneng Wen, Johns Hopkins University
  Cell Cycle Modulation as a Strategy to Enhance AAV Production Across Serotypes

- Philipp Beck, PhD, Ascend Advanced Therapies
  Small samples, big insights: accelerating AAV stability studies with high-quality, low-volume analytics

- Kevin Wong, Cirsium Biosciences
  Characterization of Plant-made AAVs and a Side-by-side Comparison with HEK-derived AAV

- Jan Carette, PhD, Stanford University
  Increasing the Susceptibility of Cell Lines to a Broad Range of Adeno-Associated Viral Vectors to Enable in Vitro Potency Assays

- Gang Li, Lonza Houston Inc.
  Development of High-performing Stable Producer Cell Lines for Therapeutic AAV Vector Manufacturing

- Efrain Guzman, PhD, MBA, NewBiologix
  Generation and Characterization of a Novel HEK293 Suspension Cell Line for rAAV Production.

- Chris Brown, ReciBioPharm
  Optimization of rAAV Production Using an In-House Suspension HEK293 Cell Line

- Leah Benedict, Sangamo Therapeutics
  Recombinant Adeno-Associated Virus (rAAV) Production in Spodoptera Frugiperda (Sf9) Cells: Viral Cathepsin Mediated Capsid Cleavage and Mitigation Strategies

- Virginia Fusco, Tigem
  Engineering a stable cell line for AAV production through synthetic biology

- EMMANUELLE CAMEAU, Cytiva Genomic Medicine
  Transient vs stable cell lines for AAV manufacturing: a cost modeling approach

- Brian Tomkowicz, PhD, SK Pharmteco - US
  Generation of a Universal AAV Packaging Cell Line for use in Commercial Gene Therapy Manufacturing

- Ryo Aoki, Gunma University Graduate School of Medicine
  Enhanced AAV Vectors for Targeted and Efficient Gene Expression in Brain Microglia

- Zhongya Wang, PhD, RenoViron
  Identification of common AAV surface residuals responsible for AAV pre-existing antibody recognition

- Ashley Morando, 4D Molecular Therapeutics
  Evaluation of Multiple Immunoassay Formats for the Detection of AAV Capsid Specific Antibodies

- Mario Mietzsch, PhD, University of Florida
  Structural Characterization of Antibody Interactions from Infants with AAV2-associated Hepatitis to Guide Rational Capsid Redesign

- Ranim Maaieh, University of Toronto
  Redesigning Immunogenic AAV Epitopes to Prevent Cell Surface Display by Human Leucocyte Antigens

- Barbara Sullivan, PhD, Ultragenyx
  The Seropositivity Dilemma for AAV Gene Therapy

Wednesday, May 14

Sponsored Symposium

MilliporeSigma: Optimizing AAV Gene Therapies: NGS applications to enhance development, manufacturing, and testing

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

- McKenzie Landgraf, MS, MilliporeSigma
  MilliporeSigma: Optimizing AAV Gene Therapies: NGS applications to enhance development, manufacturing, and testing

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
  AAV delivery to brain, peptide insertions, optimizing capsid delivery

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

Scientific Symposium

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

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

- Jeffrey Miller, MD, University of Minnesota

Developing NK Cell Therapeutics: Off-The-Shelf NK-CARs and Trispecific Killer Engagers (TriKEs) for Cancer and Autoimmune Disease

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

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

Scientific Symposium

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

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

- James Dahlman, PhD, Georgia Tech
  Nanotechnology: Discussing how nanoparticles are being used for more efficient gene delivery systems

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

- Le Cong, PhD, Stanford University
  Artificial Intelligence: Exploring how machine learning algorithms are helping to predict off-target effects in gene editing or optimize vector designs

- Shivani Srivastava, Fred Hutch
  Immunology: Investigating the integration of gene therapy with immunotherapy approaches for cancer treatment

Oral Abstract Session

Cell Therapy Product Engineering I

Location: Room 293-296

8:00 AM - 9:45 AM

- 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
  Prenatal genetic testing

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

Fireside Chat

Fireside Chat: Global Regulatory Perspectives

Location: Room 393-396
8:00 AM - 9:45 AM

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

- Yoshiaki Maruyama, PMDA

PMDA Perspective on Global Regulatory Convergence

Sponsored Symposium

Lonza & Quell Therapeutics: Large-Scale T-Cell Engineering with Non-Viral Delivery of Complex Cargos (Session 1) | Development of CAR-Treg Therapies for Transplantation and Autoimmunity (Session 2)

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

- Erin Brooks, Lonza Bioscience
  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)

Sponsored Symposium

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

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

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

Networking

Exhibit Hall

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

General Session

Presidential Symposium

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

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

- Mike McCune, MD, PhD, Bill & Melinda Gates Foundation
  Presidential Symposium Keynote

- 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

Poster Talk Session

Wednesday Poster Talks

Location: Exhibit Theater
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

Sponsored Symposium

Cytiva: Could cell line technologies bring disruptive changes to gene therapy manufacturing?

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

- Alice Giraud, Cytiva
  Cytiva: Could cell line technologies bring disruptive changes to gene therapy manufacturing?

Sponsored Symposium

BioAgilytix: Overcoming Challenges for Advanced Therapeutics: Case Studies on Navigating the Unique Bioanalytical and CMC Characterization Landscape of Cell and Gene Therapies

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

- Jessica Weaver, BioAgilytix
  BioAgilytix: Overcoming Challenges for Advanced Therapeutics: Case Studies on Navigating the Unique Bioanalytical and CMC Characterization Landscape of Cell and Gene Therapies

Sponsored Symposium

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

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

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

Sponsored Symposium

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

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

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

Oral Abstract Session

Neurologic Diseases - Vectorology and Gene Therapy

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

- Hui Yang, HuidaGene Therapeutics Co., Ltd., Shanghai, China; Shanghai Institute of Meteria Medica, Chinese Academy of Sciences, Shanghai, China
  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 to CNS

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

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

Oral Abstract Session

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

Tools & Technology Session

Tools and Technology Forum 1

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

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

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

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

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

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

- Vasileios Georgakakos, Clean Cells

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

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

Sponsored Symposium

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

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

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

Sponsored Symposium

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

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

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

Sponsored Symposium

Forge Biologics: Great Science Needs Innovative Manufacturing

Technology

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

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

Tools & Technology Session

Tools and Technology Forum 2

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

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

- Goran Sokolov
  Refeyn: Quantitative Analysis of AAV Capsid Heterogeneity with SamuxMP Mass Photometry

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

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

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

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

Oral Abstract Session

Gene Editing: New Tools and Technology Advances

Location: 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

- Geoffrey Berguig, 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, DVM, DACVIM, 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

- Ales Strancar, Sartorius BIAA Separations
  Sartorius: Optimizing AAV Production: A Holistic Approach to Upstream, Downstream, and Cost Per Dose Enhancement

Poster Abstract Session

Wednesday Poster Reception

Location: Poster Hall Hall I2
5:30 PM - 7:00 PM

- Isabelle Sansal, AlltRNA
  tRNA at the Frontier: Developing Medicines to Treat Stop Codon Disease

- Tenghui Yu, PhD, Grtigen
  AAV-mediated Gene Knock-down and Replacement Therapy for PKU was Effective with High Efficacy and Low Dosage

- Yixiong Chen, Lingyi Biotech
  LY-M003, An AAV Gene Therapy Product with Copper Responsive Engineering of ATP7B Expression (CREATE) for Wilson Disease

- Peiyi Guo, UMass Chan Medical School
  Mitochondria Targeted rAAV Gene Therapy for Cytochrome C Oxidate Deficiency

- Elena Kahn, University of Pennsylvania School of Medicine
  Correction of a Recurrent Pathogenic Variant in Methylmalonic Acidemia Using Adenine Base Editing

- Eun-Young Choi, PhD, NHGRI/NIH
  AAV9 -Mediated Gene Therapy of MMAB Deficiency: Preclinical Efficacy in Mouse models of Cobalamin B Type Methylmalonic Acidemia

- Joseph Hacia, PhD, University of Southern California
  The Mouse Peroxisome Research Resource for Testing Gene and Cell-based Therapies

- Ala'a Siam, University College London
  Systemic lentiviral and AAV gene therapies correct several metabolic imbalances in the brain of the maple syrup urine disease mouse model

- Gloria Gonzalez-Aseguinolaza, PhD, Center for Applied Medical Research (CIMA) of the University of Navarra
  AAV-Mediated Restoration of Hepatic CYP27A1 Expression in Chenodeoxycholic Acid-Pretreated Mice with Cerebrotendinous Xanthomatosis

- Gloria Gonzalez-Aseguinolaza, PhD, Center for Applied Medical Research (CIMA) of the University of Navarra
  Improvement of Progressive Familial Intrahepatic Cholestasis Type 2 Via AAV-Mediated Hepatic BSEP Expression in Mice

- Randy Chandler, PhD, National Institutes of Health
  New murine models of propionic acidemia caused by PCCB deficiency allow for the testing of genomic therapies in both severe and mild disease phenotypes

- Jae-Jun Kim, UCSF
  AAV capsid prioritization in normal and steatotic human livers maintained by machine perfusion

- Pasquale Piccolo, FONDAZIONE TELETHON
  Impact of liver fibrosis on AAV-mediated gene transfer to hepatocytes

- Jeong-A Lim, PhD, Duke University
  Determination of the Minimum Effective Dose of AAV9-LSP-hPYGL in a Glycogen Storage Disease Type VI Mouse Model

- Evangelos Pefanis, PhD, Regeneron
  Ass1 Targeted Gene Insertion in Newborn Mice Provides Effective, Long Term Disease Correction in a Lethal Mouse Model of Citrullinemia Type I

- Gloria Gonzalez-Aseguinolaza, PhD, Center for Applied Medical Research (CIMA)

of the University of Navarra
Long-term Efficacy of VTX-806(8) for the Treatment of Cerebrotendinous Xanthomatosis: Sustained Correction of Metabolic Abnormalities in Mice

- Carlo Cipriani, Department of Electronics, Information and Bioengineering, Politecnico Di Milano
  Exploring Cell-Free DNA Signatures as Biomarkers for Disease Progression in Metachromatic Leukodystrophy

- Daniel Virga, Regeneron Pharmaceuticals
  Development of a Durable Gene Therapy for Targeting CNS and Visceral Pathologies in Acid Sphingomyelinase Deficiency

- Evan Kleinboehl, University of Minnesota - Moriarity Lab
  Engineering T Lymphocytes for Enzyme Replacement Therapy

- Michael Przybilla, PhD, University of Minnesota
  Penetrating the blood-brain barrier: Utilization of the PS Gene-editing System to encode a novel fusion β-galactosidase for the treatment of GM1 gangliosidosis

- Rodrigo Miguel Dos Santos, Cedars-Sinai Medical Center
  Biological Pacemaker Activity Induced by AAV-TBX18

- John Leach, YAP Therapeutics
  Gene Therapy Targeting the Hippo Pathway for In Vivo Cardiac Regeneration in Ischemic Heart Failure

- Huanyu Zhou, PhD, Tenaya Therapeutics
  TN-501 Gene Editing Therapy for PLN-R14del-Associated Cardiomyopathy

- Laura Lalaguna, CNIC
  AAV-Based Gene Therapy Targeting p53 Signaling as a Therapeutic Strategy for Arrhythmogenic Right Ventricular Cardiomyopathy Type 5

- Matt Edwards, Affinia Therapeutics
  A Novel Investigational AAV Gene Therapy for Treatment of BAG3 Dilated Cardiomyopathy

- Juliette Hordeaux, DVM, PhD., Gemma Biotherapeutics
  Gene Replacement Therapy for Barth Syndrome Using a Novel Cardiotropic Capsid

- Mariana Argenziano, Ncardia
  Human iPSC-Derived Cardiomyocyte Disease Modeling of Friedreich's Ataxia and Duchenne Muscular Dystrophy for Therapeutic Assessment

- Taylor Anglen, Duke University
  A gene regulatory element modulates myosin expression and alters cellular stress response

- Lindsey Rollosson, Tenaya Therapeutics
  Developing In Vivo Prime Editing as a Potential Treatment Option for Heart Disease

- Timo Jonker, Amsterdam UMC
  Comparison of New and Existing Promoters for Cardiac Gene Expression and Specificity

- Md. Sohanur Rahman, Postdoctoral Associate, Florida International University Herbert Wertheim College of Medicine
  Gene Expression Alterations in HIV-Associated Chronic Obstructive Pulmonary

Disease: Insights into Molecular Pathways and Inflammation

- Kingshuk Panda, Graduate Student, Florida International University
  Single-Cell RNA Sequencing Reveals Lung T and B Cell Dysregulation in SP-C Tat Transgenic Mice Models and Cigarette Smoke Exposure

- Luis Pereira de Almeida, PhD, University of Coimbra
  CRISPR-Cas9-Mediated ATXN3 Gene Inactivation as a Promising Gene Therapy for Machado-Joseph Disease

- Dong Yang, PhD, HuidaGene Therapeutics Co., Ltd., Shanghai, China
  CRISPR-hfCas12Max-Mediated DNA Editing Restores Motor Function in Spinocerebellar Ataxia Type 3

- Anthony Donsante, Emory University School of Medicine
  The Use of AAV2.retro to Deliver Transgenes to Both Upper and Lower Motor Neurons in the Rat for the Treatment of Amyotrophic Lateral Sclerosis

- Michael Karney, Genewiz From Azenta Life Sciences
  Exploring the Role of Inverted Terminal Repeats in AAV Vector Performance for CNS Gene Therapy

- Anne-Marie Castonguay, Laval University
  Novel Noninvasive Gene Therapy for Parkinson's Disease Using Viral Encoded Single-Chain Antibody Treatment

- Samuel Hasson, Rgenta Therapeutics
  RSwitch Enabled Gene Therapy to Fine Tune Frataxin Expression for the Treatment of Friedrich’s Ataxia

- Sergi Verdés, PhD, Universitat Autònoma de Barcelona (UAB)
  Muscle-Mediated Klotho Secretion Outperforms Spinal Overexpression in Mitigating ALS Progression

- Xiupeng Chen, membership, UMass Chan Medical School
  Supraphysiological expression of SMN1 protein leads to compromised heart function improvement and cardiac thrombosis in mice

- Jeroen Bastiaans, MeiraGTx
  Preclinical Efficacy and Potency Assay Development of An Optimized AAV-hUPF1 Gene Therapy for Amyotrophic Lateral Sclerosis (ALS) and Frontotemporal Dementia (FTD)

- Paula Miranda, VectorY
  AAV-mediated delivery of an intrabody targeting TDP-43 for Amyotrophic Lateral Sclerosis therapy

- Yixiong Chen, Lingyi Biotech
  Preclinical Evaluation of LY-N001 in an innovative GBA1-Associated Parkinson's Disease Model

- Camila Vallve Maine, University College London
  Characterisation of a dnajc6^{R857G} Knock-in Mouse Model of PARK19 Towards Gene Therapy Development

- Erin Slosarek, Labcorp Drug Development
  Targeted Delivery to Putamen via MRI-Guided Surgery: Nonhuman Primate versus Göttingen minipig®

- Zhenhua Wu, Exegenesis Bio Inc.
  Next-Generation AAV-Based Gene Therapy for Spinal Muscular Atrophy: Safety

and Efficacy of EXG001-307 in Clinical Trials

- Chunjuan Song, PhD, Exegenesis Bio Inc.
  Nonclinical Evaluation of AAV Gene Therapy EXG001-307 for Spinal Muscular Atrophy

- Payam Zarin, GentiBio
  Development of Tissue Engineered Tregs for the Treatment of Ischemic Stroke and Neurodegenerative Diseases

- Luis Labrador, Cincinnati Children's Hospital Medical Center
  Development of deficits in attention and cognitive functions in Spinal Muscular Atrophy mouse model with non-physiological restoration of SMN1 transgene expression

- Rachel Eclov, PhD, Kriya Therapeutics
  Chemogenetic silencing of hyperexcitable neurons demonstrates sustained pain relief in a preclinical model of lumbosacral radiculopathy

- Neil Hackett, PhD, Neil Hackett Consulting
  AAV9-hPLA2G6 Gene Therapy Improves Survival in a Preclinical Mouse Model of Infantile Neuroaxonal Dystrophy

- Soo-Kyung Lee, PhD, University at Buffalo (SUNY)
  Human patient-specific FOXG1 syndrome mouse model reveals roles of protein homeostasis and neuroinflammation in neurodevelopmental disorder

- Mark Mizee, Amsterdam UMC
  Amsterdam Oncology and Neuroscience Research: ADORE is breaking barriers in therapy and diagnostics research in the Netherlands

- Taleen Hanania, PsychoGenics
  Characterization of a SOD1 Rat Model of ALS using Behavior, Electrophysiology, and Biomarkers: An Ideal Model to Test Novel Gene and Cell Therapies

- Geon Seong Lee, Seoul National University
  Morc2a Variants Cause Hydroxyl Radical-Mediated Neuropathy and Are Rescued by Restoring GHKL ATPase

- Ana Paula Segantine Dornellas, The Jackson Laboratory
  A humanized Atl1 mouse model for Spastic Paraplegia 3A exhibits accelerated clinical phenotypes in a Reep1 KO background.

- Matthew Simon, The Jackson Laboratory
  A PURA Knock-out mouse model for PURA syndrome exhibits fatal seizures

- Andrea Boitnott, BS, UT Southwestern Medical Center
  Gene Therapy Development for DDX3X Syndrome

- Sangeetha Hareendran, PhD, Columbia University
  AAV Gene Therapy for a Newly Identified Inherited Disorder of Copper Transport

- Gongbo Guo, Nationwide Children's Hospital
  Dissecting Molecular Mechanisms Underlying Blood-Brain Barrier Deficits in Tuberous Sclerosis Complex

- Andrew Steinsapir, BS, Deerfield Management
  Redefining TSC2 Gene Therapy: Development of a Novel Split Intein Payload and a Translational Potency Assay

- Ruby Goldstein de Salazar, UCLA

Design of Synthetic Promoters for AAV-Driven Treatment of Limb-Girdle Muscle Dystrophy Type R1

- Thierry Vandendriessche, PhD, Free University of Brussels (VUB)
  Novel Muscle-Targeted Gene Therapy Vectors with Increased Potency based on Combinatorial Assembly of Evolutionary Conserved Human Transcriptional Cis-Regulatory Elements (CRE) Identified by Comprehensive Transcriptome-Wide Data-Mining

- Daniel Maxenberger, University of Arkansas, Fayetteville
  RNA-guided Transcriptional Upregulation of Neuromuscular Disease Modifiers Using CRISPR-DREAM

- Joel Chamberlain, PhD, University of Washington School of Medicine
  RNA Polymerase II Transcription of RNAi Sequences for Myotonic Dystrophy Gene Therapy

- Warisha Faiz, UMass Chan Medical School
  Developing Suppressor-tRNAs to Treat Duchenne Muscular Dystrophy

- Hidenori Moriyama, University of Alberta
  Long-term Follow-up and Evaluation of Multi-Exon Skipping by CRISPR/Cas9-based Genome Editing in a Canine Model of Duchenne Muscular Dystrophy

- Cécile Fortuny, Scribe Therapeutics
  Advancing the Novel CasX-Editor for Precise Genome Editing to Address Muscular Dystrophies

- Shilpi Agrawal, Post doctoral fellow, University of Arkansas
  Development of Self-Delivering Gene Editors for Enhanced Non-Viral Delivery in Duchenne Muscular Dystrophy

- Hui Yang, HuidaGene Therapeutics Co., Ltd., Shanghai, China; Shanghai Institute of Meteria Medica, Chinese Academy of Sciences, Shanghai, China
  MM201-Mediated Gene Activation Therapy Enhances Utrophin Expression and Improves Muscle Function in Duchenne Muscular Dystrophy

- Jason Tucker, PhD, Atrium Health
  Design and Evaluation of FKRP Dimer Expression for Restoration of Functional Matriglycan in a LGMD 2I/R9 Mouse Model

- Claudia Huichalaf, Alexion Pharmaceuticals
  In vivo overexpression of muscleblind-like 1 leads to cardiac toxicity in mice

- Nuoying Ma, University of Washington
  Editing of Satellite Cells and Postmitotic Muscle for Continuous Correction of Dystrophin Expression in Duchenne Muscular Dystrophy

- Edo Kon, RiboX Therapeutics
  Novel Circular RNA Scaffolds Enable Efficient Circularization and Expression of Full-Length Dystrophin as an Advanced Therapeutic Approach for Duchenne Muscular Dystrophy

- Joseph Beljan, Nationwide Children's Hospital
  AAV-Delivered U7snRNA Restores Full-Length Dystrophin in Patient Cell Lines with DMD Intronic Pseudoexon Mutations

- Chen Zhang, Indiana University
  Optimized triple-vector gene therapy system enhances full-length dystrophin restoration in DMD mice

- Gretchen Thomsen, PhD, Insmed, Inc.

Administration of AAV9-Midlength-Dystrophin Gene Therapy Demonstrates Functional Efficacy in a Mouse Model of DMD

- Bryan Hu, University of Missouri
  Evaluate DWORF Gene Therapy in the Diaphragm of Dystrophin-deficient mdx Mice

- Liubov Gushchina, PhD, The Ohio State University and Nationwide Children's Hospital RI
  Characterization of New Dystrophic Mouse Models of Duchenne Muscular Dystrophy as a Tool for Therapeutic Development

- Benjamin Heithoff, REGENXBIO Inc.
  AAV-expressed Microdystrophin Containing Extended C-terminus Improves Muscle Function and Protects Against Injury in a Mouse Model of Duchenne Muscular Dystrophy

- Gretchen Thomsen, PhD, Insmed, Inc.
  CSF Delivery of INS1201 AAV9-Micro-Dystrophin Demonstrates Long-Term Durability of Efficacy in a Mouse Model of DMD

- Arnaud Valent, GENETHON
  Comprehensive analysis of longitudinal SV95C measurements, an e-digital mobility assessment in a real-life Duchenne population in the GNT-014-MDYF natural history study

- Hui Lin, Nationwide Children's Hospital
  Patient-Specific 3D Myofiber Cultures as Model for Therapeutic Development

- Anthony Blaeser, PhD, Atrium Health
  Targeting of ribitol metabolic pathway using AAV to enhance glycosylation and

therapeutic efficacy of metabolites in the FKRP-P448L mutant mouse.

- Hiromi Hayashita-Kinoh, PhD, The University of Tokyo
  Development of dosing protocol to reduce the required dose of rAAV using adult stem cells

- Marco Carpenter, BS PhD, Childrens Hospital of Philadelphia
  Base Editing Screen For Endogenous Activation of Zeta Globin Expression As A Treatment For Alpha Thalassemia Major

- Jowan Al-Nusair, Marshall University
  Identification of Genetic Variants Associated with T-Cell Lymphoma: A Case-Control SNP Analysis

- Dror Assa, PhD, Innovative Genomics Institute
  Gene Editing Primary T Cells to Enable CRISPR-On-Demand Therapy in an "Every Patient Brings Their Own Mutation" Setting: the Use Case Scenario of Hemophagocytic Lymphohistiocytosis

- Immacolata Porreca, Revvity
  A base editing platform optimised for efficient editing retains stemness and differentiation potential of hematopoietic stem cells

- Jean-Sébastien DIANA, APHP
  A new model using humanized mice to explore the fate of HIV DNA in secondary lymphoid and hematopoietic organs during the early phase of acute infection

- Saurabh Kumar, Versiti Blood Research Institute Wisconsin
  Induction of Immune Tolerance in a Food Allergy Model Utilizing Platelet-Targeted Therapy

- Estelle Berreur, Hoffmann-La Roche
  An iPSC-derived 3D liver model for safety assessment of AAV-based gene therapies

- RANRAN ZHANG, Catalent Cell & Gene Therapy
  Generation of Functional Liver Organoids Using CD34+ Cord Blood-Derived GMP Friendly iPSCs

- Sophie Tran, Institut de La Vision
  An Optimized Protocol to Transduce Photoreceptors in Human Retinal Organoids Using AAV

- Katharina Meijboom, PhD, UMASS Medical School
  Advanced Base and Prime Editing Strategies to Correct Common ALS-causing SOD1 Mutations

- Zili Qiu, MIT McGovern Institute for Brain Research
  Restoration of MeCP2 Function by converting the R270X Nonsense STOP Mutation to Tryptophan Through A-to-I RNA Editing

- Caryl Young, The Jackson Laboratory
  Development of a Humanized Mouse Model for PACS1 Syndrome and Evaluation of Base Editing as a Therapeutic Approach

- Ricardo Weinlich, Hospital Israelita Albert Einstein
  Base Editing Strategies for Restoring Functional Collagen VII Expression in Recessive Dystrophic Epidermolysis Bullosa

- Mark Osborn, PhD, University of Minnesota
  Prime Edited T-regulatory Cells for Immune Therapy

- Xuntao Sheena Zhou, UMass Chan Medical School
  Paired Prime Editors to excise GAA repeats in the Friedreich's Ataxia gene

- Daniel Brogan, Arbor Biotechnologies
  A compact RT editor built from the metagenome

- Hui Yang, HuidaGene Therapeutics Co., Ltd., Shanghai, China; Shanghai Institute of Meteria Medica, Chinese Academy of Sciences, Shanghai, China
  Cas13-Mediated RNA Base Editing Using mxABE Restores Heart Function in a Humanized Hypertrophic Cardiomyopathy Model

- Colin Robertson, PhD, University of Maryland School of Medicine
  A prime editing approach to rapidly model rare neurological disease variants in wild type animals

- Michael Kuckyr, Children's Hospital of Philadelphia
  Base Editing Premature Stop Codons Within the ATXN2 Repeat Reduces Pathogenic Polyglutamine Proteins.

- Yousif Almehza, Kennedy Krieger Institute
  Regulation of the ABCD2 uORFs as a Therapeutic Approach to Adrenoleukodystrophy

- Immacolata Porreca, Revvity
  Design and Assembly of Modular Pin-point™ Base Editors for Precise Correction of Pathogenic SNVs

- Tobias Merkle, AIRNA
  Optimized RESTORE+ oligonucleotides for an efficacious and safe RNA base editing treatment for Alpha-1 antitrypsin deficiency

- Russell Butterfield, University of Utah
  Correction of Multiple Dominant-Negative Mutations in Collagen VI-Related Muscular Dystrophy via ADAR-Mediated RNA Editing

- Hui Yang, HuidaGene Therapeutics Co., Ltd., Shanghai, China; Shanghai Institute of Meteria Medica, Chinese Academy of Sciences, Shanghai, China
  MM201-miniVPR: A Novel Single AAV-Delivered Multiplexed Gene Activation System for Therapeutic and Research Applications

- Yomira Palacios, UC Davis Health
  A Miniature CRISPR Editor for Epigenetic Rescue in Human Models of ZC4H2-associated rare disorders

- Dan Hart, PhD, Epic Bio
  Compact DNA Demethylase-Activator combination Modulators for CRISPR-Mediated Epigenetic Gene Activation

- Thyago Calvo, UC Berkeley
  CRISPR Epigenetic Reprogramming of Neuroprotective Factors as Therapy for Alzheimer's Disease

- Rachel Conover, Duke University
  Epigenetic CRISPR Editors Encoded as mRNA for Engineering Primary Human T Cells

- Da Xu, University of California, Berkeley
  Programmable Epigenome Editing by Transient Delivery of CRISPR Epigenome Editor Ribonucleoproteins

- Alison VanSchoiack, Shape Therapeutics, Inc.

Programmable RNA Editing Enables Precise Protein Modulation in the Central Nervous System

- Samuel Reisman, Duke University
  Direct Reprogramming of Primary Human Astrocytes to Functional Neurons with CRISPR-Based Epigenome Editing

- Jan Mathony, Heidelberg University
  In silico prediction of domain insertion sites for the engineering of inducible CRISPR gene editors

- Tsukasa Ohmori, MD, PhD, Jichi Medical University School of Medicine
  Phenotypic Correction of Ornithine Transcarbamylase Deficiency by Knock-in Genome Editing with Single Adeno-associated Virus Vector in Mice

- Pietro Rigoni, University of Padova
  A novel and empowered TARGETED Gene Addition Approach at a Relevant Microglia Locus for the Treatment of X-linked Adrenoleukodystrophy – Mechanistic Insights

- Marine Laurent, UMRS_951 Genethon
  Optimizing gene editing for erythroid-specific therapeutic protein expression using HSPCs

- Jeong Pil Han, Seoul National University
  In vivo therapeutic gene knock-in for hemophilia A and B using LNP and AAV

- Aurelien Colamartino, UCLA
  Autologous MPO Knock-out HematopoieticStem and Progenitor Cells for Pulmonary Arterial Hypertension

- ByoungYong Yoo, Rice University
  Highly Efficient Gene-editing with SaCas9 and AsCas12a in Patient Hematopoietic Stem and Progenitor Cells for Treating Sickle Cell Disease

- Benjamin Diner, Editas Medicine
  In Vivo CRISPR Editing of Genetic Regulatory Regions Results in Functional Upregulation of Target Protein and Meaningful Reduction of Disease-Associated Biomarker in Mice

- Ursula Andreo, Script Bioscioences
  Targeting MSH3 for Triplet Repeat Diseases Treatment using CasPlus Ursula Andreo, Menggui Huang*, Lozen Robinson, Ryan Gaudlip, Ryshone Duncan, Sapanna Chantarawong, Rebecca Haley and Ravi Iyer. Script Biosciences, Inc., 2929 Arch Street, Philadelphia PA 19104 *These authors contributed equally to this work.*

- Meghdad Rahdar, Caribou Biosciences
  Cas12a CRISPR Hybrid RNA-DNA (chRDNA)-Mediated In Vivo Genome-Editing Technology Elicits Functional Hepatic Gene Disruption with Long-term Durability

- Monica Gil-Garzon, University College London
  Generation of a de novo intronic junction in the DMD gene through CRISPR/Cas genome editing as a potential therapy for a high proportion of Duchenne muscular dystrophy patients.

- Bryan Hu, University of Missouri
  Genome Editing with Fast Degrading Cas9 Restores Dystrophin Expression in mdx Mice

- Stefan Nicolau, MD, Nationwide Children's Hospital
  Sequencing analysis of in vivo editing of the DMD gene using homology-independent targeted integration

- Adam Mischler, Precision BioSciences
  ARCUS-Mediated Gene Editing Excision of Exons 45-55 of the Human Dystrophin Gene using PBGENE-DMD Leads to Functional Dystrophin Protein and Durable Restoration of Skeletal Muscle-Function In Vivo for the Treatment of Duchenne Muscular Dystrophy

- Benjamin Persily, Children's Hospital of Philadelphia
  Targeted Integration of Large DNA Cassettes in the B2M locus of induced Pluripotent Stem Cells

- Howard Wu, Full Circles Therapeutics
  Enhanced human NK cell function by CLICK, an efficient non-viral CAR integration on CISH locus with a mini-circular single stranded DNA

- Ususa Too-Chiobi, Baylor College of Medicine
  Developing a Gene Insertion Strategy for Homozygous Familial Hypercholesterolemia

- Amanda Haupt, Revvity
  Precise and Efficient Immune Cell Engineering via Pin-pointTM Base Editing Platform: Insights from TotalSeq Single-Cell Multiomic Analysis

- Allie Crawley, Life Edit Therapeutics
  Signature of base editor RNA edits in the transcriptome and reduction of RNA editing

- Alexander Kuzin, BioReliance Corporation (MilliporeSigma/Merk KGaA)
  Improving Precision and Specificity of CRISPR Gene Editing with RGEN-seq and RGEN-mod

- Ryo Takeuchi, Excision BioTherapeutics
  Preclinical Safety Assessment of Anti-Viral Gene Editing in Chronic Hepatitis B Models

- Simon Reed, Broken String Biosciences Ltd
  INDUCE-seq®: Ensuring the Safe Development of Cell and Gene Therapies by Gene Editing

- Gavin Kurgan, Integrated DNA Technologies Inc
  Evolving Solutions for Effective Genotoxicity Assessment in CRISPR-Cas Gene Editing

- Mayuri Prasad, Labcorp Drug Development, Madison, WI
  Evaluating Optimal Volume and Dose of AAV9-mCherry in Stereotaxic targeting of Putamen in C57BL/6 mice

- Avencia Sanchez-Mejias, Integra Therapeutics
  Precise Gene Writing System for CAR-T cell therapy generation.

- Francesco Starinieri, Integra Therapeutics
  Development of viral-free FiCAT gene writing platform for liver-directed in vivo application

- Luca Brenker, University of Heidelberg
  A Versatile Anti-CRISPR Platform for Opto- and Chemogenetic Control of CRISPR-Cas9 and Cas12a across a Wide Range of Orthologs

- Laima Trinkūnienė, Vilnius University
  Engineering miniature SpaCas12f genome editing potential

- Giedrius Gasiunas, Caszyme
  Engineering a potent CRISPR-Cas12l genome editor with C-rich PAM recognition

- Noa Wertheimer, PhD, Emendo Biotherapeutics
  A Panel of Novel Engineered Nucleases Unlocks the Full Potential of Genome Editing

- Limin Wang, Cytiva
  Systematic Analysis of Oligonucleotide Approvals in the EU and US: Identifying the reasons for Non-Approval Decisions

- David Bunka, PhD, Aptamer Group
  Targeted Delivery of siRNA to Activated Hepatic Stellate Cells Using Aptamers: A Novel Approach for Fibrotic Liver Treatment

- Ashlee Colbert, Battelle Healthcare Products
  Multi-Targeted Organ Specific MicroRNA Intervention for Cancer (MOSAIC)

- Hongyu Wang, PhD, UTHealth Houston
  Aptamer-Based Discovery of Annexin A2 as a Novel Plasma Biomarker for Early Detection of Hepatocellular Carcinoma

- Bartlomiej Przychodzen, Ph.D., Vanda Pharmaceuticals Inc
  Selective ASO-based JAK2 Inhibitor for the Treatment of Polycythemia Vera

- Sebastian Arbelaez, University of South Florida
  Targeting Local Inflammation in Human Coronary Arteries Ex Vivo

- Yuhei Onizuka, NOF Corporation
  COATSOME® SS Series: Highly Versatile Ionizable Lipid as Vital Component of

Lipid Nanoparticle for Gene Vaccine Application

- Kristopher Lukas, University of Iowa
  Translational Potency of Double Stranded and Single Stranded mRNA In Vitro

- Gui Zhao, A*STAR - Agency for Science, Technology and Research
  Mannose-functionalized PEG-free LNPs targeting antigen-presenting cells for effective RNA delivery

- Yue Zhang, A*STAR - Agency for Science, Technology and Research
  Oligo(EG)-functionalized biodegradable polycarbonate-lipids for efficient mRNA delivery with alleviated anti-PEG immunity

- Patrick Thiaville, 4basebio
  Synthetic DNA for IVT mRNA production and delivery with Hermes™ lipopolyplex nanoparticles

- Natalie Orr, Cytiva
  Optimized prime: a transformative LNP composition for scalable RNA vaccines

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

- Balqees Khader, Wake Forest University School of Medicine
  High-Throughput Screening of Lipid Nanoparticles for Efficient CRISPR RNA Delivery In Vitro and In Vivo

- Alex Lagadinos, PhD, Mana.bio

Learn-Design-Make-Generate: ML Platform for Developing Novel LNP Delivery Systems

- Elisa Pagliari, University of Milan
  LNP-mRNA-Based Therapeutics for Genetic Mitochondrial Hepato-Encephalopathies

- Pengbo Guo, CybernaX Bio
  Conformation-adaptive Lipids for mRNA-Based Gene Editor Delivery via Lipid Nanoparticles (LNPs) in Non-Human Primates: Achieving a Wide Therapeutic Window

- Julie Yeo, KKH
  Evaluation of lipid nanoparticles and polymersomes for gene editing in a mouse model.

- Hilda Au, Acuitas Therapeutics
  Delivery, Potency and Tolerability of Lipid Nanoparticle Formulations of mRNA in PXB-Mice

- Michael Jaskolka, PhD, Editas Medicine
  In Vivo Gene Editing and Disease-Associated Biomarker Reduction for Multiple Liver Targets in Non-human Primate Using AsCas12a Nuclease Delivered by LNP

- Dor Breier, Tel-Aviv University, Life Sciences Department
  CRISPR/Cas9 Genome Editing Using Lipid Nanoparticles for Hepatocellular Carcinoma Therapy

- Wonbeom Park, PhD, Sungkyunkwan University
  Apolipoprotein Fusion Enables Spontaneous Functionalization of mRNA Lipid Nanoparticles with Antibody for Targeted Cancer Therapy

- Pauline Urquia, Sartorius Polyplus
  Innovative Cationic Lipids Fuel Next-Gen LNPs for Targeted Oncology and Advanced Cell Therapies

- Milena Pavlickova, 4basebio
  Delivery of synthetic DNA using Lipopolyplex nanoparticles: a thermostable alternative to mRNA-LNPs

- Jeffrey Bartlett, PhD, Rampart Bioscience
  A Non-integrating, Redosable, Non-viral DNA Vector Platform for Highly Potent, Durable, and Non-inflammatory Gene Transfer

- Zoe Hermsen, Zymo Research Corp.
  Simplifying Multigene Vector Design and Construction for Streamlined Development of Complex Therapeutics

- Rui Tang, ProBio Inc USA
  Enhanced Yield and Structural Integrity in Plasmid Biomanufacturing Employing the PowerS-ITR E. coli Platform

- Cassandra Ng, Factor Bioscience Inc.
  Incorporation of 5-methoxyuridine Enables Efficient Targeted Transgene Insertion in iPSCs and T Cells

- Reza Nouri, Abigail Wexner Research Institute at Nationwide Children's Hospital
  TREX1 Knockout Does Not Improve Gene-Length Insertions Using Single-Stranded DNA Templates

- Lina Li, PhD, MD, CELiD Biotechnologies Inc.
  Development of a Novel Full-Length F8 CELiD DNA Vector (ceFVIII) for Hemophilia

A Gene Therapy

- Silvia Rizzato, DKFZ
  Unlocking Safe and Scalable Engineering: Non-Viral and Non-Integrating SMAR DNA Vectors with Improved Payload Capacity

- Eleni Rogers, Portal
  A Next Generation Immune and Stem Cell Engineering Platform to Create Multifunctional Cells, at Clinical Scale, Using a Silicon Membrane

- Geoffrey Berguig, Evercrisp Biosciences
  Computationally Designed Miniproteins Enable Precise And Potent In Vivo Delivery Of Gene Editors To Challenging Tissues

- Zach Lamantia, Vanderbilt University
  Engineering the CRISPR-Cas9 Ribonucleoprotein Complex for Carrier-Free Gene Editing

- Tomoya Terashima, Shiga University of Medical Science
  Microglia-specific miRNA gene therapy with homing peptides for amyotrophic lateral sclerosis in mice model

- Joseph Wawrzyniak, Thermo Fisher Scientific
  Non-Viral Cell Engineering and Gene Delivery Optimization from Discovery to Clinical Applications Across Broad Cell Types

- Beeke Tappe, German Cancer Research Center
  Enhancing CAR NK Cell Production - A Comparative Analysis of Non-Viral Gene Engineering Systems

- Alison Hirukawa, Dropgenie
  Making Personalized Possible: A Miniaturized, Automation Integrated Nonviral Delivery Platform for High-Throughput Arrayed CRISPR Screening in Rare Patient-Derived Cells

- Sandra Bezemer, Thermo Fisher Scientific
  Development of Novel CaptureSelect Affinity Tools for Extracellular Vesicle Purification and Detection; Enabling a New Class of Gene Therapy Products

- Nikolia Darzenta, Auburn University
  The anti-inflammatory properties of Mescenchymal Stromal Cell derived Extracellular Vesicles on feline neuroinflammation

- Megan Keiser, PhD, Ohio State University
  Engineering Extracellular Vesicles for Neuroprotective Gene Delivery to the Central Nervous System

- Tristan Scott, Ph.D., City of Hope
  Improving and targeting programmable RNAi enriched Extracellular Vesicle for biomedical applications.

- Minjeong Kwon, Korea University
  Engineered SIRPα-Enriched Extracellular Vesicles Enable Targeted Clearance of CD47+ Pathological Fibroblasts in Fibrotic Disease

- Mitsuo Watanabe, FUJIFILM Wako Pure Chemical Corporation
  Development of a Scalable and Reproducible Large-scale Purification Method for Biologically Active Extracellular Vesicles Using Tim4-Phasphatydilserine Affinity Interaction

- Vijetha Bhat, Lonza

Designing a Plug-and-Play System to Produce Engineered Extracellular Vesicles

- Yoon Kyoung Kim, SHIFTBIO INC.
  CD47-Targeted Extracellular Vesicles Enable Enhanced Endolysosomal Escape for Oligonucleotide Therapy Delivery

- Y Savanie Fernando, Auburn University
  Extracellular Vesicle Delivery of Oligonucleotide Antiviral Therapy Against Rabies Encephalitis

- Arun Raturi, Entos Pharmaceuticals
  Targeted Extrahepatic Gene Delivery and Editing with Non-Viral Fusogenix Proteolipid Vehicles

- Ranganatha Somasagara, Ph D, Thermo Fisher Scientific
  Streamlined Cell Therapy Manufacturing: Highlighting Workflow Flexibility and Functionality with Advanced Processing and Fill Finish Instrumentation

- Henry Ordutowski, Axion BioSystems
  Evaluating CAR T-Cell Potency in 2D and 3D Models Using Fluorescent Live-Cell Imaging

- Rabinarayan Mishra, Absorption Systems, a Pharmaron Company
  Assessing In Vitro Cytotoxicity of CAR-T Cells Against Cancer Cells Using Three Assay Platforms

- Danielle Califano, Axion BioSystems
  Real-time, Label-free Assessment of HER2-targeted Antibody-drug Conjugate Therapies

- Danielle Califano, Axion BioSystems
  Development of a Faster Potency Assay for Evaluation of Immune Cell-mediated Cytotoxicity In Vitro

- Chris Lovejoy, Siren Biotechnology
  Identification of Pharmacologically Relevant Species for IND-Enabling Toxicology Studies by Deep Comparative Transcriptomic Profiling of PBMCs Treated With Recombinant Human Payloads

- William Hallows, Rampart Biosciences
  A Novel Non-Viral DNA Delivery Platform for Durable and Potent Expression of Bispecific T-cell Engagers

- Julie Tomolonis, Nationwide Children's Hospital
  Fibroblast Activation Protein (FAP) Directed Bispecific Antibodies Target Pediatric Sarcomas

- Simonne Guenette, University of Washington
  Dual Costimulatory Receptors enhance antitumor immunity through cell -extrinsic and -intrinsic signals

- Anna Hartley, German Cancer Research Centre
  Persistent Genetic Modification of NK Cells Using Non-Integrating SMAR DNA Vectors for Cancer Immunotherapy

- Situo Zheng, Kyushu University
  NK-like Cells (GAIA-102) Induce Antitumor Adaptive Immunity via the Host NK derived IFN-γ

- Adam Schieferecke, PhD, University of California, Berkeley
  CRISPR-Guided Evolution of Oncolytic Virotherapies for Improved Delivery

- Hyun Jang, Ph.D., Libentech Co.LTD.
  Molecular modification of viral surface protein enhances targeting ability of oncolytic virus

- Eric Ehrke-Schulz, Dr. rer. nat., Witten/Herdecke University
  Using primary cell culture models to study adenoviral vectors for virotherapy of head and neck squamous cell carcinoma

- Annie Yang, City of Hope Inc
  CF33-hNIS Alone and Combined with Anti-PD-L1 Eliminates Gastric Cancer Peritoneal Metastasis in an Immunocompetent Mouse Model

- Zhifang Zhang, City of Hope National Medical Center
  Intraperitoneal Delivery of Chimeric Orthopoxvirus CF17 Improves Survival in a Syngeneic Mouse Model of Gastric Cancer Peritoneal Metastasis.

- Kyla Boyd, Mayo Clinic Graduate School of Biomedical Sciences
  Arming an RNA-Based Oncolytic Picornavirus Platform Using LNPs for Replicon Co-Delivery

- Christopher LaRocca, MD, University of Minnesota
  In Vivo Efficacy of a NIS-expressing Oncolytic Adenovirus for Colorectal Cancer Treatment

- Mizuho Sato-Dahlman, PhD, University of Minnesota
  Intraperitoneal Administration of The CD133-targeted Oncolytic Adenovirus Suppresses Peritoneal Metastases of Colon Cancer in Mouse Xenograft Model.

- Alex Lagadinos, PhD, Mana.bio
  ML-Driven Design of Lipid Nanoparticles for In-Vivo T-Cell Delivery

- Alice COILLARD, PhD, Alaya.bio
  A NOVEL POLYMERIC GENE DELIVERY PLATFORM TO PRECISELY TARGET AND REPROGRAM CELLS IN VIVO FOR HAEMATOLOGICAL MALIGNANCIES & BEYOND

- Chris Lovejoy, Siren Biotechnology
  Vectorized Interferons Provide a Robust and Sustained Transcriptional Response Over Traditional Non-Vectorized Recombinant Cytokine Therapies in Human High-Grade Glioma Cells

- Céline Jaudoin, Alaya.bio
  LENTIVIRAL VECTOR ENGINEERING WITH POLYMER SURFACE MODIFICATION FOR CAR-T CELL THERAPY

- Chih Hong Lou, Chief Technology Officer, Carrigent, Inc.
  Introduction of Novel in vivo Cell Specific Payload Delivery Platform - CARGO

- Abigail Becker, M.S., Thermo Fisher Scientific
  Generation of Human iPSC-Derived Hematopoietic Progenitor Cells and Natural Killer Cells with Anti-Tumor Functionality Using a Feeder-Free Differentiation Platform

- James Eshleman, Johns Hopkins University School of Medicine
  CRISPR-Cas9-induced Double Strand Breaks Cause Chromosome Catastrophe and Eliminate Pancreatic Cancers

- Michael Launspach, Charité - Universitaetsmedizin Berlin
  Personalized CRISPR/Cas9 Cytokine Gene Therapy to Remodel Solid Tumor Microenvironment and Enhance Adoptive T Cell Therapy

- Kevin Carbajal, Inceptor Bio

OUTWIT: An In Vivo Approach to Engage Innate Immune Cells with Clinically Approved T Cell Engagers

- Marco Cortese, post doc, Candiolo Cancer Institute
  Preclinical efficacy of a HER2 synNotch/CEA-CAR combinatorial immunotherapy against colorectal cancer with HER2 amplification

- Megumi Wada, A-SEEDS Co., Ltd.
  piggyBac transposon-mediated EPH family protein-specific CAR-T cells engineered through scalable automated manufacturing with optimized T cell metabolic fitness and sustained stemness

- Xiaowen He, Oricell Therapeutics Co.,Ltd.
  Induced Pluripotent Stem Cells (iPSCs) Provide an Unlimited, Effective Claudin18.2-targed CAR-NKT cell for Allogeneic and Off-the-Shelf Immunotherapy and gastric and pancreatic cancer

- Yanping Ding, Beijing Imunopharm Technology Co. Ltd.
  Preclinical Study of Combination therapy of GUCY2C CAR-T with Immune Checkpoint Inhibitors for Metastatic Colorectal Cancer

- Xin-an Lu, Beijing Imunopharm Technology
  Glypican-3-Specific CAR-T Cells Co-expressing CD40L Exhibit Antitumor Activity Against Osteosarcoma

- Jihwan Lee, Imagen Bioworks
  Identifying genetic determinants of solid tumor infiltration of T cells using a microscopy-based cell isolation approach.

- Bryan Zimdahl, Eureka Therapeutics
  Expanding the Potential of T-Cell Therapies with the ARTEMIS Cell Receptor

Platform

- Sveta Jagannathan, George Washington University
  Antigen-Specific T cells Generated Using Photothermal Nanoparticles Demonstrate Enhanced Function Compared to Alternative Tumor Antigen Release Methods

- Xiaotong Song, PhD, Texas A&M University
  Metabolic Reprogramming Enhances HER2-CAR T Cell Therapy for HER2-ADC-resistant tumors

- Spas Markov, Fate Therapeutics, Inc.
  FT836, a Novel MICA/B-targeting CAR T-cell Therapy Engineered to Eliminate the Need for Conditioning Chemotherapy with Broad Activity Across Solid Tumor Indications

- Jingli Zhang, A2 Biotherapeutics
  Onboard, Membrane-tethered IL-12 Boosts Potency and Maintains Selectivity of a Tmod NOT Gate

- Weidong Feng, ArsenalBio
  Enhancing CAR-T Cell Therapy Efficacy Against Solid Tumors Using a Synthetic Pathway Activator (SPA)

- Henry Ordutowski, Axion BioSystems
  Label-free, High-throughput Functional Assays for Evaluating the Potency and Safety of Cell Therapies

- Yannick Schreiber, Northwestern University
  High Output, Low-Background Hypoxia Responsive Synthetic Gene Circuits for Improving the Safety and Potency of Solid Tumor CAR T Cell Therapy

- Taha Bartu Hayal, NHLBI
  In Vivo Persistence and Clonal Dynamics of Genetically Barcoded IL-15-Armored vs. Unarmored Anti-CD20 CAR-NK Cells in an Autologous Rhesus Macaque Model

- Cody Hall, Mayo Clinic
  Understanding the role of NEFM and UACA genes in CART cell effector function and dysfunction

- Rafijul Bari, Celularity
  Gamma Retroviral Vectors Pseudotyped with Either Baboon or RD114 Envelope Achieved High Transduction Efficiency and Improved Cytotoxicity of CAR-CYNK Cells

- Malte Thomas, German Cancer Research Center Heidelberg
  Methotrexate-Based Selection of S/MAR DNA Nanovectors Enhances Cytotoxic Activity of CAR-Modified Blood Cells

- Abdu Mohammed, Trinity Health System
  Cytokine Release Syndrome and Cardiovascular Outcomes: A Population-Based Comparison Between Patients Infected With Severe Acute Respiratory Syndrome Coronavirus 2 and Those Who Received Chimeric Antigen Receptor T-Cell Therapy

- Adamsegd Gebremedhen, Marshall University School of Medicine
  Exploring the Impact of Chronic Kidney Disease on In-Hospital Outcomes in Patients Receiving Chimeric Antigen Receptor T-Cell Therapy: Insights From a United States Population-Based Study

- Lucy Maynard, Fred Hutch
  Pooled CAR-T screening in nonhuman primates identifies architectures with enhanced proliferation, trafficking, and persistence

- Zhihan Chen, Huazhong University of Science and Technology
  Long-term Dynamics and Integration Site Analysis of CAR-T Cell Therapy in Relapsed/Refractory Lymphoma and Multiple Myeloma: A Retrospective Study

- Gregory Sowd, Caring Cross
  Development of Humanized VHH-Based Bi-paratopic BCMA CAR-Ts for Multiple Myeloma Treatment via a Streamlined High-Throughput CAR^2-pool Screening Platform

- Xiaowen He, Oricell Therapeutics Co.,Ltd.
  Development of a Universal, Off-the-Shelf CAR-T Cell Immunotherapy for Multiple Myeloma

- Kun Yun, Mayo Clinic
  BCMA CAR engineering arms T cells with superior functions post-ex vivo stimulation by MUC1 peptide-loaded autologous PBMCs

- Brandon Henry, IQVIA
  Pre-Existing Immunity to Adeno-Associated Virus: A Global Meta-Analysis of Anti-AAV Neutralizing Antibodies in 9,887 Patients

- Lena Winstedt, PhD, Hansa Biopharma AB
  Depletion of AAV8 Antibodies in Seropositive Individuals Treated with the IgG Cleaving Enzyme Imlifidase

- Svetlana Atasheva, PhD, Emory University
  Modulation of Adenovirus Vector Immunogenicity by Non-neutralizing Antibodies and Complement C3 Deposition

- Robert Clark, Tulane University Health Science Center
  Immunobiology of Proteasome-Targeted Strategies to Prevent Anti-AAV T-Cell Responses

- Tingxi Guo, GentiBio Inc
  A Novel NK Inhibitor Provides “Best-in-Class" Protection of HLA Knockout Allogeneic Human Engineered Regulatory T Cells to Effectively Evade Immune Rejection

- Davide D'Onghia, BIDMC Harvard Medical School
  Epigenetic targeting of macrophage polarization

- Merve Gültan, Technical University of Munich
  Targeting immune checkpoint receptor ligands to reactivate T-cell function and eliminate persistent hepatitis B virus infection

- Julia Provino, Regeneron Pharmaceuticals
  The Intracellular Fc Receptor TRIM21 Promotes Type I Interferon Responses to Antibody-complexed AAV

- Karthik Karuppusamy, PhD, University of Washington
  In vivo Engineering of HSPCs for HIV Gene Therapy using Helper-Dependent Adenoviral Vectors expressing HIV Fusion Inhibitors

- Chang Li, PhD, University of Washington
  Toward a Prophylaxis of SIV Infection in Rhesus Macaques by in vivo HSC Transduction with HDAd-S-eCD4Ig

- Darya Palianina, University Hospital Basel
  Advancing Precision Immunotherapy for EBV-Associated Malignancies Through HLA-Driven TCR Engineering

- Dominique Sims, Yale University School of Medicine
  STING activation, DNA clearance, and autoimmune disease: Investigating pathogenic mechanisms with DNAse dysfunction and novel therapeutics in SLE and AGS models

- Kate Henry, Biogen
  Selective Inhibition of Human Plasmacytoid Dendritic Cells Reduces TLR9-mediated Type I IFN to CpG DNA in AAV Gene Therapy Vectors

- Peter Cook, PhD, Seattle Childrens Research Institute
  Targeting human plasma cells using small molecule regulated BCMA CAR T cells eliminates circulating antibodies in humanized mice

- Mark Jelcic, Fate Therapeutics, Inc.
  Next-Generation Off-the-Shelf CAR T-Cell Therapies for Conditioning-Free Treatment of a Broad Spectrum of Autoimmune Diseases and Hematologic Malignancies

- Biao Zheng, Ltd
  Allogeneic CD19-targeted CAR-T Therapy for refractory Systemic Lupus Erythematosus (SLE)

- Juan Liang, GemPharmatech Co., Ltd.
  Repurposing oncology drugs to treat autoimmune disease

- Jason Stadanlick, PhD, Cabaletta Bio
  RESET-SLE^{TM}: Clinical Trial Evaluating Rese-cel (Resecabtagene Autoleucel), A Fully Human, Autologous 4-1BB Anti-CD19 CAR T Cell Therapy in Non-Renal SLE and Lupus Nephritis: Correlative Findings

- Zachary Vorndran, Cabaletta Bio Inc
  RESET-SSc^{TM}: Clinical Trial Evaluating Rese-cel (Resecabtagene Autoleucel), A Fully Human, Autologous 4-1BB Anti-CD19 CAR T Cell Therapy in Systemic Sclerosis: Correlative Findings

- Taylor Oeschger, Fred Hutchinson Cancer Center
  Modeling Innate Immune Cell Loss-of-Function in Nonhuman Primates with Anti-CD16 and Anti-NKG2A CAR T Cells

- Nélio Oliveira, PhD, The Jackson Laboratory
  A model to generate tolerogenic DCs derived from human iPSCs with CD86 biallelic deletion

- Claire Aibel, Factor Bioscience
  Neural Induction of Gene-Edited mRNA-Reprogrammed iPSCs Yields Highly Proliferative Neural Stem and Progenitor Cells That Display Persistent Transgene Expression

- Assen Roguev, GeneFab
  A Platform for Designing Neuron, Astrocyte and Hepatocyte Specific Promoters for Gene Therapy

- Jessica K Schenck, University of California, San Diego
  Investigation of the Long-Term Effects and Post-Disease Onset Impact of Wild-Type Hematopoietic Stem Cell Transplantation in a Mouse Model of Alzheimer's Disease

- Pasqualina Colella, PhD, Stanford University
  Hematopoietic Stem Cell Transplantation with Enhanced Brain Conditioning Corrects Progranulin Deficiency in mouse models

- Mayra Carrillo, UCLA
  Lentivirus-based Hematopoietic Stem/Progenitor Cell Therapy Provides Safe and Long-term Treatment in Hypophosphatasia Mouse Model

- Yuko Kasahara, PhD, The University of Tokyo, The Institute of Medical Science
  Amnion mesenchymal stromal cells have the potential to preserve muscle function in a mouse model of Duchenne muscular dystrophy

- Maria Pallarès-Masmitjà, Vall D'Hebron Research Institute
  Mesenchymal Cells as a Therapeutic Targets for ex-vivo Gene Writting in MDC1A

- Bayley Lindsay, Kennedy Krieger Institute
  Gene Supplementation via DARS2-AAV9 Rescues Phenotype in LBSL Patient Cells and in Vivo Mouse Model

- Nikitha Kota, Houston Methodist Research Institute
  Long-term Tolerance to Leydig Cell Transplantation with Localized Treg Immunomodulation Using the NanoLymph Platform

- Kyoko Tsuji-Yogo, Juntendo University
  CD80/CD86-CD28 Signal Blockade during the Mixed Lymphocyte Reaction Augments the Alloantigen-specific Inhibitory Function of Natural Regulatory T Cells

- Serap Gur, Ankara University
  The Therapeutic Effect of Adipose-Derived Stem Cells and Hydrogen Sulfide Donor Sodium Hydrogen Sulfide Combination Treatment on Erectile Dysfunction in a Rat Model Following Radical Prostatectomy

- Lola Reid, UNC School of Medicine
  Grafting Strategies for Transplantation of Organoids into Solid Organs

- Lara Campana, Resolution Therapeutics
  Development of a mode of action-based testing suite for the screening of regenerative macrophage cell therapy candidates for end-stage liver disease and beyond

- Regeant Panday, Dimension Inx
  Development of 3D Printed PLG-Based Liver Tissue Therapeutic for the Treatment of Alcoholic Liver Disease and Liver Failure

- Denny Nguyen, Genefab
  Genome-Orthogonal Gene Circuitry: Improved Synthetic Enhancer-Transcription Factor Systems with Minimal Off-Target Effects

- Russell McConnell, Kelonia Therapeutics
  Toward Treatment with Gene-Modified B Cells Engineered In Vivo Using iGPS Particles

- Pin Wang, PhD, University of Southern California
  In Vivo CAR-T Therapy Powered by Novel CLAMP Technology for T cell-Targeted mRNA Delivery

- Gilles Divita, Aanastra Inc.
  In vivo mRNA CAR-T Cell Engineering Utilizing a Novel CD3/CD5 Peptide Based Targeted Nanoparticles.

- Xiaoqian Nie, Westlake University
  A "Plug-and-Play" Erythrocyte-Mediated mRNA Delivery System Enables In Vivo Generation of CAR-Myeloid Cells for Cancer Immunotherapy

- Chantal PICHON, PhD, INSERM

Bioproduction of messenger RNA therapeutics in yeast cells: a proof of concept study.

- Andrew Tsao, GenScript USA Inc.
  Overcoming Challenges in mRNA Plasmid Preparation: Advanced Solutions for Poly(A) Tail Integrity and High-Yield Production

- Jing Zhu, ReciBioPharm
  A new linear plasmid process platform enabling small scale GMP production with significantly short lead time, high titer, superb polyA stability dedicating to nuclei acid production

- Thera Mulvania, PhD, Expression Systems, An Advancion Company
  Case Study on an Insect Cell-Produced AAV6 Gene Therapy Vector for Neuromuscular Disease

- Michela Gentile, ReiThera
  Enhanced Thermostability of a Gorilla-derived Adenoviral (GRAd) vector via E4 region chimerism

- Suddhasil Mookherjee, PhD, Catalent Pharma Solutions
  Development of a universal ddPCR based lentivirus infectivity assay

- Hicham El Bariqi, Parexel
  How Far Away are we from Global Regulatory Convergence: Analyzing CAR-T Quality Control Standards Across Six Jurisdictions`

- Geoffrey Stephens, Aicella, Inc
  Revolutionizing CAR-T Cell Therapy Manufacturing with AI

- Claire Shao, Dana-Farber Cancer Institute
  Optimizing Stem Cell Collections Using a Hematopoietic Progenitor Cell Apheresis (HPC-A) Collection Calculator Application: Web-App Development and Validation

- Krishnendu Khan, West Pharmaceutical Services
  Cyclic Olefin Polymer-based Vial Systems for Cell and Gene therapy

- Dan Pham, University of Wisconsin - Madison
  Autofluorescence Imaging Reveals the Impact of Cryopreservation on T Cell Metabolism and Activation Response

- Björn Hiller, Catalent Duesseldorf GmbH
  Off-the-Shelf GMP-Compliant iPS Cells for Application in Cell Therapy

- Huiyu Wang, PhD, Applied Cells Inc.
  A rapid and cost-effective solution for CAR- T cell manufacturing

- Priyadarshini Chatterjee, Aurigene Discovery Technologies Ltd.
  Development of Low-Cost CAR-T Process Street with Improved Manufacturing Success

- Toshihiko Okazaki, MD PhD, OSAKA University Hospital
  Challenge to Develop a GMP-compliant Integrated Manufacturing Process for Novel CAR-T Cells Using a Closed and Single-use Automated Culture System at Academic Gene and Cell Processing Facility

- Ranganatha Somasagara, Ph D, Thermo Fisher Scientific
  Automating iPSC Processing and Engineering for Allogenic Cell Therapies: The Role of Closed Modular Systems.

- Dominik Lock, PhD, Miltenyi Biotec B.V. & Co. KG
  Automated Manufacturing of T cells for Therapy by LNP-Engineering

- Vassilis Paraskevas, Orchard Therapeutics
  Toward the Development of an Automated Manufacturing Process for Haematopoietic Stem Cell Gene Therapies

- Peter Szaraz, PhD, Lonza Personalized Medicine
  Lonza Cocoon® Platform Demonstrate Compatibility with Off-the-shelf Process Analytical Technology (PAT) and Electroporation Systems for Closed and Automated In-process CPP Monitoring and Genetic Modifications.

- Rukmini Ladi, Sartorius
  Perfusion-Powered Production of Over 100 CAR-T Doses in a 2-litre Stirred-Tank Bioreactor With Automation of Downstream Harvest and Concentration Steps

- Mina Ahmadi, Thermofisher Scientific
  Improving CAR T Cell Production: Comparing Stirred Tank Bioreactors and Static Vessels

- Max Dotson, Thermo Fisher Scientific
  Scalable and Controlled Expansion of Genetically Engineered CAR-T cells in Stirred-Tank Bioreactors

- Meredith Safford, Charles River - Hanover, MD
  Scaling CAR-T Manufacturing: Redefining Large Volume Fill-Finish with ARES X20+ Automation

- Sachiko Okamoto, PhD, Takara Bio Inc.
  The Simple CAR-T Cell Production: RetroNectin can Promote Simultaneous T-cell Activation & Transduction in G-Rex Bioreactor.

- Adam Janvier, EXmoor Pharma
  Platforming iPSCs: a modular approach for all differentiation modalities

- DAVID VINCENT, EXmoor Pharma Concepts Ltd
  Platforming iPSCs: a modular approach for all differentiation modalities.

- Ching-Ying Huang, BeiGene
  Revolutionizing Allogeneic Therapies: Unveiling GMP-Grade iPSC Clones for Scalable Functional CD8 αβT and γδT Production

- Mariana Argenziano, Ncardia
  Scalable and Consistent Manufacturing of Functional Lung Cells from iPSCs: Ncardia's Advanced Differentiation Protocols for Drug Discovery and Tissue Engineering

- Micah Witte, Forge Biologics
  Development of Highly Sensitive Liquid Chromatography Assays to Ensure Excipient Clearance in AAV Products

- Clifford Froelich, ThermoFisher Scientific
  Percent Fulls – What landscape is your data painting?

- Wilhelm Paulander, Symcel
  Accelerating Sterility Testing to < 3 Days: Rapid, Real-Time Phenotypic Detection of Microbial Contamination with calScreener+

- Dan Farley, Oxford Biomedica
  The 3 Rs of Replication-Competent Lentivirus (RCL) Formation Risk: Real, Rare or Artificial?

- Patrick Kellish, PhD, Kincell Bio
  Design of Experiment (DoE) Methodology Effectively Reduces Cost and Time to Completion of Complex Analytical Method Development

- Darrick Yu, Sanofi
  Streamlined Virus Titration Workflow Enables Advancement of Upstream Process Development for AAV Producer Cell Lines

- Pouria Motevalian, Thermo Fisher Scientific
  Lentivirus Infectious titer – Scaling up and scaling out on a budget

- Jorge Santiago-Ortiz, Apertura Gene Therapy
  Fit-for-Purpose Analytics for Novel Engineered Capsids: Advancing Precision in AAV Characterization

- Victor Hernandez, PhD, Axovia Therapeutics Inc
  Development of a ddPCR Assay to Quantify AXV-101 Levels for a Mouse Biodistribution Study

- Kyoungsik Moon, Korea Institute of Toxicology
  Validation of Real-Time qPCR Method for Vector DNA Quantitation from rAAV2-shmTOR in Rat and Monkey Tissues Samples

- Sabine Geiger, Ascend Advanced Therapies
  The Fab Four -- Validated GMP QC Assays to More Reliably Assess DNA Impurities for rAAV Batch Release

- Chengjing Liu, Thermo Fisher Scientific
  Consistent rAAV In-Process Monitoring with PEA-dPCR Technology

- Tran Pham, Phenomenex, Inc. - Torrance, CA
  Determination of sgRNA and mRNA Sequence Identity by Ion-Pair Reversed-Phase Chromatography Coupled With High-Resolution Tandem Mass Spectrometry

- Zach Mason, Capsida Biotherapeutics, Inc.
  Dual-Platform NGS for Comprehensive Characterization of Engineered rAAV Vector Integrity

- Brian Tomkowicz, PhD, SK Pharmteco - US
  Adapting a Replication Competent Lentivirus (rcLVV) Assay for Commercial Manufacturing

- Alex Hale, Charles River Laboratories
  Rapid Cell-Specificity and Integration Safety Profiling of AAVs

- Kaylyn Koenig, PhD, Altasciences Preclinical Seattle LLC
  Gene Therapy Studies and Germline Integration Assessment Considerations in Preclinical Research

- Hua-Jun He, NIST: National Institute of Standards and Technology
  Interlaboratory Assessment of Candidate Reference Materials for Lentiviral Vector Copy Number and Integration Site Measurements

- Alok Upadhyay, SK Pharmteco
  Development and Validation of a Replication Competent Virus Safety assay for Adenoviral products

- Sarah Tong, Solid Biosciences
  Automation of AAV Capsid ELISA on Tecan Fluent

- Martin März, PROGEN
  Comparative Analysis of Total Antibody Titer and Neutralizing Antibody Titer: Correlation and Implications for AAV-Based Gene Therapies

- Martin März, PROGEN
  AAV Serology ELISA for Testing Pre-Existing Immunity to AAV

- Joshua Baal, PROGEN Biotechnik GmbH
  Addressing Pre-Existing Immunity in AAV Gene Therapy: Automation of Serology ELISAs

- Shian-Jiun Shih, PhD, Cellentia, Inc.
  Screening for Pre-Existing Immunity Against AAV Vectors: A Multipronged Approach

- Jeffer Bhuko, Muhimbili University of Health and Allied Sciences
  Molecular characterization of acute lymphoblastic leukemia in Tanzania

- Jian Zhao, Shanghai Clinical Research and Trial Center
  Deploying Fourier-transform infrared spectral signatures and machine learning to standard cell identities and ages (SCIAGE)

- Christopher Gough, SK Pharmteco
  Application of Mass Photometry to Characterize Adeno-associated Virus Particle Content

- John Ketz, Andelyn Biosciences
  Use of Statistical Analyses for Evaluating Large Historical Datasets of AAV Productions to Assess Process Parameters – A Case Study.

- Nandhitha Uma Naresh, Mass General Brigham GCTI
  As new gene and cell therapies emerge from academia, we must RISE to the opportunity

- Carson Greco, Andelyn Biosciences
  PPQ Ready Risk-Based Identity Testing for Ancillary Raw Materials in Gene Therapy Manufacturing

- Steven Henry, Eurofins BioPharma Product Testing
  Minimizing Volume Requirements and Turnaround Time for Compendial Testing of Cell and Gene Therapy Products

- Michela Gentile, ReiThera
  Upstream Process intensification for a Gorilla derived Adenoviral (GRAd) vector by using a Perfusion System

- David Diaz-Vera, Genzyme, a Sanofi Company
  Dissecting the effect of shear during rAAV production using perfusion bioreactors in PCLs

- Tony Cijsouw, PhD, Roche
  Establishing a Scalable rAAV Production Platform for Research-Grade Vector Supply within a Pharmaceutical Setting for rAAV Gene Therapy Early Research and Development

- Michela Gentile, ReiThera
  ReiCell-AAV: A Fully Documented High-Yield Suspension Cell Line for Scalable AAV Vector Manufacturing

- Anastassia Tselikova, FUJIFILM Irvine Scientific

Demonstrating Key Processes of the T Cell Therapy Workflow in Chemically Defined Media

- Sarah Rains, KACTUS
  High-Performing GMP CRISPR Cas9 Nuclease for Cell and Gene Therapy

- Sravya Ennamuri, Sarepta Therapeutics, Inc.
  3-Year Functional Outcomes of Patients With Duchenne Muscular Dystrophy: Pooled Delandistrogene Moxeparvovec Clinical Trial Data vs External Controls

- Yoshitsugu Aoki, MD PhD, National Center of Neurology and Psychiatry
  Brogidirsen -The First Dual-Targeting Exon 44 Skipping Drug for Duchenne Muscular Dystrophy Safety, Tolerability, and High Dystrophin Restoration in Long-Term Clinical Studies

- Mark Stahl, MD, PhD, Insmed
  Design of the Phase 1 ASCEND Study to Investigate the Safety and Biodistribution of INS1201 Gene Therapy in Males With Duchenne Muscular Dystrophy

- Sravya Ennamuri, Sarepta Therapeutics, Inc.
  Assessment of Cardiac Outcomes in Delandistrogene Moxeparvovec Clinical Trials for Duchenne Muscular Dystrophy

- Xiaodong Wang, Beijing Genecradle Therapeutics Inc.
  Innovative Intrathecal AAV9-mediated Gene Therapy for Spinal Muscular Atrophy: Phase I/II Safety and Efficacy Topline Results

- Kathrin Meyer, PhD, Alcyone Therapeutics
  Advances in AAV9 Gene Therapy for CLN3 – Update on the Phase I/II Clinical Trial Indicating Lasting Efficacy at 5 Years Post Dosing and Next Steps in Program

Development

- Florian Eichler, MD, Massachusetts General Hospital
  Completed Phase 1/2 Trial of Dual-Vector rAAVrh8 Gene Therapy for GM2 Gangliosidosis Administered by Bilateral Intrathalamic, Cisterna Magna and Intrathecal Delivery

- Amanda Nagy, Mass General Brigham
  Urine NAA Results from the CVN-101 Natural History Study for Canavan Disease: Correlation with Phenotype

- Connor Lewis, National Human Genome Research Institute
  Volumetric Magnetic Resonance Imaging, Diffusion Tensor Imaging, and Differential Tractography Metrics Correlate with Clinical Outcomes Following Gene Therapy in GM1 Gangliosidosis Patients

- Simone Seiter, Partner, (unknown)
  What is the Formula for Commercial Success of a Gene Therapy? Reflections on Approved GTx Authors: Simone Seiter, Senior Partner, Simon-Kucher Christian Hinneburg, Senior Manager, Simon-Kucher Introduction: As of today, fewer than 20 gene therapies (GTx) have been approved in the US and Europe, yet only one has reached blockbuster status, with two more expected to follow. Despite their transformative potential, the majority of gene therapies fail to achieve widespread commercial success. One of the most decisive factors influencing a GTx's market trajectory is the selection of the right indication. This analysis seeks to identify the key characteristics of commercially successful indications and how they drive adoption, pricing potential, and long-term viability. Methods: A retrospective analysis of approved gene therapies was conducted, focusing on the indications selected by manufacturers. Key variables analyzed include disease severity, prevalence, life expectancy with standard of care (SoC), quality of life (QoL) impairment, the competitive landscape, and clinical development feasibility. Structured expert interviews with industry leaders and real-world case studies were incorporated to validate insights. The session will be presented by Simone Seiter (Senior Partner, Simon-Kucher), leveraging expertise in gene

therapy commercialization. Results: Preliminary findings reveal that the selection of the right indication is one of the most critical determinants of commercial success. The following five key criteria emerged as pivotal: Life Expectancy & Disease Severity: Gene therapies targeting life-threatening diseases with limited SoC options tend to achieve higher adoption, as the urgency for a curative treatment is greater. Quality of Life (QoL) Impairment: Indications with severe, progressive QoL decline create a stronger value proposition, making them more attractive for both payers and patients. Patient Population & Age: The age of the target population plays a critical role—pediatric indications may benefit from longer lifetime value, whereas adult indications often require more established treatment paradigms for adoption. Future Treatment Alternatives: The expected evolution of the competitive landscape is a key consideration, as some indications may see disruptive alternative therapies that limit the long-term market opportunity for a GTx. Technical & Clinical Feasibility: Indications with clear, clinically validated biomarkers and regulatory precedent for approval tend to have a smoother development and commercialization path. Conclusions: The success of a gene therapy is highly dependent on indication selection—more than any other commercial factor. An indication that aligns with high unmet medical need, strong patient and provider demand, and regulatory feasibility has a significantly greater chance of commercial success. This session will provide industry leaders with a structured framework for making strategic indication selection decisions, ensuring the long-term viability of their gene therapy assets.

- Carolyn Chapman, PhD, MS, The Multi-Regional Clinical Trials Center of Brigham and Women's Hospital and Harvard
  Characteristics of Long-Term Follow-Up Studies for Gene Therapies registered in ClinicalTrials.gov

- Piotr Trzonkowski, MD, PhD, Medical University of Gdansk
  SAFETY AND EFFICACY OF THE THERAPY WITH CD4+CD25HIGHCD127- T REGULATORY CELLS – 12 YEARS AFTER ADMINISTRATION

- Alex Zhang, OneTwenty Therapeutics
  Reviving Hearts, Restoring Lives: Long-term Clinical Outcomes of Allogeneic iPSC-derived Cardiomyocytes Transplantation for Advanced Heart Failure

Patients

- Alokesh Ghosal, Leidos Biomedical Research
  A Scalable Retroviral Vector Production platform for T-cell Therapy

- Andre Raposo, OXB
  Enhancing titres of therapeutic lentiviral vectors using PKC agonists

- Mariya Viskovska, Lonza
  Large-Scale, High Titer Lentiviral Production Using Suspension Transient Transfection Platform

- Olga Makovich, Catalent Cell & Gene Therapy
  Lentiviral Vector Production Process Intensification: High-Yield and Cost-Efficient Manufacturing Solutions

- Rodrigo Nogueira, IBET - Instituto de Biologia Experimental E Tecnológica
  Exploring the potential of different point-mutated HIV-1 protease variants for continuous lentiviral vector production

- OMID TAGHAVIAN, FUJIFILM Irvine Scientific
  Chemically Defined BalanCD HEK293 Viral Feed Boosts Viral Vector Production

- Andrea Vervoort, Virica Biotech
  Increasing Lentivirus Production and T-cell Transduction Efficiency: Discovery and Validation of Next Generation Viral Sensitizer (VSE^{TM}) Compounds

- Geddy Hamblen, Cellevate
  Novel nanofiber-based microcarriers designed for improved upstream

productivity of viral vector biomanufacturing

- Michelle Hussong, Cytiva
  Using High-Throughput Genetic Screening to Improve Stable AAV Producer Cell Lines and to enhance viral vector production

- Dan Farley, Oxford Biomedica
  Optimising Production of 4th Generation Lentiviral Vectors (LV) of the TetraVecta™ System: Towards Plug-and-Play LV Manufacturing

- Shandel Pariag, Pharmaron Biologics, Liverpool
  A HTPD Approach for Rapid AAV Process Optimisation & Scale-Up

- Maria Patricio, PhD, OXGENE
  A Novel Lentiviral Vector Cargo Gene Silencing System to Benefit Manufacturing of Cell Therapies

- Shandel Pariag, Pharmaron Biologics, Liverpool
  Accelerating the AAV Path to Clinic through Targeted CMC Development

- Jona Röscheise, University of Applied Sciences Biberach
  Unveiling the Secrets: Pathway Analysis Provides Molecular Insights into Stable Lentiviral Packaging and Producer Cell Lines (GPRTG)

- Alejandro Izurieta, VectorBuilder
  Improving the safety and performance for ATMP developers using next-generation vectors

- William Weir, Ultragenyx Pharmaceutical Inc.
  Accelerating and Advancing the Pinnacle PCL™ Platform

- Hugh Murray, MilliporeSigma
  A Comprehensive Approach to Adeno-Associated Virus Upstream Process Development

- AMIR SINGH, Nanoscope Therapeutics
  Forced Degradation Studies of Multi-Characteristic Opsin Gene Therapy Drug Product

- Amita Tiyaboonchai, PhD, Oregon Health and Science University
  In Vivo Selection of Randomly Integrated rAAV Does Not Cause an Increased Risk of Tumorigenesis

- Martin Bilbao-Arribas, CIMA Universidad de Navarra
  Integration Frequency of Full and Truncated AAV Genomes in Cas9-Induced DSB Sites Is Determined by DNA Repair Pathways

- Weiya Bai, HuidaGene Therapeutics Co., Ltd., Shanghai, China
  mRNA-loaded AAV Vectors Enable Safer CRISPR Gene Editing for Neurological Diseases

- Dwaipayan Sen, Associate Director, Stanford University Laboratory for Cell & Gene Medicine
  Identification and validation of an optimal stuffer sequence for AAV gene therapy applications using AI/ML

- Sean Carrig, Children's Hospital of Philadelphia
  Comparison of AAV Expression Cassettes Modestly Above and Below AAV Packaging Capacity for Hemophilia A Gene Therapy

- Zhong-Dong Shi, PhD, Frontera Therapeutics

MAR Sequences Enhance AAV Transduction and Promote Weight Gain in a Lethal Mouse Model of Methylmalonic Acidemia

- Randolph Qian, PhD, REGENXBIO
  In Vitro and In Vivo Characterization of Oversized AAV Vectors With High Genome Integrity That Encode Microdystrophins With Extended C Terminal Sequences

- Rachana Patil, LSRP, Stanford University Dept. of Pediatrics
  Cell-line and Antibody Epitope-dependent Expression Models Inform Selection of AAV-GNE Epimerase/Kinase Transgenes to Treat GNE Myopathy Rachana Patil[1], Bradley A. Hamilton[1], John W. Day[1] Department of Neurology and Neurological Sciences, Stanford School of Medicine, Palo Alto CA[1]

- Prushti Bhavsar, Solid Biosciences
  AAV-SLB101, a Novel Muscle-Tropic Capsid, Increases Gene Delivery and Expression Versus AAV9 and AAVrh74, in Mouse Models of DMD and FSHD Muscle Disease

- Alessandro Barberis, PhD, WHITELAB GENOMICS
  Enhancing AAV gene therapy targeting retinal diseases with AI-guided rational design

- Lingjie Kong, PhD, Codone Biotechnology Co., Ltd
  A Single Suppressor-tRNA Restores Clinically Meaningful Protein Levels in Multiple Disease Models with Premature Termination Codons In Vivo

- Ana Coroadinha, PhD, IBET
  Expanding AAV vector delivery capacity: improving efficiency and vector genome cargo size

- Jonathan Boucher, Northwestern University
  A System for Distinguishing Protein and Gene Delivery Elucidates Mechanisms of EV-AAV Vehicles

- Lei Zhao, Cure Genetics
  Development of heart tropism AAV capsid by directed evolution

- Anna Schrattel, University Hospital Heidelberg
  In vivo evolution of heart-derived AAV capsids: Development of novel vectors with enhanced cardiac tropism

- Ze Cheng, PhD, Tenaya Therapeutics
  Engineering Novel AAV Capsids for Cardiac Gene Delivery

- Gang Wang, Byongen Therapeutics
  An Improved Approach Enables Efficient and Cost-effective Directed Evolution of AAV Capsids in Non-human Primates via Different Delivery Routes

- Sunayana Mitra, Massachusetts General Hospital
  In Silico and In Vitro Analysis Suggests Translational Potential of the Smooth Muscle-Tropic Engineered Capsid, AAV-PR

- Yoshihide Sehara, MD, PhD, Jichi Medical University
  Artifical Modification of Adeno-Associated Virus Type 2 for Direction to Vascular Smooth Muscle Cells

- Lei Zhao, Cure Genetics
  Development of muscle tropism AAV capsid by directed evolution

- Harry Chen, UCLA

Identifying Muscle Stem Cell Tropic AAV Variants Through Directed Evolution

- Huachun Liu, Mass Eye & Ear
  Rational Design of AAV9 Capsids for Targeted Delivery to Muscle and Heart and Detargeting of the Liver

- Bumwhee Lee, Genixcure
  AI-guided Vector Engineering for Enhanced ALS Therapy

- Motahareh Arjomandnejad, PhD, UMass Chan Medical School
  AAVrh32.33: A Promising Gene Delivery Vector for Skin Disorders

- Qiang Zheng, Chengdu Origen Biotechnology
  Engineering the AAV capsid to evade neutralizing antibody-mediated immune responses

- Patrick Schmidt, PhD, National Center for Tumor Diseases
  Neo-antigen tumor vaccination depends on CD4-licensing conveyed by adeno-associated virus like particles.

- Maria Guevara, Baylor College of Medicine
  Common AAV Gene Therapy Vectors Show Indiscriminate Transduction of Living Human Brain Cell Types

- Sara Powell, PhD, UNC Chapel Hill
  Determinants of AAV9 capsid mediated transgene suppression in the rat CNS

- Paul Heppenstall, SISSA
  Bioconjugated AAV vectors with enhanced functionality and tropism

- David Leib, PhD, Latus Bio, Inc
  Direct Comparison of the Engineered Capsid AAV-DB-3 and Wildtype AAV5 in Nonhuman Primate Basal Ganglia and Cerebral Cortex

- Irvin Garza, UT Southwestern Medical Center
  Next Generation Whole Capsid Engineering Yields Translational AAV Capsids for Neurological, Neuromuscular. and Muscular Gene Delivery

- Sai Likhith Mallannagari, Baylor College of Medicine
  Common AAV Capsid Serotypes Better Transduce Resected Glial-type Tumor Tissue, with Feasibility for Engineering Tumor-targeted Gene Therapy Vectors

- Christian LEBORGNE, GENETHON
  Shuffling of HVR in AAV capsid reveals a context-dependent role for HVR5 in peripheral tissue detargeting while ensuring CNS targeting

- Geoffrey Keeler, PhD, University of Florida
  A Rationally Designed AAV9 Capsid with Minimal Liver Tropism

- Mengyao Xu, UMass Chan Medical School
  A single amino acid variant in the variable region I of AAV capsid confers liver detargeting

- TAKESHI SATO, Astellas Gene Therapies
  Relatively Low Doses of ASP2957 AAV Gene Therapy Demonstrate Efficacy and Safety in a Mouse Model of XLMTM

- Xiupeng Chen, membership, UMass Chan Medical School
  Lowering CpG content in rAAV genomes reduces DRG toxicity without compromising therapeutic benefits in mice

- Maryam Bemanalizadeh, Tehran University of Medical Sciences
  Comparative Analysis of Transgene Expression of Neurotropic AAV Serotypes and Their Potential in Combination Treatments

- Maryam Bemanalizadeh, Tehran University of Medical Sciences
  Short-Term Hurdles, Long-Term Promise: AAV9-SMN1 Gene Therapy for SMA

- Ananya Uppalapati, UMass Chan Medical School
  Evaluating a "silence and replace" approach for development of an AAV gene therapy for Spastic Paraplegia type 4

- Agostinho Gomes Rocha, Smithers - Pharmaceutical Development Services
  Validation of a Göttingen Minipig Gene Therapy Biodistribution Assay for AVGN7 Using Droplet Digital Polymerase Chain Reaction

- Stephen Fasul, Sarepta Therapeutics, Inc.
  In Situ Biodistribution and Localization of Bidridistrogene Xeboparvovec (SRP-9003) in LGMD2E/R4 Mice After 1 Year of Follow-up

- Maximiliano Presa, PhD, The Jackson Laboratory
  CSF Delivery of AAV9/SLC52A2 is Effective for the Treatment of RTD2 in a Novel Mouse Model

- Andreia Duarte, VectorY Therapeutics
  AAV-Delivered Anti-PC-OxPL Antibody Fragments as a Novel Therapeutic Approach to Target ALS

- Kayla Mandolini, University of Florida
  Immunomodulation Approaches to Improve Safety and Efficiency of Gene Therapy in Friedreich's Ataxia

- Yasemin ÖZGÜR GÜNES, UMass Chan Medical School
  Intracisternal AAV9-MAG-hABCD1 Vector Reverses Motor Deficits in Adult Adrenomyeloneuropathy Mice.

- Eloise Hudry, PhD, Novartis
  Leveraging host cell modulators of adeno-associated vectors transduction across serotypes to detarget liver

- Remko van Vught, MIMETAS B.V
  Immunocompetent Liver-on-a-Chip Model for Preclinical Evaluation of AAV-Based Gene Therapies

- Monique Otero, UMass Chan Medial School
  AAV Gene Therapy for LMNA Associated Laminopathies

- Guy Odom, PhD, University of Washington
  Micro-utrophin Redesign Improves Physiological and Metabolomic Outcomes in Dystrophic mdx^{4cv} Mice.

- Rima Saha, PhD, University of Florida
  Hepatic-Restricted Microdystrophin Expression Induces Immune Tolerance and Enhances Transgene Expression in Dystrophin-Deficient Mdx Mice

- Jessica Hogestyn, Sanofi
  Evaluation of a Novel AAV Capsid with Widespread CNS Biodistribution Following Intra-CSF Dosing in Nonhuman Primates

- Laura Ferraiuolo, Insmed Inc
  Intrathecal delivery of INS1202 AAV9-SOD1-shRNA rescues hallmarks of neurodegeneration in a disease model of ALS and targets glia and neurons in the spinal cord and brain of non-human primates

- Pete Clarner, PSM, Biogen
  Assessing the impact of age on AAV9 biodistribution and safety following intravenous administration in infant and juvenile cynomolgus macaques

- Kimberly McDowell, Capsida Biotherapeutics, Inc.
  CAP-003, a CNS-Targeted IV-delivered AAV Gene Therapy, Safely Increases Brain GCase in NHPs to Level Supporting Potential Normalization of Activity in PD-GBA Patients

- Nick Todd, Brigham and Women's Hospital
  Focused ultrasound-mediated delivery of a novel engineered AAV capsid to the rat and non-human primate brain using a human clinical FUS system

- Marshall Goodwin, Nysnobio
  Parkin Gene Therapy for Parkinson's Disease

- Yulia Dzhashiashvili, PhD, Spark Therapeutics
  Direct Administration of AAV2-Derived Vector Library to the Putamen of Non-Human Primates Identifies Capsid Variants with Enhanced Retrograde Transport Properties

- Geary Smith, ClearPoint Neuro, Inc.
  Translational Considerations When Comparing Brain Structures in Humans, Cynomolgus Macaques and Adolescent Baboons: Implications for Anatomical Targeting and Drug Delivery

- Veda Tatavarty, Encoded Therapeutics
  Potent and Specific AAV9-miRNA Candidates Demonstrate Robust Reduction of Microtubule-associated Protein Tau (MAPT) in Non-Human Primates

- Roxanne Croze, PhD, 4D Molecular Therapeutics (4DMT)
  Alternate Reporter Genes for Safe & Durable CNS Delivery of AAV Vectors in Non-Human Primates

- Amos Gutnick, PackGene Biotech INC
  Rationally designed chimeric AAV capsids demonstrate reduced liver tropism and enhanced muscle transduction in both rodents and non-human primates

- Shaofeng Wang, Ultragenyx Pharmaceutical Inc.
  A Comparative Study of Full Adeno-Associated Virus 8 (AAV) Capsid Enrichment Platforms

- Balasubramanian Venkatakrishnan, Senior Application Scientist, Beckman Coulter Life Sciences
  Ensure Accurate Density Gradients Through a Novel Automation Platform – the OptiMATE Gradient Maker

- Mikako Wada, PhD, IMSUT
  Large-scale purification of highly effective recombinant adeno-associated virus with a zonal ultracentrifugation

- Franck Gaston, GENETHON
  Development of a Full Capsid Enrichment Polishing Step in AAV8 Purification Process With CIMmultus® PrimaT® Monolithic Column

- Benjamin Graf, Sartorius Lab Instruments GmbH & Co KG
  Development of a Novel Flow-Through Polishing Approach for Simplified Full/Empty Separation of Adeno-associated Viruses

- Lihua Yang, AbbVie
  Purification Process Toolbox Development for Gene Therapy Vector AAV

- Cecile Toussaint, VVector Bio
  Critical Considerations For Full/Empty AAV Separation

- Samit Dutta, MilliporeSigma - Carlsbad, CA
  Utilization of Real-time Multi-angle Light Scattering (RT-MALS) for Accelearted AAV Chromatography Development

- Westley Blacklock, Repligen
  A Novel Harvest-Capture Process for Rapid Clarification and Recovery of rAAV9 from Cell Lysate Using a Macroporous Filtration Device and AVIPure AAV9 Affinity Resin

- Ruud Visser, Amsterdam UMC
  Optimization of AAV6 Production at Benchtop Scale: The Role of Affinity Chromatography in Overcoming Aggregation

- Smriti Mishra, PhD, MilliporeSigma
  Affinity purification analysis: a comparison of AAV2 and AAV5 serotypes

- Yuriko Makino Manabe, PhD, Tosoh Corp.
  Performance of Alkali-Tolerant Affinity Chromatography Resins Immobilized with AAV Receptor and Full/Empty Separation of AAV1

- Tom Elich, Voyager Therapeutics, Inc.
  Enabling Large Scale Implementation of Anion Exchange Chromatography for Full Capsid Enrichment of a Novel Adeno-Associated Viral Vector

- Yuliya McAnany, MS, Tosoh Bioscience LLC
  Efficient Separation of Empty and Full AAV Capsids with Choline Chloride in Preparative Anion Exchange Chromatography

- Melanie Langhauser, Ascend Advanced Therapies
  High Yield and Full-Empty Ratios Achieved with a Scalable GMP-Ready AEX Chromatography

- Vidya Murthy, PHD, West Pharmaceutical Services, Inc.
  Maintaining Superior Viral Vector Recovery in Cell and Gene Therapy Applications by using Daikyo Crystal Zenith® Vials

- Sonia Stinus, Viralgen Vector Core
  Benefits of High-Throughput Sequencing-Based Methods for In-Depth Characterization of Recombinant AAV Products

- Shengjiang Liu, Avirmax Inc.
  Determination and Characterization of Capsid Full Ratio of the Clincal Grade rAAV Product by Transmission Electron Microscopy, Analytical Ultracentrifugation, Digital Droplet PCR, and Capsid ELISA

- Arjen van Den Berg, ThermoFisher Scientific
  Automation of Small Scale AAV Production for High Throughput Screening Using the Triple Transfection Method

- Philip Szymanski, MilliporeSigma
  Methods to Improve the Clarification Process of AAV Therapy Manufacturing

- Yeonji Kim
  Optimizing AAV8 Capsid Purification with Oversized GOI: Efficient Separation of Empty, Partial, and Full Capsids Using Anion Exchange Chromatography

- Taeho Kim, Sangamo Therapeutics
  Title: The Impact of Empty Capsids on AAV Manufacturing and Strategies for

Enhancing Yield, Purity, and Stability in the Production of a Novel Blood-Brain Barrier Penetrant AAV Capsid

- Rok Zigon, Sartorius BIA Separations
  Accelerating AAV Processing: Scalable, Serotype-Agnostic Process Eliminating 2-steps (DNase and TFF) and Optimized for Physiological-Like Conditions

- Kelly Rios, MilliporeSigma - Carlsbad, CA
  Next-Generation Sequencing for AAV Characterization

- Ricardo Correa, BioReliance Corporation (MilliporeSigma/Merk KGaA)
  Multiplex ddPCR Approach as Proof-of-Principle for Accurate AAV Genome Integrity Assessment and Enhanced Vector Production

- Suvana Lin, BS, Umass Chan Medical School
  Evaluating the Performance of Nanoneedle Assays for Robust Quantification and Determination of AAV Genome species packaged and Capsid Titers during Manufacturing Process

- Ryan Massopust, Asklepios Biopharmacuetical
  AAV Genome Integrity: Full and Partial Genome Characterization Utilizing Digital PCR and Long Read Sequencing

- Emily Phillips, Ultragenyx Pharmaceutical Inc.
  Characterization of High and Low rAAV Producer Cell Populations in Pinnacle PCL using Single Cell RNA Sequencing

- Mao Fu, Origene
  Quantification of Adeno-Associated Viral Genomes Using a Newly Developed qPCR Assay

- Conor Cox, Thermo Fisher Scientific
  Precise rAAV Capsid Characterization via Digital PCR and Statistical Modeling

- Wai Choi, Gator Bio
  GeneSwift: a PCR-Free, Hybydization Method to Determine Gene Therapy Vector Titer

- Morten LUHR, PCI Biotech
  Reducing Host-Cell Impurities and Increasing Yield In AAV Manufacturing Using the Novel Technology Photochemical Lysis (PCL)

- Akshat Gupta, MilliporeSigma
  Strategies for residual DNA Clearance in Adeno associated Virus (AAV) purification process.

- Ewan Stephenson, Evonik
  A Functional Alternative to Triton™ X-100 for Cell Lysis in Therapeutic Virus Harvesting

- Christian Gagnon, Voyager Therapeutics, Inc.
  Title: An Alternative to Detergent Lysis: Promoting rAAV Release to Media by Optimizing pH, osmolality and harvest timing

- Kate Yu, Waters Corporation
  Characterisation with Relative Quantification of AAV Capsid Particle Population Heterogeneity Using Charge Detection Mass Spectrometry in Comparison with Orthogonal Technologies

- Yuyan Wang, AAVnerGene
  Comparison of Mass Photometry (MP) and Charge Detection Mass Spectrometry (CD-MS) in AAV Characterization

- Irene Song, Packgene Biotech
  Rapid and Accurate AAV Capsid Quantification Using Multi-Angle Dynamic Light Scattering (MADLS) for In-Process Gene Therapy Manufacturing

- Benjamin Draper, PhD, Megadalton Solutions
  Highly Accurate Direct Mass Measurement of Extracted AAV Genomes Coupled with Intact Capsid Analysis to Assess Sequence Integrity and Viral Protein Ratio by Charge Detection Mass Spectrometry

- Yuki Yamaguchi, Ph.D., Osaka University
  Direct Monitoring of rAAVs in Crude Cell Lysate and Conditioned Medium

- Bryan Hassell, Nirrin Technologies
  Real-Time Monitoring of AAV Empty:Full Capsid Separations using Near Infrared High-Precision Tunable Laser Spectroscopy

- Nathan Feirer, Promega Corporation
  Leveraging TruTiter™ Technology to Overcome Thermal Stability Challenges in Engineered AAV Quantitation

- Anthony Blaszczyk, United States Pharmacopeia
  Quantification of Full, Empty, and Partial Particles of AAV8 Reference Standards From Multi-Lab Study

- Linda Chio, Gordian Biotechnology
  Comprehensive Analytic Tools Comparison for Empty, Full, and Partial Genome Characterization of Adeno-associated Viruses Show Need for Parallel Assay Verification

- Julia Zalewski, Forge Biologics

Development of a High Throughput Flow Cytometry in vitro Relative Potency Assay as an Analytical Tool to Support rAAV Process Characterization

- Thomas Quinn, MS, Takara Bio USA
  Smartphone-based Titration of AAV Vector Preparations

- Satomu Ishii, FUJIFILM
  Quality analysis and biological evaluation of AAV particle derived from AAV stable Producer cell line

- Hannah Flaherty, KACTUS
  Tailored AAV Solutions: AAV ELISA Kits and Customized Variant Kit Development

- Justin Glenn, Regenxbio
  Development of in vitro methods for the analysis of TLR9 stimulation by AAV vector genomes.

- Geoffrey Keeler, PhD, University of Florida
  Elucidating the Mechanisms of Hepatic induced Tolerance Following Liver-Directed AAV Gene Therapy

- Blake Williams, BS, UCLA
  Evaluating Immune Responses to AAV Transgenes in THP-1 Cells

- Robert Clark, Tulane University Health Science Center
  Immunosuppression with αCD20 and Rapamycin Limits Adaptive Immune Responses to AAV6.2 and Permits Unimpeded Redelivery to the Mouse Respiratory Tract

- Bradley Hamilton, PhD, Stanford

Extracellular Vesicles Reduce Type I Interferon Responses to AAV Gene Therapy in a Human Model of Innate Immunity

Thursday, May 15

Sponsored Symposium

GenScript USA Inc: Multiplex Base-Edited CAR-T Cells Overcome Glioblastoma’s Multifaceted Suppression using GenScript's sgRNA Solutions

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

- Joyce Tung, GenScript USA Inc.
  GenScript USA Inc: Multiplex Base-Edited CAR-T Cells Overcome Glioblastoma’s Multifaceted Suppression using GenScript's sgRNA Solutions

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, MD, PhD, 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

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
  Gene Therapies targeting the podocyte

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

Oral Abstract Session

Disease Models and Pre-Clinical Applications for Lysosomal Storage Diseases

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

- 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

- Sheridan Rose, 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, 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.
  IV-delivered AAV gene therapy targeting tau for the treatment of Alzheimer's disease: preclinical safety and pharmacology with translational considerations

Scientific Symposium

Cellular and Gene Therapies for Autoimmune Disease (Organized by the Hematologic and Immunologic 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
  LNP-circular RNA In Vivo CAR therapy for Autoimmune diseases

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

- Devin Rosenthal, NovaQuest Capital Management
  Beyond Traditional Metrics: A Venture Perspective on Valuing and Funding Next-Generation CGTs

Sponsored Symposium

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

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

- Pinar Boyar, FUJIFILM Irvine Scientific
  FUJIFILM Irvine Scientific: Scalable Solutions for Gene Therapy and Cell Therapy Workflows

Sponsored Symposium

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

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

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

Networking

Exhibit Hall

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

General Session

George Stamatoyannopoulos Memorial Lecture

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

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

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

Poster Talk Session

Thursday Poster Talks

Location: Exhibit Theater
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

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

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

Sponsored Symposium

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

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

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

Sponsored Symposium

Biogen: Biogen Gene Therapy

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

- Catherine Zheng
  Biogen: Biogen Gene Therapy

Sponsored Symposium

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

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

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

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

Tools & Technology Session

Tools and Technology Forum 3

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

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

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

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

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

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

- Amy Lamperti, Minaris Advanced Therapies

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

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

Sponsored Symposium

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

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

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

Sponsored Symposium

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

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

- David Ede
  Sartorius BIA Separations: Orthogonal chromatography analytics to allow for faster AAV or LNP process development and better in-process control

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

Sponsored Symposium

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

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

- Christen McDonald, STEMCELL Technologies, Inc.
  STEMCELL Technologies Inc: Cutting-Edge Tools for Hematopoietic Stem & Progenitor Cell Research

Tools & Technology Session

Tools and Technology Forum 4

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

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

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

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

- Randy Dyer, Elegen
  Elegen: Accelerating mRNA Vaccine Development with Rapid, Cell-Free Synthesis

of High-Accuracy Template DNA

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

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

Oral Abstract Session

Novel Therapeutic Gene Editing Applications

Location: 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

- Mary Barry, 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

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

- Anand Alembath, PhD, 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, 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

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

Poster Abstract Session

Thursday Poster Reception

Location: Poster Hall Hall I2
5:30 PM - 7:00 PM

- Xiaojuan Tang, UMass Chan Medical School
  An RNA switch-regulated AAV gene therapy for obesity, insulin resistance, and MASH

- Yi Gong, MGH
  Gene therapy rescues adrenocortical cells from lipid cytotoxicity in mice and humans with adrenoleukodystrophy

- John Selser, National Human Genome Research Institute
  Partial Deficiency Mouse Model of Cobalamin B Class MMA: Systemic AAV9 Gene Therapy Confers Resistance to Metabolic Crisis

- Mafalda Cacciottolo, Capricor Therapeutics
  STX-Arg1: exosome-based enzyme replacement therapy restores Arg1 activity in vivo and improves lifespan of Arg1-/- mouse model.

- Wenhao Ma, Beijing Genecradle Therapeutics Inc.
  A Novel Mmachc c.80A>G Mouse Model: Insights of Mitochondrial Dysfunction in Skeletal Muscle and Potential AAV-mediated gene therapy for cblC

- Andrea Pappas, Kriya Therapeutics

Long Term PK/PD and Preliminary Safety Study of FGF21 Following Intramuscular Administration of KRIYA-497 (AAV1.hFGF21) in Healthy NHPs

- Zhenhua Wu, Exegenesis Bio Inc.
  A GLP-compliant Toxicology Study of Intravenously Administrated EXG110 for Treating Fabry Disease

- Elizabeth Brooks, MS, DVM, Duke University Medical Center
  Long-Term Efficacy of Genome Editing in Infant Mice With Glycogen Storage Disease Type Ia

- Edo Kon, RiboX Therapeutics
  Lipid Nanoparticle Delivered Circular RNA Encoding Ornithine Transcarbamylase (OTC) for the Treatment of OTC Deficiency: A Novel and Sustainable Therapeutic Approach

- Jingsong Cao, Innorna USA, Inc
  ATP7B mRNA therapy for the treatment of Wilson's disease (WD)

- Karl-Dimiter Bissig, MD, PhD, Duke University
  Rescue of glutaric aciduria type I mice by liver directed gene editing

- June Baik, Genzyme, a Sanofi Company
  Alpha-1 antitrypsin correction by in vivo gene editing

- Zsanett Jancso, Tessera Therapeutics
  A Potential Gene Editing Approach for AATD-Associated Liver and Lung Diseases Intended to Correct the PiZ Allele

- Brian Bigger, PhD, University of Edinburgh

Anti-SGSH antibodies following Hematopoietic Stem Cell (HSC) Gene Therapy in Patients with MPSIIIA Neither Impact Engraftment of Genetically Modified HSC nor Interfere with Multi-compartment Substrate Reduction

- Slawomir Wantuch, Orchard Therapeutics
  Correction of Glycogen Accumulation in Muscle, Heart and CNS in a Pre-Clinical Model of Hematopoietic Stem Cell Gene Therapy for Pompe Disease

- Michael Przybilla, PhD, University of Minnesota
  Improving blood-brain barrier penetration in Hurler syndrome using an IDUA-ApoE fusion enzyme delivered via the PS Gene-editing System

- Fengkui Zhang, Institute of Hematology & Blood Disease Hospital, Chinese Academy of Medical Sciences & Peking Union Medical College, Tianjin, China
  Evaluation of Clinical Safety and Efficacy of LY-M001: A Phase I/II Trial of AAV8-Mediated Gene Therapy for Gaucher Disease Type I

- Joshua C. Chang, Astellas Gene Therapies
  Pharmacokinetic, Pharmacodynamic, and Toxicokinetic Analysis of an rAAV8 Gene Therapy for Friedreich Ataxia-Associated Cardiomyopathy

- Anusha Sairavi, MS
  Slow Retrograde Renal Pelvis Injection of Centrifugally Ultrafiltered AAV Miniprep Vectors Enables Rapid Capsid Screening For Kidney Gene Delivery

- Fernando Gomez Garcia, Nephrogen Inc.
  Adeno-Associated Viral Mediated Genome Editing as a Curative Therapy for Polycystic Kidney Disease

- Asmaa Mekawy, Mayo Clinic
  Enhancing Delivery Methods for Kidney-targeted Gene Therapy

- Ashley Cooney, PhD, University of Iowa
  Lentiviral complementation restores phenotypic defects of ABCA3 pathogenic variants

- Alex Lagadinos, PhD, Mana.bio
  Leveraging ML to Improve Potency and Safety of Lung-Targeted Lipid Nanoparticles

- Ashley Cooney, PhD, University of Iowa
  Peptide insertions enhance AAV capsid tropism for airway epithelia

- Martin Donnelley, PhD, University of Adelaide
  Optimising Magnetic-Guidance of Lentiviral Vectors for Improved Airway Gene Therapy Efficacy

- Autumn Greco, Johns Hopkins University School of Medicine
  Development of Lung-tropic Lipid Nanoparticles for Endothelial Cell Nucleic Acid Delivery

- Ri Tang, University of Michigan
  Pulmonary delivery of circular RNA therapeutics for cystic fibrosis

- Anais Amaya, Stanford University
  A hybrid gene correction strategy for Cystic Fibrosis

- Maegan Hoover, GentiBio
  Allogeneic Engineered T Regulatory Cells Improve Disease Outcome in Preclinical Models of Acute Lung Injury

- Darryl Narcisse, Opsin Biotherapeutics
  Ultrasound-Guided Nano-Enhanced Optical Delivery of Optogenes in the Central Nervous System for Modulating Pain

- Anthony Delalande, University of Orléans
  Evaluating Behavioral Outcomes of Whole-Brain AAV9 Gene Therapy for Fragile X Syndrome via Focused Ultrasound and BBB Opening

- Christopher Davis, Apertura Gene Therapy
  Intravenous Delivery of a CNS-Penetrant AAV Gene Therapy Provides Survival Benefit in a TSC1 Disease Model

- Deeann Wallis, University of Alabama At Birmingham
  Identification of AAV engineered capsids that allows efficient CNS transduction through comparative biodistribution studies.

- David Cameron, University of California Davis Department of Neurology
  Multivariate Analysis of Routes of Administration and AAV Capsid Selection for Efficient Brain Transduction in the FVB Strain of Mice

- Barbara Bailus, Keck Graduate Institute
  A Minimally Invasive Enzyme Replacement Therapy for Angelman Syndrome

- Fatemeh Mehryab, Nationwide Children's Hospital
  A MyoAAV Capsid Variant Revealed Schwann Cell Specific Colocalization in AAV Serotype Screening Studies for Peripheral Nervous System Gene Therapies

- Mathieu Desclaux, PhD, Regeneron
  Development of an antibody-conjugated AAV platform for non-invasive and targeted delivery of therapeutics to the peripheral nervous system.

- Marius Walter, PhD, Fred Hutchinson Cancer Center
  Gene drive viral vector suppress recurrences of herpes simplex virus 1 and 2 in mice

- Tomeh Tomeh, Mass General Brigham
  Gene Therapy for Tuberous Sclerosis Complex 1: AAV-Mediated Hamartin Delivery Enhanced via Extracellular Vesicles in a Mouse Model of TSC1.

- Joan Roig-Soriano, Universitat Autonoma de Barcelona
  Human Secreted Klotho Expression Improves Cognition and CNS Status in Aged Non-Human Primates

- Matthew Simon, The Jackson Laboratory
  A new, patient-derived SYNGAP1 mouse model demonstrates multipledisease-relevant phenotypes.

- Aura Kullmann, NeuroOne
  Enhancing Chemotherapeutic Efficacy by Combining Efflux Transporter Inhibition with CED Using a Novel sEEG-based Drug Delivery System

- Raegan Adams, UT Southwestern Medical Center
  Comparative Gene Therapy Efficacy for KO and KI Models of SLC13A5 Citrate Transporter Disorder

- Wuh-Liang Hwu, PhD, MD, China Medical University Hospital and National Taiwan University Hospital
  Gene therapy for mouse models of 6-pyruvoyl-tetrahydropterin synthase deficiency

- Jordan Dattero, MIT McGovern Institute
  Utilizing Cytosine Base Editors to Generate MECP2-R270X Knock-in Models of Rett

Syndrome

- Joshua Bonkowsky, University of Utah
  AAV-Mediated Gene Supplementation in Combination with Microglial Replacement for Vanishing White Matter Disease Using Circulation-Derived Myeloid Cells (CDMCs)

- Gaia Colasante, PhD, San Raffaele Scientific Institute
  Selective reactivation of Scn1a gene in GABAergic interneurons to explore symptomatic reversibility in Dravet Syndrome

- Karin Morandell, Maxwell Biosystems
  The Importance of High-Density Microelectrode Arrays for Recording Multi-Scale Extracellular Potential and Label-Free Characterization of Network Dynamics in iPSC-Derived Neurons

- Casiana Gonzalez, UC Davis
  Novel Stem Cell Model for CASK-Related Disorders and Evaluation of a CRISPR/dCas9 Based Rescue

- Adele Bubnys, Arbor Biotechnologies
  Disruption of UBE3A-ATS by Gene Editing with a Type V CRISPR-Cas Enzyme as a treatment for Angelman Syndrome

- Morgan Moser, PhD, Nationwide Children's Hospital
  AI-Guided Gene Therapy Strategy for Treating Patients with Tuberous Sclerosis Complex Type 2

- Keneth Munoz, University of California San Diego
  Impact of FXN gene editing in Friedreich’s Ataxia patient-derived lymphoblasts and iPSC-derived microglia

- Siyuan Hao, PhD, UT Southwestern Medical Center
  AAV9-based gene therapy for PDHA1 deficiency.

- Sarah Holbrook, PhD, UMass Chan Medical School
  Investigating allotopic expression and mitochondria-targeted base editors as therapeutic strategies to alleviate MTATP6 associated Leigh Syndrome

- Jessica Herstine, Nationwide Children's Hospital
  Two Years of Efficacy: Astrocyte-Targeted Gene Replacement for Vanishing White Matter Disease Exposes Uncorrected Disease Mechanisms

- Robert Thompson, Boston Children's Hospital/Mass General Brigham
  Exploring Enzymatic Restoration and Sphingolipid Modulation through AAV-Mediated Gene Addition of a Subunit of Serine Palmitoyltransferase

- Rafik Boudra, Sensorion
  Safety and efficacy of GJB2-GT, an adeno associated vector-based gene therapy treatment candidate for the autosomal recessive non-syndromic deafness 1A (DFNB1A)

- Domenica Karavitaki, Skylark Bio
  Novel Gene Therapy Shows Cell Specific Transduction in Non-Human Primate Cochlea and Provides A Robust and Durable Rescue of Hearing in Mouse Models of GJB2 Related Deafness

- David Corey, PhD, Harvard Medical School
  Cell-Specific Delivery of GJB2 Restores Auditory Function in Mouse Models of DFNB1 Deafness and Mediates Appropriate Expression in NHP Cochlea

- Rafik Boudra, Sensorion

GJB2 gene therapy-response of two pre-clinical mouse models of the most frequent form of human deafness, DFNB1A.

- Heon Yung Gee, Yonsei University College of Medicine
  Gene Replacement Therapy Ameliorates Hearing Loss in a Mouse Model with an Mpzl2 East Asian Founder Variant.

- Erdem Yildiz, Medical University of Vienna
  Large animal model for human inner ear gene therapy: Transgene expression of viral vectors in pigs

- Kayo Takashima, Kyoto University
  Supporting the Informed Consent Process in Clinical Trials of Induced Pluripotent Stem Cell–Derived Corneal Epithelium Transplantation

- Cecilia Marinova, Medasol
  IMPROVING ACCESSIBILITY TO CELL AND GENE THERAPY FOR RARE DISEASES

- Ivan Fernandez-Bueno, Universidad de Valladolid
  Generation of induced Pluripotent Stem Cell (iPSC)-derived Retinal Pigment Epithelium (iRPE) from a patient with Retinitis Pigmentosa associated with a mutation in PROM1

- Xiaoming Gong, Akron Children's Hospital
  A Novel AAV2 Capsid Variant-based Dual-Acting Ocular Gene Therapy Targeting VEGF and Inflammatory factors for Neovascular Age-related Macular Degeneration

- Lovisa Selander, Neurotech Pharmaceuticals, Inc.
  NT-501 Capsule Stability and Encapsulated Cell Technology Platform Capabilities

- Sasha Makohon-George, Kriya Therapeutics
  Analysis of Spatial Transcriptomic Data in a Non-Human Primate Ophthalmology Model

- Ian McHugh, Neurotech
  Encapsulated Cell Technology Enables Steady State Delivery of a Wide Range of Therapeutic Targets

- Jiansen Yan, Baylor College of Medicine
  Intra-Articular Re-administration of High-Capacity Adenovirus (HCAd) for Gene Therapy in Post-traumatic Osteoarthritis

- Susi Feng, University of North Carolina at Chapel Hill
  Treatment of Rheumatoid Arthritis in a Collagen-Induced Arthritis Mouse Model Using Intra-Articular Injection of AAV6-Delivered sIL17RA

- Yangjin Bae, Ph.D., Baylor College of Medicine
  High Capacity Adenoviral Intra-articular NFkB-responsive IL1RA gene therapy on a genetic osteochondrodysplasia model

- Yahui Lan, Regeneron
  Antibody-Based AAV Retargeting to CACNG1 Mediates Enhanced Skeletal Muscle Transduction and Reduced Hepatotoxicity in Non-Human Primates

- Madeleine Landau, Duke University
  AAV Gene Therapy for Digit Regeneration Informed by Spatial Transcriptomics

- Stefano Cagnin, University of Padova
  Secreted microRNAs are implicated in the reversal of myogenesis defects in ALS-derived myocytes.

- Kuo-An Liao, Duke University
  A Highly Potent MyoAAV4A Vector Achieved Long-Term Correction of Muscle Disease in Young Adult GSD IIIa Mice and Reversed Muscle Dysfunction in Aged Mice

- Eric Ehrke-Schulz, Dr. rer. nat., Witten/Herdecke University
  In vitro models and viral vectors and for targeted gene replacement therapy in Calpainopathy (LGMD2A/R1)

- Ivan Krivega, SonoThera
  Non-Viral Genetic Medicine for Targeted Delivery of Full-Length Dystrophin to Skeletal, Cardiac, and Diaphragm Muscles in DMD Mouse Models and Non-Human Primates.

- Sarah Nath, DVM, University of Massachusetts Chan Medical School
  Development and Characterization of a Mouse Model, Cell Model, and AAV-Based Gene Therapies for TNNT1 Nemaline Rod Myopathy

- Sonia Albini, GENETHON
  Therapeutic Screening of Dual AAV-Split Intein MIDI Dystrophins by Analysis of Mechanical Properties in Vivo and in Human Muscle Organoids

- Cristina Antich Acedo, NIH
  3D Bioprinted Platform as a High-Throughput Model for Drug Screening and Discovery for Myopathies and Neuromuscular Diseases

- Ricardo Weinlich, Hospital Israelita Albert Einstein
  Optimizing CRISPR/Cas9 and AAV6-Based Gene Therapy for Sickle Cell Disease: The Dual Impact of Polyvinyl Alcohol on HDR Efficiency and HSC Viability.

- M Graça Almeida-Porada, MD, PhD, Wake Forest School of Medicine

Introduction of the Human Sickle Cell Mutation in Sheep Beta-globin by CRISPR/Cas9 Models Human SCD in Cloned Sheep

- Seyeon Bae, CellFE
  Robust Gene Editing of CD34+ Hematopoietic Stem Cells Using a Microfluidics-Based Gene Delivery Technology

- Vivien Sheehan, PhD, MD, Emory University School of Medicine
  Impact of novel mobilizers and disease modifying agents in the sickle mouse

- Carson Stoker, Utah State University
  Heterozygous SCD Sheep Produced by Breeding Exhibit Sickle Cell Traits

- Cassady Rupert, PhD, Propria LLC
  Human Engineered Heart Tissues to Model Genetic Dilated, Hypertrophic, and Arrhythmogenic Cardiomyopathies.

- Sabrina Santos, Children's Hospital of Philadelphia
  Exploring the Genetic Landscape of Neurodevelopmental Disorders: Novel Insights and Therapeutic Avenues through JNK Pathway Analysis

- Daniel Paull, The New York Stem Cell Foundation
  Leveraging automation and AI to streamline and scale iPSC-based drug screening

- Hristina Gaydarska, Kyoto University, Center for IPS Research and Application
  Ethical and Social Considerations of Heritable Genome Editing in Human Embryos: A Focus on UNESCO Initiatives

- Vasu Kommireddy, LifeEDIT Therapeutics
  Deep CRISPR Nuclease Portfolio and Multiple Editing Modalities Accelerates

Identification of Viable Clinical Candidates

- Immacolata Porreca, Revvity
  A single-step process for non-viral engineering of hypoimmunogenic pluripotent stem cells with the Pin-point™ base editing platform

- Siyu Chen, Stanford School of Medicine
  CRISPR-mediated gene therapy and read-through therapeutics for Lowe Syndrome

- Chunyan He, Suzhou GenAssist Therapeutics Co., Ltd
  Expanding Base Editor Scope with Embedding Strategy

- Elena Benvenuto, Vita-Salute San Raffaele University
  Tricking Protein Translation: A Novel Base Editing Strategy to treat Dravet Syndrome

- Sarah Rains, KACTUS
  The Second-Generation High-Fidelity Cytosine Base Editor AccuBase™ Can Proficiently Modify Multiple Genes Within A Human Primary T cell While Minimizing Off-target Effects

- Immacolata Porreca, Revvity
  Minimal activation of the p53 DNA damage response by a modular cytosine base editor enables effective multiplexed gene knockout in induced pluripotent stem cells.

- Immacolata Porreca, Revvity
  One-step engineering of allogeneic CAR-T cells by simultaneous multiplex knockout and site-specific transgene integration with the Pin-point™ base editing platform configured with OpenCRISPR-1, an AI-engineered CRISPR-Cas enzyme

- Immacolata Porreca, Revvity
  Customizing the versatile Pin-point™ platform: Modular components for tailored base editing in genomic applications

- Yongzhong Wang, PhD, Accuredit Therapeutics US
  Engineering TadA-Derived Base Editors with Enhanced Potency and Safety for non-viral In Vivo Gene Editing

- Louisa Mayer, Cellectis
  High fidelity C-to-T editing with TALE base editors

- Amanda Haupt, Revvity
  Optimization of the modular Pin-pointTM base editing platform for an engineered Type V CRISPR-Cas effector

- Shutan Jiang, Epigenic Therapeutics
  EPIREG®: A Novel Epigenetic Platform for Safe and Efficient Multi-Gene Silencing in Universal CAR-T Cell Therapies

- Kiriaki Paschoudi, School of Biology, Artistotle University of Thessaloniki and "Gene and Cell Therapy Center George Papanikolaou Hospital
  Epigenetic Suppression of HBF Modifiers, Efficiently Reactivates Gamma Globin Expression in Primary Cells

- Alejandro Tapia, Indiana University School of Medicine
  Development of a Novel Chimeric Suppressor tRNA Successfully Rescues Functional Protein in Nonsense Harboring TP53 Mutants

- Hui Yang, HuidaGene Therapeutics Co., Ltd., Shanghai, China; Shanghai Institute

of Meteria Medica, Chinese Academy of Sciences, Shanghai, China
CRISPR-hfCas13Y RNA-targeting Therapy Restores Cognitive Function by Reducing BACE1 or MAPT in Alzheimer's Disease Model

- Daniel Reed, Rice University
  Engineering CRISPR/dCas9-based modalities in primary neurons for gene modulation of ΔFosb in the context of cocaine substance use disorders (SUD)

- Wang Shimin
  Efficacy and Safety of a CasRm-Based Therapy for Parkinson's disease in Non-Human Primates

- Abbey Stokes, Student, University of Arkansas
  A Skeletal Muscle-Specific dCas9-KRAB Mouse Model for Precision Gene Silencing

- Renee Napoliello, University of California, Davis
  A Multi-Omic Deep Learning Approach to Predict CRISPR Activation and Interference Efficacy

- Matthew Dale, Concinnity Genetics
  AI-Driven Engineering of RNA Control Systems for Precise Regulation of Gene Therapy Activity

- Gauri Bora, Northwestern University
  Engineering Synthetic Epigenetic Regulation to Enable State-Switching Genetic Programs in Mammalian Cells

- Carrie Ziemniak, MS, Azenta Life Sciences
  The Long and Short of It: Comparing Next Generation Sequencing Methods for Quality Control in AAV Development

- Sören Turan, Bayer AG
  B-GEn is an Innovative Bayer Proprietary Genome Editor Suitable for CRISPR Applications and Therapeutic Developments

- Yifan Ding, Brigham and Women's Hospital
  Engineering a Compact Gene Switch for Temporal Control of AAV-CRISPR Gene Editing

- Michael Molnar, Synthego Corporation
  hfCas12Max: Engineered CRISPR Nuclease for Safer and Effective CGT

- Bastian Nießing, Fraunhofer Institute for Production Technology
  Automated CRISPR/Cas9-based genome editing of human pluripotent stem cells using the StemCellFactory

- Catherine Morffy Smith, Synthego Corporation
  PsCas9 (eSpOT-ON): An Engineered High-Fidelity CRISPR Nuclease for Therapeutic Applications.

- Yuanhao Qu, Stanford University
  CRISPR-GPT: LLM Agents for Automated Design of Gene-Editing Experiments

- Karthik Murugan, Integrated DNA Technologies
  Standardized methods in iPSC for CRISPR-based editing and Homology-Directed Repair (HDR)

- Francesco Puzzo, PhD, Stanford University
  Tales from the R-loops: how RNA/DNA hybrids may influence CRISPR/Cas9-mediated homologous recombination gene editing outcomes

- Minghong Zhong, PhD, GeneLancet Biosciences, Inc.
  Chemically Modified and Ligated Clustered Regularly Interspaced Short Palindromic Repeats (CRISPR) Guide RNAs (LgRNA) and Their Anti-HBV Activities

- Yueyang Jin, University of North Carolina, Chapel Hill
  Constructs of Chemically Ligated Guide RNAs (LgRNA) for Precise Clustered Regularly Interspaced Short Palindromic Repeats (CRISPR) Gene Editing

- Max Levine, Tenaya Therapeutics
  Engineering the sgRNA Scaffold to Enhance Editing Efficiency in CRISPR/Cas9-Based Gene Therapy

- Steve Glenn, Integrated Dna Technologies
  Engineering SpCas9 to be Compatible with sgRNAs with Shorter Constant Regions

- Dabbu Jaijyan, Virginia Commonwealth University
  Infusion of Sso7d DNA-Binding Protein Boosts Gene Editing Efficiency of the Engineered Hypercompact OsCas12f (enOsCas12f) in Mammalian Cells

- Federica Esposito, MS, TIGEM
  Optimizing knock-in efficiency of therapeutic transgenes using novel Cas9 fusion proteins

- Joe OBrien, Integrated DNA Technologies
  Engineering an Improved Inhibitor of 53BP1 to Enhance HDR Efficiency

- Laura Hartmann, Baylor College of Medicine
  Optimizing phenotype and function of non-viral CRISPR-Cas9 TRAC-replaced CAR T cells by pharmacological tyrosine kinase inhibition during ex vivo expansion

- Kezhi Yan, Cystic Fibrosis Foundation
  Therapeutic transgenes need optimization – lessons from Cystic Fibrosis

- Giandomenico Turchiano, University College London
  DNA repair precision after gene editing, unveiled

- Erin Cross, Kromatid Inc
  The missing link: Kromatid's Next-Gen Cytogenetic Platform, KROMASURE™, Provides Critical Insight for Evaluating Cell and Gene Therapy Products for Chromosome Structural Variation, including Edit Integrity and Genomic Stability.

- David Kuo, Tessera Therapeutics
  Diji: The First Integrated Method to Analyze Single and Double-strand Breaks in GUIDE-seq and Digenome-seq Data for Unbiased Assessment of Editing Outcomes

- Charles Blanluet, QUiCKR Bio
  Fast Quantification of Genome-Editing Outcomes using CRISPR-based detection

- SHU WANG, Mission Bio
  Comprehensive On- and Off-target Validation Using Integrated rhAmpSeq and Targeted DNA Resequencing Single-Cell Technology for Gene Editing Applications

- Haiwang Yang, MilliporeSigma
  RGENtool - a CRISPR/Cas double-strand cleavage evaluator

- Kejia Kan, Shanghai Waker Bioscience Co., Ltd
  Enhancing Precision and Safety in CRISPR/Cas Gene Editing: A Combined Off-Target Detection Strategy

- Ivan Kristanto, Arbor Biotechnologies
  Type V CRISPR nuclease edit patterns enable highly sensitive off-target detection

- Douglas Smith, SeQure DX
  Sensitive Rearrangement Detection at CRISPR On- and Off-target Editing Loci Using SAFER Detection

- Chengzu Long, NYU School of Medicine
  Preventing large deletions and chromosome loss in engineered human primary T cells by CasPlus with optimized guide RNAs

- Jennifer Cherone, Altius Institute for Biomedical Sciences
  Potent and specific activation of fetal hemoglobin expression by cis-regulatory interference

- Selami Demirci, PhD, National Institutes of Health (NIH)
  Robust Genome Editing with Reduced Cytotoxicity and Enhanced Engraftment Using Protein-Assisted Delivery of Cas12a-RNP into Human HSPCs

- Daniel Brenner, Rice University
  Engineered Extrachromosomal Technologies for Tunable and Persistent Control of Therapeutic Gene Expression in Human Cells

- Kathleen Christie, Scribe Therapeutics
  Self-Targeting CRISPR-CasX-Editor AAV Vectors Enable Potent and Controllable On-target Editing in the Murine CNS

- Naseem Maghzian, Children's National Hospital
  CRISPR/Cas9 enables efficient knockout of CD52 without CD3/CD28 in expanded CMV-specific T cells

- Gerard Platenburg, PhD, ProQR Therapeutics B.V.
  ADAR-Mediated RNA Editing of Premature Termination Codon Results in Functional Correction in MECP2 for Rett Syndrome

- ARUN PARIPATI, Postdoctoral researcher, Nationwide Children's Hospital
  Novel U7 Small RNA mediated modulation of TCF4 Promoter activity as a therapeutic strategy for Pitt-Hopkins syndrome

- M. Leontien Van Der Bent, PhD, UniQure Biopharma B.V.
  Combined Targeting of HTT, HTT1a and Somatic Instability in Huntington's Disease using LinQURE®: a Feasibility Study in Multiple HD Mouse Models

- Jiayin Tian, PYC Therapeutics
  PYC-002, a Novel Antisense Oligonucleotide Targeting SHANK3 Haploinsufficiency in Phelan-McDermid Syndrome

- Christina Tyner, Vanda Pharmaceuticals Inc.
  First in Class ASO Targeting A53T Allele: Preclinical Efficacy

- Xiang Li, Entrada Therapeutics
  Exon 45 Skipping, Dystrophin Production, and Functional Improvement with ENTR-601-45 in Preclinical Models of Duchenne Muscular Dystrophy

- Caroline Johnson, Vanda Pharmaceuticals Inc.
  Translating IGHMBP2 Variants with a CMT2S Patient-Specific Model: Personalized Medicine Rescue

- John Lueck, University of Rochester
  Functionalized Nonsense Suppressor tRNA Picovectors Represent a Novel Therapeutic Cargo for the Treatment of PTC-associated Diseases

- Rashida Khan, Senior Scientist, Institute of Biomedical & Genetic Engineering Islamabad Pakistan
  Micro RNAs in the core arsenal of Molecular Paradigms in Papillary Thyroid Cancer Modalities

- Gerard Platenburg, PhD, ProQR Therapeutics B.V.
  ADAR-Mediated RNA Editing-Based Correction of PNPLA3 I148M Functionality to Address Hepatic Steatosis

- Mengyao Xu, UMass Chan Medical School
  Developing suppressor tRNAs to target the most common type of pathogenic nonsense mutation

- Adam Brooks, Wyatt Technology
  Optimizing LNP-RNA Formulations: Key Insights into Size, Zeta Potential, and Stability

- Danylo Sirskyj, Cytiva
  Large-scale Transfection of T cells in a Xuri™ Cell Expansion System W25 Bioreactor Using LNPs

- Jing Zhu, ReciBioPharm
  Insights of CMC, Quality & regulatory for mRNA-based gene editing program – A case study and lessons learned from early phase development to final GMP manufacturing for IND application

- Jing Zhu, ReciBioPharm
  Comprehensive evaluation of T7 polymerase for enhanced yield and quality in mRNA production

- Shilpi Agrawal, Post doctoral fellow, University of Arkansas

Developing a Cost-Effective and Reproducible Protocol for Lipid Nanoparticle Synthesis and Characterization to Advance Preclinical Research and Education

- Huu Thuy Trang Duong, Regeneron Pharmaceuticals
  Key learnings during mRNA-LNP drug product process development

- Alvin Jogasuria, ProBio Inc USA
  Enzymatic Linearized DNA – A Novel, Cell-Free Platform for Accelerated mRNA Therapeutic Development

- Andrea O'Hara, PhD, Azenta
  Effective Quality Control is Crucial Throughout mRNA Product Development

- Linda Gombos, Biomay
  mRNA Manufacturing: from Template DNA to Lipid Nanoparticles – an Integrated Platform Approach

- Reka Geczy, Precision NanoSystems Inc
  Versatile Lipid Nanoparticle Platform for Efficient CRISPR-Cas9 Gene Editing in Primary T Cells and CD34+ Hematopoietic Stem Cells

- Bryan Dafniet, WhiteLab Genomics
  Novel In Silico Generation of a Synthetic Peptide Library Derived from Biological Complexes for Protein and Vector Engineering

- Dylan SERILLON, WhiteLab Genomics
  Revolutionizing Peptide Discovery: AI-Driven Drug Design Paving the Way for the Future of Cell & Gene Therapy

- Chris DJamoos, Msc, Promega Corporation - Madison, WI

A Novel Technology for Sequence-Specific Determination of Lipid Nanoparticle Encapsulation Efficiency **  **

- Teresa Einhaus, Fred Hutchinson Cancer Center
  Comprehensive Imaging Methods to Quantify mRNA Lipid Nanoparticle Trafficking and Expression

- Laura Philips, PhD, Spheryx, Inc.
  Total Holographic Characterization: A New Tool for Empty/Full Ratio Determination

- Alex Schaffer, BS, ChromaTan Inc.
  High throughput process development for column-free purification of mRNA via BioRMB™ platform

- Yizong Hu, Massachusetts Institute of Technology
  Heterogeneous RNA Loading and Size Correlations in Lipid Nanoparticles Revealed by Single-Particle Spectroscopic Chromatography

- Ales Strancar, Sartorius BIAA Separations
  Orthogonal chromatography analytics to allow for faster AAV or LNP process development and better in-process control

- Rui Zhang, PhD, XGen Bio
  An End-to-End High-Throughput LNP Formulation Device Platform for mRNA & LNP Screening

- Jerilyn Izac, NIST: National Institute of Standards and Technology
  Advanced mRNA Lipid Nanoparticle Analysis by Flow Cytometry

- Xiulian Sun
  A Novel HPLC-ELSD Method for Lipid Identification and Quantification in mRNA-LNP Complexes

- Eric Ouellet, STEMCELL Technologies, Inc.
  Optimized CRISPR-Cas9 Editing of Primary Human Immune and CD34+ Hematopoietic Stem and Progenitor Cells using a Mechanoporation Platform

- Leonie Binder, German Cancer Research Center
  Exploring Mechanoporation for Efficient Genetic Modification Across Diverse Cell Types Using SMAR Vectors

- Christie Ciarlo, CellFE
  Improving Workflow Efficiency of CAR T Cell Gene Editing Using Microfluidic Delivery Technology

- Andy Tay, National University of Singapore
  Non-viral, high throughput genetic engineering of diverse human primary immune cells using nanostraw-mediated transfection

- Sven Mentink, Uneedle
  Precise intradermal and suprachoroidal delivery of gene and cell therapeutics using a silicon microneedle

- Kathrin Meyer, PhD, Alcyone Therapeutics
  ThecaFlex DRx™: Implantable Intrathecal Catheter and Subcutaneous Port System for Repeated Intrathecal Delivery of Nusinersen

- Michael Templin, Charles River - Reno
  MRI-Guided Focused Ultrasound: A New Drug Delivery Modality Addressing the Blood Brain Barrier

- Kourtney Kostecki, OpenCell Technologies
  Scalable Softporation™ platform for intracellular delivery to primary natural killer (NK) cells

- Jarryd Campbell, PhD, Recombinetics
  Swine Reporter Model for In Vivo Vector Characterization

- Bradley Davis, University of Guelph
  Charge Detection Mass Spectrometry of Large Biomolecular Assemblies: A Novel Tool for the Characterization of Gene Therapy Vectors

- Lohra Miller, Megadalton Solutions
  Advancements in Charge Detection Mass Spectrometry for Characterizing Large, Intact mRNA Structures and Optimizing mRNA Therapeutic Analytics.

- Steffen Honrath, ETH Zurich
  Closing the Gap: Boosting the TFAMoplex Transfection System with bZIP Domains allows Comparison with AAVs

- Barbara Bailus, Keck Graduate Institute
  Title ZIP Delivery: A Versatile Delivery Platform for the Central Nervous System

- Ross Wilson, University of California Berkeley
  Engineered CRISPR enzymes mediate highly efficient editing of neurons in mice and pigs

- Joseph Lavalla, Clemson University
  Peptide-Based Nanoplatform for Improved Cas9 RNP Delivery and Editing

- Rachel Cunningham, BS, Fred Hutchinson Cancer Center
  Non-viral Co-delivery of Long DNA Template and Cas9 Ribonucleoprotein into Primary Blood Cells

- Dylan Dautel, NChroma Bio
  E4/K4 Coiled-coils Improve Cargo Loading in Engineered Virus-Like Particles

- Karol Budzik, PhD, NChroma Bio
  Engineered Virus-like Particles with Minimal Viral Components for Improved Base Editing Efficiency in vivo

- Natalie Correa, Emory University School of Medicine
  Regulation of rAAV transgene expression using nanoparticle-delivered Cre-recombinase protein

- Léa DANDAN, Alaya.bio
  A VERSATILE PLATFORM FOR THE MANUFACTURING OF LENTIVIRAL VECTORS-BASED NANOPARTICLES FOR IN SITU GENE DELIVERY - AN INTEGRATED SOLUTION TO ACCELERATE ADVANCED THERAPIES INTO THE CLINICS

- Isabel Daher, MaxCyte, Inc.
  Efficient, Large-Scale Virus-Like Particle Manufacturing for Gene Editing by a GMP-Compliant Flow Electroporation Platform

- Lavelay Kizekai, Waters Corporation
  Capsid and Virus-Like Particle Biophysical Characterization by High Sensitivity, High Resolution SEC-MALS: Case Studies on QBeta, Hepatitis B, and Recombinant Protein VLP Control

- Erin McAllister, Waters Corporation
  Virus-Like Particles: Advanced Insights through Charge Detection Mass

Spectrometry (CDMS)

- Tim Fouts, ABL Inc.
  Development of a Stable Lyophilized Formulation of a Quadrivalent Frame-Shift Peptide Vaccine for Cancer Prevention in Lynch Syndrome

- Sacha Robert, University of Minnesota
  Evaluation of Immunomodulatory Effects of Oncolytic Adenovirus Therapy in Immunocompetent Hamster Models of Metastatic Breast Cancer.

- Rachel Ginther, MRIGlobal
  Development and Validation of a Six-Plex Quantitative PCR Potency Assay for an Anti-Breast Cancer DNA Plasmid Vaccine

- Jason Ballon, Sarcoma Oncology Center
  Phase 2 Study Using Talimogene Laherparepvec, Nivolumab, and Trabectedin for Advanced Leiomyosarcoma and Liposarcoma (NCT03886311)

- Judit Sanchez Gil, MASSACHUSETTS GENERAL HOSPITAL
  Targeting Adenosine Immunosuppression in Glioblastoma with Oncolytic Virotherapy

- Ying Cheng Chen, Purdue University
  Evaluating IL27-expressing Oncolytic Adenovirus for Treating Advanced Prostate Cancer

- A-Rum Yoon, Hanyang University
  Intranasal Delivery of IR-780-Conjugated Oncolytic Adenovirus: A Non-Invasive Approach Combining Virotherapy and Photothermal Therapy for Glioblastoma Treatment

- Soojin Kim, Articure Inc.
  Overcoming Recombination in Semi-Replicating Retroviral Vectors: A Novel Double Suicide Gene Therapy for Glioblastoma

- Jacqueline Lara, City of Hope
  Stem Cell Delivered Viro-immunotherapy Advancements for Ovarian Cancer Treatment

- Margarita Romanenko, PhD, University of Minnesota
  A Novel Oncolytic Adenovirus-based Therapy for Pediatric Neuroblastoma

- Yoya Vashi, City of Hope
  A Highly Tumor-Specific Oncolytic Virus in Combination with a Viral Protein-Targeted T Cell Engager for the Treatment of Triple-Negative Breast Cancer

- Gurmeet Kaur, City of Hope
  Neural Stem Cell Delivered CRAd-S-pk7 Dose Interval Optimization for Ovarian Cancer in an Immunocompetent Mouse Model

- Derek Korpela, Recombinetics Inc
  Somatic Cell Gene Editing for Translational Preclinical Cancer Modeling in Swine

- Felipe Montano-Campos, University of Washington
  Cost-Effectiveness of Gene Therapy for Sickle Cell Disease in Uganda: Tailoring High-Income Evidence to Uganda's Context

- Ceera Manikandan, Senior Research Fellow, Vellore Institute of Technology
  Therapeutic Potential Of NF-κB Inhibition In Glioblastoma: Gene Therapy Approach Using rAAV-5 Mediated IκBαM Overexpression

- Rashmi Rana, Institute of Liver and Biliary Sciences
  AK2, COL1A1, and PLG protein signatures: targeted therapeutics for meningioma

- Lei Zhao, Cure Genetics
  Tumor-specific AAV Delivery of Interleukin-12 Enhances Antitumor Immunity and Safety in Ovarian Cancer

- Vlad Seitan, Laverock Therapeutics
  Programmable iPSC-derived monocytes for the treatment of solid tumours

- Ilana Chefetz Menaker, Mercer University
  Identifying a target for CAR therapy in tumor-initiating cells

- Scott Leighow, PhD, Penn State University
  Programmed evolution: Delivery of asexual gene drives to sculpt tumor populations and combat genetic diversity

- Wonbeom Park, PhD, Sungkyunkwan University
  DEC205-Targeting mRNA Cancer Vaccines for Enhanced Antigen Presentation in Dendritic and Tumor Cells

- Nagarathinam Selvaraj, Biopharmaceutical Development program, Frederick National Laboratory for Cancer Research (FNCLR), NIH
  Development of a Novel Process which Combines Lentiviral Transduction and Base Editing for the Production of Highly Effective and Potent TCR T Cell therapy Products

- Jill Rosenberg, Regeneron Pharmaceuticals
  COMET, a novel TCR-enabled architecture, combines the sensitivity and intracellular targetability of a TCR with the ease of engineering of a CAR

- Hua Zhang, SPH Biotherapeutics (HK) Limited
  DLL3-targeting CAR co-deletion of PD1 results in enhanced T cell cytotoxicity and persistence in a pre-clinical model for small cell lung cancer.

- Collin Walter, Regeneron Cell Medicines
  A Novel Approach To Preclinical Safety Assessment Of TCRs, Pairing Quantitative Immunopeptidomics With Cell-Based Functional Assays, Identified a Development-Limiting Risk Of Cardiotoxicity for a PRAME TCR

- Julie Garcia, Moonlight Bio
  *CARD11-PIK3R3 Potency Enhanced p53R175H-HLA-A*02:01 TCR T Cell Therapy: Overcoming Barriers in Solid Tumor Treatment*

- Yuxiao Wang, PhD, MyeloidTx
  Novel Innate Immune Receptor-Based Chimeric Antigen Receptors For In Vivo Programming Of Myeloid Cells Show Potent Anti-Tumor Activity In Preclinical Solid Tumor Models

- Thomas Treiber, Immatics Biotechnologies GmbH
  ACTallo: Developing an Allogeneic T Cell Therapy Platform Using Engineered γδ T Cells

- Marta Soria Castellano, IDIBAPS
  Engineering Inducible Signal3 Chimeric Cytokine and Antigen Receptors for T Cell Therapy in Solid Tumors

- Sebastian Snedal, H. Lee Moffitt Cancer Center and Research Institute
  A Novel T Cell Receptor Targeting Cancer Testis Antigen NY-ESO-1

- Mayra Cruz Tleugabulova, Genentech, Inc
  Multiplexed Engineering for Generation of HLA-Class I Restricted CD4 T Cells

- Qi Zhao, Enrich Biosystems Inc.
  AI guided tumor reactive cell isolation using immune cell tumor microcultures

- Robert Keefe, PhD, BluesphereBio
  Development of Novel TCR Based Cell Therapies Targeting Tumor Specific Mutation in AML.

- Amrik Kang, BS, University of California San Francisco
  Deep Learning Algorithm-Guided Optimization of a Natural Ligand-based CAR-T against CD70 in Acute Myeloid Leukemia

- Laura Griffin, Taconic Biosciences
  Super Immunodeficient NOG-EXL Mice Have Enhanced Patient-Derived Xenotransplantation of Acute Myeloid Leukemia: An Ideal Model to Test Novel Therapies

- Quanjun Yang, Shanghai Jiao Tong University Affiliated Sixth People’s Hospital
  Asparaginase Coupled CAR-T Cells Overcome ASNS Mediated Resistance to CAR T Cell Therapy in Leukemia

- Rachel Bito, Children's Hospital Los Angeles
  Advancing AML CAR-T: Transposon Based CAR-T Cells

- Marta Soria Castellano, IDIBAPS
  Development of ARI-007: CD7negative Anti-CD7 CAR-T Cells for the Treatment of T-Cell Malignancies

- Haifeng Chen, PhD, Virovek Incorporation
  In Vivo CAR-T Cell Therapy Using Modified AAV Capsids Targeting Human T Cells

- Michael Coffey, Poseida Therapeutics
  Low Dose Methotrexate as a Combinatorial Therapy with mDHFR^{+} Allogeneic CAR-T Cells

- Ambalika Chowdhury, Graduate Student, Indian Institute of Technology Bombay
  Novel Humanized anti-CD20 CAR-T cells Demonstrate Robust Efficacy in B-cell Lymphoma in Preclinical Studies.

- Shlomo Elias, Hadassah Medical Center
  The human natural killer (NK) cell line YTS as a novel platform for cellular therapy

- Atsushi Okuma, Hitachi, Ltd.
  DesignCell® Platform for Optimal CAR Discovery

- Megan Blair, Verismo Therapeutics
  SynKIR-310 Provides a Novel Split-Signal CAR T Platform (KIR-CAR) Based upon Natural Killer Cell Signaling to Prolong Anti-Tumor Treatment in B-NHL

- Rachel Hedinger, AskBio
  Immunobiology of Adeno-Associated Virus (AAV) in Murine Liver: A Cross-Sectional Comparison of AAV8, AAV6, and a Chimeric Liver-Tropic Capsid in a Preclinical Model

- Anne-Sophie Kuhlmann, PhD, Fred Hutchinson Cancer Center
  A Multitherapeutic Approach Combining Ex vivo and In vivo Cell Engineering to Treat HIV in Nonhuman Primates

- Leah Cardwell, PhD, Ultragenyx
  Cross-reactive Immunologic Material (CRIM) Analyses for AAV Gene Therapy

- Raphaela Bento, Massachusetts General Hospital - Harvard Medical School
  Genetically Engineered Organs for Early Reporting of Transplant Rejection

- Hannah Pizzato, University of Arizona
  Towards a Universal Cell Replacement Therapy: Engineering Human Pluripotent Stem Cell Lines to Evade Xenogeneic Transplantation Barriers

- Jocelyn Schroeder, Medical College of Wisconsin and Versiti Blood Research Institute
  The Effects of Antigen-Specific $CD8^+$ T Cells on Successful Engraftment in Platelet-Targeted Gene Therapy

- Taishi Kimura, HDT Bio
  A localizing nanocarrier formulation enables anti-enterovirus antibody responses from multivalent replicon RNA by limiting systemic interferon responses

- Irafasha Casmil, The University of British Columbia
  Alphaviral Genetic Background of Self-Amplifying RNA Influences Protein Expression and Immunogenicity Against SARS-CoV-2 Antigen

- Manfred Kraus, Kernal Biologics
  T Cell Targeted mRNA tLNPs Drive Fast, Selective and Highly Effective in situ CAR T Cell Programming

- Robert Freeborn, Tr1X, Inc.
  CD19-CAR and IL-10 Reprogramming of $CD4^+$ T Cells Generates Type 1 Regulatory CAR-T Cells for the Treatment of Autoimmune Disease

- Sachie Ikegami, Hoxworth Blood Center/University of Cincinnati

Role of $CD4^+$ T cell and NK cell subpopulations in CMV IFN-γ production in CMV-specific T-cell products

- Lisa Schiller, Technical University of Munich
  Overcoming Barriers for T-cell Therapy of Chronic Hepatitis B Virus Infection

- Ian Hay, Factor Bioscience
  UltraSlice Gene-Editing mRNA Enables Efficient Transgene Insertion into TRAC Locus in Primary Human T Cells

- Reyes Acosta, University of Pennsylvania School of Medicine
  Second Generation $CD4^+$ CAR T Cells Incorporating a 2B4 Cosignaling Domain Exhibit Enhanced Proliferation and $CD8^+$ CAR Help

- Hang Su, Albert Einstein College of Medicine
  Synergizing Broadly Neutralizing Antibody-Secreting T Cells and CAR-T Cells Against HIV-1 Infection

- Atishay Mathur, USC
  Enhancing Engineered B Cells through Expression of B Cell Activating Molecules

- Luhan Yang, PhD, Qihan Biotech
  Enhanced CART Function by Arming CAR-T Cells with Cytokines

- Edo Kon, RiboX Therapeutics
  In Vivo Generation of anti-CD19 CAR T Cells Utilizing Circular RNA Encapsulated in Targeted Lipid Nanoparticles

- Lawrence Lamb, MN, PhD, In8Bio
  INB-600: A Novel T Cell Engager Platform Specific for gamma-delta (γδ) T cells

- Jia Wei, Sonoma Biotherapeutics
  Treg Specific Synthetic Promoters (TRSP) for Antigen Specific Treg Therapy

- Amanda Conerty, BS, CLS (ASCP) CABP, Artiva Biotherapeutics
  AlloNK: A Scalable and Consistent NK Cell Therapy in Development for Treatment of Oncology and Autoimmune Diseases

- WEIWEI MA, Unicet Biotech
  Preclinical Development of Allogeneic CD19 CAR-γδT Cells for the Treatment of B-cell Malignancies and Autoimmune Diseases

- Tea Soon Park, SereNeuro Therapeutics
  Advancing Non-Opioid Pain Management for Osteoarthritis: SN101 Nociceptor Cell Therapy Approach

- Jean-Sébastien DIANA, APHP
  ex vivo Gene therapy for Artemis-SCID and leaky-SCID patients: results of the French ARTEGENE phase I/II clinical trial

- Payam Zarin, GentiBio
  A stable Engineered Regulatory T cell therapy platform with modular targeting for autoimmune and inflammatory diseases

- Mary Spellman, Panclarity LLC
  A Novel Staphylococcus epidermidis Compound for the Topical Treatment Epidermal Growth Factor Receptor (EGFR) Inhibitor-Induced Dermal Toxicity

- Steven Montecinos, NJ Child Health Institute
  Reaching Remission: Harnessing Master Regulator Transcription Factor Zbtb20 for the Development of a Cellular Therapy against IBD

- Louai Labanieh, PhD, Mount Sinai School of Medicine
  STASH-Select: a platform for multi-vector engineering and single-step selection of cell therapies bearing multiple enhancements

- Anushka Dikshit, Advanced Cell Diagnostics, Inc. - Newark, CA
  Cell therapy biodistribution and efficacy assessment using a highly sensitive spatial multiomics assay

- WEIWEI MA, Unicet Biotech
  Allogeneic CAR-γδT cells armored with 'super-mbIL15' demonstrates enhanced efficacy towards both liquid and solid tumors

- Chen Huang, PhD, Shanghai Juncell Therapeutics
  Development of A Feeder-Free Process for IL-2-Independent TIL Expansion

- Pin Wang, PhD, University of Southern California
  Enhancing Production and Functional Potential of Tumor-Infiltrating Lymphocytes via Lipid Nanoparticle-Mediated CRISPR/Cas9 Gene Editing

- Kenta Ishimoto, Kyushu University
  Metabolic Alterations in Highly Activated NK-like Cells (GAIA-102) Through Cryopreservation

- Liz Mutter-Rottmayer, PhD, Genentech
  Nonclinical Safety Evaluation of a KRAS G12D Neoantigen-Targeting TCR

- Patrick Au, PhD, Ensoma
  Acute safety and biodistribution profile of hematopoietic stem cell (HSC) targeting virus-like particles based on helper-dependent adenovirus serotype 5/35++ in non-human primates

- Sravya Kattula, Ensoma
  Novel In Vivo Gene Therapy Approach to Hematopoietic Stem Cell (HSC) Engineering Creates Durable HSC-Derived Neutrophils to Treat X-Linked Chronic Granulomatous Disease

- Denise Klatt, PhD, Boston Children's Hospital
  In Vivo Hematopoietic Stem Cell Gene Therapy for Sickle Cell Disease Using BaEVRLess-pseudotyped Viral Vectors

- Karthik Karuppusamy, PhD, University of Washington
  In Vivo Truncation of the EpoR Gene Allows for Expansion of Therapeutically Edited Erythroid Cells after HSC Gene Therapy with HDAd Vectors

- Chirayu Chokshi, Ensoma
  In Vivo Engineering of Hematopoietic Stem Cells with Virus-Like Particles to Generate Multi-Lineage CAR Immune Cell Therapy for Cancer

- Esther Chen, Capstan Therapeutics
  Effective Gene Editing in Hematopoietic Stem and Progenitor Cells (HSPCs) through a Novel Targeted Lipid Nanoparticle

- Nafiseh Nafissi, University of Waterloo/Mediphage Bioceuticals
  Chromatographic purification of linear DNA from supercoiled plasmid DNA

- Yeonji Kim
  Enhancing Lentivirus Depth Filtration Purification: Strategies for Improved Recovery and Quality for Downstream

- Sujeong Yang, Astrea Bioseparations Ltd
  Advancing Purification Strategies for Large Viral Modalities Using Nanofiber

Adsorbent Technology

- Glenda Dickson, ViroCell Biologics, 12-18 Theobalds Road, London, WC1X 8SL, UK
  Lenti Viral Vectors Displaying Ligands for Enhanced Capture, Purification, Concentration and Targeted Infection of Specifically Selected Cells

- Shanshan Zhu, ABL Inc.
  ABL's HIV p24 ELISA Kit: a Solution for Lentivirus Quantification in Industrial Processes

- Yanis Habtoun, WhiteLab Genomics
  A Lentiviral Vector Downstream Processing Analytic Tool Powered by Knowledge Graph and LLM Technology

- Julie Erwin, ProBio Inc USA
  Quality by Design in Plasmid DNA and Gene Therapy: ProBio CDMO’s Digital QMS for Risk-Based Manufacturing Excellence

- Luca Nanni, NewBiologix
  A Novel Pipeline for the Genomic Characterization of Cell Lines.

- Marian McKee, Eurofins BioPharma Product Testing
  Harnessing the Power of Next Generation Sequencing and Bioinformatics to Propel Viral Safety of Cell Therapies

- Philemon Asfeha, ProBio Inc USA
  Optimization and Comparative Analysis of Host Cell DNA Quantification and Plasmid Supercoiled Percentage: Cost-Effective Agarose Gel Electrophoresis vs. High-Sensitivity qPCR and IEC-HPLC

- Erin Cross, Kromatid Inc
  Genomic Integrity Analysis of Starting Material using Advanced Cytogenetic Analysis for Donor, Cell Line, and/or Clone Screening and Qualification

- Michele Vicentini-Hogan, Lonza
  End-to-End Production of T Cells on the Cocoon® Platform, Using Buoyancy-Activated Cell Sorting™ for T Cell Isolation from Leukopaks

- Crisha Barrett, MS, Akadeum Life Sciences
  Healthier Cells with Akadeum's Alerion Microbubble Separation System: A Novel Approach to Gentle and Efficient Cell Isolation

- Supriya Prakash, ElevateBio
  Peripheral Blood Mononuclear Cell (PBMC) Isolation Using Cell Fractionation Filters

- Bob Snyder, Ph.D., MBA, Proteios Technology, Inc.
  Rapid Isolation of Immune Cells for Cellular Therapeutics Using Multiple Antigen Criteria

- Ping Wei, Catalent Pharma Solution
  Comparison of Isolation Methods of CD3+ T Cells from Leukapheresis

- Eleni Papanikolaou, PhD, Miltenyi Biotec B.V. & Co. KG
  Novel Automatic Application for Enrichment of $CD34^+$ Hematopoietic Stem and Progenitor Cells from Apheresis Products

- Pushpalatha Chaluvappa, Thermo Fisher Scientific
  From Fresh to Frozen and Everything in Between: Revolutionizing WBC Isolation from Leukopak and Whole Blood with the Rotea Counterflow Centrifugation System

- Maetja Verbarendse, Thermo Fisher Scientific
  Novel Detection Method for Residual Beads in CAR T-Cell Manufacturing Using Imaging Flow Cytometry

- Donald Beers, Entegris Inc.
  Method Development and Scale Prediction for Separation Systems to Solve Challenges in Allogeneic Scale-up

- Thomas Quinn, MS, Takara Bio USA
  Streamlined Ex-Vivo Engineering of Human T Cells: A Single-Step Approach to Activation and Lentiviral Transduction

- Eleni Papanikolaou, PhD, Miltenyi Biotec B.V. & Co. KG
  Maximizing Safety and Efficacy in Hematopoietic Stem Cell Gene Therapy

- Ashley Strickland-Dietz, Na
  Optimizing CRISPR/Cas-mediated CAR Knock-in in Primary Human T Cells Using MaxCyte® Electroporation

- Brock Binkowski, Promega Corporation
  A Versatile Platform for Cell Therapy Cytotoxicity Assays Using mRNA Transfection

- Tianqi Jiang, Lingyi
  Establishment of The Inducible AAV Production Cell Line 293RSS2.0

- Xiaofei Gao, Westlake University
  The First Coacervate-Based Delivery System for CAR-T Cell Engineering and Manufacturing

- Caitlin Edwards, Charles River
  Development of a Quantitative Screen to Assess AAV On/Off-Target Binding and Transduction.

- Isabel Brandtjen, Rutgers University
  A Kinetic Perfusion Assay to Measure Gene Switch Responses in vitro

- Despina Lymperopoulou, Thermo Fisher Scientific
  Rapid Mycoplasma Detection Techniques for Facilitating Quality in Low-Volume T Cell Therapies

- Julyana Acevedo, Sangamo Therapeutics Inc
  Assessment of Adeno-Associated Virus (AAV) Purity by Capillary Electrophoresis-Based Western

- Gregory Preston, Alexion Pharmaceuticals Inc.
  Seroprevalence, Correlation, and Clinical Relevance of Total and Neutralizing Anti-AAV Antibodies and Assay Formats

- Jeff Nelson, PhD, Promega Corporation
  Novel Bioluminescent Assays for Detecting Anti-AAV Neutralizing and Total Antibodies in Serum Samples

- Joshua Baal, PROGEN Biotechnik GmbH
  Anti-Pan AAV – New Versatile Antibody for the Detection of Various AAV Serotypes Including Novel Capsids

- Jordi Rodo, Svar Life Science
  Developing a methodology for seropositivity assessment through sensitive anti-AAV total antibodies ELISA

- Jordi Rodo, Svar Life Science
  A Novel Tool for Complement Activation Profiling in AAV Gene Therapy

- Kara Machleidt, PhD, Promega Corporation
  Development of a Novel Luminescent Assay for Sensitive and Specific Quantitation of Double-stranded RNA

- Praveensingh Hajeri, PhD, University of Minnesota
  A Novel Next-Gen Sequencing Library Preparation Method To Sequence Low-Quality (Fragmented/ Degraded) With Low (Pico-Grams) Quantity Of DNA/RNA Templates For Better Diagnostics, Biomarker Discovery, And Research.

- Johannes Solzin, Boehringer Ingelheim Pharma GmbH & Co KG
  Development of a Novel High-Throughput Determination of Infectious Virus Titers by Kinetic Measurement of Infection-Induced Changes in Cell Morphology

- Chengjing Liu, Thermo Fisher Scientific
  Enhancing Robustness in rAAV Characterization with Optimized Workflows and PEA Technology

- Renzo Adilardi, Advanced Cell Diagnostics, Inc.
  Automated co-detection of small RNAs, mRNAs and proteins with a new RNAscope™ assay on the Roche DISCOVERY™ ULTRA using translucent chromogens

- Aparajita Chatterjee, MilliporeSigma
  Strategies for Overcoming Analytical Challenges in Complex Upstream AAV Samples

- Anna Baoutina, PhD, National Measurement Institute

Massively parallel sequencing-based method to detect misuse of gene transfer technologies in sport

- Amanda Oran, SKPT
  Confirmation of Lentiviral Vector Identity Using Short-Read Next-Generation Sequencing

- Swetha Devi Velivela, AskBio
  Optimizing AAV Sample Preparation Workflow for Short and Long Read NGS for AAV Characterization.

- Kevin Chen, Genzyme, a Sanofi Company
  Development of a Novel Scale-Down Model for High-Throughput Clone Selection for Adeno-Associated Virus (AAV) Production

- Francesca Rossetti, AGC Biologics - Milan
  Automated solutions for characterization and release of ATMPs

- Ali Fallahi, Northeastern University
  A Solid-state Nanopore Mesh Device for Quantification and Quality Control of Carrier Particles Used in Gene Therapy

- Xi Qin, National Institutes for Food and Drug Control of China
  Methodological validation of sedimentation velocity analytical ultracentrifugation method for Adeno-Associated Virus and collaborative calibration of system suitability substance

- Varun Gejji, Capsida Biotherapeutics Inc
  Development of a Novel Automated Loading Approach Which Significantly Reduces Processing Time for Enriching Full AAV Capsids Using Ultracentrifugation.

- Xiaoling Chen, Wuxi Extpure Solutions Co., Ltd.
  Purifying AAV Free of Endonuclease Treatment & Pre-Capture TFF

- Anne Blackwell, Agilent Technologies
  Size Exclusion Chromatography of messenger Ribonucleic acid, Adeno-Associated Virus and Virus-Like Particle Analysis using Newly Developed Wide Pore Size Exclusion Chromatography Columns

- Rachel Ginther, MRIGlobal
  High Throughput Analysis of CRISPR sgRNA using Ultrashort Ion Pairing Reversed Phase Column Chromatography and High Resolution Mass Spectrometry

- Brittany Sangiovanni, Bachelors, Akron BioManufacturing
  Dual Cation Exchange coupled with Multi-Modal Chromatography Allows the Production of High-Purity and Low residuals cGMP-Grade Cas9 Nuclease for Cell Therapy Applications

- Jacob Cardinal, Alexion Pharmaceuticals Inc.
  High-Throughput Developability Assessment of AAV Drug Products: A Predictive Approach

- Marco Thomann, Roche
  Upgrading Our Toolbox: New Analytical Tools to Support rAAV Vector Bioprocess Development and Product Understanding

- Benjamin Graf, Sartorius Lab Instruments GmbH & Co KG
  Next Generation digital PCR Technology for Sterility Testing of Cell and Gene Therapy Products

- Brianna McManus, MilliporeSigma

Using Next Generation Sequencing to Test Raw Materials used in Biologics Manufacturing

- Sabine Geiger, Ascend Advanced Therapies
  Extraction-free ddPCR quantification of host-cell derived E1A impurities in rAAV samples

- Jesse Kay, PhD, Progen Biotechnik GmbH
  Rapid and Versatile Analysis of AAV Samples Using Lateral Flow Assays: Applications in Gene Therapy Development

- Xiulian Sun
  A Novel AAV TCID50 Testing Method Using Replication-Deficient Helper Adenovirus and Inducible 293 Cell

- Alison Hannah, Consultant
  Phase 1 Study of GEN2, A Personalizing Gene Therapy Vector, in Adult Patients with Locally Advanced or Metastatic Solid Tumor Malignancies

- Sant Chawla, Sarcoma Oncology Center
  Analysis of Clinical Benefit for a Phase 2 Basket Study Using DNG64 Cyclin G1 Inhibitor Gene Vector + Immunotherapy for CCNG1 Oncogene Expressing Tumors Predicts Successful Efficacy Endpoints (NCT04091295)

- Dae-Won Kim, PhD, ICM Co., Ltd. & Yonsei University
  Interim Analysis of a Phase 1/2a Study of ICM-203 AAV Gene Therapy for Osteoarthritis

- Allen Reha, Vironexis Biotherapeutics
  Design of the First Clinical Trial of AAV Gene Therapy in Immuno-Oncology (SENTRY-ALL): A Single Dose CD3/CD19 Bi-Specific T-Cell Engager for the

Treatment of CD19+ B-Cell Acute Lymphoblastic Leukemia

- Annahita Keravala, PhD, Genascence Corporation
  A Randomized, Double-Blind, Placebo-Controlled Dose-Ranging Phase 1b Study Evaluating Safety, Tolerability, and Pharmacodynamics of a Local AAV-Mediated Anti-Interluekin-1 Gene Therapy in Subjects With Knee Osteoarthritis: 6-Month Interim Results

- Cecilia Marinova, Medasol
  Patients perspective on their cross-border personalized treatment experience

- Michelle Lorentzos, Sydney Children’s Hospitals Network
  The Kids Advanced Therapeutics Program – Responding to the Need for Systems Readiness in the Gene Therapy Translation Pathway

- Maryam Bemanalizadeh, Tehran University of Medical Sciences
  Over a Decade of Gene Therapy Trials: Evolution from 2010 to 2025

- Carolyn Chapman, PhD, MS, The Multi-Regional Clinical Trials Center of Brigham and Women's Hospital and Harvard
  Ethical, legal, and social issues (ELSI) in human somatic gene therapy clinical research: A Scoping Review

- Andrea Boitnott, BS, UT Southwestern Medical Center
  Safety and efficacy results from a phase I/II open label gene therapy trial for Spastic Paraplegia 50 (NCT05518188).

- Steve Winitsky, Parexel International
  Deconstructing FDA’s Expedited Program Determinations: Analyzing how the Agency may Set the Bar for RMAT Designation

- Ananta Ayyagari, Nanoscope Therapeutics
  Accelerated Regulatory Approvals in Cell and Gene Therapy

- Rafael Escandon, PhD, DGBI Consulting
  Trade Secret Protections in Early Stage Gene Therapy Research: Patient Harm Through Limited Transparency?

- Biao Zheng, Ltd
  Efficacy and Safety of BRL-101, CRISPR-Cas9-mediated Gene Editing of The BCL11A Enhancer in Transfusion-dependent β-thalassemia and Severe Sickle Cell Disease

- Patrick Derigs, German Cancer Research Center, Heidelberg, Germany
  Preclinical Development of CD33-Null Hematopoietic Stem and Progenitor Cells for Enhanced CD33-Directed Immunotherapy in Acute Myeloid Leukemia

- Zixuan He, Changhai Hospital of Shanghai
  Safety and Efficacy of Umbilical Cord-Derived Mesenchymal Stem Cells (TH-SC01) in Patients with Perianal Fistula in Crohn's Disease: Phase 1 and 2 Clinical Trials

- Alice Aguiar, OXB
  Evaluation of Salt-Tolerant Endonucleases for Lentiviral Vector Purification

- Melissa Rangel, C-LEcta GmbH
  High Salt, High Impact: An Endonuclease Engineered for High-Efficiency DNA Removal in Viral Vector Manufacturing

- Emma Gerstmann, University Hospital Heidelberg
  Characterization of 27 Primate and Non-Primate rAAV/BoV Vectors in vitro

- Tim McClain, C-LEcta GmbH
  The Challenge of Comparability: The benefit of common release standards for Serratia marcescens endonucleases.

- Abdullah Sufan, Tozaro Limited
  A New Approach to Designing Synthetic Affinity Ligands for Enhanced Viral Vector Purification

- Shandel Pariag, Pharmaron Biologics, Liverpool
  State-of-the-Art Mass Spectrometry Characterisation of Adeno-associated Virus (AAV) by Intact Mass, Peptide Mapping, Host Cell Protein and Metabolomics Analysis

- Jae Hwang, SK Pharmteco - US
  Streamlining Downstream Lentiviral Vector Production: A Novel IEX Platform for Improved Recovery, Infectivity, and Purity

- Luka Bevc, Sartorius BIA Separations
  Advancements in Lentiviral Purification: From High Throughput Screening to Scalable Solutions

- Alan Griffith, VectorBuilder
  Hedge Your Bets in Genetic Roulette with cliniVec: Accelerating Cell and Gene Therapy Development Through Optimized Vector Design and Characterization

- Praveensingh Hajeri, PhD, University of Minnesota
  Host and viral (non-viral genomic) DNA/RNA contamination in viral particles produced for clinical applications under GLP/GMP and profiling of RNA/DNA packaged inside viral particles.

- Miroslav Vranes, QIAGEN

Enhanced Lentiviral Vector Characterization Using Digital PCR: Genome Titer, VCN, and RCL Detection

- Agnieszka Lass-Napiorkowska, PhD, Eng, MilliporeSigma
  Importance of AAV sample preparation for gc/ml titer determination using PCR.

- Henry Kang, SCIEX
  A new approach for high-resolution full and empty AAV capsid analysis using a high throughput method for comprehensive AAV evaluation on a single CE platform

- Tomas Andreani-Faborni, Sarepta Therapeutics, Inc.
  Enhancing rAAV genome integrity through process development optimization

- Steven Le, Halo Labs
  Counting, Sizing, Imaging, and Identifying Viral Vector Product Aggregates at Ultra Low Volume

- Martine Barros, Sartorius
  Tangential Flow Filtration membranes for robust ultrafiltration and diafiltration in Lentiviral vectors downstream processing

- Giuliana Vallanti, Dr., AGC Biologics
  Development of a Fully Closed and Sterile Manufacturing Process for Lentiviral Vectors: Eliminating the Need for Sterile Filtration

- Tahsin Jahan, MilliporeSigma - Carlsbad,CA
  High-Throughput Flow Cytometry Assay for Quantifying Infectivity of AAV, Adenovirus, and Lentivirus Vectors in Gene Therapy Applications

- Jakob Shoti, University of Florida
  STRATEGY FOR IMPROVING THE RESCUE, REPLICATION, AND PACKAGING OF GENERATION Z (GenZ) SINGLE-STRANDED AAV VECTORS

- Chunyan He, Suzhou GenAssist Therapeutics Co., Ltd
  A Novel AAV Vector Design for Reducing Cross-Packaged ITR Promoter Activity

- Tam Duong, Lonza Houston Inc.
  Adeno-Associated Virus characterization using Nanopore long read sequencing and analysis

- Taylor Gunnels, Eli Lilly and Company
  Evaluating Plasmid Architectures for Improved rAAV Production in HEK293s

- Holly Appleton, Novartis Gene Therapies
  Differential Effects of AAV GOI Plasmid Input on the Yield and Impurity Profiles of Single-Stranded Versus Self-Complementary AAV

- Kelly Fagan, Gordian Biotechnology
  Long-Read Sequencing Identifies Novel Class of Contaminants in Adeno-associated Viruses

- Yuanmei Ma, PhD, Eli Lilly and Company
  Characterization of the AAV production kinetics from a recombinant baculovirus and insect cell platform

- Sven Mathias, Sartorius Stedim Cellca GmbH
  Balance is Key: Optimization of Rep Protein Levels for Superior rAAV Production.

- Aysegul Atasoy Zeybek, Assistant Professor of Physical Medicine & Rehabilitation,

Mayo Clinic
Encapsulation of Adeno-Associated Virus-2.5 in Extracellular Vesicles Improves Transduction Efficiency to Human Chondrocytes in vitro

- Risa Shibuya, Osaka University
  Serotype-Dependent Optimization of Formulation for AAV Vectors Based on Stability against Aggregation

- Katrina Costa-Grant, Oxford Biomedica Solutions LLC
  The Impact of Individual Helper Genes on AAV Productivity

- Yi Zhao, Takara Bio USA
  Expedited Quantification of AAV Titers Using a Single-Wash ELISA Assay

- Zhuolun Yang, Osaka University
  Correlation between Deamidation and Structure of Adeno-Associated Virus Vectors

- Daniel Koback, Sartorius
  Advancing Gene Therapy: Stabilization of Viral Formulations with Recombumin® Human Albumin

- Kirk Twaroski, PhD, FUJIFILM Cellular Dynamics
  Human iPSC-derived Cells as a Platform to Determine AAV Transduction Efficiency

- Angga Kusuma, PhD, UniQure Biopharma
  Enhanced Gene Transfer to Brain Parenchyma via Next-Generation AAV5 Capsids

- Patrick Aldrin-Kirk, RAAVen Therapeutics

Rewriting Tropism: Engineered AAV-Kingfisher Capsids show Superior Oligodendrocyte Targeting and Spread

- David Ojala, Sangamo Therapeutics, Inc.
  Characterization of Receptor-Targeted Blood-Brain Barrier Penetrant AAV Capsids

- Changfan Lin, Caltech
  Harnessing Human Brain-Specific Carbonic Anhydrase IV for Targeted AAV Gene Delivery

- Xiaodong Lu, MS, Biogen
  Engineering AAV vectors with TfR1-targeting domains for CNS gene delivery in cynomolgus macaques and humanized mice

- Elad Firnberg, PhD, REGENXBIO Inc.
  Blood-Brain Barrier Crossing AAV Vectors Targeting the Transferrin Receptor Engineered Using Two Different Approaches

- Andrew Steinsapir, BS, Deerfield Management
  Expanding Access to Gene Therapies for Neurometabolic Disorders: A Platform Approach Using TfR1 CapX

- Sherry Cao, Affinia Therapeutics
  Novel AAV capsids that bind human transferrin receptor (TFRC) demonstrate widespread and preferential CNS tropism in TFRC-KI mice after low dose systemic dosing

- Ken Chan, PhD, Broad Institute of MIT and Harvard
  Second Generation Human Transferrin Receptor Targeted AAV Capsids With Enhanced CNS Tropisms and Liver Detargeting In Vivo

- Allison Dane, AskBio
  Combining In Vivo and In Vitro Screening Approaches for Identification of BBB-Crossing Capsids Across Species

- Sherry Cao, Affinia Therapeutics
  Engineered AAV capsids that target a novel human brain endothelial receptor achieve robust transduction in non-human primate central nervous system after intravenous dosing

- Yuan Yuan, PhD, Biogen
  Optimized CNS-targeting AAV Capsids with NHP and Human Cross-reactivity

- Bing Wang, AAVnerGene Inc
  AAV-ShDs: Liver-detargeted AAV Capsids with Strong Cross-species Lung Tropism and High BBB Activity in Non-human Primate and In Vitro Human Models

- Xinxu Yuan, Virginia Commonwealth University
  Cross-species Compatibility of Novel AAV-R2e-MAC to Cross Blood-brain Barrier and Transduce Myeloid Cells in the Central Nervous System

- Sachiko Okamoto, PhD, Takara Bio Inc.
  Efficient Neuronal Transduction of AAV2-derived CereAAV.YN Vector in Cynomolgus Macaque Brain without Liver Transduction by Systemic Injection.

- Matthew Tiffany, PhD, Sangamo Therapeutics
  Fitness Maturation of STAC-BBB Yields Second-Generation Capsid Variants with Enhanced Delivery to the Central Nervous System

- Hye-Kyung Oh, AAVATAR Therapeutics,Inc
  Machine Learning-Driven Engineering of AAV Capsids for Enhanced Blood-Brain Barrier Penetration and Optimized Tissue Specificity

- Daniel Cox, Voyager Therapeutics, Inc.
  Machine-Learning for AAV9 Mutant-Capsid Screening for both Production and ALPL-Mediated Transduction Efficiency

- Angga Kusuma, PhD, UniQure Biopharma
  Rapid Discovery of Novel AAV Capsids Using the BRAIN-X Insect Cell Library and Q-CAST

- Sirimar Laosinwattana, University College London
  Enhancing AAV2 Capsid Engineering Through Co-Evolutionary Coupling and Predictive Modelling

- Benjamien Moeyaert, PhD, KU Leuven
  A Novel Conjugation Technology for Enhanced Specificity and Potency in AAV Gene Therapy

- Tristan Cai, Changenic Biotech
  The generation of a versatile AAV engineering platform via the separated capsid protein expression

- Lingying Tong, Nationwide Children's Hospital
  Functional, Electrophysiological, and Histopathological Improvements in a Mouse model of CMT2A Following AAVrh74.tMCK.NT-3 Surrogate Gene Therapy

- Kristina Zhelcheska, University College London
  Targeting Oligodendrocytes in Neonatal Wildtype C57BL/6 Mice

- Raj Putatunda, Astellas Gene Therapies
  Human Frataxin Gene Transfer to the Cerebellar Dentate Nucleus Improves Motor Coordination in a Mouse Model of Friedreich Ataxia

- Sergiy Chornyy, Abigail Wexner Research Institute at Nationwide Children's Hospital
  Comparative analysis of cell-specific promoters in AAV9-mediated gene therapy targeting the central nervous system.

- Andreia Duarte, VectorY Therapeutics
  Targeting misfolded mutant HTT protein with vectorized antibodies for the treatment of Huntington Disease

- Ken Inoue, PhD, MD, National Center of Neurology and Psychiatry
  Focused ultrasounds enhance efficacy of AAV transduction via intracerebroventricular administration in mice

- Chinwendu Chukwu, Washington University in St. Louis
  Comparative Evaluation of Intranasal and Intravenous AAV Delivery for Safe and Targeted CNS Gene Therapy

- Amrutha Pattamatta, Arbor Biotechnologies
  Preclinical development of AAV mediated gene editing for SOD1-ALS using a Type V CRISPR-Cas enzyme

- Katherine Villa, Latus Bio
  Preclinical Mouse Pharmacology Studies Evaluating the Biodistribution and Pharmacodynamics of LTS-101, a Novel Gene Therapy Candidate for CLN2 Batten Disease

- Chao Tai, Encoded Therapeutics
  Developing an AAV-based Gene Therapy for Chronic Pain Through Identification of Potent and Selective Artificial miRNA Candidates to Knockdown SCN9A

- Yasemin ÖZGÜR GÜNES, UMass Chan Medical School
  Gene Therapy for ZNF526 Deficiency Using Endogenous Regulatory Elements

- Victor Hernandez, PhD, Axovia Therapeutics Inc
  A codon-optimised human sequence of human MC4R regulates cAMP levels and normalise weight in Mc4r mutant obese males and females when delivered with as a self-complementary AAV9 (scAAV9) with an intracerebroventricular delivery.

- Federica Ghersa, University of Minnesota Twin Cities
  Ongoing Preclinical Study of an AAV vaccine in Spontaneous Canine Model of Oral Melanoma: Safety and Efficacy Evaluation

- Glen Banks, Solid Biosciences
  Cardiac catheter delivery of AAV5 to Goettingen Minipig hearts

- Samantha Smith, PhD, Carbon Biosciences
  CGT-003: A novel recombinant parvovirus gene therapy for the treatment of hypertrophic cardiomyopathy resulting from genetic variants of myosin binding protein C3 (MYBPC3)

- Jang-Ho Cha, Latus Biosciences
  LTS-101: An Intracerebroventricular Delivered AAV Gene Therapy Using a Novel Capsid Variant for the Treatment of CLN2 Batten Disease

- Elizabeth Brooks, MS, DVM, Duke University Medical Center
  AAV Gene Therapy with a Bacterial Glycogen Debranching Enzyme in a Dog Model of GSD IIIa

- Nicole Zielinska, University of Guelph
  Optimizing Vector Administration of an AAV Gene Therapy for Surfactant Protein B Deficiency in a Neonatal Pig Model

- David Apiyo, Sartorius Stedim Biotech GmbH
  Extending Biolayer Interferometry (BLI) Principles to Develop a Simple Non – Cell Lysis Method for the Detection of Empty vs Full AAV Capsids

- Isai Leguizamo, Emory University School of Medicine
  Characterization and Optimization of AAV Transgene Cassettes Expressing HIV-1 Broadly Neutralizing Antibody 10-1074 for AAV9-Mediated expression in Non-Human Primates

- Adam Scheidegger, Ginkgo Bioworks
  A Cis-Regulatory Element Discovery Platform: Machine Guided Design and NHP Validation

- Azadeh Sarfallah, Spark Therapeutics
  Optimizing Plasmid Design to Enhance Safety and Efficiency in rAAV Production

- Corben Davis, Forge Biologics
  Fueling Technological and Process Advancements for Increased rAAV Productivity to Meet the Industry's Growing Demand

- Surabhi Godbole, Epicrispr Biotechnologies
  EPI-321 Development: Strategies to Establish a Scalable and Robust rAAVrh74 Upstream Manufacturing Process from 0.5 L to 1000 L Scale

- Itsasne Arangoa, Viralgen Vector Core - San Sebastian
  Leveraging Historical Data and Percentile based Modeling for Improved AAV Production Scaling

- Melanie Langhauser, Ascend Advanced Therapies
  Gaining control of rAAV production: real-time monitoring with Raman

Spectroscopy

- Daniel Brownell, BSc, Vinta Bio, Inc.
  Optimizing Harvest Time for AAV Full Capsids through Analysis of Production Kinetics in an Industrial System

- Ainara Apezteguia, Viralgen Vector Core
  Enhancing the Upstream Performance of Adeno-Associated Virus (AAV) Vector Manufacturing via Multivariate Data Analysis

- Silvia Gómez, PhD, Viralgen
  Streamlining Advanced Therapy Medicinal Product Manufacturing: A Platform Approach for Accelerating Gene Therapy Commercialization at Viralgen

- Nazgul Wagner, Sartorius
  Accelerating AAV manufacturing timelines via a novel Rolling Seed Approach

- Ana Mena Amores, Keck Graduate Institute
  Optimizing Recombinant AAV Vector Production: Ehnacing Upstream and Downstream Processes for Improved Yield and Quality

- Sydney Bear, Isolere Bio by Donaldson
  Combining Fixed Bed Bioreactor and Liquid-Phase Affinity Purification Technologies for Next-Generation, Low-Cost Lentiviral Manufacturing

- Vedud Purde, Eli Lilly and Company
  Optimization of AAV Upstream Production for a Low Producing Vector

- Arthur Rostovtsev, Catalent Cell & Gene Therapy
  Adeno-Associated Viral Vector Production Process Intensification

- Ken Prentice, Shape Therapeutics, Inc. - Seattle, WA
  Comprehensive Evaluation of TruStable^{TM} vs Transient Production Systems for AAV Vector Manufacturing

- Jessie Chua, Forge Biologics
  Recent Advancements in Enhanced Starting Materials and Process Optimization for Manufacturing High Yield rAAV in Suspension HEK293 Cells in Single-Use Bioreactors

- Megan Del Greco, MilliporeSigma
  Optimizing Upstream Process Inputs to Enhance Viral Yields and Ensure Product Quality in Adeno-Associated Virus (AAV) Manufacturing

- Jose Romero Sanchez, FUJIFILM Irvine Scientific
  Scalable Bioprocessing Strategies for Intensified Viral Vector Production in HEK293 Cell Culture

- Hung-Lun Hsu, Voyager Therapeutics, Inc.
  Assessment of two HEK293 Cell Line Cloning strategies to improve AAV yield

- Takanori Ieki, Astellas Gene Therapies
  High cell density AAV production

- Oliver Varette, Virica Biotech
  Increased AAV Yield: From Screen to Scale Up to Cost Savings Using Viral Sensitizer (VSE^{TM}) Technology

- Thomas Cummings, Trisk Bio Ltd
  Production of large 1e14-1e15 NHP-grade batches of Adeno-associated viruses

via fast intensification and parallelized manufacturing

- Eric Lin, PhD, ProBio Inc
  Scalable Production of Recombinant AAV (rAAV) in HEK293 Suspension Cells: From Shake Flask to 10L Bioreactor Scale-Up

- Tala Calvi, Cytiva
  Optimized cell culture media for scalable rAAV bioreactor production

- Thomas Robert, PhD, Univercells Technologies
  Optimizing Serum-Free Conditions for Scalable AAV Production in Fixed-Bed Bioreactors

- Kelly Cybulski, Cytiva
  Scale-up of an ELEVECTA™ Transient rAAV Production Process in the Xcellerex™ X-platform Single-use Bioreactor

- Do Hyun Park, Massachusetts Institute of Technology
  Pseudo-Continuous Manufacturing of Adeno-Associated Virus (AAV) Using a Dual-Bioreactor System with Inertial Microfluidic Cell Retention Device

- Masafumi Nishino, FUJIFILM Corporation
  Downsizing Process for a Large Amount of rAAV Vector Manufacturing by Combination of Perfusion Culture and Transfection to High Densified Cells

- Kyle Burrell, Cytiva
  N-1 Perfusion Process for Intensification of AAV Production

- Adam Osborne, Sanofi
  Development of a Perfusion Process for AAV Production Using High-Throughput

Bioreactor Systems

- Stefan Seeber, PhD, Roche PRED
  Enhanced AAV-Vector Production Using a Novel Stable Producer Cell Line and Optimized Perfusion Processes

- Pouria Motevalian, Thermo Fisher Scientific
  Process and Quality Considerations for Recombinant Adeno-Associated Virus (rAAV) Manufacturing Platforms

- Andrew Detzel, SK Pharmteco - US
  Development of a Serotype-Agnostic AAV Platform for Suspension Cell Culture

- Femke Hoeksema, VectorY Therapeutics
  An Optimized Baculovirus Generation Process and Novel ITR-Transgene Design for High Quality AAV Production Using the Insect Cell Production Platform.

- Franziska Bollmann, Sartorius Stedim Biotech GmbH
  Development of a Robust and Scalable AAV8 Production Platform: Enhancing Vector Yield and Purity through Optimized Bioprocessing Techniques

- Arjun Kumar Mishra, Frederick National Laboratory for Cancer Research
  Platform Development for Production and Purification of AAV9

- Eric Lin, LEXEO Therapeutics
  Development of a Novel High-Yielding Scalable Sf9-Baculovirus Platform to Produce Quality AAV at 200L Scale

- Larry Forman, CHO Plus
  Novel Cell Engineering Platform for High-Yield AAV Production and Improved

Manufacturability via Engineered HEK-293 Cells.

- Rongze Yang, AAVnerGene
  AAVone System Generates over 5E15 VG/L AAV9 Vectors in Suspension HEK 293 Cells

- Megan Del Greco, MilliporeSigma
  Process Optimization of a Baculovirus-Insect Cell Expression System for Scalable rAAV Production

- David Ede
  Development of a Robust AAV Upstream Platform Using next generation Raw material, a Comprehensive Design-of-Experiment (DoE) approach & High-Throughput Automated Bioreactors

- DAVID VINCENT, EXmoor Pharma Concepts Ltd
  Fast-Tracking AAV Process Development Using Innovative and Universal Approaches – The Platform of the Future

- Xiaobin He, Genevoyager
  Scaling Up AAV Manufacturing: Expanding AAV-based Therapies Beyond Rare Diseases with Genevoyager Bac/Sf9 System

- Yash Shah, AskBio
  Strategies to Remove Pre-Existing AAV Antibodies: IgG Enzymatic Degradation vs FcRn inhibition.

- Yunxia (Erica) Xu, Bao Pharma
  Short-Term Repeat Dosing of KJ103 (Ricefidase) Enables Sustained IgG Reduction for Enhanced AAV Gene Therapy

- Yunxia (Erica) Xu, Bao Pharma
  Clinical Validation of Short-Term Triple Dosing of KJ103 (Ricefidase) for Sustained IgG Reduction in AAV Gene Therapy

- Surendra Sharma, UNC Gene Therapy Vector Core
  Rapid Transient Antibody Depletion using Genetically Engineered IgG-Degrading Enzyme allows efficient rAAV9 Gene Delivery in an α-AAV9-Ab^{+} rabbit model

- Brian Long, PhD, 4DMT
  Low Pre-existing AAV-Neutralizing Titers Detected Using a Cell-Based Assay Did Not Impact 4D-310 Safety or Efficacy in Fabry Cardiomyopathy Patients

- Irene Song, Packgene Biotech
  Evaluating Neutralizing Antibodies Against AAVs Using a High-Sensitivity LacZ Reporter Assay

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

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

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

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

- Michael Klichinsky, PharmD, Carisma Therapeutics
  CAR Macrophages

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

Scientific Symposium

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

Location: 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 and globalization of GCTs; how approved therapies are reaching patients in leading markets {the US, Europe} (evaluated through a DEI lens); The promise of one and done therapies in developing countries and the significant challenges to bring them there

Member-Submitted Proposal

Translational Strategies for Cell and Gene Therapies in Inherited Neurotransmitter Disorders

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

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

- Brad Elder, MD, The Ohio State University College of Medicine
  Gene Therapy for AADC

- Dimitri Kullmann, University College London
  Self-regulated closed-loop gene therapy for disorders of circuit excitability

- Cory Nicholas, Neurona Therapeutics

First-in-human clinical investigation of GABAergic inhibitory interneuron cell therapy for drug-resistant epilepsy

Scientific Symposium

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

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

- Ryuichi Morishita, MD, PhD, Center of Medical Innovation & Translational Research, University of Osaka
  HGF gene therapy- Phase II data in USA, and FDA approved Breakthrough therapy

- Alberto Auricchio, PhD, MD, TIGEM, Telethon Institute of Genetics and Medicine
  Clinical Trials of AAV gene therapy directed to liver and retina

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

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

Scientific Symposium

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

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

- Kimberly Haugstad, MBA, RareRising
  Engaging in Patient-Centered Scientific Communication

- Allyson Berent, DVM, DACVIM, Foundation for Angelman Syndrome Therapeutics
  Translating Clinical Developments for Families and Patients with Rare Diseases

- Benjamin McLeod, Convey Bio
  Communicating Complex Science on Social Media

Scientific Symposium

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

Location: Room 393-396
8:00 AM - 9:45 AM

- Uta Griesenbach, 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

Sponsored Symposium

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

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

- Laura Juckem, PhD, Mirus Bio
  MilliporeSigma: To 1000L and Beyond: Introducing the first-and-only transfection complex stabilizer for simple AAV manufacturing scale-up

Sponsored Symposium

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

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

- Matthew Lauber, PhD, Waters Corporation
  Sponsor

- Noah Miller-Medzon, LEXEO Therapeutics
  Sponsor

Networking

Exhibit Hall

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

General Session

Outstanding New Investigator Symposium

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

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

Therapy

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

- Xavier Anguela, Nava Therapeutics
  Talk Title Forthcoming

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

Scientific Symposium

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

Sponsored Symposium

Pacira Biosciences: High-Capacity Adenoviral Vectors: Advancing Gene Therapy Beyond AAV to Deliver Cost-effective Therapies for Common Diseases

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

- Derek Jackson

Pacira Biosciences: High-Capacity Adenoviral Vectors: Advancing Gene Therapy Beyond AAV to Deliver Cost-effective Therapies for Common Diseases

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

- Maria Ester Bernardo, MD, PhD, San Raffaele Telethon Institute
  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.

Tools & Technology Session

Tools and Technology Forum 5

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

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

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

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

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

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

VSVg. That is the question.

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

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

Scientific Symposium

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

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

- Carmen Sivakumaren, IQVIA
  BGTC Coordination Center Rep

- James Noll, IQVIA
  BGTC Coordination Center Rep

- Sharon King, Aldevron
  BGTC/FNIH Partner

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

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

- Leah Byrne, UC Berkeley
  BGTC AAV Biology Awardee

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

- Courtney Coates, Hope in Focus
  Hope in Focus

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, 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

- 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

- 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

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

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

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

- 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

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

- 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 Urea Cycle 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

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

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

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.

- Aphrodite Georgakopoulou, PhD, University of Washington
  In vivo HSC Transduction in Humanized Mice Mediated by Novel Capsid-modified HDAd Vectors

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: Exhibit Hall
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

- Mickey Lorenzini, Salk Institute
  Joint Single-Cell Profiling of CRISPR-Cas9 Edits and Transcriptomes Reveals Widespread Off-Target Events and Affected Gene Expression

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, 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 Rheumatoid Arthritis - early Phase 1b results

- Vijay Bhoj, MD PhD, University of Pennsylvania
  CAR-T for alloantibodies prior to organ transplant

- Nicole Paulk, PhD, Siren Biotechnology
  SRN-101 universal AAV gene therapy for solid tumors

Scientific Symposium

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

Location: Room 383-385
8:00 AM - 9:45 AM

- Alexandra Piotrowski-Daspit, PhD, University of Michigan
  Multi-organ nucleic acid delivery/ overcoming macrophage phagocytosis

- Marianne Carlon, KU Leuven
  Base and prime editing mutant CFTR in patient-derived cell model to treat cystic fibrosis

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

- Stephen Hyde, PhD, University of Oxford

Lentiviral gene transfer for CF and SP-B deficiency

Oral Abstract Session

Chemistry, Manufacturing, and Controls

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

- 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

- Dmitry Zabezhinsky, Merck Life Science KGaA
  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, 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

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
  Pharmacological Strategies to Shift DNA Repair Pathway Choice in Gene Editing

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

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