PROGRAM GUIDE

Los Angeles Convention Center

ASGCT 26TH ANNUAL MEETING Los Angeles

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A WELCOME LETTER FROM HANS-PETER KIEM, MD, PHD

Dear Colleagues,

On behalf of the American Society of Gene & Cell Therapy (ASGCT), I'd like to welcome you all to our 26th Annual Meeting! I'm thrilled to be with you in sunny Los Angeles, CA for an expanded 4.5 days of the latest discoveries in gene and cell therapy. This year we're privileged to bring you more content than ever before. The record-breaking amount of research we received shows how many of you are dedicated to the Society's mission of advancing knowledge, awareness, and education to expand the discovery and clinical application of gene and cell therapies to alleviate human disease.

Whether you're gathering with us in person in Los Angeles or virtually, I hope you're looking forward to meeting new colleagues and connecting with old ones while learning about new developments in the field. Please remember that all attendees will have on-demand access to the keynote speaker talks, oral abstract presentations, scientific symposia, education sessions, virtual poster hall, and more, for 30 days following the meeting.

I would like to extend a warm welcome to Founders Award recipient David Williams, MD, who we'll hear from on Tuesday, the first day of the meeting. On Wednesday afternoon, I'm also excited to welcome Jennifer Doudna, PhD, and David R. Liu, PhD, who will speak during the Presidential Symposium. Finally, on Thursday morning, Crystal Mackall, MD, and Stanley Riddell, MD, will present the George Stamatoyannopoulos Memorial Lectures.

I'd like to congratulate our Annual Meeting award winners: J. Keith Joung, MD, PhD, recipient of the Outstanding Achievement Award; Manuela Corti, PhD, Justin Eyquem, PhD, Maksim "Max" Mamonkin, PhD, and Shondra Pruett-Miller, PhD, recipients of the Outstanding New Investigator Award; Mark Dudley, PhD, Bruce Levine, PhD, and Isabelle Rivière, PhD, recipients of the Jerry Mendell Award for Translational Science; Anthony Fauci, MD, recipient of the Sonia Skarlatos Public Service Award; Joy Cavagnaro, PhD, recipient of the Catalyst Award; and David A. Williams, MD, recipient of the Founders Award. Thank you for the important work you do to move the field forward.

Finally, I would like to thank all of you involved in the planning and execution of the Annual Meeting, especially the Program Committee, Scientific and Education Committees, abstract reviewers, staff, and all of our volunteers. We couldn't make this happen without all of you and I'm grateful for your commitment. Thank you for supporting ASGCT and enjoy the meeting.

Sincerely,
Hans-Peter Kiem, MD, PhD
President, ASGCT

GENERAL MEETING INFORMATION

Mission + Vision

The mission of ASGCT is to advance knowledge, awareness, and education leading to the discovery and clinical application of genetic and cellular therapies to alleviate human disease. ASGCT's strategic vision is to be a catalyst for bringing together scientists, physicians, patient advocates, and other stakeholders to transform the practice of medicine by incorporating the use of genetic and cellular therapies to control and cure human disease.

Abstract Publications

All abstracts accepted for presentation at the ASGCT 26th Annual Meeting have been published in a May supplement of *Molecular Therapy* and are searchable for free on the Annual Meeting Platform.

Continuing Medical Education

The ASGCT Board of Directors decided that the Society will not offer CME credit for the 26th Annual Meeting.

Disclosure

In keeping with good practices, ASGCT requires faculty to disclose any relevant financial interest or other relationship with the manufacturer(s) of any commercial product(s) and/or provider(s) of commercial services that are discussed in this educational activity.

Educational Objectives

At the conclusion of the activity, the participant should be able to:

- Provide advice to patients who inquire about the potential of gene and cell therapy or the availability of open clinical trials, based on their exposure to the current clinical trials in gene and cell therapies.
- Better instruct their students in medical school and other health venues using the state-of-the-art basic science and clinical trials data presented at the meeting.
- Use the latest advances in gene and cell therapy to enhance their research mission, as physician scientists conducting basic and clinical research.
- Demonstrate improved regulatory compliance in conducting gene and cell therapy clinical trials, through exposure to NIH and FDA faculty during the educational program.

Evaluation Method

An evaluation of the Annual Meeting is live and will be available for 30 days after the meeting's conclusion. The evaluation will address program content, presentation, and possible bias, and will be sent to all attendees at the conclusion of the event.

Needs

Clinical gene transfer has become increasingly complex due to ongoing developments in the fields of gene and cell therapy, together with bioethics, research integrity, and financial conflicts, as well as federal mandates, regulations, and guidelines. Oligonucleotide therapies, novel vector development, host-vector interactions, and vaccine therapies will be discussed, as well as many other scientific topics. This meeting will provide an educational forum for scientists and clinicians to expand their knowledge about the broad developments in these fields.

Target Audience

The target audience includes basic science and translational researchers, clinical investigators, physicians, postdoctoral fellows, graduate students, employees of federal government and regulatory agencies, patient advocates, and other health care professionals with an interest in the latest advancements in the fields of gene and cell therapy.

Dates + Location

The ASGCT 26th Annual Meeting will be a hybrid event. The in-person portion will be held at the Los Angeles Convention Center in Los Angeles, CA. A virtual registration will also be offered. Both virtual and in-person attendees will have access to the virtual platform and on-demand content.

The meeting will begin on the morning of Tuesday, May 16, 2023, and continue through 12 p.m. (PT) on Saturday, May 20, 2023. The Exhibit Hall will be open on Tuesday, May 16, from 5:30 p.m. to 7 p.m., and from Wednesday, May 17, through Friday, May 19, from 9:45 a.m. to 7 p.m. All ASGCT content and select sponsor-generated content will be available to registrants on-demand for 30 days following the conclusion of the meeting.

Photography + Video Recording

ASGCT contracts with photographers and videographers throughout the Annual Meeting to assist in the creation of future marketing materials. By attending the 26th Annual Meeting you understand and agree that your image may be recorded and used in ASGCT promotional materials.

Printing Instructions

To print this program:

1. Right click on the program and select "Print."
2. In the print settings, select "Fit to printable area."
3. Select "Print."

REGISTRATION + EXHIBIT HALL

Registration

WEST LOBBY

Tuesday, May 16 | 7:00 a.m.–7:00 p.m.
Wednesday, May 17 | 7:00 a.m.–6:00 p.m.
Thursday, May 18 | 7:00 a.m.–6:00 p.m.
Friday, May 19 | 7:00 a.m.–6:00 p.m.
Saturday, May 20 | 7:00–10:00 a.m.

Visit the Exhibit Hall

DATES AND TIMES

Tuesday, May 16 | 5:30 p.m.–7:00 p.m.
Wednesday, May 17 | 9:45 a.m.–7:00 p.m.
Thursday, May 18 | 9:45 a.m.–7:00 p.m.
Friday, May 19 | 9:45 a.m.–7:00 p.m.

For more information, view the exhibitor listing on the Annual Meeting website.

ALL TIMES LISTED IN PT

PROGRAM COMMITTEE + ABSTRACT PLANNING COMMITTEE

2023 ASGCT Program Committee

Thank you to the following individuals for serving on the ASGCT Program Committee and helping with the overall development of the ASGCT 26th Annual Meeting.

CHAIR
Hans-Peter Kiem, MD, PhD, *Fred Hutchinson Cancer Research Center*

MEMBERS
Chiara Bonini, MD, *Università Vita Salute San Raffaele*
Paula M. Cannon, PhD, *University of Southern California*
Jeffrey S. Chamberlain, PhD, *University of Washington*
Beverly L. Davidson, PhD, *Children's Hospital of Philadelphia*
Terence R. Flotte, MD, *University of Massachusetts Medical School*
Philip D. Gregory, DPhil, *2seventy bio*
Helen E. Heslop, PhD, *Baylor College of Medicine*
Maritza C. McIntyre, PhD, *Advanced Therapies Partners*
Carol H. Miao, PhD, *Seattle Children's Research Institute*
Isabelle Rivière, PhD, *Takeda*

2023 Abstract Planning Committee

Thank you to the following individuals for reviewing and selecting abstracts for presentation in the Presidential Symposium and Clinical Trials Symposium!

PRESIDENT
Hans-Peter Kiem, MD, PhD, *Fred Hutchinson Cancer Research Center*

ASGCT PRESIDENT ELECT
Jeffrey S. Chamberlain, PhD, *University of Washington*

ASGCT SECRETARY + ABSTRACT CHAIR
Isabelle Rivière, PhD, *Takeda*

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SIREN BIOTECHNOLOGY

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TERUMO BLOOD AND CELL TECHNOLOGIES

uniQure

ThermoFisher SCIENTIFIC

GenScript

GenScript PROBIO

AWARD RECIPIENTS

Career Development Award

Jacquelyn Bower, PhD
Assistant Professor at University of North Carolina at Chapel Hill

Y. Esther Tak, PhD
Instructor and Research Fellow at Harvard Medical School and Massachusetts General Hospital

Liubin Yang, MD, PhD
Reproductive Endocrinology and Infertility Clinical Fellow at Baylor College of Medicine

Tristan Scott, PhD
Assistant Research Professor and Staff Scientist at City of Hope

Martin Kang, PhD
Assistant Professor of Pediatrics at Darby Children's Research Institute at the Medical University of South Carolina

Alma-Martina Cepika, MD, PhD
Instructor at Stanford University School of Medicine

Underrepresented Population Fellowship in Gene and Cell Therapy - Any Indication

Miguel Santoscoy, PhD
Postdoctoral Fellow at Harvard Medical School

Underrepresented Population Fellowship in Gene and Cell Therapy - Oncology

Karina Vázquez-Arreguín, PhD
Postdoctoral Researcher, Department of Pathology, Georgia Cancer Center, Kaur Laboratory

Mavis Agbandje-McKenna Scholarship

Alan Rosales
PhD Student at Duke University

Outstanding Achievement Award

Outstanding Achievement Award is supported by: Spark Therapeutics

J. Keith Joung, MD, PhD
Colvin Endowed Chair in Pathology at Massachusetts General Hospital & Professor of Pathology at Harvard Medical School

Outstanding New Investigator Award

Maksim "Max" Mamonkin, PhD
Assistant Professor at Baylor College of Medicine and Co-founder and CSO at March Bio

Justin Eyquem, PhD
Assistant Professor at University of California, San Francisco and Cofounder of Mnemo Therapeutics

Shondra Pruett-Miller, PhD
Associate Director, Shared Resources at St. Jude Children's Research Hospital Comprehensive Cancer Center and Founding Director of the Center for Advanced Genome Engineering at St. Jude Children's Hospital

Manuela Corti, PhD
Associate Professor and Associate Director, Powell Gene Therapy Center at the University of Florida

Excellence in Research Award

Martina Fiumara, SR-TIGET
Alan Rosales, Duke University
William Feist, Stanford University
Cesare Canepari, SR-TIGET
Chun Huang, University of Southern California
Royce Ma, Baylor College of Medicine
Timothy Smith, Duke University
Geoffrey Rogers, PhD, University of Southern California
Ai Vu Hong, GENETHON
Gabriele Casirati, Dana Farber Cancer Institute
Junping Zhang, PhD Indiana University School of Medicine
Nadja Meumann, Hannover Medical School
Cassie Turnage, University of Utah
Rrita Daci, UMass Chan Medical School
William Nyberg, UCSF

Sonia Skarlatos Public Service Award

Anthony Fauci, MD
Former Director of the National Institute of Allergy and Infectious Diseases (NIAID)

Jerry Mendell Award for Translational Science

Mark Dudley, PhD
Chief Scientific Officer of Instil Bio

Bruce Levine, PhD
Barbara and Edward Netter Professor in Cancer Gene Therapy and Founding Director of the Clinical Cell and Vaccine Production Facility at the University of Pennsylvania

Isabelle Rivière, PhD
Head of Cell Therapy Technology & Product Engine at Takeda

Catalyst Award

Joy Cavagnaro, PhD
President at Access BIO

Founders Award

David A. Williams, MD
Chief of the Division of Hematology/Oncology at Boston Children's Hospital; Associate Chairman of the Department of Pediatric Oncology at the Dana-Farber Cancer Institute; and Leland Fikes Professor of Pediatrics at Harvard Medical School

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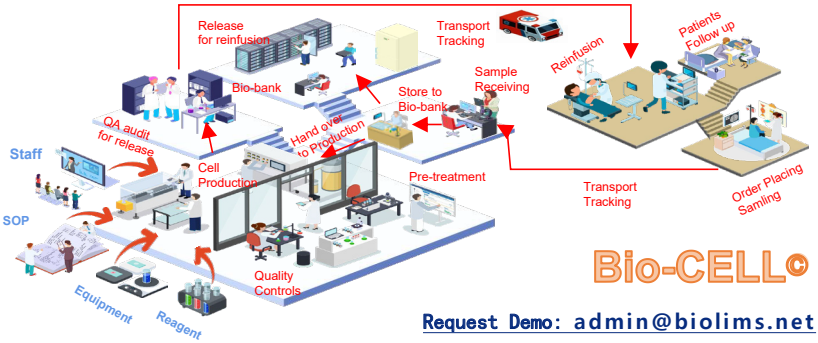
Basic Regulation:
21 CFR Part11, ALCOA+CCEA, CSV;

GMP Specification:
ISPE GAMP5-2, FDA & EU GMP, WHO;

Lab Specification:
GLP, CAP/CLIA, ISO17025, ISO15189;

HOW Bio-CELL Cell and Gene Therapy Digitalize System Works?



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Bio-LIMS© Bio-LIMS, the informatics solution for the molecular and genomic labs, helps your lab run more efficiently, automate tasks and integrate with your instruments and systems. With our flexible, robust system and experienced professional services team, you can minimize the risk of project delays or failures. Once deployed, the system can also easily adapt to your organization's evolving business requirements.

Bio-Research© Bio-Research system can optimize biotech lab's R&D works and data analysis. With Bio-Rearch system, you can easily standardize your data collection, automate data analysis, and create visual reports, all while maximizing the value of your research data.

Bio-CELL© Bio-CELL is cell and gene therapy digital solution, support COI (Chain of Identity) COC (Chain of Custody).The compliance standard of Bio-CELL is FDA Alcoa + CCEA. Combined with the requirements of ISPE GAMP5-2, the electronic data (EDP) that basically meets the requirements is constructed.

Bio-Bank© Bio-BANK system adopts graphical touch-screen drag and drop operation mode, which is suitable for all biological banks, cells, sample storage and application enterprises.The system is quite easy to extend, it used the relative sample for extension management. It's simple but it is powerful.

ASGCT 26TH ANNUAL MEETING Los Angeles

SCHEDULE

All Times Listed in PT

SCHEDULE (ALL TIMES LISTED IN PT)

TUESDAY, MAY 16, 2023

WORKSHOP

ROOM 408 AB

Accessibility of Gene Therapies: Exploring the Continuum from Patient-Specific to Market Approved Therapies

Co-chairs: Allison Bradbury, PhD, Nationwide Children's Hospital, The Ohio State University Wexner Medical Center; and Michelle Berg

8:00–8:35 AM	**Exploration of Patient Specific Medicines** *Joe Gleeson, MD, N-Lorem Foundation*
8:35–9:00 AM	**Exploration Patient Specific Medicine: Ethical Perspective** *Alison Bateman-House, PhD, MPH, NYU Grossman School of Medicine*
9:00–9:30 AM	**Conventional Clinical Trial Design and Expanded Access Clinical Trials** *Barry Byrne, MD, PhD, University of Florida*
9:40–10:05 AM	**Enrolling a Full Clinical Trial/Conventional Clinical Trial Design** *Rae Blaylark, Sickle Cell Foundation of Minnesota*
10:05–10:30 AM	**Patient Advocate Perspectives on Conventional Clinical Trial Design to Cover Other Technologies** *Clark Paramore, MSPH, bluebird bio*
10:30–10:55 AM	**Market Approved Therapies - Focus on ex vivo Lenti Gene Therapy** *Jasmine Greenamyer, Bristol Myers Squibb*
11:05 AM–12:00 PM	**Panel Discussion** *Joe Gleeson, MD* *Barry Byrne, MD, PhD* *Rae Blaylark* *Alison Bateman-House, PhD, MPH* *Jasmine Greenamyer* *Clark Paramore*

SCHEDULE (ALL TIMES LISTED IN PT)

TUESDAY, MAY 16, 2023

WORKSHOP

ROOM 502 AB
CMC Development Challenges and How to Avoid Them
Co-chairs: Angela Whatley, PhD, PTC Therapeutics and Dan Leblanc, Ensoma

8:00–8:25 AM	**Introduction to the Issues: Common CMC Issues and Best Practices** *Jessica Chery, PhD, FDA CBER*
8:25 –8:50 AM	**Case Study on Early-Phase Development: Verve Therapeutics** *Chris Cheng, PhD, Verve Therapeutics*
8:50–9:15 AM	**Case Study on Early-Phase Development: Kite** *Alison Holzer-Speed, PhD, Kite Pharma*
9:15–9:40 AM	**Case Study on Later-Phase Development for Upstaza** *Michael Chang, PTC Therapeutics*
9:40–10:15 AM	**Panel Discussion** *Jessica Chery, PhD* *Chris Cheng, PhD* *Alison Holzer-Speed, PhD* *Michael Chang*
10:35–11:20 AM	**Attendee Breakout Discussions**
11:20 AM–12:00 PM	**Reconvene and Closing Remarks** *Angela Whatley, PhD and Dan Leblanc*

SCHEDULE (ALL TIMES LISTED IN PT)

TUESDAY, MAY 16, 2023

WORKSHOP

CONCOURSE HALL 152 & 153
How to Become a Site for AAV Gene Therapy Trials
Co-chairs: Nicolas Abreu, MD, NYU Langone and Charles Venditti, PhD, MD, Stanford University School of Medicine

8:00–8:25 AM	**Preclinical/IND-enabling Basics of AAV Biology and GT Vectors Preclinical Enablement and the IB** *Mark Kay, MD, PhD, Stanford University School of Medicine*
8:25–8:50 AM	**Regulatory Considerations for Establishing Gene Therapy Trial Sites** *Sandra Nino-Siddens, PharmD, Ultragenyx*
8:50–9:15 AM	**Clinical Protocol Implementation: Who sees the Patient and What is Needed? Clinical Infrastructure Treating the Patient/Acute Monitoring Phase I/II vs III vs IV** *Kimberly Goodspeed, MD, University of Texas Southwestern Medical Center*
9:15–9:40 AM	**Follow up and Long-term Toxicity: What Needs to Be Followed for Follow Up?** *Denise Sabatino, PhD, The Children's Hospital of Philadelphia*
9:55–11:55 AM	**Panel Discussion** *Sandra Nino-Siddens* *Kimberly Goodspeed, MD, MSCS* *Denise Sabatino, PhD* *Mark Kay, MD, PhD*

SCHEDULE (ALL TIMES LISTED IN PT)

TUESDAY, MAY 16, 2023

WORKSHOP

PETREE HALL C
How to Make the Most of Your Annual Meeting Experience
Co-chairs: Marina Pavlou, PhD, University of Washington and Nerea Zabaleta, PhD, Mass Eye and Ear/Harvard Medical School

8:00–8:25 AM	**How to Network at a Conference** *Hildegard Buning, PhD, Hannover Medical School*
8:25–8:50 AM	**How to Maximize your Use of Social Media During the Conference** *Emily Walsh, PhD, Tremont Therapeutics*
8:50–9:15 AM	**Anticipating Where the Field is Going** *Federico Mingozzi, PhD, Spark Therapeutics*
9:15–9:40 AM	**Diversifying Your Network to Improve Collaboration and Innovation** *Avery Posey, PhD, University of Pennsylvania*
9:55–11:55 AM	**Panel Discussion** *Hildegard Buning, PhD* *Avery Posey, PhD* *Emily Walsh PhD* *Federico Mingozzi, PhD*

SCHEDULE (ALL TIMES LISTED IN PT)

TUESDAY, MAY 16, 2023

WORKSHOP

ROOM 515 AB
Immune Effector Cells: 2023 and Beyond!
Co-chairs: Nirali Shah, MD, National Cancer Institute and Daniel Powell, PhD, University of Pennsylvania

8:00–8:20 AM	**TILs in 2023: State of the Art and Future Directions** *Stephanie Goff, MD, National Cancer Institute (NIH)*
8:20–8:40 AM	**TCR based strategies: State of the Art and Future Directions** *Aude Chapuis, MD, Fred Hutchinson Cancer Research Center*
8:40–9:00 AM	**HSCT and Gene Editing to Optimize IEC Therapy** *Saar Gill, PhD, University of Pennsylvania*
9:00–9:15 AM	**Panel Discussion** *Stephanie Goff, MD* *Aude Chapuis, MD* *Saar Gill, MBBS, PhD, FRACP*
9:15–9:35 AM	**GD2 Targeting and Midline Brainstem Tumors** *Sneha Ramakrishna, MD, Stanford University*
9:35–9:55 AM	**CD19-palooza: Summary of Indications and Factors Impacting Outcomes** *MIchael Jain, MD, PhD, Moffitt Cancer Center*
9:55–10:15 AM	**BCMA CAR T-cells: Summary of Indications, Use and Outcomes in Multiple Myeloma** *Yi Lin, MD, PhD, Mayo Clinic*
10:15–10:30 AM	**Panel Discussion** *Michael Jain* *Yi Lin, MD, PhD* *Sneha Ramakrishna, MD*

SESSION CONTINUED ON NEXT PAGE

SCHEDULE (ALL TIMES LISTED IN PT)

TUESDAY, MAY 16, 2023

SESSION CONTINUED FROM PREVIOUS PAGE

WORKSHOP

ROOM 515 AB
Immune Effector Cells: 2023 and Beyond!
Co-chairs: Nirali Shah, MD, National Cancer Institute and Daniel Powell, PhD, University of Pennsylvania

10:45–11:05 AM	**Beyond T-Cells: CAR-Macs, CAR-NKs** *Michael Klichinsky, PharmD, Carisma Therapeutics*
11:05–11:25 AM	**CD 19 CAR T-Cells for SLE** *Samik Basu, MD, Cabaletta Bio*
11:25–11:45 AM	**Harnessing Synthetic Biology and Gene Editing Technologies to Engineer Next Generation CAR T-Cells** *Justin Eyquem, PhD, UCSF*
11:45 AM–12:00 PM	**Panel Discussion** *Samik Basu, MD* *Justin Eyquem, PhD,* *Michael Klichinsky, PharmD*

SCHEDULE (ALL TIMES LISTED IN PT)

TUESDAY, MAY 16, 2023

WORKSHOP

PETREE HALL D

Precise Genome Editing: One Goal, Multiple Avenues

Co-chairs: Alexis Komor, PhD, University of California, San Diego and Claudio Mussolino, PhD, Institute for Transfusion Medicine and Gene Therapy at Center for Translational Cell Research

8:00–8:20 AM	**Genome Editing Using TALENs** *Alexandrea Juillerat, PhD, Cellectis Inc.*
8:20–8:40 AM	**Harnessing the Diversity of CRISPR-Cas Proteins for Genome Editing** *Giedrius Gasiunas, PhD, Caszyme*
8:40–9:00 AM	**Harnessing Novel CRISPR Systems to Detect and Cure Genetic Disease** *Janice Chen, PhD, Mammoth Biosciences*
9:00–9:20 AM	**Stem Cell Genome Editing to Treat Neurodegenerative Disorders** *Natalia Gomez-Ospina, MD, PhD Stanford University*
9:20–9:45 AM	**Panel Discussion** *Janice Chen, PhD, Natalia Gomez-Ospina, MD, PhD, Giedrius Gasiunas, PhD, Alexandre Juillerat, PhD*
10:00–10:20 AM	**Clinical Exploitation of SB Editing Platform** *Zoltan Ivics, PhD, Paul Ehrlich Institut*
10:20–10:40 AM	**Base Editing for Therapeutic Applications** *Holly Rees, PhD*
10:40–11:00 AM	**Base Editing CAR-T** *Branden Moriarity, PhD, University of Minnesota*
11:00–11:20 AM	**Prime Editing** *Julian Grünewald, MD, Technical University of Munich*
11:20–11:45 AM	**Panel Discussion** *Branden Moriarity, PhD* *Julian Grünewald, MD* *Zoltan Ivics, PhD* *Holly Rees, PhD*

SCHEDULE (ALL TIMES LISTED IN PT)

TUESDAY, MAY 16, 2023

WORKSHOP

CONCOURSE HALL 150 & 151

The Magic Year - Founders Tips for What to do in Your Last Six Months in Academia and First Six Months in Industry

Co-chairs: Melissa Kotterman, PhD, Iris Medicine and Nicole Paulk, PhD, University of California San Francisco

8:00–8:20 AM	**IP/Contracts (getting legal representation, MTA, option/license)** *Sabrina Poulos, JD, Goodwin Procter LLP*
8:20–8:40 AM	**Money (dilutive vs non-dilutive, angels, grants): Successful Operator-to-VC** *Chris Garabedian, Xontogeny and Perceptive VC Firm*
8:40–9:00 AM	**How to Vet if Your Idea is Worthy of Starting a Startup** *Nicole Paulk, PhD, University of California San Francisco*
9:00–9:20 AM	**Defining Milestones for Program Development & Successful Fundraising** *Melissa Kotterman, PhD, Iris Medicine, Inc.*
9:20–9:40 AM	**Don't Worry, Just Do It: Go Ahead and Create a Successful Biotech Startup** *Douglas Crawford, PhD, MBC BioLabs*
9:40–10:00 AM	**Building the Team That's Right for Your Startup** *Eric Kelsic, PhD, Dyno Therapeutics*
10:15 AM–12:00 PM	**Panel Discussion** *Nicole Paulk, PhD* *Sabrina Poulos, JD* *Chris Garabedian* *Eric Kelsic, PhD* *Melissa Kotterman, PhD* *Douglas Crawford, PhD*

SCHEDULE (ALL TIMES LISTED IN PT)

TUESDAY, MAY 16, 2023

SCIENTIFIC SYMPOSIUM

ROOM 408 AB
Building Successful Careers for the New Investigator
Co-chairs: Afrooz Rashnonejad, PhD, Ohio State University and Rachel Bailey, PhD, UT Southwestern Medical Center

1:31–1:55 PM	**Creating a Roadmap (Strategic Plan) in Academia for Early-Stage Investigators** *Terence Flotte, MD, UMass Chan Medical School*
1:55–2:20 PM	**Navigating and Networking in Gene and Cell Therapy Space** *Denis Phichith, PhD, Roche Innovation Center*
2:20–2:45 PM	**Wearing Multiple Hats: Building an Alternative Career INSIDE Academia** *Shondra Pruett-Miller, PhD, St. Jude Children's Research Hospital*
2:45–3:10 PM	**Navigating the NIH as a New Investigator** *Maureen Shuh, PhD, Center for Scientific Review, National Institutes of Health*

SCHEDULE (ALL TIMES LISTED IN PT)

TUESDAY, MAY 16, 2023

SCIENTIFIC SYMPOSIUM

PETREE HALL D
Comparability Challenges for CMC
Co-chairs: Katy Spink, PhD, Dark Horse Consulting Group and John Tomtishen, Cellares

1:30–1:50 PM	**Overview of Comparability for CGT Products: Considerations for Developers** *Katie Miller, PhD, Bayer Biologics*
1:50–2:10 PM	**CGT Comparability from Here and There: Perspective of an Ex-FDA Reviewer** *Tal Salz, PhD, Dark Horse Consulting Group*
2:10–2:30 PM	**Case Study on Comparability Issues for Cell Therapies: Atara's Experience** *Jim Sesic, Atara Biotherapeutics*
2:30–2:50 PM	**Case Study on Comparability Issues for AAV Products: Solid Biosciences' Experience** *Brian Collins, PhD, Solid Biosciences*
2:30–2:55 PM	**Panel Discussion** *Katie Miller, PhD* *Tal Salz, PhD* *Jim Sesic* *Brian Collins, PhD*

SCHEDULE (ALL TIMES LISTED IN PT)

TUESDAY, MAY 16, 2023

SCIENTIFIC SYMPOSIUM

ROOM 501 ABC

Implementing Approved Gene Therapies in Clinical Care: Considerations for Patients

Co-chairs: Betsy Bogard, Ensoma and Nicolas Abreu, MD, NYU Langone

1:30–1:55 PM	**Patient Advocate Perspectives on Approved Gene Therapies: Coverage and Conversations with Stakeholders** *Barbara Lavery, ACGT Foundation*
1:55–2:20 PM	**Value Demonstration and Access Considerations for Gene Therapies** *Clark Paramore, MSPH, bluebird bio*
2:20–2:45 PM	**Clinician Perspectives and Patient Care Considerations for Implementing Cancer Gene Therapy** *Stephen Russell, MD, PhD, Vyriad Inc.*
2:45–3:10 PM	**Perspectives on Payment for Launching Approved Gene Therapies** *Patrick DeMartino, MD, Oregon Health and Science University*

SCHEDULE (ALL TIMES LISTED IN PT)

TUESDAY, MAY 16, 2023

SCIENTIFIC SYMPOSIUM

ROOM 502 AB
Pricing Considerations in Genetic Therapies - Reassessing Access and Privilege
Co-chairs: David Segal, PhD, University of California, Davis and Rachel Salzman, DVM, Armatus Bio

1:30–1:45 PM	**Setting the Stage: Outlining the Ethical Dilemmas Involved in Pricing and Access** *Rachel Salzman, DVM, Armatus Bio*
1:45–2:00 PM	**Pricing of and Access to Gene Therapies - Opportunities and Challenges** *Sarah Emond, Institute for Clinical and Economical Review (ICER)*
2:00–2:15 PM	**Pricing and Valuation of CGTs from the Developer Perspective** *Mary-Lacey Reuther, CSL Behring*
2:15–2:30 PM	**The Promise and Challenges of Accessing Innovative High-cost Therapies: Angelman Syndrome** *Allyson Berent, DVM, DACVIM, Foundation for Angelman Syndrome Therapeutics*
2:30–2:45 PM	**Assessing the American Health Care System: How Did We Get Here?** *Aaron Seth Kesselheim, MD, JD, Harvard Medical School*
2:45–3:15 PM	**Panel Discussion** *Rachel Salzman, DVM* *Sarah Emond* *Mary-Lacey Reuther* *Allyson Berent, DVM* *Aaron Seth Kesselheim, MD, JD*

SCHEDULE (ALL TIMES LISTED IN PT)

TUESDAY, MAY 16, 2023

SCIENTIFIC SYMPOSIUM

PETREE HALL C

Risks of Autologous ex vivo Gene Therapy and Considerations for Improvement

Co-chairs: Stefano Rivella, PhD, Children's Hospital of Philadelphia and Melissa Bonner, PhD, bluebird bio

1:30–1:55 PM	**Alternative Conditioning Strategies and New Antibody Based Regimens** *Megan Hoban, PhD, Magenta Therapeutics*
1:55–2:20 PM	**Risk of Clonal Hematopoiesis from Autologous Gene Therapies (and Baseline)** *Coleman Lindsley, MD, PhD, Dana Farber Cancer Institute*
2:20–2:45 PM	**Therapeutic Chimerism Levels in SCD (haplo)** *Francis Pierciey Jr.*
2:45–3:10 PM	**Ab Conditioning for Primary Immunodeficiencies** *Judith Shizuru, MD, PhD, Stanford University School of Medicine*

SCHEDULE (ALL TIMES LISTED IN PT)

TUESDAY, MAY 16, 2023

ORAL ABSTRACT SESSION

CONCOURSE HALL 152 & 153
AAV Capsid Biology
Co-chairs: Leszek Lisowski, MBA, PhD, Children's Medical Research Institute and Elena Balkanska-Sinclair, Ultragenyx Pharmaceutical Inc.

1:30–1:45 PM	**Identification of Host Factors Critical for rAAV2.5T Transduction of Polarized Human Airway Epithelia** *Siyuan Hao, PhD, University of Kansas Medical Center*
1:45–2:00 PM	**AAV2's Defective Trafficking in the Absence of the AAV-Receptor is Due to a Block in Endosomal Escape** *Marti Cabanes, PhD, Children's Medical Research Institute*
2:00–2:15 PM	**Modifying Immune Responses to Adeno-Associated Virus (AAV) Vectors by Capsid Engineering** *Martin Bentler, Hannover Medical School*
2:15–2:30 PM	**Structural and Kinetic Characterization of Anti-AAV9 Monoclonal Antibodies derived from Patients Post-Zolgensma Treatment** *Jane Hsi, University of Florida*
2:30–2:45 PM	**Co-identification and Characterization of Host and Viral Protein Interactomes During AAV Transduction by Two Different Proximity Labeling Methods** *Jisun Lee, PhD, UMass Chan Medical School*
2:45–3:00 PM	**Multiplexed Characterization of AAV Cellular Tropism and Promoter Expression in Large Animals Using Single-Cell RNA Sequencing** *Linda Chio, PhD, Gordian Biotechnology*
3:00–3:15 PM	**A New Class of AAVR Binders: Structure of a Goat Adeno-Associated Virus (AAVGo.1) Complexed with the Human Adeno-Associated Virus (AAVR) Receptor** *Edward Large, University of Missouri*

SCHEDULE (ALL TIMES LISTED IN PT)

TUESDAY, MAY 16, 2023

ORAL ABSTRACT SESSION

ROOM 515 AB
AAV Vectors - Product Development Manufacturing: Downstream
Co-chairs: Scott Loiler, PhD, Apic Bio
and Nikitha Nimmagadda, St. Jude Children's Research Hospital

1:30–1:45 PM	**Exploring the Robustness and Throughput of Size Exclusion Chromatography of Recombinant AAVs** *Matthew Lauber, PhD, Waters Corporation*
1:45–2:00 PM	**Novel Repression System for Gene Expression Regulation during Recombinant Adeno-Assoiciated Virus and Lentiviral Vector Manufacturing** *Weiran Shen, OBiO Technology (Shanghai) Corp., Ltd.*
2:00–2:15 PM	**Ahead of the Game: A Scalable Purification AEX Platform Capable of Achieving <10% Empty Capsids** *Ashish Sharma, Oxford Biomedica Solutions*
2:15–2:30 PM	**Impact of Downstream Buffer pH and Ionic Strength on rAAV Capsid Stability** *Vijesh Kumar, PhD, Spark Therapeutics*
2:30–2:45 PM	**Challenging the Bind and Elute Convention: A Novel Approach to Partitioning Method for Full rAAV Capsid Enrichment Using AEX Chromatography** *Tamara Zekovic, PhD Biochemistry, AskBio*
2:45–3:00 PM	**Preserving High Transduction Efficiency - Serotype Agnostic Purification of Adeno-Associated Virus Using Adsorbents Functionalized with Insilco Discovered Peptide Ligand** *Shriarjun Shastry, North Carolina State University*
3:00–3:15 PM	**IsoTag AAV - An Innovative, Scalable, Non-Chromatographic Method for Streamlined AAV Manufacturing** *Michael Dzuricky, Isolere Bio*

SCHEDULE (ALL TIMES LISTED IN PT)

TUESDAY, MAY 16, 2023

SCIENTIFIC SYMPOSIUM

CONCOURSE HALL 150 & 151

Tumor Microenvironment as a Barrier to the Success of Immunotherapies and Strategies to Overcome

Co-chairs: Sunil Raikar, MD, Emory University and Saad Kenderian, MD, Mayo Clinic

1:30–1:55 PM	**Review of Current Status of Tumor Immunologies and Obstacles to Tumor Microenvironment** *Maria Castro, PhD, University of Michigan Medical School*
1:55–2:20 PM	**Armoring T-Cells** *Joseph Fraietta, PhD, University of Pennsylvania*
2:20–2:45 PM	***In Vivo* Macrophage Engineering Reshapes the Tumor Microenvironment Leading to Eradication of Liver Metastases** *Mario Leonardo Squadrito, PhD, San Raffaele Telethon Institute for Gene Therapy*
2:45–3:10 PM	**Combinational Oncolytic Viruses** *Paola Grandi, PhD, CG Oncology*

ASGCT 26TH ANNUAL MEETING Los Angeles

SCHEDULE (ALL TIMES LISTED IN PT)

TUESDAY, MAY 16, 2023

EDUCATION SESSION

ROOM 403 AB
Vector-Derived Gene Therapy
Co-chairs: Dan Manning, PharmD, Spark Therapeutics and Donald Kohn, MD, University of California, Los Angeles

1:30–2:00 PM	**CMC: Vectors, Cells. What to Know to Leverage Early Phase Studies to Support an Eventual Biologic License Application** *Graeme Price, PhD, FDA, CBER, OTP, OGT CMC*
2:00–2:30 PM	**Overview of Lentiviral Vector Therapies: From Bench to Clinic** *John Tisdale, MD, NIH, NHLBI*
2:30–3:00 PM	**Overview of AAV Gene Therapies: From Bench to Clinic** *Allison Bradbury, PhD, Nationwide Children's Hospital; The Ohio State University Wexner Medical Center*

SCHEDULE (ALL TIMES LISTED IN PT)

TUESDAY, MAY 16, 2023

GENERAL SESSION

WEST HALL B
3:45–5:30 pm
Founders and Catalyst Award Lectures
Speakers: David Williams, MD, Boston Children's Hospital and Joy Cavagnaro, PhD, Access Bio

3:55–4:40 PM	**The Maturation of Hematopoietic Stem Cell Gene Therapy: Of Mice and Men** *David Williams, MD, Boston Children's Hospital*
4:40–5:30 PM	**It's All About The Dose** *Joy Cavagnaro, PhD, Access Bio*

ASGCT 26TH ANNUAL MEETING
Los Angeles

SCHEDULE (ALL TIMES LISTED IN PT)

TUESDAY, MAY 16, 2023

RECEPTION

EXHIBIT HALL
5:30–7:00 pm
Welcome Reception

SCHEDULE (ALL TIMES LISTED IN PT)

WEDNESDAY, MAY 17, 2023

SCIENTIFIC SYMPOSIUM

ROOM 515 AB
Accelerated Approval for Cell and Gene Therapies
Co-chairs: Keith Wonnacott, PhD, Lexeo Therapeutics and Kristin Van Goor, PhD, Vertex Pharmaceuticals

8:00–8:20 AM	**Considerations for Accelerated Approval for Gene Therapy** *Shelby Elenburg, MD, FDA CBER*
8:20–8:40 AM	**Bluebird's Experience with Accelerated Approval and Efficacy Data—Discussion on the Skysona (eli-cel) Experience** *Leslie Wilder, bluebird bio*
8:40–9:00 AM	**The Scientific Underpinnings of Accelerated Approval: Validity of Biomarkers and Clinical Data to Demonstrate Relevance** *Aaron Seth Kesselheim, MD, JD, Harvard Medical School*
9:00–9:45 AM	**Panel Discussion** *Adora Ndu, PharmD, JD* *Aaron Seth Kesselheim, MD, JD* *Peter Marks, MD, PhD* *Leslie Wilder* *Courtney Silverthorn, PhD* *Shelby Elenburg, MD*

SCHEDULE (ALL TIMES LISTED IN PT)

WEDNESDAY, MAY 17, 2023

SCIENTIFIC SYMPOSIUM

PETREE HALL C

Advances of Gene and Cell Therapy in Europe (ESGCT)

Co-chairs: Alberto Auricchio, MD, PhD, Fondazione Telethon and Juan Bueren, PhD, CIEMAT/CIBERER/Fundación Jiménez Diaz

8:00–8:25 AM	**Advances of Gene and Cell Therapy in Europe (ESGCT)** *Vania Broccoli, Ospedale San Raffaele*
8:25–8:50 AM	**Dressing Up Viruses to Fool Cancer: Towards Personalised Oncolytic Vaccines** *Vincenzo Cerullo, PhD, University of Helsinki*
8:50–9:15 AM	**Making Effective Gene Therapies Available For Patients With Rare Diseases** *Claire Booth, PhD, UCL GOS Institute of Child Health*
9:15–9:40 AM	**Advancing Genetic Engineering of Hematopoietic Stem Cells for Treating Genetic Diseases and Cancer** *Luigi Naldini, PhD, SR-TIGET*

SCHEDULE (ALL TIMES LISTED IN PT)

WEDNESDAY, MAY 17, 2023

SCIENTIFIC SYMPOSIUM

ROOM 411

Advancing Novel Delivery Tools and Treatments for Neurodegenerative Disorders

Co-chairs: Fredric Manfredsson, PhD, Barrow Neurological Institute and Megan Keiser, PhD, Children's Hospital of Philadelphia

8:00–8:25 AM	**Discovery and Characterization of Novel Cross-Species BBB-Penetrant Capsids** *Brett Hoffman, PhD, Voyager Therapeutics*
8:25–8:50 AM	**Exosomes for Delivery to the CNS** *Dhanu Gupta, University of Oxford*
8:50–9:15 AM	**A Novel Delivery Approach to Support Development of Genetic Medicines for Human Inner Ear Conditions** *Michelle Valero, PhD, Akouos, a wholly owned subsidiary of Eli Lilly*
9:15–9:40 AM	**Pushing the Envelope: Will Novel Gene Knockdown Strategies Get us to a Cure for Motor Neuron Disease?** *Defne Amado, MD, PhD, University of Pennsylvania*

SCHEDULE (ALL TIMES LISTED IN PT)

WEDNESDAY, MAY 17, 2023

SCIENTIFIC SYMPOSIUM

CONCOURSE HALL 152 & 153
Refinement of NHP Usage in Gene Therapy Drug Development
Co-chairs: PJ Brooks, PhD, NCATS, NIH
and Nicole Paulk, PhD, University of California San Francisco

8:00–8:25 AM	**Diversifying Away from NHPs** *Christine Fuentes, PhD, Dark Horse Consulting*
8:25–8:50 AM	***In Vitro* Pharmacology Study of Gene Therapy Using Retina Organoids of CLN2 Patient-Derived Induced Pluripotent Stem Cells** *Kwi Hye Kim, PhD, REGENXBIO Inc*
8:50–9:15 AM	**Ocular Anatomy and Relevance to Gene Therapy & Alternative Animal Models for Ocular Gene Therapy** *Timothy MacLachlan, PhD, Novartis* *and Krishna Yekkala, PhD, Janssen Pharmaceuticals*
9:15–9:40 AM	**Use of Rat Data to Support an IND for a Rare Disease Trial (NGLY1)** *Becky Schweighardt, PhD, Grace Science, LLC*

SCHEDULE (ALL TIMES LISTED IN PT)

WEDNESDAY, MAY 17, 2023

SCIENTIFIC SYMPOSIUM

ROOM 403 AB
Storytelling as a Scientist
Co-chairs: Catherine Gillespie, Houston Methodist Hospital Foundation and Lynnea Olivarez, Encoded Therapeutics

8:00–8:25 AM	**Genomics for Everyone? Perspectives from an Indigenous Geneticist-Ethicist** *Krystal Tsosie, PhD, MPH, Arizona State University*
8:25–8:50 AM	**How to Form a Narrative Arc when You're Talking or Writing about Science from a Scientist-Turned-Filmmaker-Turned-Author** *Randy Olson, PhD, Randy Olson Productions*
8:50–9:15 AM	**Explaining Key Messages in Gene Therapy Effectively to a Lay v. Scientist Audience with a Focus on Viral Vectors** *Semih Tareen, PhD, Sana Biotechnology*
9:15–9:40 AM	**Communicating Science on Social Media** *Nicole Paulk, PhD, University of California San Francisco*

SCHEDULE (ALL TIMES LISTED IN PT)

WEDNESDAY, MAY 17, 2023

SCIENTIFIC SYMPOSIUM

ROOM 408 AB

How Novel Technologies Will Advance Understanding of Neurological Disorders and Development of Transformative Therapies (CIRM)

Co-chairs: Abla Creasey, PhD, CIRM and Shyam Patel, PhD, CIRM

8:00–8:10 AM	**Accelerating the Development of Cell and Gene Therapies in California** *Shyam Patel, PhD, California Institute for Regenerative Medicine and Abla Creasey, PhD, CIRM*
8:10–8:40 AM	**Developing AAV Gene Therapies for Movement Disorders** *Krystof Bankiewicz, MD, PhD, The Ohio State University*
8:40–9:40 AM	**Panel Discussion** *Claire Henchcliffe and Hartmuth Kolb*

SCHEDULE (ALL TIMES LISTED IN PT)

WEDNESDAY, MAY 17, 2023

SCIENTIFIC SYMPOSIUM

ROOM 502 AB

The 5-Minute Presentation Challenge: How to Present Your Ideas

Co-chairs: Sarwish Rafiq, PhD, Emory University and Jaquelyn Zoine, PhD, St. Jude Children's Research Hospital

8:00–9:45 AM	**The 5-Minute Thesis Challenge: How to Present Your Ideas** **Judges:** *Ashvin Bashyam, PhD, Ensoma* *Marina Pavlou, PhD, University of Washington* *Ananya Zutshi, Guardian Bio*
8:15–8:30 AM	**Sergi Verdés, Universitat Autònoma de Barcelona (UAB)**
8:30–8:45 AM	**Sandhya Sharma, Baylor College of Medicine**
8:45–9:00 AM	**John Riley, Children's Hospital of Philadelphia**

SCHEDULE (ALL TIMES LISTED IN PT)

WEDNESDAY, MAY 17, 2023

SCIENTIFIC SYMPOSIUM

CONCOURSE HALL 150 & 151

Predicting and Overcoming Immune Responses in Gene and Cell Therapies

Co-chairs: Matthew Gardner, PhD, Emory University and Abraham Scaria, PhD, AGTC (Applied Genetic Technologies Corporation)

8:00–8:24 AM	**Predicting Immune Responses and Toxicity in Ocular Gene Transfer** *Kathryn Pepple, MD, PhD, University of Washington*
8:25–8:50 AM	**Delivery of AAVs to the Central Nervous System Using MRI-Guided Focused Ultrasound and Immunological Considerations** *Isabelle Aubert, PhD, Sunnybrook Research Institute/ University of Toronto*
8:50–9:15 AM	**Assessing Transgene Immunogenicity in the Context of (LV) Gene Therapies** *Andrea Annoni, PhD, SR-TIGET*
9:15–9:40 AM	**Innate Immunity to AAV** *Roland W. Herzog, PhD, IU School of Medicine*

SCHEDULE (ALL TIMES LISTED IN PT)

WEDNESDAY, MAY 17, 2023

EDUCATION SESSION

PETREE HALL D
Gene Editing Technologies
Co-chairs: Kimberly Nye, TESS Research Foundation and Renata Stripecke, PhD, University of Cologne

8:00–8:30 AM	**CRISPR Off-Target Activity and Mitigation Strategies** *Kyle Cromer, PhD, UCSF*
8:30–9:00 AM	**Prime Editing Corrections of Point Mutations Responsible for Duchenne Muscular Dystrophy, Central Core Disease, Ataxia-8 and Malignant Hyperthermia** *Jacques Tremblay, PhD, Laval University*
9:00–9:30 AM	**CRISPR-derived Genome Editing Therapies: Progress from Bench to Bedside** *Zoya Gluzman-Polorak, PhD, SonoThera*

SCHEDULE (ALL TIMES LISTED IN PT)

WEDNESDAY, MAY 17, 2023

EXHIBITOR SHOWCASES

8:15–8:45 AM
ROOM 511
PTC Therapeutics, Inc.:
Pioneering in Targeted Gene Delivery to the Brain

8:15–8:45 AM
ROOM 409
Repligen: Vector manufacturing process:
addressing bottlenecks with innovative solutions

9:15–9:45 AM
ROOM 511
WuXi Advanced Therapies: Understanding the shortest route
to approval for Advanced Therapies

9:15–9:45 AM
ROOM 409
Forge Biologics: Building an Improved Platform Process for
AAV Manufacturing

SCHEDULE (ALL TIMES LISTED IN PT)

WEDNESDAY, MAY 17, 2023

OUTSTANDING ACHIEVEMENT AWARD IS SUPPORTED BY:

Spark THERAPEUTICS

AWARD SYMPOSIUM

WEST HALL B
Outstanding Achievement Award Symposium
Chair: Jeffrey S. Chamberlain, PhD, University of Washington

10:35–11:15 AM	**Gene Editing Voyages from Z to C (Zinc fingers to CRISPR)** *J. Keith Joung, MD, Massachusetts General Hospital*
11:15–11:30 AM	**Translating T Cell Therapies From Boutique to Global (Inveniemus Aut Faciemus)** *Bruce Levine, PhD, University of Pennsylvania*
11:30–11:45 AM	**Building a Highway for CARs** *Isabelle Rivière, PhD, Takeda*
11:45 AM–12:00 PM	**One Patient, One Product - The Development of Adoptive T-cell Therapy** *Mark Dudley, PhD, Instil Bio*

SCHEDULE (ALL TIMES LISTED IN PT)

WEDNESDAY, MAY 17, 2023

INDUSTRY SPONSORED SYMPOSIA

12:00 PM–1:30 PM

PETREE HALL C
Oxford Biomedica: Moving Gene Therapy Forward - How Process Improvements Drive Productivity to Reach More Patients

PETREE HALL D
Miltenyi Biotec: Latest Advances in Cell and Gene Therapy

ROOM 408 AB
Vertex Pharmaceuticals: Targeted Delivery of Cellular and Genetic Therapies: Challenges and Advances

ROOM 409
Terumo Blood and Cell Technologies: Hollow Fiber Bioreactor Expansion of CD3+ T Cells and CD34+ HSCs

ROOM 511
MilliporeSigma

ROOM 403
Bio-Techne: Supporting Development of Next Generation Cell and Gene Therapies that Seamlessly Scale to Commercialization: From Cell Engineering Solutions to Pre-Clinical Efficacy Testing

ROOM 501
Siren Biotechnology: Universal AAV Immuno-Gene Therapy: Sounding the Alarm Against Cancer

SCHEDULE (ALL TIMES LISTED IN PT)

WEDNESDAY, MAY 17, 2023

STARTUP SPOTLIGHT

Presentation Theater in Exhibit Hall
Chair: Xiaojing Shi, Capsida Biotherapeutics, Inc.

12:00–12:10 PM	OcyonBio: PDMOs is a Novel Business Model That Can Help Companies Advance Their Pipelines.
12:10–12:20 PM	Logomix, Inc: Precise and Mb-scale iPSC Genome Engineering for Cell Therapy & Regenerative Medicine by Logomix Geno-Writing™ Platform
12:20–12:30 PM	Repair Biotechnologies: Clearance of Excess Free Cholesterol Using LNP-mRNA Therapy Reverses NASH Pathology
12:30–12:40 PM	Rejuvenate Bio: Targeting the Core Drivers of Chronic Age-related Diseases Through Gene Expression and Epigenetic Reprogramming
12:40–12:50 PM	CellFe Biotech: Microfluidics Delivering the Next Generation of Cell Therapies
12:50–1:00 PM	SeQure Dx: Off-target Risk and Genetic Diversity: Is Your Therapy Ready Everyone?
1:00–1:10 PM	Triovance: Genetically Modified Skin Graft for Accelerating Wound Healing
1:10–1:20 PM	Watershed Informatics: Scalable, Accessible and Reproducible Multi-omics Analyses for Advancing Personalized Cell Therapies
1:20–1:30 PM	Northern Biomolecular Services

ASGCT 26TH ANNUAL MEETING
Los Angeles

SCHEDULE (ALL TIMES LISTED IN PT)

WEDNESDAY, MAY 17, 2023

POSTER SESSION I

12:00-1:30 PM
EXHIBIT HALL/WEST HALL A

SCHEDULE (ALL TIMES LISTED IN PT)

WEDNESDAY, MAY 17, 2023

PRESIDENTIAL SYMPOSIUM IS SPONSORED BY:

Biogen

GENERAL SESSION

WEST HALL B

Presidential Symposium and Presentation of Top Abstracts

Speakers: David R. Liu, PhD, Broad Institute, Harvard University, and HHMI and Jennifer Doudna, PhD, UC Berkeley

1:30–1:50 PM	**CRISPR Chemistry and Applications in the Clinic** *Jennifer Doudna, PhD, UC Berkeley*
1:50–2:20 PM	**Base Editing and Prime Editing: Correcting Mutations that Cause Genetic Disease in Cells, Animals, and Patients** *David R. Liu, PhD, Broad Institute, Harvard University, and HHMI*
2:20–2:38 PM	**Efficacy and Safety of a Single Dose of Exagamglogene Autotemcel for Transfusion-Dependent β-Thalassemia and Severe Sickle Cell Disease** *Haydar Frangoul, Children's Hospital Tristar Centennial*
2:38–2:53 PM	**Split Intein-Mediated Protein*trans-Splicing* to Express Large Dystrophins** *Hichem Tasfaout, PhD, University of Washington School of Medicine*
2:53–3:09 PM	**Novel AAV-Capsid-Mediated Delivery of an RNAi Targeting Atxn2 Extends Survival and Improves Strength and Neuroinflammation in a Mouse Model of Sporadic ALS** *Defne Amado, MD, PhD, Neurology University of Pennsylvania*

SCHEDULE (ALL TIMES LISTED IN PT)

WEDNESDAY, MAY 17, 2023

ORAL ABSTRACT SESSION

CONCOURSE HALL 150 & 151
AAV Library Technology
Co-chairs: Mathieu Nonnenmacher, PhD, Voyager Therapeutics and Anna Maurer, PhD, UCBerkeley

3:45–4:00 PM	**Parallel Engineering and Recombination of Adeno-Associated Virus Variable Regions Enables Multisite Library Production** *David Goertsen, California Institute of Technology*
4:00–4:15 PM	**APPRAISE: Fast, Accurate Ranking of Engineered Proteins by Receptor Binding Propensity Using Structure Modeling** *Xiaozhe Ding, California Institute of Technology*
4:15–4:30 PM	***In Vivo* AAV Capsid Selection at Spatial and Single-Cell Resolution** *Ashley Robbins, University of Pennsylvania*
4:30–4:45 PM	**Antibody Conjugated AAV Vectors for Efficient and Specific Skeletal Muscle Directed Gene Delivery Across Species** *Leah Sabin, PhD, Regeneron Pharmaceuticals*
4:45–5:00 PM	**Generative Networks Create Novel Receptor Targeted AAVs with only 1,200 Training Examples** *Andrew Barry, PhD, Broad Institute of MIT and Harvard*
5:00–5:15 PM	**Development of Stabilized AAV Capsids for Vector Engineering** *Simon Pacouret, PhD, Broad Institute of MIT and Harvard*
5:15–5:30 PM	***In Vivo* Targeting of HSCs by Capsid-engineered AAV Vectors** *Nadja Meumann, Hannover Medical School*

SCHEDULE (ALL TIMES LISTED IN PT)

WEDNESDAY, MAY 17, 2023

ORAL ABSTRACT SESSION

CONCOURSE HALL 152 & 153
AAV Vectors - Product Development Manufacturing: Upstream
Co-chairs: Anna Ucher, UMass Chan Medical School and Xin Chen, UT Southwestern Medical Center

3:45–4:00 PM	**Development of a Novel Plug-and-Play Upstream Process Offering Scalable, High-Yield Production of High-Quality AAV Vectors** *Klaudia Szymczak, Oxford Biomedica (OXB) Solutions*
4:00–4:15 PM	**Analysis of Mechanisms Driving Imrpoved Adeno-Associated Vector Production by the Addition of Small Molecules** *Nicholas Dietrich, Kriya Therapeutics, Inc*
4:15–4:30 PM	**A Modified Triple Transfection Method Produces High-Quality AAV Vectors with Much Reduced Plasmid Demand** *Hao Liu, PhD, UMass Chan Medical School*
4:30–4:45 PM	**Adenovirus L4 22 and 33K Proteins are Essential for Episomal Amplification of Integrated rep/cap Genes and rAAV Production from Stable AAV Packaging Cells** *Weiheng Su, OXGENE*
4:45–5:00 PM	**A Combined Transcriptomics and Compound Screening Approach to Increasing AAV Productivity in Gene Therapy Process Development** *Maria Choi-Ali, Biogen*
5:00–5:15 PM	**Development of AAV Continuous High Production Technology by FF Original Flow Electroporation Method** *Yoichi Nagai, Fujifilm*
5:15–5:30 PM	**Synthetic Cell Lines for Recombinant AAV Production** *Min Lu, University of Minnesota*

SCHEDULE (ALL TIMES LISTED IN PT)

WEDNESDAY, MAY 17, 2023

ORAL ABSTRACT SESSION

PETREE HALL C

Synthetic/Molecular Conjugates and Physical Methods for Delivery of Gene Therapeutics I

Co-chairs: Loree Heller, University of South Florida and Carol Miao, PhD, Seattle Children's Research Institute

3:45–4:00 PM	**Focused Ultrasound for Improved Delivery of AAV Vectors to the Brain for the Treatment of Huntington's Disease** *Nick Todd, Brigham and Women's Hospital*
4:00–4:15 PM	**Liver-specific Targeting CRISPR/Cas9 mRNA LNPs Achieve Long-term FVIII Expression in Hemophilia A Mice** *Chun-Yu Chen, PhD, Seattle Children's Research Institute*
4:15–4:30 PM	**A Novel Multitargeted Peptide-siRNA Complex for Simultaneous Inhibition of SARS-CoV-2 Host Cell Entry and Intracellular Viral Replication** *Martina Tuttolomondo, University of Southern Denmark*
4:30–4:45 PM	**Isolation of Natural and Biologically Active EV-AAVs for Brain Gene Delivery** *Rui Nobre, Center for Neuroscience and Cell Biology*
4:45–5:00 PM	**Shuttle Peptides-mediated Local RNP Delivery of Editing Complex in the Sensory Organs of the Inner Ear and Retina In Vivo in Adult Mice** *Wan Du, MD, PhD, Harvard Medical School/Massachusetts Eye and Ear*
5:00–5:15 PM	**Hematopoietic Stem and Progenitor Cell Specific Antibodies and Intraosseous Administration Enhance Delivery of a CRISPR Nanoformulation** *Daniel Lane, PhD, Fred Hutchinson Cancer Center*
5:15–5:30 PM	**NHEJ Gene Editing of Hemophilia A Mice Show Therapeutic Levels of FVIII Following Ultrasound Mediated Gene Delivery of CRISPR/Cas9 Plasmid** *Savannah Lawton, Seattle Children's Research Institute*

SCHEDULE (ALL TIMES LISTED IN PT)

WEDNESDAY, MAY 17, 2023

ORAL ABSTRACT SESSION

PETREE HALL D
Epi-/Genetic Editing and New Methods to Target the Nervous System
Co-chairs: Vania Broccoli, Ospedale San Raffaele and Monika Chauhan, University of Minnesota

3:45–4:00 PM	**Zinc Finger Transcriptional Regulator Mediated Repression of *SCN9A* as a Therapeutic Approach for Painful Peripheral Neuropathies** *Mohammad Samie, PhD, Sangamo Therapeutics, Inc.*
4:00–4:15 PM	**Delivery of BDNF Through a Pluripotent Stem Cell-based Platform Ameliorates Behavioral Deficits in a Mouse Model of Huntington's Disease** *Sridhar Selvaraj, Stanford University School of Medicine*
4:15–4:30 PM	**RNA Editing Therapy in a Humanized Mouse Model of MECP2 Duplication Syndrome and Non-Human Primates** *Xiaoqing Wu, HuidaGene Therapeutics Co., Ltd.*
4:30–4:45 PM	**The Interplay Between Central Nervous System and Peripheral Organs: The Lesson from the Treatment of Canavan Disease with rAAV Gene Therapy** *Jie Wang, PhD, UMass Chan Medical School*
4:45–5:00 PM	**Gene Editing Strategies to Treat Spinocerebellar Ataxia type 1** *Kelly Fagan, University of Pennsylvania*
5:00–5:15 PM	**Single-Cell RNA-sequencing to Evaluate rAVV transduction in the CNS and its Therapeutic Implications for Canavan Disease Gene therapy** *Jie Wang, PhD, UMass Chan Medical School*
5:15–5:30 PM	**Ionizable Lipid Nanoparticles for Therapeutic Base Editing of Congenital Brain Disease** *Rohan Palanki, University of Pennsylvania*

SCHEDULE (ALL TIMES LISTED IN PT)

WEDNESDAY, MAY 17, 2023

ORAL ABSTRACT SESSION

ROOM 403 AB
Immunotherapy, Oncolytic Viruses, and Cytokines
Co-chairs: Barbara Savoldo, MD, PhD, UNC Lineberger Comprehensive Cancer Center
and Marcela Maus, MD, PhD, Massachusetts General Hospital

3:45–4:00 PM	**UB-VV200 is a Novel Surface-engineered Lentiviral Product Candidate for *In Vivo* Engineering of Universal TagCAR T cells for the Treatment of Solid Tumors** *Alyssa Sheih, PhD, Umoja Biopharma*
4:00–4:15 PM	**TNT: Talimogene Laherparepvec (an Oncolytic Virus Expressing GM-CSF), Nivolumab and Trabectedin for Advanced Leiomyosarcoma: A Phase 2 Study *[NCT# 03886311]*** *Neal Chawla, MD, City of Hope*
4:15–4:30 PM	**Cytokine Encoding Adenoviruses Advance T cell Recruitment To Cancerous Tumors Of Renal Cell Carcinoma** *Michaela Feodoroff, University of Helsinki*
4:30–4:45 PM	**PD-L1 siRNA and Cyclic Dinucleotide Based Immune Reprogramming for Cancer Immunotherapy** *Fanfei Meng, Purdue University*
4:45–5:00 PM	**Cis-targeted Cytokines for Specific Stimulation of CAR T cells** *Sara Sleiman, MD, University of Pennsylvania*
5:00–5:15 PM	**Correlative Results of a Phase 1/2 Study of Pembrolizumab Combined with Blinatumomab in Patients with Relapsed/Refractory (r/r) ALL** *Alan Macias, City of Hope*
5:15–5:30 PM	**Epigenetic-mediated Cancer Rewind Enhanches Oncolytic Immunovirotherapy** *Salvatore Russo, University of Helsinki*

SCHEDULE (ALL TIMES LISTED IN PT)

WEDNESDAY, MAY 17, 2023

ORAL ABSTRACT SESSION

ROOM 408 AB
AAV Vectors - Preclinical and Proof-of-Concept *In Vivo* Studies I
Co-chairs: Phillip Tai, PhD, UMass Chan Medical School and Kruti Patel, PhD, Pfizer

3:45–4:00 PM	**AAV Immuno-Gene Therapy Delivers Vectorized Cytokines to Effectively Treat High-Grade Gliomas** *Nicole Paulk, PhD, Siren Biotechnology*
4:00–4:15 PM	**Durability of Bicistronic AAV Gene Therapy for Tay-Sachs Disease in the Sheep Model** *Toloo Taghian, PhD, UMass Chan Medical School*
4:15–4:30 PM	**More than Nine Year Survival of a GRMD Dog After Injection of AAV-Microdystrophin Gene Therapy** *Caroline Le Guiner, PhD, Nantes Université, CHU de Nantes, INSERM*
4:30–4:45 PM	**Intravitreal Gene Therapy of Retinitis Pigmentosa (RP) Associated With Mutations in the *CNGA1* gene (*CNGA1*-RP)** *Stylianos Michalakis, LMU Munich*
4:45–5:00 PM	**AAV Vaccine in a Preclinical Spontaneous Canine Model of Oral Melanoma** *Ester Molina, PhD, The Hormel Institute, University of Minnesota*
5:00–5:15 PM	**The Longitudinal Kinetics of AAV5 Vector Integration Profiles in Mice** *Ashrafali Mohamed Ismail, BioMarin Pharmaceutical Inc.*
5:15–5:30 PM	**A Novel Gene Therapy for X-Linked Neurological Disorders Including Rett Syndrome Through Reactivation of the Silent X Chromosome** *Kathrin Meyer, PhD, Nationwide Children's Hospital*

SCHEDULE (ALL TIMES LISTED IN PT)

WEDNESDAY, MAY 17, 2023

ORAL ABSTRACT SESSION

ROOM 409 AB
RNA Virus Vectors for *In Vivo* and *Ex Vivo* Applications
Co-chairs: Christian Brendel, PhD, Boston Children's Hospital/ Dana-Farber Cancer Institute and Melissa Kotterman, PhD, Iris Medicine

3:45–4:00 PM	**Liver-Directed Lentiviral Gene Therapy is Safe and Curative in Argininosuccinic Aciduria** *Loukia Touramanidou, Great Ormond Street Institute of Child Health, University College London*
4:00–4:15 PM	**Macrophage Inhibitor Clodronate Enhances Liver Transduction of Lentiviral but Not AAV Vectors or mRNA Lipid Nanoparticles *In Vivo*** *Loukia Touramanidou, University College of London*
4:15–4:30 PM	**Characterization and Cure of a New Mouse Model of Alpha-Thalassemia Major** *Stefano Rivella, PhD, Children's Hospital of Philadelphia*
4:30–4:45 PM	**Liver-Directed Lentiviral Gene Therapy For ARC Syndrome** *Andrei Claudiu Cozmescu, University College London*
4:45–5:00 PM	**Improving The Efficiency Of *In Vivo* Lentiviral Gene Transfer To Hepatocytes, By Targeting Anti-Viral Pathways, And Application To Familial Hypercholesterolemia** *Cesare Canepari, San Raffaele Telethon Institute for Gene Therapy*
5:00–5:15 PM	**Development of an *Ex Vivo* Gene Therapy for Infantile GM1 Gangliosidosis** *Valentina Poletti, Dana-Farber/Boston Children's Cancer and Blood Disorders Center, Harvard Medical School*
5:15–5:30 PM	**Lentiviral Gene Therapy for CARD9 Deficiency** *Karissa Bever, NIAID, National Institutes of Health*

SCHEDULE (ALL TIMES LISTED IN PT)

WEDNESDAY, MAY 17, 2023

ORAL ABSTRACT SESSION

ROOM 411
AAV Vectors: Clinical and Non-Human Primate Studies
Co-chairs: Ana Buj Bello, MD, PhD, GENETHON and Amber Van Laar, Asklepios BioPharmaceutical, Inc.

3:45–4:00 PM	**Large-scale Characterization of the Location and Expansion of AAV Integrations in Macaques and Humans Following *In Vivo* Exposure** *Kelly Martins, University of Pennsylvania*
4:00–4:15 PM	**The Safety and Efficacy of Pre-Treatment with Imlifidase Prior to Adeno Associated Virus (AAV)-Based Gene Therapy in Non-Human Primates with Pre-existing Anti-AAVrh74 Antibodies** *Rachael Potter, Sarepta Therapeutics*
4:15–4:30 PM	**Bilateral Ixo-vec NHP Tolerability and Efficacy Following a Staggered Dosing Interval Between Eyes - Gene Therapy nAMD** *Kris Poulsen, Adverum Biotechnologies*
4:30–4:45 PM	**Monitoring Fetal and Infant Somatic Cell Genome Editing in Rhesus Monkeys with Total-Body PET** *Alice Tarantal, PhD, University of California, Davis*
4:45–5:00 PM	**AAV Serotype Tropism and Editing in Young Rhesus Monkeys** *Alice Tarantal, PhD, University of California, Davis*
5:00–5:15 PM	**The Machine Learning-Guided Fit4Function Platform Quantitatively Profiles the Biodistribution of Peptide-Modified AAV Capsids After a Single Round of Screening in Macaque** *Fatma Elzahraa Eid, Broad Institute of MIT and Harvard*
5:15–5:30 PM	**AAV-barcoding for High-throughput Screening of Vector Transduction Efficiency in Cynomolgus Macaques Compared to C57BL/6 Mice** *Frederick Ashby, University of Florida*

SCHEDULE (ALL TIMES LISTED IN PT)

WEDNESDAY, MAY 17, 2023

ORAL ABSTRACT SESSION

ROOM 501 ABC
Nucleic Acid Therapeutics
Co-chairs: Bruce Sullenger, PhD, Duke University Medical Center and Isabel Aznarez, Stoke Therapeutics

3:45–4:00 PM	**Transferrin Receptor-Targeted RNA Aptamer Enhanced Blood-Brain Barrier Penetration in Brain Metastases Occurring from Triple-Negative Breast Cancer** *Minsun Song, PhD, City of Hope*
4:00–4:15 PM	**The FORCE™ Platform Delivers Oligonucleotides to the Brain in a DM1 Mouse Model and in NHPs** *Stefano Zanotti, PhD, Dyne Therapeutics*
4:15–4:30 PM	**Long-circulating Lipid Nanoparticles for Agnostic Nucleic Acid Delivery to the Bone Marrow and Beyond** *Kevin An, NanoVation Therapeutics*
4:30–4:45 PM	**RNA Base Editing For The Treatment Of Alpha-1 Antitrypsin Deficiency** *Prashant Monian, PhD, Wave Life Sciences*
4:45–5:00 PM	**Phosphoryl Guanidine-Containing Oligonucleotides Support Exon Skipping in Skeletal Muscle in Mice and Boys with DMD** *Pachamuthu Kandasamy, Wave Life Sciences*
5:00–5:15 PM	**A Novel Small Molecule-Aptamer Pair for Regulating Gene Expression *In Vitro* and *In Vivo*** *Yohei Yokobayashi, , Okinawa Institute of Science and Technology Graduate University*
5:15–5:30 PM	**tRNA Picovectors Represent a New Therapeutic Approach for the Rescue of Premature Termination Codons** *Joseph Porter, University of Rochester*

SCHEDULE (ALL TIMES LISTED IN PT)

WEDNESDAY, MAY 17, 2023

ORAL ABSTRACT SESSION

ROOM 502 AB
CAR Engineering and Production Advances for Targeting Hematologic and Solid Tumor Malignancies
Co-chairs: Saad Kenderian, Mayo Clinic and Rima Saha, PhD, University of Florida

3:45–4:00 PM	**CD5 CAR T-Cells Promote Selection of Fratricide-Resistant Circulating T-Cells Thus Avoiding Global T-Cell Aplasia** *Royce Ma, Baylor College of Medicine*
4:00–4:15 PM	**CAR Signaling Drives Distinct Immunological Synapse Dynamics That Influence the T Cell Behavior in Killing Cancer** *Ahmed Gad, Baylor College of Medicine*
4:15–4:30 PM	**Potent *In Vivo* Transduction by iGPS Particles Generates CAR T-Cells with Durable Anti-Tumor Activity** *Shannon Contrastano, Kelonia Therapeutics*
4:30–4:45 PM	**Engineering Optimal CAR T Cells to Overcome Pancreatic Tumors with Secreted Antagonistic Peptides** *Heather Lin, Emory University School of Medicine*
4:45–5:00 PM	**Harnessing CD39 For The Treatment Of Colorectal Cancer And Liver Metastases By Engineered T Cells** *Alessia Potenza, IRCCS San Raffaele Scientific Institute*
5:00–5:15 PM	**Enhancement of K-Ras Neo-Antigen Targeting CAR-T Cells via Homogenous Knock in of Inducible IL-12** *Alex Benton, University of Pennsylvania*
5:15–5:30 PM	**AMELI-01: A Phase I Trial of UCART123v1.2, an Anti-CD123 Allogeneic CAR-T Cell Product, in Adult Patients with Relapsed or Refractory (R/R) CD123+ Acute Myeloid Leukemia (AML)** *Daniel Lee, MD, Cellectis, Inc*

SCHEDULE (ALL TIMES LISTED IN PT)

WEDNESDAY, MAY 17, 2023

ORAL ABSTRACT SESSION

ROOM 515 AB
Genome Editing Therapies & Safety I
Co-chairs: Shengdar Tsai, PhD, St. Jude Children's Research Hospital and Mark Osborn, PhD, University of Minnesota

3:45–4:00 PM	**Epitope Edited Hematopoietic Stem Cells Allow Immune-Based *In Vivo* Selection of Genome-Engineered Cells** *Gabriele Casirati, MD, PhD, Dana Farber Cancer Institute*
4:00–4:15 PM	**Development of Prime Editing Strategies for the Treatment of B-Hemoglobinopathies** *Anne Chalumeau, Imagine Institute*
4:15–4:30 PM	**CHANGE-seq-BE Enables Sensitive and Unbiased Genome-Wide Profiling of Adenine Base Editors *in vitro*** *Cicera Lazzarotto, DVM, PhD, St. Jude Children's Research Hospital*
4:30–4:45 PM	**Uncovering Upsides and Pitfalls of Base and Prime Editing in Hematopoietic Stem Cells** *Martina Fiumara, SR-TIGET*
4:45–5:00 PM	**Sniper2L, a High-Fidelity Cas9 Variant with High Activity** *Jungjoon Lee, PhD, Toolgen*
5:00–5:15 PM	**Genetic Variant Off-Target Editing Assessment by Lentiviral Proxy Assay** *My Anh Nguyen, Boston Children's Hospital*
5:15–5:30 PM	**Prime Editing of Human CD34$^+$ Long-Term Hematopoietic Stem Cells Precisely Corrects the Causative Mutation of p47phox Chronic Granulomatous Disease and Restores NADPH Oxidase Activity in Myeloid Progeny** *Jennifer Gori, PhD, Prime Medicine, Inc.*

SCHEDULE (ALL TIMES LISTED IN PT)

WEDNESDAY, MAY 17, 2023

TOOLS AND TECHNOLOGY FORUM

PRESENTATION THEATER IN EXHIBIT HALL
Tools and Technology Forum I
Chair: Li Ou, PhD, Genemagic Bio

3:45–4:00 PM	Forge Biologics: Building an Improved Platform Process for AAV Manufacturing
4:00–4:15 PM	WuXi Advanced Therapies
4:15–4:30 PM	MilliporeSigma
4:30–4:45 PM	Polyplus-transfection: Introducing FectoVIR®-LV: The Next-Generation Transfection Reagent for of Large Scale Therapeutic Lentivirus Production
4:45–5:00 PM	SCIEX: Purity Analysis of Long RNA Oligos to Support and Investigate new CRISPR Systems
5:00–5:15 PM	PathoQuest: Sensitivity, Scalability & Speed: The Value of NGS for Identity Testing of Plasmids & Viral Vectors
5:15–5:30 PM	Thermo Fisher Scientific: Screening for Success with Gibco™ Media by Design™ Services

SCHEDULE (ALL TIMES LISTED IN PT)

WEDNESDAY, MAY 17, 2023

EXHIBITOR SHOWCASES

3:45–4:15 PM

ROOM 511

Oxford Biomedica Solutions: Advancing Your Gene Therapy Game - Lessons Learned from the AAV Experts

4:45–5:15 PM

ROOM 511

OBiO Technology: Novel Process Development and Large Scale Manufacturing System of LV and rAAV Vectors

SCHEDULE (ALL TIMES LISTED IN PT)

WEDNESDAY, MAY 17, 2023

CAREER FAIR

3:15–5:15 PM
CONCOURSE FOYER

RECEPTION

POSTER VIEWING AND NETWORK RECEPTION 1
5:30–7:00 PM
EXHIBIT HALL/WEST HALL A

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

SCIENTIFIC SYMPOSIUM

PETREE HALL D
Advances in Respiratory and GI Tract Gene and Cell Therapy
Co-chairs: Maria Limberis, PhD, Spirovant Sciences Inc. and Uta Griesenbach, PhD, Imperial College London

8:00–8:25 AM	**Hepatocyte transplantation: Overview and Recent Advances** *Markus Grompe, MD, Yecuris Corporation*
8:25–8:50 AM	**Targeted Insertion of CFTR using CRISPR RNA-guided Integrases** *Sam Sternberg, PhD, Columbia University*
8:50–9:15 AM	**MSCs for CF Lung Disease and the Overall Field of Cell-Based Therapies for Lung Diseases** *Tracey Bonfield, PhD, Case Western University*
9:15–9:40 AM	**AAV Mediated Gene Therapy for Cystic Fibrosis (4D-710)** *Jennifer Taylor-Cousar, MD, National Jewish Health*

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

SCIENTIFIC SYMPOSIUM

ROOM 502 AB
DEI Roundtable
Chair: Rayne Rouce, MD, Baylor College of Medicine

8:20–8:40 AM	**Diversity in Clinical Trials** *Alex Herrara, MD, City of Hope*
8:40–9:00 AM	**Recognizing and Addressing Subtle Acts of Exclusion** *Jennifer Adair, PhD, University of Oklahoma Health Sciences*
9:00–9:20 AM	**Scientific Societies and Inclusion** *Dee Terrell, PhD, University of Oklahoma Health Sciences Center*
9:20–9:45 AM	**Panel Discussion** *Grant Holley, PhD* *Alex Herrera, MD* *Jennifer Adair, PhD* *Dee Terrell, PhD*

ASGCT 26TH ANNUAL MEETING Los Angeles

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

FIRESIDE CHAT

ROOM 515 AB
A Fireside Chat on Emerging Regulatory Trends
Moderator: Keith Wonnacott, PhD, Lexeo Therapeutics
8:00 AM–9:45 AM
Speakers: Peter Marks, MD, PhD, US Food and Drug Administration (FDA) and Yoshiaki Maruyama, PhD, Japanese Pharmaceuticals and Medical Devices Agency (PMDA)

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

EDUCATION SESSION

ROOM 403 AB
Formalizing Education in Gene and Cell Therapies
Co-chairs: Juliana Alvarez Argote, MD, Medical College of Wisconsin and Christopher LaRocca, MD, University of Minnesota

8:00–8:30 AM	**Hands-on Education in Gene and Cell Therapies: Bench-to-Bedside** *Maria-Grazia Roncarolo, MD, Stanford University*
8:30–9:00 AM	**Models of Graduate and Postgraduate Curriculum for Cell Therapy and Cell-Manufacturing Research** *Krishnendu Roy, PhD, Georgia Institute of Technology*
9:00–9:30 AM	**Laboratory and Mentoring Program Development for Cell and Gene Therapy Research** *Giedre Krenciute, PhD, St. Jude Children's Research Hospital*

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

SCIENTIFIC SYMPOSIUM

ROOM 411

Immunization Strategies and Their Impact on Containing Recent Pandemics: Lessons Learned and Steps Going Forward

Co-chairs: David Weiner, PhD, The Wistar Institute and Claire Evans, PhD, Ichor Medical Systems

8:00–8:35 AM	**COVID Vaccines** *Hildegund Ertl, MD, Wistar Institute, University of Pennsylvania*
8:35–9:10 AM	**Lessons Learned from Rapid Deployment of Ebola Vaccines and Platforms Used** *Ruxandra Draghia-Akli, MD, PhD, Johnson & Johnson*
9:10–9:45 AM	**Gene Therapy and Genetic Deliveries for Risk Groups** *Paul Leon, PhD, AstraZeneca*

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

SCIENTIFIC SYMPOSIUM

ROOM 408 AB

Recent Advances in the Therapy of Musculoskeletal and Neuromuscular Diseases

Co-chairs: Chunping Qiao PhD, Novartis and John Gray, PhD, Vertex Cell and Genetic Therapies

8:00–8:25 AM	**Opportunities and Challenges in Pursuing Gene Therapy for Charcot-Marie-Tooth Diseases** *Marina Stavrou, PhD, The Cyprus Institute of Neurology and Genetics*
8:25–8:50 AM	**WNT-modulating Gene Silencers as a Gene Therapy for Osteoporosis, Bone Fracture, and Critical-sized Bone Defects** *Jae-hyuck Shim, PhD, UMass Chan Medical School*
8:50–9:15 AM	**Alternatives in the Delivery of the CRISPR-Cas9/sgRNA System to Skeletal Muscle Tissues, IPS Cells** *Akitsu Hotta, PhD, Kyoto University*
9:15–9:40 AM	**Modeling FSHD in Mouse Therapeutic Interventions Targeting Muscle and Environment** *Michael Kyba, PhD, University of Minnesota*

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

SCIENTIFIC SYMPOSIUM

PETREE HALL C
Targeting Organs with Non-viral Delivery Systems
Co-chairs: Jonathan Hoggatt, PhD, Moderna Therapeutics and Daniel Scherman, PhD, CNRS-Inserm

8:00–8:25 AM	**DNA Barcoding for Optimized LNP Delivery *In Vivo*** *Kalina Paunovska, PhD, Nava Therapeutics*
8:25–8:50 AM	**Hydrodynamic Delivery to the Kidneys** *Lauren Woodard, PhD, Vanderbilt University Medical Center*
8:50–9:15 AM	**Using Extracellular Vesicles for Targeted Gene Delivery** *Angela Pannier, PhD, University of Nebraska-Lincoln*
9:15–9:40 AM	**Next Generation Electroporation Device for *In Vivo* Gene Delivery** *Mark Jaroszeski, PhD, University of South Florida*

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

ORAL ABSTRACTS

CONCOURSE HALL 152 & 153
Clinical Trials Spotlight Symposium
8:00–9:45 AM
Co-Chairs: Rebecca Ahrens-Nicklas, MD, PhD, Children's Hospital of Philadelphia and Carsten Bonnemann, MD, NINDS/NIH

8:00–8:15 AM	**Efficacy and Safety at Week 52 and up to Four Years in Adults with Glycogen Storage Disease Type IA (GSDIa): Results from a Phase 1/2 Clinical Trial and Long-Term Follow-Up Study of DTX401, an AAV8-Mediated, Liver-Directed Gene Therapy** *Rebecca Riba-Wolman, University of Connecticut*
8:15–8:30 AM	**Early Skeletal Outcome after Hematopoietic Stem Cell Gene Therapy for Mucopolysaccharidosis Type I Hurler** *Maria Ester Bernardo, MD, PhD, San Raffaele Telethon Institute for Gene Therapy*
8:30–8:45 AM	**Midbrain Gene Therapy for AADC Deficiency** *Krystof Bankiewicz, MD, PhD, The Ohio State University*
8:45–9:00 AM	**Gene Therapy for Adenosine Deaminase Deficiency: Long-Term Outcome and Post-Marketing Experience** *Maddalena Migliavacca, SR-TIGET*
9:00–9:15 AM	**GPC3-CAR T Cells Co-Expressing IL15 Mediate Potent Antitumor Activity in Liver Cancer Patients Associated with Toxicity That Can Be Mitigated Using iC9 Safety Switch** *David Steffin, MD, Baylor College of Medicine*
9:15–9:30 AM	**Danon Disease Phase 1 RP-A501 Results: The First Single-Dose Intravenous (IV) Gene Therapy with Recombinant Adeno-Associated Virus (AAV9:LAMP2B) for a Monogenic Cardiomyopathy** *Joseph Rossano, MD, Children's Hospital of Philadelphia*
9:30–9:45 AM	**Clinical Dose-Response Relationship and Safety Profile of rAAVrh8-HexA/HexB** *Terence Flotte, MD, UMass Chan Medical School*

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

EXHIBITOR SHOWCASES

8:15–8:45 AM
ROOM 511
MilliporeSigma: Utilization of DoE for AAV Process Development

8:15–8:45 AM
ROOM 409
AAVnerGene: Solving AAV Bottleneck Problems: Novel AAVone All-in-one Production System and ATHENA Capsid Selection Platform

9:15–9:45 AM
ROOM 511
Exothera: High-performance, scalable process for cost-effective AAV manufacturing - A case study of transfer from bench-scale to 1000L, presented by Polyplus and Exothera

9:15–9:45 AM
ROOM 409
GenScript USA Inc.: Comprehensive Solution for Cell and Gene Therapies Development

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

GEORGE STAMATOYANNOPOULOS MEMORIAL LECTURE AND AWARD PRESENTATION IS SPONSORED BY:

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GENERAL SESSION

WEST HALL B
George Stamatoyannopoulos Memorial Lecture and Award Presentation

10:30–11:15 AM	**Realizing the Potential of Genetically Engineered Immune Cells in Cancer Therapy** *Stanley Riddell, MD, Fred Hutchinson Cancer Research Center*
11:15 AM–12:00 PM	**Expanding the Reach and Efficacy of CAR T Cell Therapies** *Crystal Mackall, MD, Stanford University*

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

INDUSTRY SPONSORED SYMPOSIA

12:00 PM–1:30 PM

PETREE HALL C
Sarepta Therapeutics: Future Considerations for Gene Therapies: Exploring Suppression of Pre-existing Immunity to Vector

PETREE HALL D
Shape Therapeutics: Advancing programmable medicine through AAVId capsid engineering and TruStable cell line manufacturing

ROOM 409
Thermo Fisher Scientific: Exploring the Potential of AAV-Based Gene Therapy: Advances and Challenges

ROOM 501
MaxCyte

ROOM 511
Cytiva: Flexible end-to-end gene therapy solutions for successful manufacturing

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

POSTER SESSION II

12:00–1:30 PM
EXHIBIT HALL/WEST HALL A

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

ORAL ABSTRACT SESSION

CONCOURSE HALL 150 & 151

AAV Vectors - Product Development Manufacturing: Analytics & Stability Studies

Co-chairs: Lauren Drouin, PhD, LogicBio Therapeutics and Guocai Zhong, PhD, UMass Chan Medical School

1:30–1:45 PM	***In Vivo* and *In Vitro* Assessment of Residual DNA Impurity-Derived Transcriptions** *Hsin-I Jen, Ultragenyx*
1:45–2:00 PM	**AAV Vector DNA Carrying Two Concatenated miRNA Stem-Loops is Highly Homogenous and Stable in GLP-Grade AAV Product** *Chenxia He, Corlieve Therapeutics, AG, a wholly owned subsidiary of uniQure N.V.*
2:00–2:15 PM	**A Rapid, Serotype Specific Approach to AAV Formulation Development** *Robert Damitz, Kriya Therapeutics, Inc.*
2:15–2:30 PM	**Formulate for Success: Early Investment in Drug Product Development Enables Platform Stabilization of Multiple AAV Serotypes, Refrigerated Supply Chain, and Accelerated Dosing Timelines** *Kelly Walsh, Oxford Biomedica Solutions*
2:30–2:45 PM	**Systematic Comparison of rAAV Vectors Manufactured by Large-scale Suspension Cultures of Sf9 and HEK293 Cells** *Shengjiang Liu, Avirmax Biopharma Inc.*
2:45–3:00 PM	**Deamidation Analysis for Supporting AAV Gene Therapy Development** *Yu Zhou, Novartis Pharmaceuticals*
3:00–3:15 PM	**A Comparative Analysis of Recombinant AAV9 Product Generated from Insect and Mammalian Bioproduction Processes** *Phuong Nguyen, Neurogene Inc.*

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

ORAL ABSTRACT SESSION

CONCOURSE HALL 152 & 153
Genome Editing Therapies & Safety II
Co-chairs: Jennifer Gori, PhD, Prime Medicine Inc. and Isaac Hilton, PhD, Rice University

1:30–1:45 PM	**Pathogenic Exon Deletion Using Prime Editing for Correcting Primary Recessive Dystrophic Epidermolysis Patient Cells** *Mark Osborn, PhD, University of Minnesota*
1:45–2:00 PM	**Genome Editing B Cells to Express Custom Heavy Chain Antibodies with Modified Antigen Specificity and Fc Function** *Chun Huang, University of Southern California*
2:00–2:15 PM	**A Targeted Non-Viral CRISPR-based *In Vivo* Gene Editing Strategy for the Treatment of Sickle Cell Disease** *Christophe Lechauve, Sanofi*
2:15–2:30 PM	**Precise CRISPR/Cas9 Genome Engineering of Primary Human B Cells Enables a New Class of Cellular Medicines Designed for Sustained Delivery of Therapeutic Biologics** *Anja Hohmann, PhD, Be Biopharma*
2:30–2:45 PM	**Restoration of Full-Length Dystrophin Using AAV-Delivered U7snRNA in Patient Cells with a *DMD* pseudoexon: a Model for Bespoke Gene Therapies in an Orphaned Mutational Class** *Joseph Beljan, Nationwide Children's Hospital*
2:45–3:00 PM	**Large-scale GUIDE-seq-2 Profiling Reveals Effects of Human Genetic Variation on Cellular Off-target Genome Editing Activity** *Yichao Li, St. Jude Children's Research Hospital*
3:00–3:15 PM	**Modeling Gene Editing Outcomes in Microphysiological Human Tissue System Models of Duchenne Muscular Dystrophy** *Madeleine Sitton, Duke University*

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

ORAL ABSTRACT SESSION

ROOM 403 AB

Metabolic, Storage, Endocrine, Liver and Gastrointestinal Diseases I

Co-chairs: Dao Pan, PhD, Cincinnati Children's Medical Center and Cristina Baricordi, Beam Therapeutics

1:30–1:45 PM	**Oligodendrocyte Toxicity by Guanidino Compounds, not Arginine, Fosters Dysmyelination in Arginase Deficiency and is Prevented by AAV-based Gene Therapy** *YuChen Zhang, University of California, Los Angeles*
1:45–2:00 PM	**Enhanced Enzyme Uptake and Glycogen Clearance in Muscles and CNS of Pompe Mice Administered AAV9 Expressing Novel Engineered GAA** *Sury Somanathan, PhD, Pfizer Inc.*
2:00–2:15 PM	**Rescue of a Lethal Murine Model of Methylmalonic Acidemia by AAV Mediated Delivery of PiggyBac Transposase and a Therapeutic Transgene** *Randy Chandler, PhD, National Institutes of Health*
2:15–2:30 PM	**New Mouse Models of, and AAV9 Gene Therapeutics to Treat, Isolated Methylmalonic Acidemia, Cobalamin B (*cblB*) Type** *Eun-Young Choi, PhD, MMB, OARS, NHGRI, NIH*
2:30–2:45 PM	**Preclinical Proof-of-Concept: A Novel Hybrid Gene Therapy approach to Treat Severe Early-Onset Ornithine Transcarbamylase Deficiency** *Bernard Kok, Poseida Therapeutics*
2:45–3:00 PM	**Long-term Safety and Efficacy of DTX301 in Adults with Late-Onset Ornithine Transcarbamylase (OTC) Deficiency: A Phase 1/2 Trial** *Cary Harding, MD, Oregon Health & Science University*
3:00–3:15 PM	**Single AAV Vector Gene Therapy with a Mini-GDE Transgene Corrects Muscle Impairment in Mouse, Rat and Human Cellular Models of Glycogen Storage Disease Type III** *Antoine Gardin, Genethon*

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

ORAL ABSTRACT SESSION

ROOM 408 AB

Gene Therapy Approaches for Muscle and Skeletal Diseases

Co-chairs: Caroline Le Guiner, PhD, TaRGeT Lab - Translational Research for Gene Therapies - Nantes, France
and Shin'ichi Takeda, MD, PhD, National Institute of Neuroscience, NCNP

1:30–1:47 PM	**AAVrh74.tMCK.hBAG3 Gene Therapy Improves Function and Decreases MYOT Aggregate Formation in TgT57I Mice** *Burcak Ozes, PhD, Nationwide Children's Hospital*
1:47–2:04 PM	**Toxicology, Pharmacokinetics and Biodistribution of a PATrOL™-Enabled Investigational Genetic Therapy for Myotonic Dystrophy, Type 1** *William Riedl, NeuBase Therapeutics*
2:04–2:21 PM	**Management Of Patients Following Investigational Delandistrogene Moxeparvovec Gene Therapy For Duchenne Muscular Dystrophy: Delphi Panel Consensus Considerations Based on Clinical Trial Experience** *Crystal Proud, MD, Children's Hospital of the King's Daughter*
2:21–2:38 PM	**Lentivirus-based Hematopoietic stem/progenitor Cell Therapy Provides Safe and Long-term Treatment of Hypophosphatasia in Murine Models** *Anjie Zhen, PhD, UCLA*
2:38–2:55 PM	**AUF1 Gene Therapy for Duchenne Muscular Dystrophy Increases Durable Endogenous Utrophin Expression, Muscle Regeneration and Muscle Function Performance in Pre-Clinical Animal Studies** *Dounia Abbadi, NYU Grossman School of Medicine*
2:55–3:12 PM	**Intra-Articular Delivery of AAV Vectors Encoding Immune Checkpoint Protein as a Novel Treatment for Arthritis** *Wenjun Li, University of North Carolina at Chapel Hill*

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

ORAL ABSTRACT SESSION

ROOM 411

Understanding and Modeling Immune Responses to Gene Therapy and Vaccines

Co-chairs: Helena Costa Verdera, PhD, SR-TIGET and Ying Kai Chan, PhD, Harvard University

1:30–1:45 PM	**High-dose AAV Toxicity in Mice: Serotype-dependent Hepatocellular Damage and Complement Deposition and Activation** *George Buchlis, PhD, University of Pennsylvania*
1:45–2:00 PM	**Effectiveness of Engineering AAV Vector Genomes to Evade TLR9 Signaling for Prevention of $CD8^+$ T Cell Responses in Muscle Gene Transfer is Vector Dose Dependent** *Sandeep Kumar, PhD, Indiana University School of Medicine*
2:00–2:15 PM	**Identification of Dose-Dependent Immune Landscape Signatures Following Administration of Ixo-vec in Non-Human Primates** *Julian Ramos, PhD, Adverum Biotechnologies*
2:15–2:30 PM	**Humoral Arms Of The Innate And Adaptive Immune Systems Define The Severity Of The Cytokine Storm Response To Human Adenovirus Type 5 Vectors** *Svetlana Atasheva, PhD, Emory University*
2:30–2:45 PM	**Causal Role for Immune Response in AAV-Mediated DRG Toxicity in NHPS** *Kate Henry, PhD, Biogen*
2:45–3:00 PM	**A Human PBMC Assay of Type 1 Interferon Responses to Closely Related AAV Vectors** *Bradley Hamilton, PhD, Stanford*
3:00–3:15 PM	**Pre-Existing Maternal Immunity to Adeno-Associated Virus Causes Fetal Mortality after *In Utero* Gene Editing Mediated by Maternal Interleukin 6 and CXCL1** *John Riley, Children's Hospital of Philadelphia*

SESSION CONTINUED ON NEXT PAGE

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

SESSION CONTINUED FROM PREVIOUS PAGE

ORAL ABSTRACT SESSION

ROOM 411

Understanding and Modeling Immune Responses to Gene Therapy and Vaccines

Co-chairs: Helena Costa Verdera, PhD, SR-TIGET and Ying Kai Chan, PhD, Harvard University

3:15–3:30 PM	**Reducing Cas9 Immunogenicity In Vivo Leveraging Cell-Endogenous Protein Degradation Pathway** *Roberto Nitsch, PhD, AstraZeneca*
3:30–3:45 PM	**Immune Responses Observed to AAV Gene Therapy for the Treatment of GM2 Gangliosides** *Allison Keeler-Klunk, PhD, UMass Chan Medical School*

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

ORAL ABSTRACT SESSION

ROOM 502 AB
Next Generation CAR, TCR, and AAV Technologies for Solid Tumors
Co-chairs: Michael Milone, PhD, MD, University of Pennsylvania and Aude Chapuis, MD, Fred Hutchinson Cancer Research Center

1:30–1:45 PM	**Adeno-associated Virus-mediated Targeting of HER2+ Brain Metastasis** *Shweta Aras, PhD, University of Pennsylvania*
1:45–2:00 PM	**Safety Evaluation of Intra-cisterna Magna (ICM) Delivery of a Novel AAV-trastuzumab Vector to Target HER2+ Breast-to-Brain Metastasis in Rhesus Macaques** *Marcela Werner, PhD, University of Pennsylvania*
2:00–2:15 PM	**Designed Tumor Microenvironment Responsive Biosensors Enhance Chimeric Antigen Receptor T Cell Therapy for Cancer** *Jan Rath, Lausanne University Hospital and University of Lausanne*
2:15–2:30 PM	**Development of Adeno-Associated Virus (AAV) Vectors for Intratumoral Gene Replacement Therapy in a Novel Mouse Model of Neurofibromatosis Type 1 (NF1)** *Rrita Daci, MD, UMass Chan Medical School*
2:30–2:45 PM	**Enhanced Anti-AML Potency of DARIC33 by iSynPro-IL-15*: an IL-15 Expression Module Driven by a Tightly Regulated Synthetic Promoter Activated by Antigen Receptor Signaling** *Jacob Appelbaum, MD, PhD, University of Washington*
2:45–3:00 PM	**Preclinical Development of AB-1015, an Integrated Circuit T Cell Therapy Containing an ALPG/MSLN Logic Gate and FAS/PTPN2 shRNA-miR, for the Treatment of Ovarian Cancer** *Jun Feng, PhD, Arsenal Biosciences*
3:00–3:15 PM	**Engineering Pharmacologically Relevant, FDA-Approved Small-Molecule-Regulated Gene Circuits for Therapeutic Applications in the Brain** *Rebecca Cottman, PhD, Senti Biosciences*

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

ORAL ABSTRACT SESSION

ROOM 515 AB
Gene Targeting and Gene Correction: Liver
Co-chairs: Bruce Torbett, PhD, MSPH, University of Washington and Michela Milani, PhD, SR-TIGET

1:30–1:45 PM	**Effective Treatment of Mitochondrial Complex III Deficient Mice with Hepatocyte-Targeted Gene Therapy** *Rishi Banerjee, University of Helsinki*
1:45–2:00 PM	**An Epigenetic Hit-and-Run Platform for Durable and Effective Silencing of Pcsk9 *In Vivo*** *Martino Alfredo Cappelluti, SR-TIGET*
2:00–2:15 PM	**Optimizing Therapeutic Base Editing for Hereditary Tyrosinemia Type 1** *Ana Maria Dumitru, MD, PhD, Children's Hospital of Philadelphia*
2:15–2:30 PM	**RNA Gene Writers Drive Therapeutically Relevant Levels of Correction of the PAH Gene Responsible for Phenylketonuria in Mouse and Non-human Primate Models** *Anne Bothmer, Tessera Therapeutics*
2:30–2:45 PM	**Development of a Human PCSK9-Targeting Epigenetic Editor With Durable, Near-Complete In Vivo Silencing Efficiency** *Aron Jaffe, Chroma Medicine*
2:45–3:00 PM	**Temporally Restricting Cas9 Expression Improves Deletion Frequency in the Liver** *Jesse Weber, University of Pennsylvania*
3:00–3:15 PM	**Cas-CLOVER Technology Enables Precise Gene Editing and Site-Specific Transgene Insertion in Mouse Liver** *Oscar Alvarez, PhD, Poseida Therapeutics*

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

ORAL ABSTRACT SESSION

WEST HALL B
AAV Engineering for CNS Targeting
Co-chairs: Josef El Andari, Dinaqor and Yuan Yuan, PhD, Biogen

1:30–1:45 PM	**Structure-Activity Relationships Guided Engineering of AAV Capsids with Optimized Skeletal Muscle, Cardiac Muscle, and CNS Tropism** *Charles Albright, PhD, Affinia Therapeutics*
1:45–2:00 PM	**The Use of Transcription-Dependent Directed Evolution (TRADE) to Identify a Novel AAV Capsid that Transduces Motor Neurons with High Efficiency** *Christina Chatzi, PhD, PharmD, Capsigen Inc.*
2:00–2:15 PM	**AAV-Mediated CNS-Wide Gene Delivery via a de Novo Engineered Capsid-Human Receptor Interaction** *Ken Chan, PhD, Broad Institute of MIT and Harvard*
2:15–2:30 PM	**Directed Evolution of an AAV9 Library Identifies a Capsid Variant with Enhanced Brain Tropism and Liver De-targeting in Non-Human Primates and Mice Following Systemic Administration** *Tyler Moyer, PhD, Voyager Therapeutics*
2:30–2:45 PM	**Developing Blood-Brain Barrier-Crossing AAV9 Variants with Reduced Sensitivity to Neutralizing Antibodies** *Simon Pacouret, PhD, Broad Institute of MIT and Harvard*
2:45–3:00 PM	**Machine-Learning Guided Design of Cell-Specific AAVs in Human Central Nervous System (CNS)** *Danqing Zhu, HKUST*
3:00–3:15 PM	**Functional Gene Delivery to and Across Brain Vasculature of Systemic AAVs with Endothelial-Specific Tropism in Rodents and Broad Tropism in Primates** *Xinhong Chen, Caltech*

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

TOOLS AND TECHNOLOGY FORUM

PRESENTATION THEATER IN EXHIBIT HALL
Tools and Technology Forum II
Chair: Dominic J. Gessler, UMass

2:00–2:15 PM	PlasmidFactory: Large scale plasmid DNA production in High Quality
2:15–2:30 PM	Cytiva: AAV production with inducible stable ELEVECTA producer cell lines - high vector quality at large scale
2:30–2:45 PM	Refeyn: The Versatile AAV Platform - Using Mass Photometry from R&D to Manufacturing
2:45–3:00 PM	ScaleReady
3:00–3:15 PM	STEMCELL Technologies: Hematopoietic Cell Therapy: A Standardized Workflow for Culture and Functional Assessment

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

TOOLS AND TECHNOLOGY FORUM

PRESENTATION THEATER IN EXHIBIT HALL
Tools and Technology Forum III
Co-chairs: Blythe Sather, PhD, Tune Therapeutics and Stefan Radtke, PhD, Fred Hutchinson Cancer Research Center

3:45–4:00 PM	Lonza: Superior T-cell Expansion Using TheraPEAK® T-VIVO®, a Chemically Defined Cell Culture Medium for Cell Therapy
4:00–4:15 PM	ReiThera: ReiThera's Technology Development and CDMO Capabilities
4:15–4:30 PM	BriGene Biosciences
4:30–4:45PM	PerkinElmer: Advances in Manufacturing Best-in-Class rAAV Vectors
4:45–5:00 PM	Wyatt Technology: AAV Multi-attribute Quantification (MAQ) by SEC-MALS
5:00–5:15 PM	CERBA Research: Assessment of the Tumor Infiltrating Lymphocytes (TILs) Profile, an Immune Fingerprint of the Solid Tumor
5:15–5:30 PM	10x Genomics: Acceleration of Immune Cell Therapies with Single Cell and Spatial Resolution

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

EXHIBITOR SHOWCASES

2:30–3:00 PM
ROOM 511
PackGene Biotech INC: Current Challenges and Our Approaches to Developing Best-in-Class rAAV Manufacturing Processes for Large-scale GMP production

3:45–4:15 PM
ROOM 511
Resilience: Resilience Technologies for mRNA, AAV, and Cell Therapy: Integration of Three Discrete Modalities to Deliver Advanced Cell Therapies

4:45–5:15 PM
ROOM 511
Center for Breakthrough Medicines: Reimagining Advanced Therapies Manufacturing Through Innovation, Digitization, and Supply Chain Simplification

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

ORAL ABSTRACT SESSION

CONCOURSE HALL 150 & 151
AAV Vector Genome Biology and Engineering I
Nadja Meumann, Hannover Medical School
and Steven Gray, PhD, University of Texas Southwestern

3:45–4:00 PM	**Machine-Guided Design of Tissue-Specific Promoters** *Alec Nielsen, Asimov*
4:00–4:15 PM	**A New CCEAAV Vector Without a Mutant ITR is Efficient for Reducing Subgenome Generation** *Junping Zhang, PhD, Indiana University School of Medicine*
4:15–4:30 PM	**Characterization of Promoter and Intron Interactions Affecting Transgene Expression in AAV Cassettes** *Ferzin Sethna, AskBio*
4:30–4:45 PM	**Improving Codon Optimization for Gene Therapy Vectors Using Deep Learning** *Sandhiya Ravi, UMass Chan Medical School*
4:45–5:00 PM	**Searching the Hairpin in the Haystack: Tracing the Impact of AAV-ITR Mutations** *Felix Bubeck, University of Heidelberg*
5:00–5:15 PM	**Engineering mRNA Stability with Flaviviral Genomic Elements to Improve AAV Transduction** *Roza Ogurlu, Duke University*
5:15–5:30 PM	**Long Read Sequencing of RAAV Vectors Illuminates Origins of Contaminating Genomic Species** *Lauriel Earley, PhD, Shape Therapeutics, Inc. - Seattle, WA*

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

ORAL ABSTRACT SESSION

CONCOURSE HALL 152 & 153
Pharmacology/Toxicology Studies: Methodology
Chair: Jenny Greig, PhD, University of Pennsylvania

3:45–4:02 PM	**Assessing *In Vivo* Recombinant AAV DNA by Long-read Sequencing After Gene Therapy** *Jason Lamontagne, University of Pennsylvania*
4:02–4:19 PM	**Retrieval and Quantification of Vector Integration Sites by Sonication Linker Mediated-PCR (SLiM-PCR): Efficiency and Applications** *Fabrizio Benedicenti, SR-TIGET*
4:19–4:36 PM	**Cell Isolation Following Apheresis Using an Immunomagnetic Beads Approach in Non Human Primates** *Vincent Sénécal, PhD, Charles River*
4:35–4:53 PM	**Characterization of AAV Integrations and Rearrangements from Long and Short Reads with RAAVioli** *Carlo Cipriani, San Raffaele Telethon Institute for GT (Sr-Tiget), IRCCS San Raffaele Scientific Institute, Milan, Italy*
4:53–5:10 PM	**CISC: A Multi-Purpose Enrichment Tool for HDR-Edited Human T-Cells Applicable for Treg and CAR-T Therapeutic Cell Products** *Annaiz Grimm, Seattle Childrens Research Institute*
5:10–5:27 PM	**Novel Tools for Gene and Cell Therapy Safety: Optical Long-Read Genomics for Detection and Characterization of On- and Off-Target Transgene Integrations and Off-Target Structural Variants** *Saumyaa Saumyaa, PhD, Astrazeneca*

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

ORAL ABSTRACT SESSION

PETREE HALL C

Synthetic/Molecular Conjugates and Physical Methods for Delivery of Gene Therapeutics II

Co-chairs: Lauren Woodard, PhD, Vanderbilt University Medical Center and Liwei Hui, MilliporeSigma

3:45–4:00 PM	**Acoustofluidic Sonoporation-Mediated Gene Delivery Utilizing DNA-encapsulated Supramolecular Nanoparticles** *Yao Gong, UCLA*
4:00–4:15 PM	**Development of Gold Nanoparticles for *In Vivo* CRISPR Delivery - Improving Nuclease Loading and Stability with Pre-formed RNP Complexes** *Karthikeya Gottimukkala, Fred Hutchinson Cancer Center*
4:15–4:30 PM	**Molecular Chameleon Carriers for Nucleic Acid Delivery: The Sweet Spot Between Lipoplexes and Polyplexes** *Simone Berger, Ludwig-Maximilians-Universität Munich*
4:30–4:45 PM	**Environmentally-Sensitive Polymer-Based Nanoparticles for Intravitreal Gene Delivery** *Gijung Kwak, Johns Hopkins University School of Medicine*
4:45–5:00 PM	**Altered Localization of Plasmid DNA Within the Nucleus Affects Gene Expression** *Ningyang Gu, University of Rochester*
5:00–5:15 PM	**Neuroprotection by Optical Delivery of Therapeutic PEDF Gene into Retina** *Sanghoon Kim, Nanoscope Instruments, Inc.*
5:15–5:30 PM	**Development of a Monoparticle-Based Crispr Gene Editing and Intraparenchymal Delivery System for Neurological Applications** *Victor Van Laar, PhD, The Ohio State University*

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THURSDAY, MAY 18, 2023

ORAL ABSTRACT SESSION

PETREE HALL D

Neurological Gene Therapies in Advanced Stages of Clinical Translation

Co-chairs: Miguel Sena-Esteves, PhD, UMass Chan Medical School and Kleopas Kleopa, MD, PhD, The Cyprus Institute of Neurology & Genetics

3:45–4:00 PM	**Lowering of Toxic Dipeptide Proteins and Phenotype Rescue in an ALS Mouse Model Treated with AAV-miQURE® Targeting the Repeat Expansion-Containing C9orf72 Transcripts** *Vanessa Zanvanella, PhD, UniQure Biopharma, B.V.*
4:00–4:15 PM	**Long-Term Efficacy of Gene Therapy for AADC Deficiency, Including Patients with a Moderate Phenotype** *Marina Hashiguchi, Jichi Medical University*
4:15–4:30 PM	**Intraparenchymal Convection Enhanced Delivery of AAV in Sheep to Treat Mucopolysaccharidosis IIIC** *Brian Bigger, PhD, University of Manchester*
4:30–4:45 PM	**Subacute Liver Injury In Two Young Infants Following Gene Replacement Therapy For Spinal Muscular Atrophy** *Cassie Turnage, MD, University of Utah*
4:45–5:00 PM	**Efficacy and Safety of a Novel *FXN* Gene Therapy (AVB-202) for the Treatment of Friedreich's Ataxia** *Grace Pavlath, PhD, Solid Biosciences*
5:00–5:15 PM	**Preclinical Safety Assessment of NGN-401, a Clinical-Stage Gene Therapy Product for Rett Syndrome** *Suzanne Burstein, PhD, Neurogene Inc.*
5:15–5:30 PM	**Comparison of Dorsal Root Ganglion Toxicity in the Development of AAV9/MFSD8 and AAV9/AP4M1 Gene Therapies for CLN7 and SPG50** *Xin Chen, UT Southwestern Medical Center*

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

ORAL ABSTRACT SESSION

ROOM 403 AB
Metabolic, Storage, Endocrine, Liver and Gastrointestinal Diseases II
Co-chairs: R. Scott McIvor, University of Minnesota and Lili Wang, PhD, University of Pennsylvania

3:45–4:00 PM	**Phase 1/2 Clinical Trial of Autologous Hematopoietic Stem and Progenitor Cell Gene Therapy for Cystinosis** *Stephanie Cherqui, PhD, University of California, San Diego*
4:00–4:15 PM	**Manipulating Macrophage Phenotype to Improve Inflammatory Bowel Disease Outcomes** *Adele Mucci, Dana Farber/Boston Children's Cancer and Blood Disorders Center, Harvard Medical School*
4:15–4:30 PM	**Impact of Genetic Diversity on Gene Therapy Efficacy** *Joerg Votteler, AskBio*
4:30–4:45 PM	**Effective Readministration of AAV Gene Therapy Prevents Tumorigenesis in Infant PFIC3 Mice, a Mouse Model Prone to Hepatocellular Carcinoma** *Nicholas Weber, Vivet Therapeutics*
4:45–5:00 PM	**mRNA-3927 Therapy for Propionic Acidemia: Interim Data From a Phase 1/2 Study** *Gerald Lipshutz, MD, David Geffen School of Medicine at UCLA*
5:00–5:15 PM	**A Pancreatic Gene Therapy Delivery Platform for the Treatment of Type 2 Diabetes** *Harith Rajagopalan, Fractyl Health, Inc.*
5:15–5:30 PM	**Rescue of Glutaric Aciduria Type I Mice by Liver Directed Therapies** *Mercedes Barzi, PhD, Duke University*

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

ORAL ABSTRACT SESSION

ROOM 408 AB
Novel Genetic Approaches for Muscle Diseases
Co-chairs: Christopher Nelson, PhD, University of Arkansas and Joel Chamberlain, PhD, University of Washington School of Medicine

3:45–4:00 PM	**CRISPR/Cas9 Homology-Independent Targeted Integration of Exons 1-19 Restores Full-Length Dystrophin in Mice** *Stefan Nicolau, MD, Nationwide Children's Hospital*
4:00–4:15 PM	**Orthogonal Long- and Short-read Sequencing after AAV-CRISPR Reveals On-target Heterogeneity and Chimeric AAV-*Dmd* Transcripts** *Mary Jia, University of Arkansas*
4:15–4:30 PM	**AAV-NoSTOP Gene Therapy for Nonsense Mutation Mediated Dysferlinopathy** *Mengyao Xu, UMass Chan Medical School*
4:30–4:45 PM	**Development of Myorganoids for Therapeutic Evaluation of Gene Therapy Products in Duchenne Muscular Dystrophy** *Laura Palmieri, Genethon*
4:45–5:00 PM	**CRISPR-Mediated Insertion of a Transferrin Receptor-Targeted *GAA* Transgene into Hepatocytes Provides Effective, Long-Lasting Treatment of Muscles and the Central Nervous System in Neonate and Adult Pompe Disease Mice,** *Katherine Cygnar, PhD, Regeneron Pharmaceuticals*
5:00–5:15 PM	**Improved AAV-Mediated Systemic Gene Therapy to Striated Muscles through Increased Tissue Perfusion** *Fatemeh Khadir, PhD, University of Minnesota*
5:15–5:30 PM	**PGN-EDODM1 Nonclinical Data Demonstrate Potential for Meaningful Impact in Myotonic Dystrophy Type 1 (DM1): Support for Phase 1 Clinical TrialDesign** *Jaya Goyal, PhD, PepGen*

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

ORAL ABSTRACT SESSION

ROOM 409 AB

Breaking Gene Editing and Other Barriers for Cardiovascular and Pulmonary Diseases

Chair: Ashley Cooney, PhD, University of Iowa

3:45–4:00 PM	**Gene Transfer Efficiency in Models of Increased Secreted Airway Mucins** *Kenan Najdawi, University of Iowa*
4:00–4:15 PM	***SCN10A-short* Gene Therapy for the Treatment of Cardiac Conduction Disorders** *Jianan Wang, Amsterdam UMC*
4:15–4:30 PM	**EV-AAVs as a Novel Gene Delivery Vector to the Heart: New -Evaluations for EV-AAVs and free AAV Separation** *Sabrina La Salvia, PhD, Icahn School of Medicine at Mount Sinai*
4:30–4:45 PM	**Proliferation and Differentiation Potential of CFTR-Expressing Epithelial Cells in Mouse Airways** *Jia Ma, University of Iowa*
4:45–5:00 PM	**AAV Gene Editing Extends Survival in a Mouse Model of Surfactant Protein B Deficiency** *Sylvia Thomas, University of Guelph*
5:00–5:15 PM	**LX2020-An AAV Based Gene Therapy Improves The Arrhythmogenic Right Ventricular Cardiomyopathy Phenotype In A Severe Mouse Model Harboring Human PKP2 Mutation** *Farah Sheikh, University of California San Diego*
5:15–5:30 PM	**Efficient *In Vivo* Base Editing Prevents Hypertrophic Cardiomyopathy in Mice** *Gregory Newby, Broad Institute of MIT and Harvard*

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

ORAL ABSTRACT SESSION

ROOM 411

Modulating and Engineering Immune Responses to Gene Therapy and Vaccines

Co-chairs: Allison Keeler-Klunk, PhD, UMass Chan Medical School and Gwladys Gernoux, PhD, Nantes University

3:45–4:00 PM	**Combination Immunosuppression Prevents Toxicity and Increases Liver Transduction in Cynomolgus Macaques Administered with High Dose AAV Vector** *Kruti Patel, PhD, Pfizer Inc*
4:00–4:15 PM	**Inhibition of AAV-Specific Adaptive Immune Response by Co-Stimulation Blockade with CTLA4-Ig Fusion Protein** *Audry Fernandez, PhD, Asklepios BioPharmaceutical, Inc.*
4:15–4:30 PM	**Engineered Human B cells Respond to Antigen-Specific Immunization in a Tonsil Organoid Model** *Geoffrey Rogers, PhD, University of Southern California*
4:30–4:45 PM	**Newly Engineered IgM and IgG Cleaving Enzymes for AAV Gene Therapy** *Timothy Smith, Duke University*
4:45–5:00 PM	**A Biosynthetic Approach for CpG Methylation of AAV Expression Cassettes** *Fabian Huellen, Stanford*
5:00–5:15 PM	**B-cell Targeted Therapies Deplete Neutralizing Antibodies to AAV** *Bhavya Doshi, MD, Children's Hospital of Philadelphia*
5:15–5:30 PM	**Redosing of AAV Vectors Containing TLR9 Inhibitory Oligonucleotides Improves Survival in a Mouse Model of Respiratory Surfactant Deficiency** *Martin Kang, PhD, Medical University of South Carolina*
5:30–5:45 PM	**Establishing Multilayered Genetic Resistance to HIV-1 by Engineering Hematopoietic Stem and Progenitor Cells for B Cell Specific Secretion of Therapeutic Antibodies** *William Feist, Stanford University*

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THURSDAY, MAY 18, 2023

ORAL ABSTRACT SESSION

ROOM 501 ABC
Hematologic and Immunologic Diseases
Co-chairs: Axel Schambach, MD, PhD, Hannover Medical School and Kit Shaw, PhD, Rejuvitas

3:45–4:00 PM	**mRNA Delivery to Hematopoietic Stem Cells by Targeted Lipid Nanoparticles Allows In Vivo Genome Engineering and Control of Cell Fate** *Michael Triebwasser, University of Michigan*
4:00–4:15 PM	**First Case of Leukaemia in Retroviral Gene Therapy for ADA-SCID** *Daniela Cesana, PhD, San Raffaele Telethon Institute for GT (Sr-Tiget), IRCCS San Raffaele Scientific Institute*
4:15–4:30 PM	**Lentiviral Vector Mediated *in Vivo* Gene Transfer into Hematopoietic Stem and Progenitor Cells** *Michela Milani, PhD, San Raffaele Telethon Institute for Gene Therapy*
4:30–4:45 PM	**Lentiviral-Mediated Gene Therapy for Fanconi Anemia [Group A]: Results From Global RP-L102 Clinical Trials** *Agnieszka Czechowicz, MD, PhD, Stanford School of Medicine*
4:45–5:00 PM	**Global Phase 1 Study Results of Lentiviral Mediated Gene Therapy for Severe Pyruvate Kinase Deficiency** *Ami Shah, MD, Lucile Packard Children's Hospital*
5:00–5:15 PM	**Unraveling the Effect of Proliferative Stress *in Vivo* in Hematopoietic Stem Cell Gene Therapy Mouse Study** *Francesco Gazzo, San Raffaele Telethon Institute for GT (Sr-Tiget), IRCCS San Raffaele Scientific Institute*
5:15–5:30 PM	**Highly Efficient Correction of the Sickle Cell Disease Mutation in Patient HSC Using an RNA Gene Writing System, an RNA-based, Nuclease-free Approach to Genome Editing** *Giulia Schiroli, PhD, Tessera Therapeutics*

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

ORAL ABSTRACT SESSION

ROOM 502 AB
Cell Therapy Product Engineering and Development | Cancer
Co-chairs: Patrick Hanley, PhD, Children's National Medical Center and Daniela Bischof, PhD, Indiana University School of Medicine

3:45–4:00 PM	**Allogeneic CAR T cells with Regulated NEF Expression Display Immune Evasion and Optimized Signaling** *Karlo Perica, MD, PhD, Memorial Sloan Kettering Cancer Center*
4:00–4:15 PM	**Mechanistic Characterization of Afamitresgene Autoleucel (Afami-cel; Formerly ADP-A2M4)** *Laura Hudson, Adaptimmune*
4:15–4:30 PM	**Cytosine Base Editor Ameliorates the Safety Profile of (TCR) - Transgenic T Cells for the Adoptive Cell Therapy of Gastrointestinal Tumors** *Martina Spiga, San Raffaele Scientific Institute*
4:30–4:45 PM	**CD19/BCMA Dual-targeting CAR-T Cells Generated by Co-transduction for the Treatment of Non-Hodgkin Lymphoma** *Mireia Bachiller, Fundacio Clinic per la Recerca Biomedica*
4:45–5:00 PM	**High-Throughput Screen of Chimeric Antigen Receptor (CAR) T Cells with Native and Non-Native Costimulatory Domains** *Conor Kelly, National Institutes of Health (NIH)*
5:00–5:15 PM	**Epitope Editing in Hematopoietic Cells Enables Universal Blood Cancer Immune Therapy** *Nils Wellhausen, University of Pennsylvania*
5:15–5:30 PM	**Co-expression of the IL15-IL15_Complex with a 41BB-based Chimeric Antigen Receptor Promotes Superior Antitumor Activity in NKT Cells** *Gabriel Barragan Bravo, Baylor College of Medicine*

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

ORAL ABSTRACT SESSION

ROOM 515 AB
Gene Targeting and Gene Correction: New Technologies
Co-chairs: Megan Hoban, PhD, UCLA
and Zulema Romera Garcia, PhD, UCLA

3:45–4:00 PM	**Shuttle Peptide Delivers Base Editor RNPs to Rhesus Monkey Airway Epithelial Cells *In Vivo*** *Katarina Kulhankova, MD, PhD, University of Iowa*
4:00–4:15 PM	**A Novel Type V CRISPR System with Potent Editing Activity in Mice and Non-Human Primates** *Alan Brooks, PhD, Metagenomi*
4:15–4:30 PM	**An *In Vivo* CAST-Seq Workflow Identifies and Quantifies Off-target Activity as well as Chromosomal Translocations in Organs Edited *In Vivo* with CRISPR-Cas Nucleases or Nickases** *Julia Klermund, Institute for Transfusion Medicine and Gene Therapy, Medical Center - University of Freiburg*
4:30–4:45 PM	**Writing a Chimeric Antigen Receptor (CAR) into T Cell Genomes Using RNA-Based Gene Writing Systems** *Alberto De Iaco, Tessera Therapeutics*
4:45–5:00 PM	**Cell Type-Programmable CRISPR-Cas9 Delivery for Human T Cell Engineering** *Jennifer Hamilton, PhD, UC Berkeley*
5:00–5:15 PM	**Efficient and Safe RNA Editing in Non-Human Primates Using AAV Delivered LEAPER Agents** *Pengfei Yuan, PhD, EdiGene Inc.*
5:15–5:30 PM	**Co-LOCKR-Mediated, Highly Specific *In Vivo* Targeting without Off-Targets** *Kurt Berckmueller, Fred Hutchinson Cancer Research Center*

SCHEDULE (ALL TIMES LISTED IN PT)

THURSDAY, MAY 18, 2023

CAREER FAIR	3:15–5:15 PM CONCOURSE FOYER
RECEPTION	POSTER VIEWING AND NETWORK RECEPTION 2 5:30–7:OO PM EXHIBIT HALL/WEST HALL A

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

SCIENTIFIC SYMPOSIUM

ROOM 408 AB

Beyond the Liver: Decorated Carriers for Targeted *In Vivo* Delivery

Co-chairs: Stefan Radtke, PhD, Fred Hutchinson Cancer Center and Anastasia Khvorova, PhD, University of Massachusetts Medical School

8:00–8:35 AM	**LNP Targeting and *In Vivo* Applications** *Hamideh Parhiz, University of Pennsylvania*
8:35–9:10 AM	**CoLOCKR tools for *In Vivo* T Cell and HSC Targeting** *Jilliane Perkins*
9:10–9:45 AM	**Approaches and Considerations for *In Vivo* Gene Delivery** *Semih Tareen, Sana Biotechnology*

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

SCIENTIFIC SYMPOSIUM

PETREE HALL C

Fostering Global Collaborations to Advance Gene and Cell Therapy Research in LMICs

Co-chairs: Jayandharan Rao, Indian Institute of Technology and Ulrike Reiss, MD, St. Jude Children's Research Hospital

8:00–8:25 AM	**Clinical Gene Therapy for Monogenetic Disorders - Progress in Thailand** *Usanarat Anurathapan, MD, Faculty of Medicine Ramathibodi Hospital, Mahidol University*
8:25–8:50 AM	**Diversification of Genetic Therapies: Barriers and Opportunities - an Industry Perspective** *Walter Gunzburg, PhD, University of Veterinary Medicine, Vienna*
8:50–9:15 AM	**CAR-T Cell Therapy - Global Collaborations and Clinical Service Scale up in a Tertiary Care Center in India** *Vikram Mathews, MD, PhD, FASc, Christian Medical College, Vellore*
9:15–9:40 AM	**Lessons Learnt from Gene Therapy of Hemophilia in Brazil** *Margareth Ozelo, MD, PhD, Hemocentro UNICAMP, University of Campinas*

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

SCIENTIFIC SYMPOSIUM

ROOM 403 AB
Medicaid Coverage and Reimbursement of Cell and Gene Therapies
Chair: Francesca Cook, Regenxbio

8:00–8:15 AM	**Current Challenges in Patient Access and the Implications for Scientists** *Rayne Rouce, MD, Baylor College of Medicine*
8:15–8:30 AM	**The Role of Medicaid in Ensuring Timely, Equitable Access to Care** *Diane Berry, PhD, Sarepta Therapeutics*
8:30–8:45 AM	**Payer Considerations for Medicaid Fee-for-service Outcomes** *Denise Pierce, DK Pierce Consulting*
8:45–9:00 AM	**Payer Perspective on Outcomes Based Arrangements: Managed Care Organization** *Ross Hoffman, MD, Centene Corp*
9:00–9:15 AM	**Payer Perspective on Outcomes Based Arrangements: Reinsurance for States and MCOs** *Ashley Hume, Emerging Therapy Solutions*
9:15–9:45 AM	**Panel Discussion** *Rayne Rouce, MD* *Diane Berry, PhD* *Denise Pierce* *Ross Hoffman, MD* *Ashley Hume*

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

SCIENTIFIC SYMPOSIUM

ROOM 411

New Approaches in Cell and Gene Therapy for Cardiovascular Disease

Co-chairs: Michael Laflamme, MD, PhD, McEwen Stem Cell Institute, University Health Network, University of Toronto and Jeffrey Ellsworth, PhD, Stellar Research and Development, LLC

8:00–8:25 AM	**Correction of Multisystem Smooth Muscle Dysfunction Syndrome in a Mouse Model via Base Editing** *Mark Lindsay, MD, PhD, Massachusetts General Hospital*
8:25–8:50 AM	**Developing the DWORF Microprotein as a Gene Therapy Target for Heart Failure** *Catherine Makarewich, PhD, Cincinnati Children's Hospital Medical Center*
8:50–9:15 AM	**Cell Cycle Reactivation in Cardiomyocytes** *Mauro Giacca, MD, PhD, King's College London*
9:15–9:40 AM	**Engineering AAVs to Target and Cross the Brain Vasculature** *Benjamin Deverman, PhD, Broad Institute*

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

SCIENTIFIC SYMPOSIUM

CONCOURSE HALL 150 & 151

N=1 and Beyond: Small Patient Studies to Enable New Therapies for Genetic Disorders

Co-chairs: Rebecca Ahrens-Nicklas, PhD, MD, Children's Hospital of Philadelphia and Gerald Lipshutz, MD, University of California, Los Angeles

8:00–8:25 AM	**From Mila to Millions: Opportunities for Individualized Medicines** *Julia Vitarello, Mila's Miracle Foundation*
8:00–8:25 AM	**N=1 and Milosen** *Timothy Yu, MD, PhD, Boston Children's Hospital*
8:25–8:50 AM	**Bespoke Program** *PJ Brooks, PhD, NIH / NCATS*
8:50–9:15 AM	**Considerations for Navigating a Regulatory Pathway for Ultra-Rare Diseases** *Kevin Whittlesey, PhD, Dark Horse Consulting*
9:15–9:40 AM	**Panelist - N=1 and Beyond: Small Patient Studies to Enable New Therapies for Genetic Disorders** *Petra Kaufmann, MD, Affinia Therapeutics, Inc.* *Kevin Whittlesey, PhD, Dark Horse Consulting Group* *Amanda Haidet-Phillips, PhD, Sarepta Therapeutics*

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

SCIENTIFIC SYMPOSIUM

WEST HALL B

Expanding the Genome Editor Toolbox and Translation Towards the Clinic

Co-chairs: Benjamin Kleinstiver, PhD, Massachusetts General Hospital & Harvard Medical School and Mark Osborn, PhD, University of Minnesota

8:00–8:25 AM	**Prime Editing** *Andrew Anzalone, PhD, Prime Medicine*
8:25–8:50 AM	**Development and Characterization of Multiplexed Orthogonal Base Editing Systems** *Alexis Komor, PhD, University of California, San Diego*
8:50–9:15 AM	**Writing DNA with RNA: Genome Engineering by Target Primed Reverse Transcription** *Cecilia Cotta-Ramusino, PhD, Tessera Therapeutics*
9:15–9:40 AM	**From N=1 to N=All: Expanding the Clinical Impact of CRISPR** *Fyodor Urnov, PhD, University of California, Berkeley*

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

SCIENTIFIC SYMPOSIUM

CONCOURSE HALL 152 & 153
Venture Capital in the Gene and Cell Therapy Space
Co-chairs: Robert Peters, PhD, Ensoma and Eric David, MD, JD, BridgeBio

8:00–8:35 AM	**Venture Capital in the Gene and Cell Therapy Space - 5AM Ventures** *Deborah Palestrant, PhD, 5AM Ventures*
8:35–9:10 AM	**Venture Capital in the Gene and Cell Therapy Space - BridgeBio** *Neil Kumar PhD, BridgeBio*
9:10–9:45 AM	**Venture Capital in the Gene and Cell Therapy Space - Rejuvenate Bio** *Dan Oliver, Rejuvenate Bio*

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

FIRESIDE CHAT

PETREE HALL D

A Fireside Chat with Siddhartha Mukherjee

8:00 AM–9:00 AM

Panel Participants: Hans-Peter Kiem, MD, PhD, Fred Hutchinson Cancer Center, Siddhartha Mukherjee, DPhil, MD, Columbia University, Fyodor Urnov, PhD, UC Berkeley, and Maria-Grazia Roncarolo, MD, Stanford University

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

AWARDS

ROOM 502 AB
CDA/DEI Awards I
Chair: Li Ou, PhD, Genemagic Biosciences

8:00–8:25 AM	**Domain Insertional Profiling of AAV** *Mareike Hoffmann, PhD, University of Minnesota*
8:25–8:50 AM	**Developing CRISPR/AAV-mediated** *Kyle Cromer, PhD, UCSF*
8:50–9:15 AM	**Liver Repopulation by Acetaminophen Selection of Hepatocytes Edited *Ex Vivo* Using Nonviral Delivery Approaches** *Renee Cottle, PhD, Clemson University*
9:15–9:40 AM	**AA, BB, CC, D Deciphering the Alphabet of the AAV ITR** *Liujiang Song, PhD, University of North Carolina*

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

ORAL ABSTRACT SESSION

ROOM 515 AB
Late Breaking Abstracts I
8:00–9:45 AM
Co-Chairs: Terence Flotte, MD, UMass Chan Medical School and Jeffrey S. Chamberlain, PhD, University of Washington

8:00–8:15 AM	**Two-Year Clinical Outcomes with Fordadistrogene Movaparvovec for Duchenne Muscular Dystrophy (DMD) and Contextualization with External Controls** *Perry Shieh, MD, PhD, UCLA*
8:15–8:30 AM	**Preclinical Efficacy of AAVrh.74-PKP2a (RP-A601): Gene Therapy for PKP2-Associated Arrhythmogenic Cardiomyopathy** *Christopher Herzog, PhD, Rocket Pharmaceuticals*
8:30–8:45 AM	**Universal Survival and Superior Immune Reconstitution after Lentiviral Gene Therapy with Low Dose Conditioning for X-linked SCID (SCID-X1)** *Sung-Yun Pai, MD, NIH*
8:45–9:00 AM	**Safety and Survival Outcomes in Recurrent High-Grade Glioma Patients Treated with CAN-3110, a First-in-Class ICP34.5 Expressing Oncolytic HSV1** *Francesca Barone, Candel Therapeutics*
9:00–9:15 AM	**Transient Delivery of Epigenome Editors Stably Represses PCSK9 and Lowers LDL Cholesterol in Non-Human Primates** *Jennifer Kwon, Tune Therapeutics*
9:15–9:30 AM	**BCMA-Targeted CART (HBI0101), a Safe and Efficacious Novel Modality of Treatment for LC Amyloidosis Patients** *Nathalie Asherie, Hadassah Medical Center-HUJI*
9:30–9:45 AM	**Rhesus Antibody Secreting Cells Differentiated Ex Vivo from B Cells Engraft without Preconditioning in an Autologous Host and Represent a Novel Modality for Cell and Gene Therapy** *David Young, MD, PhD, NIH*

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

EXHIBITOR SHOWCASES

8:15–8:45 AM
ROOM 511
Viralgen: A Platform Approach for Adeno-Associated Virus Manufacturing to Support Gene Therapy Products

9:15–9:45 AM
ROOM 511
AGC Biologics: Navigating Complexities of Viral Vector Production for Gene Therapies

10:15–10:45 AM
ROOM 511
PerkinElmer: Toward Developing Best-in-Class rAAV Vectors with PerkinElmer

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

TOOLS AND TECHNOLOGY FORUM

PRESENTATION THEATER IN EXHIBIT HALL
Tools and Technology Forum IV
Chair: Sarwish Rafiq, PhD, Emory University

10:15–10:30 AM	Promega Corporation: Expanding the AAV Toolbox: A Dual Luminescence and Fluorescence Reporter System
10:30–10:45 AM	PBS Biotech: Systematic Bioprocess Design for the Reproducible and Robust Workflow of iPSC Expansion and Scale-Up in the Vertical-Wheel Bioreactor
10:45–11:00 AM	Gyros Protein Technologies: Accelerate downstream process development with automated analysis of viral vector titer and process-related impurities.
11:00–11:15 AM	PROGEN Biotechnik: Humanized Chimeric AAV Antibodies—Advanced Standard for Serological Assay
11:15–11:30 AM	Synthego: Choosing the right GMP partner for your CRISPR-enabled cell and gene therapy

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

SCIENTIFIC SYMPOSIUM

WEST HALL B
Somatic Cell Genome Editing (SCGE) Program
Chair: Marrah Lachowicz-Scroggins, PhD, NIH, NHLBI and Felicia Qashu, PhD, NIH

10:16–10:25 AM	**SCGE Introduction** *Felicia Qashu, PhD, National Institutes of Health*
10:25–10:37 AM	**Technologies to Facilitate Translation of Somatic Cell Editing** *Charles Gersbach, PhD, Duke University*
10:37–10:49 AM	**Delivery Initiative Highlights (SCGE Consortium)** *Ross Wilson, PhD, University of California Berkeley*
10:49–11:01 AM	**Develop Combinatorial Non-Viral and Viral CRISPR Delivery for Lung Diseases (SCGE Consortium)** *Shunqing Liang, PhD, UMass Chan Medical School*
11:01–11:13 AM	**Animal Initiative Highlight** *Cathleen Lutz, PhD, The Jackson Laboratory*
11:13–11:48 AM	**Biological Initiative Highlight** *Bruce Conklin, MD, Gladstone Institute of Cardiovascular Medicine*
11:48 AM–12:00 PM	**Phase 2 of the NIH's Somatic Cell Genome Editing Program** *Marrah Lachowicz-Scroggins, NIH, NHLBI*

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

SCIENTIFIC SYMPOSIUM

ROOM 411
Evolution of Genetic Medicines: Navigating the Challenges and Synergies Among Therapeutic Modalities
Co-chairs: Thomas Wechsler, PhD, Janssen BioTherapeutics and Kaye Spratt, PhD, Rejuvenate Bio

10:15–10:46 AM	**The Importance of Modality Selection for Success in Drug Discovery** *Emma Parmee, DPhil, Janssen Pharmaceutical Companies of Johnson & Johnson*
10:46–11:18 AM	**Therapeutic Modalities: One Size Does NOT Fit All** *Vanessa Newman, BioMarin Pharmaceuticals*
11:18–11:50 AM	**How Patients View Therapeutic Modalities** *Glenn Pierce, MD, PhD, World Federation of Hemophilia*

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

SCIENTIFIC SYMPOSIUM

CONCOURSE HALL 150 & 151
From the Needle to the Nucleus: Exploring Key Points of Vector Design & Development
Co-chairs: Joseph Lee, PhD, Affini-T Therapeutics and Adam Cockrell, PhD, Solid Biosciences

10:16–10:40 AM	**AAV *In Vivo* Tracking and Microscopy** *Katherine Ferrara, PhD, Stanford University*
10:40–11:05 AM	**Disease Gene-agnostic Gene Therapy to Prolong Vision** *Connie Cepko, PhD, Harvard Medical School*
11:05–11:30 AM	***In Vivo* Cell-specific Gene Delivery Achieved Through a Re-targetable Fusogen Platform** *Jagesh Shah, PhD, Sana Biotechnology*
11:30–11:55 AM	**Spatial Transcriptomics for Pooled AAV Profiling** *Min Jee Jang, PhD, Caltech*

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

SCIENTIFIC SYMPOSIUM

ROOM 408 AB

Accelerate Your Drug Development Program in Rare Disease with Robust Natural History Data: An Evolving Space

Chair: Kathleen Kirby, Viridian

10:16–10:25 AM	**Natural History Studies - Introduction** *Kathleen Kirby, Viridian*
10:25–10:37 AM	**How to Design an NH Study that Regulators and Clinicians Support** *Adam Shaywitz, MD, PhD, BridgeBio Pharma*
10:37–10:49 AM	**Designing a Development Plan for a Robust Control Data Source** *John Balser, PhD, Veristat LLC*
10:49–11:01 AM	**Listening to the Public/Patients/Caregivers** *Cara O'Neill, MD, Cure Sanfilippo Foundation*
11:01–11:13 AM	**Running NH Studies in a Robust and Verifiable Way** *Anne Pariser, MD, Alltrna*
11:13–11:48 AM	**Panel discussion** *John Balser, PhD* *Cara O'Neill, MD,* *Adam Shaywitz, MD PhD,* *Anne Pariser, MD* *Kristen Pappas* *Daniel Chung, DO, SparingVision*
11:48 AM–12:00 PM	**Key take-aways; Future directions** *Samantha Parker, Innoskel*

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

SCIENTIFIC SYMPOSIUM

ROOM 403 AB

Made in Japan: Leaders in Gene & Cell Therapy from The Land of the Rising Sun

Co-chairs: Takafumi Nakamura, PhD, Tottori University and Noriyuki Kasahara, MD, PhD, University of California, San Francisco (UCSF)

10:16–10:40 AM	**Gene Therapy for Neurodegenerative Disorders at Osaka University** *Hideki Mochizuki, MD, PhD, Osaka University Gradual School of Medicine*
10:40–11:05 AM	**Oncolytic Adenoviruses for Pancreatic Cancers** *Masato Yamamoto, MD, PhD, University of Minnesota*
11:05–11:30 AM	**New Targets for CAR-T Cell Therapy Against Multiple Myeloma** *Naoki Hosen, PhD, Osaka University Graduate School of Medicine*
11:30–11:55 AM	**Development of Hematopoietic Stem Cell-targeted Gene Therapy for Sickle Cell Disease** *Naoya Uchida, MD, PhD, University of Tokyo, Japan*

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

AWARDS

ROOM 502 AB
CDA/DEI Awards 2
Chair: Agnieszka Czechowicz, MD, PhD, Stanford University School of Medicine and Mark Brimble, PhD, St. Jude Children's Research Hospital

10:16–10:35 AM	**Investigation of Contributing Factors to Human Immune Responses Against AAV-Mediated Gene Therapy** *Mark Brimble, PhD, St. Jude Children's Research Hospital*
10:35–10:55 AM	**Engineering hematopoietic Stem Cell Specific CRISPR Delivery Vehicles (Exo-CRISPR)** *Reza Shabazi, PhD, Indiana University*
10:55–11:15 AM	**Longitudinal Profiling of Active Fetal Immunity with Single-Cell and Spatial Resolution** *Enrico Barrozo, PhD, Baylor College of Medicine*
11:15–11:35 AM	**Engineering "Off the Shelf" scFv-dsTCR Tregs with TCR Like Signaling for Tolerance to Anti-Drug Antibody Formation** *Moanaro Biswas, PhD, Indiana University*
11:35–11:55AM	**Engineering Exosomes for Delivery of Biotherapeutics** *Andrew Hamann, PhD, University of Nebraska - Lincoln*

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

SCIENTIFIC SYMPOSIUM

ROOM 515
Optimizing CAR T for Treatment of Cancer and Autoimmune Diseases
Chair: Cynthia Dunbar MD, National Institutes of Health (NIH)

10:15–10:40 AM	**Engineering Next-Generation CAR T-Cells for Cancer Immunotherapy** *Yvonne Chen, PhD, UCLA*
10:40–11:05 AM	**Treatment of Murine Autoimmunity via Autoreactive T-Cell-specific CAR T-Cells** *Chyi Hsieh, Washington University*
11:05–11:30 AM	**New Directions in CAR T-Cell Design** *Michel Sadelain, MD, PhD, Memorial Sloan Kettering Cancer Center*
11:30-11:45AM	**Panel Discussion** *Yvonne Chen, PhD* *Chyi Hsieh* *Michel Sadelain, MD, PhD*

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

ORAL ABSTRACT SESSION

CONCOURSE HALL 152 & 153
Late Breaking Abstracts II
10:15 AM–12:00 PM
Co-Chairs: Philip Gregory, DPhil, 2seventy Bio and Isabelle Rivière, PhD, Takeda

10:15–10:30 AM	**Chimeric Antigen Receptor (CAR)-Modified Stem Cells Generate Superior Anti-HIV Efficacy Compared to CAR-Modified Autologous Peripheral T Cells in Preclinical Animal Models** *Mayra Carrillo, UCLA*
10:30–10:45 AM	**ARCUS-Mediated Excision of the "Hot Spot" Region of the Human Dystrophin Gene for the Treatment of Duchenne Muscular Dystrophy (DMD)** *Gary Owens, Precision BioSciences*
10:45–11:00 AM	**Advancing Epigenetic Editing with CRISPR-GNDM: Novel Muscle-Tropic AAV Vectors Deliver Promising Single-Dose Treatment for LAMA2-CMD** *Yuanbo Qin, Modalis Therapeutics Inc.*
11:00–11:15 AM	**Long Term Lineage Commitment is Modulated by the Underlying Disease in Hematopoietic Stem Cell Gene Therapy Patients** *Eugenio Montini, PhD, San Raffaele Telethon Institute for Gene Therapy*
11:15–11:30 AM	**Cerebrospinal Fluid Enables Potent AAV Gene Delivery to the Primate Inner Ear** *Paul Ranum, Children's Hospital of Philadelphia*
11:30–11:45 AM	**First in Human Studies Show Activation of SC-DARIC33, a Rapamycin-Regulated Anti-CD33 CAR T Cell Therapy, in Patients with AML** *Jacob Appelbaum, MD, PhD, University of Washington*
11:45 AM–12:00 PM	**A Novel Therapeutic Safely Suppresses Pre-Existing Neutralizing Antibodies and Enables Re-Administration of Hemophilia A Gene Therapy in Large Animals** *Charles Askew, PhD, University of North Carolina*

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

POSTER SESSION III

12:00–1:30 PM
EXHIBIT HALL/WEST HALL A

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

INDUSTRY SPONSORED SYMPOSIA

12:00 PM–1:30 PM

PETREE HALL C

Dyno Therapeutics: The Capsids You Need: AI-guided Design and in vivo Validation of AAV Capsids for Better Delivery to Muscle, Eye and CNS

PETREE HALL D

Takeda: Bringing Functional Cures to Patients with Next-generation Gene Therapies

ROOM 409

Labcorp Drug Development: Enabling Next Generation Gene Therapies by Leveraging Strategic Collaborations

ROOM 501

Myrtelle Inc.: Gene Therapies Targeting Oligodendrocytes: The Expanding Role of Oligodendrocytes in Brain Function & Disease States

ROOM 511

Capsida Biotherapeutics: Unlocking the Potential of Gene Therapy: A Targeted Next Generation Platform

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

AWARD SYMPOSIUM

WEST HALL B
Outstanding New Investigator Symposium
Chair: Paula Cannon, PhD, University of Southern California

1:30–1:40 PM	**Introduction and Presentation of New Awards** *Hans-Peter Kiem, MD, PhD, Fred Hutchinson Cancer Research Center*
1:40–2:02 PM	**Strategies to Manage Immunological Challenges of AAV-mediated Gene Therapy** *Manuela Corti, PhD, University of Florida*
2:02–2:24 PM	**Engineering T-cells against malignant and pathogenic T-cells** *Maksim Mamonkin, PhD, Baylor College of Medicine*
2:24–2:46 PM	**Integrating Gene Editing and Synthetic Immunology to Improve T Cell Therapies** *Justin Eyquem, PhD, UCSF*
2:46–3:08 PM	**Modeling and Correcting Disease with Genome Editing** *Shondra Pruett-Miller, PhD, St. Jude Children's Research Hospital*

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

ORAL ABSTRACT SESSION

CONCOURSE HALL 150 & 151
AAV Vector Genome Biology and Engineering II
Co-chairs: Mathieu Nonnenmacher, PhD, Voyager Therapeutics and Victoria Madigan, PhD, MIT and Sandhya Pande, Shape Tx

3:45–4:00 PM	**Mechanistic Evaluation of Liver-specific Transgene Repression from AAV Vectors in Non-Human Primates and Minipigs** *Zhuo Wang, PhD, REGENXBIO Inc.*
4:00–4:15 PM	**HICOS: High-Throughput Screening of Codon Optimized Sequences Using Protein Barcoding** *Jang Hwan Cho, PhD, Spark Therapeutics*
4:15–4:30 PM	**Tissue Regeneration Enhancer Elements Enable Injury Responsive AAV Gene Expression** *David Wolfson, Duke University School of Medicine*
4:30–4:45 PM	**Strong Compact Neuron Specific Promoters for AAV Gene Transfer** *Xiupeng Chen, UMass Chan Medical School*
4:45–5:00 PM	**Capsid Mediated Control of Adeno-associated Viral Genome Transcription** *Ezra Loeb, Duke University*
5:00–5:15 PM	**DNA Damage Repair Pathways Have Opposing Effects on Concatenation and Expression from rAAV Vectors** *Anna Maurer, PhD, University of California, Berkeley*
5:15–5:30 PM	**Should You Judge an AAV by its Cover? The Role the AAV Capsid Plays in Setting up the Vector Epigenome** *Eirini Vamva, Stanford University*

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

ORAL ABSTRACT SESSION

CONCOURSE HALL 152 & 153
Genome & Epigenome Editing Technologies I
Co-chairs: Benjamin Kleinstiver, Massachusetts General Hospital and Marcello Maresca, PhD, AstraZeneca-Gothenburg

3:45–4:00 PM	**A Metagenomics-Derived Gene-Editing Toolbox Enables Efficient Genome Engineering with Nucleases and Base-Editors in Primary Cells and *In Vivo*** *Gregory Cost, PhD, Metagenomi*
4:00–4:15 PM	**Programmable A-to-Y Base Editing by Fusing an Adenine Base Editor with an N-methylpurine DNA Glycosylase** *Huawei Tong, HuidaGene Therapeutics Co., Ltd.*
4:15–4:30 PM	**Multiplexed Epigenome Editing to Induce Sustained Silencing of Immune Checkpoints' Expression in Car T Cells** *Maria Silvia Roman Azcona, PhD, Medical Center - University of Freiburg*
4:30–4:45 PM	**Novel Cas9 Orthologs Expand The Genome Editing Toolbox For CRISPR-Based Therapeutics** *Laura Pezzè, Alia Therapeutics*
4:45–5:00 PM	**Multiplexed Editing Without Chromosomal Rearrangements Using Epigenetic Editors** *Sahar Abubucker, Chroma Medicine*
5:00–5:15 PM	**Metagenomic Mning and Engineering of Single-Strand Annealing Proteins for Cleavage-Free Genomic Knock-In of Long Sequence in Mouse Hepatocytes via dCas9 Targeting** *Le Cong, PhD, Stanford University*
5:15–5:30 PM	**Comprehensive Engineering of CasX Holoenzyme to Create an X-Editor with High Activity, Specificity and Deliverability** *Addison Wright, Scribe Therapeutics*

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

ORAL ABSTRACT SESSION

PETREE HALL C
Pharmacology/Toxicology Studies: Bio Distribution
Co-chairs: Kah-Whye Peng, PhD, Mayo Clinic and Daniela Cesana, PhD, SR-TIGET

3:45–4:00 PM	**PK/PD Modeling to Inform Clinical Development of an Adeno-Associated Virus Gene Transfer Therapy for Duchenne Muscular Dystrophy** *Lilly East, Sarepta Therapeutics*
4:00–4:15 PM	**Vector Shedding in Patients with DMD Treated with Delandistrogene Moxeparvovec and Seroconversion from Shed Vector in Naïve Mice** *Rachel Potter, PhD, Sarepta Therapeutics*
4:15–4:30 PM	**Development of an Immunosuppressed Murine Model for AAV based Gene Therapy Biodistribution Studies** *Mayuri Prasad, PhD, Labcorp Drug Development*
4:30–4:45 PM	**Assessment via Positron Emission Tomography of Adenovirus Capsid Biodistribution Following Intramuscular Versus Intravenous Administration to Nonhuman Primates** *Edward Fung, Weill Cornell Medicine*
4:45–5:00 PM	**Neural Stem Cell Mediated Oncolytic Virotherapy for Ovarian Cancer** *Karen Aboody, MD, City of Hope National Medical Center & Beckman Research Institute*
5:00–5:15 PM	**A Novel Technique to Detect Peripheral Blood CAR+ T Cells using RNAscope *In Situ* Hybridization (ISH) in Non-Human Primates and Mice** *Sundeep Chandra, Sana Biotechnology*
5:15–5:30 PM	**Nonclinical Pharmacology, Biodistribution, and Safety Studies Supporting the Clinical Development of DB-OTO (AAV1-Myo15-hOTOFv5) for Hearing Loss due to Genetic Otoferlin Protein Deficiency** *Tyler Gibson, PhD, Decibel Therapeutics*

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

ORAL ABSTRACT SESSION

PETREE HALL D

Emerging Preclinical Gene and Cell Therapy Approaches for Neurological Disorders

Co-chairs: Andrew Mercer, PhD, REGENXBIO Inc. and Heather Gray-Edwards, DVM, PhD, UMass Chan Medical School

3:45–4:00 PM	**Transplantation of Wild-Type Hematopoietic Stem and Progenitor Cells Rescue Alzheimer's Disease in a Mouse Model and Highlights the Central Role of Microglia in Disease Pathogenesis** *Priyanka Mishra, University of California, San Diego*
4:00–4:15 PM	**Intracerebroventricular Delivery of SLC6A8 by Self Complimentary AAV9 Restores Brain Creatine and Reduces Hyperactivity in a Mouse Model of SLC6A8 Deficiency** *Troy Webster, Queen's University*
4:15–4:30 PM	**AAV9-Mediated Expression of Secreted Klotho Reduced Several Aging-Associated Phenotypes and Increased Longevity** *Joan Roig-Soriano, Universitat Autònoma de Barcelona*
4:30–4:45 PM	**A System for Whole-Body Gene Delivery Rescues Wolfram Syndrome II** *Michael Florea, Harvard*
4:45–5:00 PM	**A Gene Therapy Approach to Treat PMLD1** *Kleopas Kleopa, MD, PhD, The Cyprus Institute of Neurology & Genetics*
5:00–5:15 PM	**Gene Therapy for Mucolipidosis IV Using Novel CNS-Targeted AAV Capsid** *Yulia Grishchuk, PhD, Massachusetts's General Hospital*
5:15–5:30 PM	**Development of an *Ex Vivo* Gene Therapy for Frontotemporal Dementia (FTD)** *Yuri Ciervo, The University of Padua*

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

ORAL ABSTRACT SESSION

ROOM 403 AB
CAR Immunotherapy
Co-chairs: Renier Brentjens, MD, PhD, Roswell Park Comprehensive Cancer Center
and Sarwish Rafiq, PhD, Emory University

3:45–4:00 PM	**Epitope Engineered Hematopoietic Stem and Progenitor Cells to Enable Multi-Specificity Car-T-Cell Immunotherapy for Acute Myeloid Leukemia** *Gabriele Casirati, MD, PhD, Dana Farber Cancer Institute*
4:00–4:15 PM	**IL-12 Reprograms CAR-Expressing NKT Cells To Long-Lived Th1-polarized Antitumor Effector Cells** *Elisa Landoni, PhD, UNC Lineberger Cancer Center*
4:15–4:30 PM	**Knocking-Down Expression of BTG1, a Key Driver of NKT Cell Exhaustion, Promotes Durable Tumor Control by CAR-NKTs in a Xenogeneic Neuroblastoma Model** *Andras Heczey, MD, Baylor College of Medicine*
4:30–4:45 PM	**Elucidating the Role of PD-1/PD-L1 Axis in Solid Tumour CAR-T Cell Therapy** *Irene Andreu Saumell, Hospital Clínic*
4:45–5:00 PM	**Non-Virally Engineered Polyclonal γδ T Cells Exhibit Potent Anti-Tumor Activity *In Vivo*** *Jacob Bridge, University of Minnesota*
5:00–5:15 PM	**Combining Anti-CD33 Chimeric Antigen-Receptor T cells with the Hypomethylating Agent Decitabine to Treat Acute Myeloid Leukemia** *Kenneth Ng, City of Hope*
5:15–5:30 PM	**Removal of Endogenous T Cell Receptor (TCR) May Inadvertently Compromise Allogeneic CAR T Cell Function** *Hongjing Xia, Novartis Institute of Biochemical Research*

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

ORAL ABSTRACT SESSION

ROOM 408 AB
AAV Vectors - Preclinical and Proof-of-Concept *In Vivo* Studies II
Co-chairs: Satinder Rawat, PhD, Taysha Gene Therapies and Eva Andres-Mateos, PhD, Atsena Therapeutics

3:45–4:00 PM	**Adeno-Associated Virus Serotype 5 is a Suitable Vector for S100A1-Based Gene Therapy of Post-Ischemic Chronic Cardiac Dysfunction** *Patrick Most, MD, Heidelberg University Hospital*
4:00–4:15 PM	**Expression and Function of Guanidinoacetate Methyltransferase (GAMT) is Effectively Restored in Cellular and Murine Models of GAMT Creatine Deficiency Following Treatment with *scAAV9.hGAMT*** *Robyn Binsfeld, Queen's University*
4:15–4:30 PM	**Comparative Transcriptomic Analysis Identifies Rescue of Dysregulated Signatures by ATXN1 Knockdown and ATXN1L Overexpression in a Mouse Model of SCA1** *Ashley Robbins, University of Pennsylvania*
4:30–4:45 PM	**Optimizing Base Editing Strategies in a Mouse Model of Zellweger Syndrome** *Cathleen Lutz, The Jackson Laboratory*
4:45–5:00 PM	**Therapeutic Effect of Linker Protein-Mediated Gene Therapy in a Mouse Model for LAMA2-Related Muscular Dystrophy** *Judith Reinhard, University of Basel*
5:00–5:15 PM	**AAV-mediated Monoclonal Antibody Expression for the Prevention and Treatment of *Pseudomonas Aeruginosa* Infections** *Jordyn Lopes, University of Guelph*
5:15–5:30 PM	**AAV9-delivered Artificial miRNA Gene Therapy Rescues the PS19 Mouse Model of Tauopathy** *Huei-Bin Wang, UT Southwestern Medical Center*

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

ORAL ABSTRACT SESSION

ROOM 409 AB

Vector Product Engineering, Development, and Manufacturing

Co-chairs: Johannes Van Der Loo, PhD, Children's Hospital of Philadelphia and Stephen Russell, MD, PhD, Mayo Clinic

3:45–4:00 PM	**A Sequence-Upgraded PolyA LTR (SupA-LTR™ Imparts Enhanced Transcriptional Insulation To New 4th Generation Lentiviral Vectors From Both 5' and 3' Genic Regions in Target Cells** *Dan Farley, Oxford BioMedica*
4:00–4:15 PM	**Fitting-Out 4th Generation Lentiviral Vectors: New Technology To Improve Their Production, Quality, Safety, Capacity and Utility** *Dan Farley, Oxford Biomedica*
4:15–4:30 PM	**Suspension-Based Adenovirus Production Process Development and Scale-Up in Scale-™ Fixed-Bed Bioreactors** *Hanna Lesch, PhD, Exothera*
4:30–4:45 PM	**Lentiviral Vector Production Using Suspension Adapted HEK293T Cells in Serum Free Media** *Matthew Welty, IU School of Medicine*
4:45–5:00 PM	**Development of a Scalable Lentiviral Manufacturing Upstream Process with a Suspension Producer Line for Cell Therapy Applications** *Bianca Volkmann, SIRION Biotech (a PerkinElmer company)*
5:00–5:15 PM	**Intensified Production Process for Gorilla Adenoviral Vector GRAd: Yield, Purity, Potency and Stability Results** *Stefano Colloca, ReiThera*
5:15–5:30 PM	**Lentiviral Vector Engineering: Impact on Manufacturing and Vector Performance** *Ana Coroadinha, PhD, iBET*

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

ORAL ABSTRACT SESSION

ROOM 411
Preclinical Development of Novel Cell Therapies for Human Disease
Co-chairs: Richard James, PhD, Seattle Children's Research Institute and Daisuke Araki, NIH

3:45–4:00 PM	**Gene-Editing to Eliminate Automaticity Prevents Arrhythmias after Engrafting Human Pluripotent Stem Cell-Derived Cardiomyocytes** *Kenta Nakamura, MD, University of Washington*
4:00–4:15 PM	**Engineering Stage-Specific Developmental Cues to Generate Non-allogenic, iPSC-derived CAR T cells for Immunotherapy** *Sang Pil Yoo, UCLA*
4:15–4:30 PM	**Electrophysiological Maturation Of Human Pluripotent Stem Cell-Derived Cardiomyocytes *via* Gene Editing** *Silvia Marchiano, University of Washington*
4:30–4:45 PM	**Induced Human Pluripotent Stem Cells Differentiation to Syndetome Informed by Single Cell Analysis** *Victoria Yu, Cedars-Sinai Medical Center*
4:45–5:00 PM	**Microglia Replacement With Bone Marrow-Derived Cells After Transient Inhibition of the Colony-Stimulating Factor 1 Receptor (CSF1R) Is Superior to Standard Myeloablative Conditioning in Neuropathic Lysosomal Storage Diseases** *Pasqualina Colella, PhD, Stanford University*
5:00–5:15 PM	**Constitutive IL-7 Receptor (C7R) Signaling Enhances the Persistence of EBV-Specific T-Cells** *Sandhya Sharma, Baylor College of Medicine*
5:15–5:30 PM	**Stomach-Derived Human Insulin-Secreting Organoids for Diabetes Cell Therapy** *Joe Zhou, Weill Cornell Medical College of Cornell University*

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

ORAL ABSTRACT SESSION

ROOM 501 ABC
Cancer - Oncolytic Viruses
Co-chairs: John Bell, PhD, Ottawa Hospital Research Institute and Evanthia Galanis, MD, Mayo Clinic

3:45–4:00 PM	**A Personalised Neo-Antigen Viro-Immunotherapy Platform for Triple-Negative Breast Cancer** *Yaohe Wang, MD, PhD, Barts Cancer Institute, Queen Mary University of London*
4:00–4:15 PM	**Emerging Data from the Use of TILT-123, an Oncolytic Adenovirus Armed with TNFa and IL-2 in Patients with Solid Tumors** *Victor Cervera-Carrascon, PhD, PharmD, TILT Biotherapeutics Ltd*
4:15–4:30 PM	**Addition of αTIGIT Checkpoint Blockade To MV-s-NAP-uPA + αPD-1 Combination Immunotherapy Enhances Long-lasting Immunity In A Syngeneic Murine Glioblastoma Model** *Susanna Concilio, PhD, Mayo Clinic*
4:30–4:45 PM	**A First-in-Human Phase I Clinical Trial of Intratumoral Administration of a Measles Virus Derivative Expressing the *Helicobacter Pylori* Neutrophil-Activating Protein in Patients with Metastatic Breast Cancer** *Ianko Iankov, MD, PhD, Mayo Clinic*
4:45–5:00 PM	**Temozolomide Chemotherapy Enhances Dual blockade of an Oncolytic Virus Expressing an Anti-CD47 Antibody for Breast Cancer Brain Metastases** *Tasha Barr, City of Hope*
5:00–5:15 PM	**Clinical Activity of Oncolytic Virus Voyager-V1 in Patients with Relapsed Refractory Lymphoma** *Nora Bennani, MD, Mayo Clinic*
5:15–5:30 PM	**Novel Oncolytic Therapy VET3-TGI Restricts TGFb1 and Augments Type-1 Immune Response in TME, Leading to Superior Therapeutic Efficacy in Multiple Preclinical Tumor Models** *Ravi Muthuswamy, PhD, KaliVir Immunotherapeutics, Inc.*

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

ORAL ABSTRACT SESSION

ROOM 502 AB
Cell Therapy Product Engineering and Development | Non-Cancer
Co-chairs: Chiara Magnani, PhD, Universitätsspital Zurich, University of Zurich and Moanaro Biswas, PhD, Indiana University

3:45–4:00 PM	**Immune Profiling of Respiratory Syncytial Virus (RSV) for the Development of Targeted Immunotherapy** *Penelope Papayanni, Center for Cell and Gene Therapy, Baylor College of Medicine, Texas Children's Hospital and Houston Methodist Hospital*
4:00–4:15 PM	**Co-Engineering Synthetically Programmed Cells and Biomaterials for Regenerative Medicine** *Joanne Lee, Vanderbilt University*
4:15–4:30 PM	**SEC-seq: Association of Molecular Signatures with Antibody Secretion in Thousands of Single Human Plasma Cells** *Rene Cheng, PhD, Seattle children research institute*
4:30–4:45 PM	**Pancreatic Islet-Specific Engineered Treg are Therapeutic in Mouse Models of Type 1 Diabetes** *Travis Drow, Seattle Childrens Research Institute*
4:45–5:00 PM	**New Generation Trained Mesenchymal Stromal Cell Therapy for Treatment for Rare Autoimmune Diseases** *Hulya Bukulmez, MD, Case Western Reserve University*
5:00–5:15 PM	**Human Chimeric Antigen Receptor-Mesenchymal Stem Cells (CAR-MSCs) display enhanced Trafficking and Immunosuppressive Efficacy** *Olivia Sirpilla, Mayo Clinic*
5:15–5:30 PM	**Engineered Microenvironment Converters (EM-Cs): Macrophages Expressing Synthetic Cytokine Receptors Rebalance Pro-/Anti-Inflammatory Signals in Disease Microenvironments** *David Sloas, PhD, Carisma Therapeutics*

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

TOOLS AND TECHNOLOGY FORUM

PRESENTATION THEATER IN EXHIBIT HALL
Tools and Technology Forum V
Co-chairs: Edith L. Pfister, PhD, UMass Medical School and Lauren Woodard, PhD, Vanderbilt University

3:45–4:00 PM	Parexel: Use Statistical Modeling to Project Future Cost-Savings of Cell and Gene Therapies
4:00–4:15 PM	GenScript USA Inc.: Expedite Your Cell and Gene Therapy Pipelines with GenScript's Non-viral GMP CRISPR Solutions
4:15–4:30 PM	Repligen: AAV Titer Quantitation in <2 Min. Using Variable Pathlength Spectroscopy
4:30–4:45 PM	Catalent: One-stop Integrated Viral Vector Platform for Speed to Clinic
4:45–5:00 PM	Unchained Labs: The AAV Analytical Tool You Should Use Before You Run Anything Else
5:00–5:15 PM	Avantor: Novel Solutions in Sampling to Overcome Challenges Associated with Manufacturing and Commercialization of Cell and Gene Therapies
5:15–5:30 PM	Sartorius: Rapid AAV Titer for Bioprocessing in Gene Therapy Using Octet® BLI Platform

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

EXHIBITOR SHOWCASES

3:45–4:15 PM

ROOM 511

Precision NanoSystems: Multi-Step Engineering Using RNA-Lipid Nanoparticles and Scale Up Manufacturing for Non-Viral Cell and Gene Therapies

4:45– 5:15 PM

ROOM 511

Precision for Medicine: Considerations for AAV ELISpot Assays

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

ORAL ABSTRACT SESSION

ROOM 515 AB

Gene Targeting and Gene Correction: Hemoglobin, Muscle, and Eye

Co-chairs: Luca Biasco, PhD, Sana Biotechnology Inc. and Gretchen Lewis, Kytopen

3:45–4:00 PM	**Exceptional Fetal Hemoglobin Induction by *BCL11A* +58/+55 Combined Enhancer Editing and Hydroxyurea Treatment in Rhesus Monkeys** *Selami Demirci, PhD, NHLBI/NIH*
4:00–4:15 PM	**EPI-321, A POTENTIAL CURE FOR FSHD** *Alexandra Collin de L'Hortet, PhD, Epic-Bio*
4:15–4:30 PM	**Efficient Engraftment of Genome Edited CD34+ HSPCS in CD45 Antibody-Drug Conjugate (ADC) Conditioned Non-Human Primates** *Selami Demirci, PhD, NHLBI/NIH*
4:30–4:45 PM	***In Vivo* Genetic Eye Disease Correction Using Split AAV-Mediated Adenine Base Editing** *Jack Sullivan, Beam Therapeutics*
4:45–5:00 PM	***In Vivo* HSC Base Editing for Gene Therapy of Sickle Cell Disease in a Mouse Model** *Chang Li, PhD, University of Washington*
5:00–5:15 PM	**Optimization and Characterization of Genome Editing of Human Hematopoietic Stem Cells to Induce Fetal Hemoglobin Towards SAGES-1 Clinical Trial** *Varun Katta, St. Jude Children's Research Hospital*
5:15–5:30 PM	**Correction of Multisystem Smooth Muscle Dysfunction Syndrome in a Mouse Model via Base Editing** *Patricia Musolino, Massachusetts General Hospital*

SCHEDULE (ALL TIMES LISTED IN PT)

FRIDAY, MAY 19, 2023

CAREER FAIR

3:15–5:15 PM
CONCOURSE FOYER

RECEPTION

POSTER VIEWING AND NETWORK RECEPTION 3
5:30–7:OO PM
EXHIBIT HALL/WEST HALL A

SCHEDULE (ALL TIMES LISTED IN PT)

SATURDAY, MAY 20, 2023

SCIENTIFIC SYMPOSIUM

ROOM 408 AB

Advances in Gene Therapies to Address Gaps in Veterinary Medicine for our Four-Legged Furry Friends

Co-chairs: Thomas Conlon, PhD, Michelson Found Animals and Noah Davidsohn, PhD, Rejuvenate Bio

8:00–8:20 AM	**OPENING REMARKS** **George Church, PhD, Harvard Medical School** **Overview of Animal Health Industry - Similarities and Differences from Human Health** *Mark Heffernan, PhD, Scout Bio*
8:20–8:40 AM	**Use of a Gene Delivery Approach for Single Dose Lifetime Sterility in Female Cats: An Alternative to Spay** *David Pepin, PhD, Massachusetts General Hospital, Harvard Medical School*
8:40–9:05 AM	**CVM and CBER Commonalities and Opportunities to Collaborate in the Development of Gene Therapies for Human and Veterinary Use** *Lisa Troutman, DVM, FDA, Ann Lund, DVM, FDA, Anne Van Auken, DVM, FDA, Kate Dabirsiaghi, VMD, FDA*
9:05–9:25 AM	**Durability and Safety of AAV Gene Therapy in Hemophilia A Dogs** *Denise Sabatino, PhD, The Children's Hospital of Philadelphia*
9:25–9:45 AM	**Relevance of Animal Data for Human Health Programs** *Noah Davidsohn, PhD, Rejuvenate Bio*

SESSION CONTINUED ON NEXT PAGE

SCHEDULE (ALL TIMES LISTED IN PT)

SATURDAY, MAY 20, 2023

SESSION CONTINUED FROM PREVIOUS PAGE

SCIENTIFIC SYMPOSIUM

ROOM 408 AB

Advances in Gene Therapies to Address Gaps in Veterinary Medicine for our Four-Legged Furry Friends

Co-chairs: Thomas Conlon, PhD, Michelson Found Animals and Noah Davidsohn, PhD, Rejuvenate Bio

9:45–10:10 AM	**Disease Indications in Animal Health - One Medicine Approach in Gene Therapy** *Anne Traas, DVM, DACT, Scout Bio*
10:10–10:45 AM	**CMC Strategy - Decreasing Cost of Goods for Animal Health Market** *Matthew Wilson, MD, PhD, Braidwell*
10:45 AM–12:00 PM	**Panel Discussion** *Lisa Troutman, DVM* *Ann Lund, DVM* *Anne Van Auken, DVM* *Kate Dabirsiaghi, VMD* *Matt Wilson, MD, PhD,* *Mark Heffernan, PhD*

SCHEDULE (ALL TIMES LISTED IN PT)

SATURDAY, MAY 20, 2023

SCIENTIFIC SYMPOSIUM

ROOM 403 AB

Accelerating Medicines Partnership Program - Bespoke Gene Therapy Consortium (AMP BGTC) Annual Meeting

Co-chairs: Michael Lehmicke, Alliance for Regenerative Medicine and Courtney Silverthorn, PhD, Foundation for the National Institutes of Health (FNIH)

8:00–8:30 AM	**BGTC Program Overview** *Courtney Silverthorn, PhD, Foundation for the National Institutes of Health (FNIH)*
8:30–8:50 AM	**Overview of AAV Biology Awards** *Junghae Suh, PhD, Biogen*
9:20–9:40 AM	**California Institute for Regenerative Medicine (CIRM)** *Shyam Patel, PhD, CIRM and Abla Creasey, PhD, California Institute for Regenerative Medicine (CIRM)*
10:00–10:45 AM	**Playbook User Engagement** *Amritha Jaishankar, PhD, IQVIA*
10:45–11:45 AM	**Panel Discussion** *Sadik Kassim, PhD* *PJ Brooks, PhD* *Peter Marks, MD, PhD* *Debra Miller*

SCHEDULE (ALL TIMES LISTED IN PT)

SATURDAY, MAY 20, 2023

ORAL ABSTRACT SESSION

CONCOURSE HALL 150 & 151
AAV Development for Eye, Muscle, Kidney and CNS
Co-chairs: Hossein Ameri, MD, PhD, USC Roski Eye Institute and Darren Murrey, Forge Biologics

8:00–8:17 AM	**Massive Diversity Capsid Screening and Machine Learning Identify Next-Generation AAV for Targeted Tissue Biodistribution** *Kevin Stein, PhD, Shape Therapeutics, Inc.*
8:17–8:34 AM	**Side-by-side Comparison of Systemic AAV8, 9 and rh74 Transduction in Human Muscle** *Matthew Burke, University of Missouri School of Medicine*
8:34–8:51 AM	**Identification of Natural Retinotropic AAV2 Variants with Functionally Enhanced VP1 Unique Regions** *Mengtian Cui, PhD, UMass Chan Medical School*
8:51–9:08 AM	**Modification Of Capsid Surface Residues In AAV2 For Improved Retinal Tropism** *Jacqueline Bogedein, University Hospital, LMU Munich*
9:08–9:25 AM	**Evolving Nephrotropic AAV Variants Using *Ex Vivo* NHP Kidney Perfusion and Human Kidney Organoids** *Alan Rosales, Duke University*
9:25–9:42 AM	**An Integrin-Targeting AAV Developed by a Novel Computational Rational Design Methodology Presents an Improved Targeting to the Skeletal Muscle and Reduced Tropism to the Liver** *Ai Vu Hong, GENETHON*

SCHEDULE (ALL TIMES LISTED IN PT)

SATURDAY, MAY 20, 2023

ORAL ABSTRACT SESSION

CONCOURSE HALL 152 & 153
Genome & Epigenome Editing Technologies II
Co-chairs: Le Cong, PhD, Stanford University
and Maria Grazia Roncarolo, MD, Stanford University

8:00–8:15 AM	**A Novel Engineered U7 Small Nuclear RNA (snRNA) Scaffold Increases ADAR-Mediated Programmable RNA Base Editing** *Susan Byrne, Shape Therapeutics, Inc.*
8:15–8:30 AM	**Improving the Precision and Fidelity of Cas9 and PEn mediated gene editing through Pharmacological Treatment** *Marcello Maresca, PhD, AstraZeneca-Gothenburg*
8:30–8:45 AM	**Nuclease-free Gene Editing with Peptide Nucleic Acids: A New Class of *In Vivo* Gene Editors** *Dani Stoltzfus, NeuBase Therapeutics*
8:45–9:00 AM	**Compact Engineered Human Mechanosensitive Transactivation Modules Enable Potent and Versatile Synthetic Transcriptional Control** *Isaac Hilton, PhD, Rice University*
9:00–9:15 AM	**CRISPR Assisted Trans-splicing of RNA Fragments** *David Fiflis, Duke University*
9:15–9:30 AM	**Precise Cut-and-Paste DNA Insertion Using Engineered Type V-K CRISPR-Associated Transposases** *Benjamin Kleinstiver, Massachusetts General Hospital*
9:30–9:45 AM	***De novo* Design of 26S Proteasome Recruiters with Language Model-Derived Guide Peptides** *Vivian Yudistyra, Duke University*

SCHEDULE (ALL TIMES LISTED IN PT)

SATURDAY, MAY 20, 2023

ORAL ABSTRACT SESSION

PETREE HALL C
Pharmacology/Toxicology Studies: *In Vitro* and *In Vivo* Safety
Chair: Jeff Moffit, PhD, Carbon Biosciences

8:00–8:15 AM	**Tissue-Restricted Promoter Selection as a Mitigation Strategy for Dorsal Root Ganglia Toxicity Due to AAV9 Treatment of Yucatan Pigs** *Harith Rajagopalan, Fractyl Health, Inc.*
8:15–8:30 AM	**Preclinical Safety Assessment of an Investigational Gene Replacement Therapy for the Treatment of *RPE65*-Mediated Inherited Retinal Dystrophies** *Weiwei Wu, HuidaGene Therapeutics Co., Ltd.*
8:30–8:45 AM	***In Vitro* Toolbox to Investigate AAV-induced Immune-associated Hepatotoxicity** *Fabrice Müller, PhD, University of Applied Sciences and Arts Northwestern Switzerland*
8:45–9:00 AM	**Investigating the Impact of Empty AAV Capsids on Safety and Efficacy Following Intracisterna Magna Administration in New Zealand White Rabbits** *Pete Clarner, Biogen*
9:00–9:15 AM	**Intraparenchymal Dosing in Beagle Dogs using Convection Enhanced Delivery Guided by Real Time MRI** *Srishti Vats, Charles River Laboratories*
9:15–9:30 AM	**Target Cell and Tissue Specificity of a Novel CD8-Targeted Fusosome for Direct *In Vivo* Delivery of CD19 or a CD20 CAR to CD8+ T cells** *Sundeep Chandra, Sana Biotechnology*
9:30–9:45 AM	**Safety and Biodistribution Study of Intra-articular Delivered ICM-203, an AAV Gene Therapy Vector for Osteoarthritis, in Normal Rodents and Osteoarthritis-induced Beagle Dogs** *Minsun Park, ICM Co., Ltd.*

SCHEDULE (ALL TIMES LISTED IN PT)

SATURDAY, MAY 20, 2023

ORAL ABSTRACT SESSION

PETREE HALL D
Cell Therapy Product Manufacturing
Co-chairs: Maksim Mamonkin, PhD, Baylor College of Medicine and Seunga Choo, Fred Hutchinson Cancer Center

8:00–8:15 AM	**Efficient and Minimally Perturbative CAR-T Cell Engineering Using Peptide-Enabled CRISPR RNP Delivery** *Joseph Muldoon, UCSF*
8:15–8:30 AM	**A Minimal Workflow for *Ex Vivo Magnetically-Assisted* Lentiviral Vector Transduction of Hematopoietic Stem and Progenitor Cells for Gene Therapy Applications** *Lois Bayigga, PhD, Fred Hutchinson Cancer Research Center*
8:30–8:45 AM	**'Off the shelf' CD62L-Selected Multiplex Edited Umbilical Cord Blood CAR T Cells** *Christos Georgiadis, PhD, UCL Great Ormond Street Institute of Child Health*
8:45–9:00 AM	**Live Imaging on Single Cell Arrays (LISCA) as a Platform to Study Dynamics of mRNA Expression in Mammalian Cells** *Judith Müller, Ludwig-Maximilians-Universität München*
9:00–9:15 AM	**Development of a GMP-Compatible Manufacturing Process for Highly-Edited, Stem-like, Metabolically Fit, Virus Free CRISPR CAR T Cells** *Dan Cappabianca, University of Wisconsin-Madison*
9:15–9:30 AM	**Improving Genome Editing Efficiency and *In Vivo* Potency of CAR T Cells via Lable Free Metabolic Imaging** *Dan Pham, University of Wisconsin-Madison*
9:30–9:45 AM	**Evolution of a Hyperactive TcBuster Transposase Enables Highly Efficient Non-Viral Generation of CAR-NK and CAR-T Cells for Combination Cell Therapies** *Joseph Skeate, PhD, University of Minnesota-Moriarity Lab*

SCHEDULE (ALL TIMES LISTED IN PT)

SATURDAY, MAY 20, 2023

ORAL ABSTRACT SESSION

ROOM 502 AB
Ophthalmic and Auditory Diseases
Co-chairs: Killian Hanlon, PhD, Harvard Medical School and Nerea Zabaleta Lasarte, PhD, Mass Eye and Ear/Harvard Medical School

8:00–8:15 AM	**A Mutation-Independent RNA Replacement Approach Based on a RHO-Targeting Trans-Splicing Ribozyme for the Treatment of Autosomal Dominant Retinitis Pigmentosa (adRP)** *Jihyun Kim, PhD, Rznomics Inc.*
8:15–8:30 AM	**Massively Parallel and Systematic Engineering Platform for Highly Compact, Cell-Type Specific, and Potent Smart Sensor Promoters For Precision Retinal Gene Therapies** *Magdalena Cichewicz, Senti Biosciences, Inc.*
8:30–8:45 AM	**Rescue of *OTOF* Q829X Mutation-Induced Hearing Loss by *In Vivo* Delivery of Mini-dCas13X-Derived RNA Base Editor** *Yuanyuan Xue, HuidaGene Therapeutics Co., Ltd.*
8:45–9:00 AM	**Indirect Comparison of Lenadogene Nolparvovec Gene Therapy Versus Natural History in m.11778mt-ND4 Leber Hereditary Optic Neuropathy Patients** *Thomas Klopstock, University Hospital, LMU*
9:00–9:15 AM	**Development of a Mutation Independent Gene Therapy for Cone Reactivation in the Treatment of Retinitis Pigmentosa** *Hanen Khabou, SparingVision*
9:15–9:30 AM	**Precise Targeting of GJB2 Cells Resulted in Safe and Efficacious Gene Therapy in a Rodent Model of Hearing Loss Due to GJB2 Deficiency** *Gabriela Pregernig, Decibel Therapeutics*
9:30–9:45 AM	**A Novel Stem Cell Therapy for Treating Patients with Retinitis Pigmentosa** *Deepti Singh, Harvard Medical School*

SCHEDULE (ALL TIMES LISTED IN PT)

SATURDAY, MAY 20, 2023

ORAL ABSTRACT SESSION

ROOM 515 AB
Gene Targeting and Gene Correction: CNS
Co-chairs: TJ Cradick, PhD, Excision BioTherapeutics and David Segal, PhD, UC Davis Genome Center

8:00–8:15 AM	**High Efficiency *In Vivo* RNA Editing in the CNS with AAV-Delivered ADAR gRNAs** *Stephen Burleigh, Shape Therapeutics, Inc.*
8:15–8:30 AM	**Targeting CX3CR1 Gene to Improve Microglia Reconstitution And Transgene Delivery Into The CNS Upon Hematopoietic Stem and Progenitor Cell Transplant** *Annita Montepeloso, PhD, Dana-Farber/Boston Children's, Harvard Medical School*
8:30–8:45 AM	**A Synthetic miRNA IFFL Circuit Module for Rett Syndrome Gene Therapy** *Michael Flynn, California Institute of Technology*
8:45–9:00 AM	**CRISPR-SKIP 2.0: a Highly Efficient Base Editing Toolbox for Exon-Skipping That Enables a Therapeutic Approach for Alzheimer's Disease** *Angelo Miskalis, University of Illinois at Urbana-Champaign*
9:00–9:15 AM	**Rewriting *ABCA4* RNA for the Treatment of Stargardt Disease** *Robert Bell, PhD, Ascidian Therapeutics*
9:15–9:30 AM	**Transcriptional Upregulation of α-L-iduronidase Utilizing Small-Activating RNA Following Bone Marrow Transplant in a Murine Model of Hurler Syndrome** *Michael Przybilla, PhD, University of Minnesota*
9:30–9:45 AM	**Epigenome Therapy Approach to Tune *SNCA* Expression in Parkinson's Disease: Pre-Clinical Proof of Concept in PD Mouse Model** *Ornit Chiba Falek, Duke University*

SCHEDULE (ALL TIMES LISTED IN PT)

SATURDAY, MAY 20, 2023

SCIENTIFIC SYMPOSIUM

CONCOURSE HALL 152 & 153

Emerging Opportunities in Cellular Therapies Derived from Stem Cells

Co-chairs: Dan Kaufman, MD, PhD, University of California - San Diego and Agnieszka Czechowicz, MD, PhD, Stanford University School of Medicine

10:16–10:40 AM	**iPSC to HSC** *Elizabeth Ng, PhD, Murdoch Children's Research Institute*
10:40–11:05 AM	**Engineering iPSCs to Generate Therapeutic T Cells** *Gay Crooks, MD, David Geffen School of Medicine at UCLA*
11:05–11:30 AM	**A Combined Stem Cell and Gene Therapy Approach for the Treatment of ALS and Other Neurological Diseases** *Clive Svendsen, PhD, Cedars-Sinai Medical Center*
11:30–11:55 AM	**Basic and Translational Stem Cells to Tendons** *Alice Huang, PhD, Columbia University*

SCHEDULE (ALL TIMES LISTED IN PT)

SATURDAY, MAY 20, 2023

SCIENTIFIC SYMPOSIUM

PETREE HALL C

In Vivo Delivery: Lessons From the World

Co-chairs: Claire Booth, PhD, UCL Great Ormond Street Institute of Child Health and Assumpció Bosch, PhD, Universitat Autònoma de Barcelona

10:16–10:40 AM	**RNA Therapy Targeting Liver Urea Cycle Defects, in Collaboration with Moderna** *Julien Baruteau, MD, PhD, Great Ormond Street Institute of Child Health, University College London*
10:40–11:05 AM	**Non-pathogenic Clostridia as a Vector for Solid Tumors** *Alexandra Mowday, PhD, Maastricht University*
11:05–11:30 AM	***In Vivo* CAR T for B-NHL** *He Huang, Zhejiang University*
11:30–11:55 AM	***In Vivo* CRISPR-LNP for Hemophilia** *Jeong Pil Han, Seoul National University*

SCHEDULE (ALL TIMES LISTED IN PT)

SATURDAY, MAY 20, 2023

SCIENTIFIC SYMPOSIUM

ROOM 502 AB

Strategies to Deliver Genetic Therapies for Cystic Fibrosis

Co-chairs: Bridget Gomperts, MD, UCLA
and John Lueck, PhD, University of Rochester, School of Medicine and Dentistry

10:16–10:35 AM	**Ferrets as Preclinical Model Genetic Therapies Development** *John Engelhardt, PhD, University of Iowa*
10:35–10:55 AM	**Cellular Diversity of the Airway** *Brigitte Gomperts, MD, UCLA*
10:55–11:15 AM	**Nanoparticle-Mediated Delivery of mRNA to Airway Epithelial Cells** *Gaurav Sahay, PhD, Oregon State University*
11:15–11:35 AM	**Polymeric Nanoparticle Strategies to Restore CFTR Function** *Alexandra Piotrowski-Daspit, PhD, University of Michigan*
11:35–11:55 AM	**Strategies to Restore Functional CFTR in Epithelial Cells** *Sriram Vaidyanathan, PhD, Nationwide Children's Hospital*

SCHEDULE (ALL TIMES LISTED IN PT)

SATURDAY, MAY 20, 2023

EDUCATION SESSION

PETREE HALL D

Trending Topics in Gene and Cell Therapies

Co-chairs: Maksim Mamonkin, PhD, Baylor College of Medicine and Satiro DeOliveria, MD, UCLA

10:16–10:50 AM	**Management of CRS, ICANS, and HLH-like Syndromes Following Adaptive T-Cell Therapy** *Nirav Shah, MD, Medical College of Wisconsin*
10:50–11:25 AM	**Pericardium-derived Extracellular Vesicles** *Costanza Emanueli, PhD, Imperial College London National Heart & Lung Institute*
11:25 AM–12:00 PM	**Microglial Vesicles: Role in Preventing Immune Cell Senescence and Myelin Repair** *Marta Fumagalli, PhD, Università degli Studi di Milano*

SCHEDULE (ALL TIMES LISTED IN PT)

SATURDAY, MAY 20, 2023

SCIENTIFIC SYMPOSIUM

ROOM 515 AB

tRNAs: Biology, Pathophysiology, and Potential Therapeutic Applications

Co-chairs: Paloma Giangrande, PhD, Wave Life Sciences and Mark Kay, MD, PhD, Stanford University School of Medicine

10:16–10:40 AM	**Overview of Genomic Arrangements of tRNAs** *Todd Lowe, PhD, University of California, Santa Cruz*
10:40–11:05 AM	**tRNA Derived Small RNAs** *Saumya Das, MD, PhD, Massachusetts General Hospital*
11:05–11:30 AM	**Therapeutic Applications of tRNAs** *Theonie Anastassiadis, Alltrna*
11:30–11:55 AM	**mRNA Spell-checking with tRNA to Read-through Premature Termination Codons** *Christopher Ahern, PhD, University of Iowa*

SCHEDULE (ALL TIMES LISTED IN PT)

SATURDAY, MAY 20, 2023

ORAL ABSTRACT SESSION

CONCOURSE HALL 150 & 151
Gene and Cell Therapy Trials in Progress
Chair: David Steffin, MD, Baylor College of Medicine

10:15–10:30 AM	**Phase 1/2a Trial of Delandistrogene Moxeparvovec in Patients with DMD: 4-Year Update** *Linda Lowes, PhD, Nationwide Children's Hospital*
10:30–10:45 AM	**Autologous Stem Cell Transplantation in HIV Infected Recipients Using Zinc-Finger Nuclease-Based CCR5 Edited CD34+ Cells: Persistence of Hematopoietic Progeny and Stem Cells at 5 Year Follow-Up** *Angelo Cardoso, MD, PhD, City of Hope*
10:45–11:00 AM	**An Updated Follow-Up of BRL-101, CRISPR-Cas9-Mediated Gene Editing of the BCL11A Enhancer for Transfusion-Dependent β-thalassemia** *Biao Zheng, BRL Medicine Inc*
11:00–11:15 AM	**Supraphysiological Enzyme and Early Neurocognitive Outcomes after Autologous Hematopoietic Stem Cell Gene Therapy in Patients with Mucopolysaccharidosis IIIA** *Brian Bigger, PhD, University of Manchester*
11:15–11:30 AM	**Initial Biomarker and Clinical Findings from the CANaspire Canavan Disease Gene Therapy Trial: Exploration of Connections between NAA and Disease Severity** *Florian Eichler, MD, Massachusetts General Hospital*
11:30–11:45 AM	**First in Human RESKUE Phase 1/2 Clinical Trial of Intravenous FBX-101 (AAVrh10.hGALC) Administered after Immune and Myeloablation for Unrelated Umbilical Cord Blood Transplantation Prevented Immune Responses, Increased GALC Activity, Restored Normal Brain Development, and Normalized Motor Function in Patients with Infantile Krabbe Disease** *Maria Escolar, MD, Forge Biologics*
11:45 AM–12:00 PM	**A Phase 1 Dose Escalation Study of GCC19CART - A Novel CoupledCAR Therapy for Subjects with Metastatic Colorectal Cancer** *Lei Xiao, Innovative Cellular Therapeutics Inc.*


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