
ANNUAL MEETING
27TH
2024
BALTIMORE, MD
PROGRAM GUIDE
MAY 7-11

Pioneering innovative science to transform patients' lives

A proud sponsor of the ASGCT 27th Annual Meeting

biogen.com

Biogen

POLICY SUMMIT

American Society of Gene + Cell Therapy

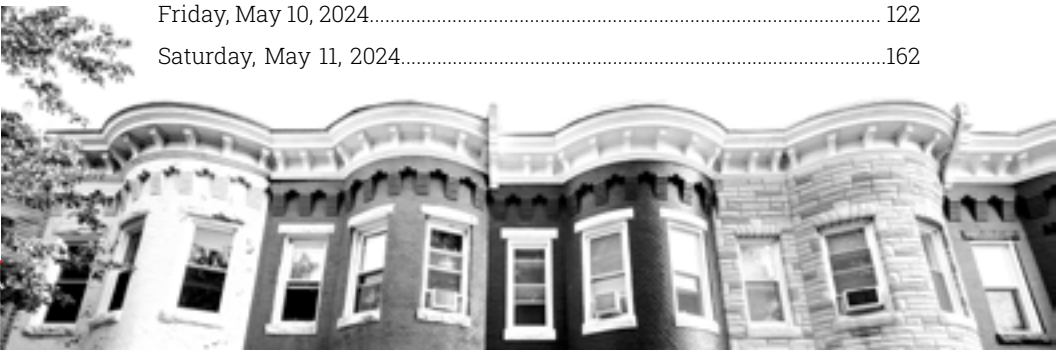
SAVE THE DATE

SEPTEMBER 23 + 24, 2024

The Westin Washington, DC Downtown

asgct.org/PolicySummit

Table of Contents



A WELCOME FROM JEFFREY S. CHAMBERLAIN, PHD

Dear Colleagues,

On behalf of the American Society of Gene & Cell Therapy (ASGCT), I'd like to welcome you all to our 27th Annual Meeting! I'm really excited to be together again, this time in Baltimore, MD, for more than four days of learning from the latest research and discoveries in gene and cell therapy!

Whether you're gathering with us in person in Baltimore or virtually, I hope you're looking forward to meeting new colleagues and reconnecting with old ones this week. 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 Kathy High, MD, who we'll hear from on Tuesday, the first day of the meeting. On Wednesday morning, I'm also excited to welcome Kevin Campbell, PhD, and Charles Murry, MD, PhD, who will speak during the Presidential Symposium. Finally, on Thursday morning, Beverly Davidson, PhD, and Philip Gregory, DPhil, will present the George Stamatoyannopoulos Memorial Lectures. I'd like to congratulate all the other Annual Meeting award winners: David Liu, PhD, recipient of the Outstanding Achievement Award; Agnieszka Czechowicz, MD, PhD; Pietro Genovese, PhD; Jennifer Hamilton, PhD; and Sharif Tabebordbar, PhD, recipients of the Outstanding New Investigator Award; John Tisdale, MD, recipient of the Jerry Mendell Award for Translational Science; Pat Furlong, recipient of the Sonia Skarlatos Public Service Award; and Maritza McIntyre, PhD, recipient of the Catalyst Award; Cindy Dunbar, MD, recipient of the George Stamatoyannopoulos Mentorship Award; and Roland Herzog, PhD, recipient of the Exemplary Service 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 hold this meeting without all of you and I'm grateful for your commitment. Thank you for supporting ASGCT and enjoy the meeting.

Sincerely,

Jeffrey S. Chamberlain, PhD

President, ASGCT

GENERAL MEETING INFORMATION

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 27th Annual Meeting have been published in the May supplement of *Molecular Therapy* and are searchable for free on the Annual Meeting platform.

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.

Education Methods + Materials

Enhance your expertise and connect with leading professionals at the forefront of gene and cell therapy at ASGCT's 27th Annual Meeting through thoughtfully curated experiences such as Workshops, General Sessions, Scientific Symposia, Education Sessions, Oral Abstract Sessions, Networking Opportunities, Sponsored Symposia, and Tools and Technology Forums.

GENERAL MEETING INFORMATION

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 and Feedback

ASGCT's goal is to create the best possible education, and your thoughts, ideas, and suggestions play a major role in shaping the future of ASGCT's education. We strongly encourage all attendees to fill out the short 3-question evaluation at the end of each session in the meeting app, as well as the evaluation you will receive 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.

GENERAL MEETING INFORMATION

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 27th Annual Meeting will be a hybrid event. The in-person portion will be held at the Baltimore Convention Center in Baltimore, MD. 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 7, 2024 and continue through 12 p.m. (ET) on Saturday, May 11, 2024. The Exhibit Hall will be open on Tuesday, May 7, from 5:30 p.m. to 7 p.m., and from Wednesday, May 8 through Friday, May 10, from 12 p.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 and Video Recording

ASGCT contracts with photographers and videographers throughout the Annual Meeting to assist in the creation of future marketing materials. By attending the 27th 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."

GENERAL MEETING INFORMATION

REGISTRATION

Located in the Pratt Street Lobby, Level 300

Monday, May 6 | **5–7 p.m.**

Tuesday, May 7 | **7 a.m.–7 p.m.**

Wednesday, May 8 | **7 a.m.–6 p.m.**

Thursday, May 9 | **7 a.m.–6 p.m.**

Friday, May 10 | **7 a.m.–6 p.m.**

Saturday, May 11 | **7–11 a.m.**

VISIT THE EXHIBIT HALL

Located in Hall C-G & Swing Hall, Level 100

Tuesday, May 7 | **5:30–7 p.m.**

Wednesday, May 8 | **12–7 p.m.**

Thursday, May 9 | **12–7 p.m.**

Friday, May 10 | **12–7 p.m.**

For more information, view the exhibitor listing.

THE EXHIBIT HALL PRESENTATION THEATER IS LOCATED IN SWING HALL

ABSTRACT POSTERS

Located in the Exhibit Hall (Hall C-G & Swing Hall, Level 100)

Wednesday, May 8 | **12–7 p.m.**

Thursday, May 9 | **12–7 p.m.**

Friday, May 10 | **12–7 p.m.**

PROGRAM COMMITTEE + ABSTRACT PLANNING COMMITTEE

2024 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 27th Annual Meeting.

Chair

Jeffrey S. Chamberlain, PhD, University of Washington

Members

Chiara Bonini, MD, Università Vita Salute San Raffaele

Hildegard Büning, PhD, Hannover Medical School

Barry J. Byrne, MD, PhD, University of Florida

Paula M. Cannon, PhD, University of Southern California

Terence R. Flotte, MD, UMass Chan Medical School

Lindsey A. George, MD, UPenn

Philip D. Gregory, DPhil, Regeneron

Roland W. Herzog, PhD, IU School of Medicine

Helen E. Heslop, MD, Baylor College of Medicine

Maritza C. McIntyre, PhD, Advanced Therapies Partners

Carol H. Miao, PhD, Seattle Children's Research Institute

Federico Mingozzi, PhD

Isabelle Rivière, PhD, Takeda

Rayne H. Rouce, MD, Baylor College of Medicine

John Tisdale, MD, National Heart, Lung, and Blood Institute (NHLBI)

PROGRAM COMMITTEE + ABSTRACT PLANNING COMMITTEE

2024 ABSTRACT PLANNING COMMITTEE

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

President
Jeffrey S. Chamberlain, PhD, University of Washington

ASGCT President Elect
Paula M. Cannon, PhD, University of Southern California

ASGCT Secretary + Abstract Chair
Isabelle Rivière, PhD, Takeda

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

Lindsey George, MD, UPenn

Helen E. Heslop, MD, Baylor College of Medicine

Frederico Mingozzi, PhD

John Gray, PhD

SPONSORS

THANK YOU to our 27th Annual Meeting sponsors!

Diamond

ANEMOCYTE

Manufacturing Biotech Innovation

Kriya

Platinum

biotechne®

cytiva

FUJIFILM

Diosynth biotechnologies

SPONSORS

THANK YOU to our 27th Annual Meeting sponsors!

Gold

DYNO THERAPEUTICS

CITELINE a norstella company

Lilly

MYRTELLE

Oxford Biomedica

Pfizer

RESILIENCE

sanofi

SAREPTA THERAPEUTICS

SIREN BIOTECHNOLOGY

Spark THERAPEUTICS

uniQure

VERTEX THE SCIENCE of POSSIBILITY

VIRALGEN

Silver

CAPSIDA BIOTHERAPEUTICS

Catalent CELL & GENE THERAPY

Fortrea

FORGE BIOLOGICS

GINKGO BIOWORKS

Mass General Brigham Gene and Cell Therapy Institute

MaxCyte

MilliporeSigma

parexel

AWARD RECIPIENTS

CAREER DEVELOPMENT AWARD

Pradip Bajgain, PhD
Center for Cancer Research, National Cancer Institute

Gabriele Casirati, MD, PhD
Boston Children's Hospital

Leonid Cherkassky, MD
Roswell Park Comprehensive Cancer Center

Ashley Cooney, PhD
University of Iowa

Tomas Gonzalez-Fernandez, PhD
Lehigh University

Denise Klatt, PhD
Boston Children's Hospital

Min-Sun Song, PhD
Beckman Research Institute, City of Hope

Wenlin Zhang, MD, PhD
Stein Eye Institute, UCLA

This year, ASGCT has partnered with the Cystic Fibrosis Foundation (CF Foundation) & the Children's Tumor Foundation (CTF) to award six additional Career Development Awards. The Cystic Fibrosis Foundation is the world's leader in the search for a cure for CF and supports a broad range of research initiatives to tackle the disease from all angles. The Children's Tumor Foundation's mission is to drive research, expand knowledge, and advance care for the NF community.

ASGCT-CF FOUNDATION AWARDEES

Aysegul Atasoy-Zeybek, PhD
Mayo Clinic

Sandhiya Ravi, PhD
UMass Chan Medical School

Di Yin, PhD
Stanford University

AWARD RECIPIENTS

ASGCT-CTF AWARDEES

Lukas Landegger, MD, PhD
Stanford University

Chance Meers, PhD
Columbia University Irving Medical Center

Ngoc Tam Le Tran, PhD
UMass Chan Medical School

For more information on these organizations, please visit https://www.cff.org/ and https://www.ctf.org/.

RESEARCH ON CONDITIONS AFFECTING MINORITIES AWARD

Jose Martinez-Navio, PhD
UMiami Miller School of Medicine

UNDERREPRESENTED POPULATION FELLOWSHIP IN GENE AND CELL THERAPY – ONCOLOGY

Erik Kimble, MD
Fred Hutchinson Cancer Center

MAVIS AGBANDJE-MCKENNA SCHOLARSHIP

Esther Alao
UT Southwestern Medical Center

AWARD RECIPIENTS

OUTSTANDING ACHIEVEMENT AWARD

David Liu, PhD
Broad Institute of MIT and Harvard

OUTSTANDING NEW INVESTIGATOR AWARD

Agnieszka Czechowicz, MD, PhD
Stanford University

Pietro Genovese, PhD
Dana-Farber Cancer Institute + Harvard Medical School

Jennifer Hamilton, PhD
Azalea Therapeutics

Sharif Tabebordbar, PhD
Kate Therapeutics

EXCELLENCE IN RESEARCH AWARD

Christian McRoberts Amador, Duke University
Elena Barbon, PhD, SR-Tiget
Xiupeng Chen, PhD, UMass Chan Medical School
Su Jin Choi, PhD, Duke University Medical Center
Sébastien Levesque, PhD, Boston Children's Hospital
Hao Liu, PhD, Horae Gene Therapy Center, UMass Chan Medical School
Sandeep Mangrati, University College London
Kamran Miah, PhD, University of Oxford
Daisuke Morita, MD, PhD, Baylor College of Medicine
Suk Namkung, Horae Gene Therapy Center, UMass Chan Medical School
Aadit Shah, Stanford University School of Medicine
Jakob Shoti, University of Florida
Georgios Stergiopoulos, MD, Mayo Clinic
Fang Wan, UMass Chan Medical School

AWARD RECIPIENTS

SONIA SKARLATOS PUBLIC SERVICE AWARD

Pat Furlong
Parent Project Muscular Dystrophy (PPMD)

JERRY MENDELL AWARD FOR TRANSLATIONAL SCIENCE

John Tisdale, MD
National Heart, Lung, and Blood Institute (NHLBI)

CATALYST AWARD

Maritza McIntyre, PhD
Advanced Therapies Partners LLC

FOUNDERS AWARD

Kathy High, MD
Rockefeller University

EXEMPLARY SERVICE AWARD

Roland Herzog, PhD
Indiana University School of Medicine

AWARD FOR EXCELLENCE IN ADVANCING DIVERSITY, EQUITY, AND INCLUSION

Innovative Genomics Institute (IGI)

GEORGE STAMATOYANNOPOULOS MENTORSHIP AWARD

Cynthia "Cindy" Dunbar, MD
National Institute of Health (NIH)

NETWORKING OPPORTUNITIES

TUESDAY, MAY 07

5:30–7:00 PM
Welcome Reception
Exhibit Hall

WEDNESDAY, MAY 08

9:15–10:15 AM
Career Fair
Camden Lobby (Level 300)

9:45–10:15 AM
Coffee Break
Charles Street Lobby

3:15–3:45 PM
Exhibit Hall Coffee Break
Exhibit Hall

3:15–4:15 PM
Career Fair
Camden Lobby (Level 300)

5:30–7:00 PM
Poster Reception
Exhibit Hall
Wednesday Posters 436 – 926 and Late-Breaking 15 - 25

THURSDAY, MAY 09

9:15–10:15 AM
Career Fair
Camden Lobby (Level 300)

9:45–10:15 AM
Coffee Break
Charles Street Lobby

3:15–3:45 PM
Exhibit Hall Coffee Break
Exhibit Hall

3:15–4:15 PM
Career Fair
Camden Lobby (Level 300)

NETWORKING OPPORTUNITIES

THURSDAY, MAY 09

5:30–7:00 PM
Poster Reception
Exhibit Hall
Thursday Posters 927 – 1418 and Late-Breaking 26 – 36

6:30–8:00 PM
Women in Gene and Cell Therapy Reception
Outdoor Terrace

FRIDAY, MAY 10

7:00–8:00 AM
Global Gathering Breakfast
Room 307-308

9:45–10:15 AM
Coffee Break
Charles Street Lobby

12:00–1:00 PM
Meet the Editors of The Molecular Therapy Family of Journals
ASGCT Central - Booth #815

3:15–3:45 PM
Exhibit Hall Coffee Break
Exhibit Hall

5:30–7:00 PM
Poster Reception
Exhibit Hall
Friday Posters 1419 – 1908

6:00–7:00 PM
Committee Reception
Outdoor Terrace

8:00–11:00 PM
Closing Night Reception
Power Plant Live!
This event can be added to your current registration for an additional fee. Update your online registration or stop by the Registration Desk for assistance.

SATURDAY, MAY 11

7:00–8:00 AM
Society Business Meeting
Room 337-338

9:45–10:15 AM
Coffee Break
Levels 300 + 400


American Society of Gene + Cell Therapy
An ASGCT Hybrid Conference
Advancing Gene + Cell Therapies for Cancer
October 16-17, 2024 | Philadelphia + Online
asgct.org/events

New in 2024


American Society of Gene + Cell Therapy
An ASGCT Hybrid Conference
BREAKTHROUGHS IN
MUSCULAR DYSTROPHY
NOVEMBER 19 + 20, 2024 | CHICAGO + ONLINE
asgct.org/events

We're bringing cures from bench to bedside to help patients like Mia.

BEST CHILDREN'S HOSPITALS
U.S.News & WORLD REPORT
CANCER
2023-2024

Our open trials include:

- αβ T-cell depleted haploidentical stem cell transplantation and solid organ transplantation for Schimke immuno-osseous dysplasia (SIOD), cystinosis, and focal segmental glomerulosclerosis (FSGS)
- αβ+T-cell/CD19+ B-cell depleted haploidentical stem cell transplant in children and young adults affected by malignant or non-malignant hematological disorders
- Gene therapy for sickle cell disease, PKD, and IPEX syndrome
- CD19/22 CAR T-cell therapy for children and young adults with B-cell malignancies
- GD2 CAR T-cell therapy for brain and spine tumors
- Tr1 cells to suppress allogenic responses in stem cell transplantation
- TCRab+ T-cell/CD19+ B-cell depleted hematopoietic grafts in combination with JSP191 to treat Fanconi anemia

To learn more, call **(650) 497-8953** or visit **basscenter.stanfordchildrens.org**.

Stanford MEDICINE
Children's Health

TUESDAY, MAY 07

WORKSHOP*

Delivery & Development of Precision Genome Editing Technologies

Co-chairs: Holly Rees, PhD, Beam Therapeutics and
Julian Grünewald, MD, PhD, Technical University of Munich

BALLROOM 1

8:00–8:05 AM — **OPENING REMARKS**

8:05–8:25 AM — ***In Vivo* Gene Editing in Porcine Models of Heart Disease, also in Comparison to "Classic" AAV Gene Therapy**
Christian Kupatt, MD, TUM

8:25–8:45 AM — **Leveraging on the LNP/mRNA Technology for *Ex-Vivo* Gene Editing**
Samuele Ferrari, PhD, SR-Tiget

8:45–9:05 AM — **Next Generation Epigenetic Editing**
Y. Esther Tak, PhD, Massachusetts General Hospital

9:05–9:25 AM — ***In Vivo* Genome Editing Agent Delivery, Particularly Virus-Like Particles**
Samagya Banskota, PhD, Broad Institute

9:25–9:45 AM — **LNP Development and Screening**
James Dahlman, PhD, Georgia Tech

9:45–10:05 AM — **Development and Delivery of Prime Editors**
Jonathan Levy, PhD, Prime Medicine

10:05–10:25 AM — **BREAK**

10:25–10:45 AM — ***In Vivo* AAV Delivery for Genome Editing**
Amy Wagers, PhD, Harvard University

TUESDAY, MAY 07

WORKSHOP (CONTINUED)

Delivery & Development of Precision Genome Editing Technologies

Co-chairs: Holly Rees, PhD, Beam Therapeutics and
Julian Grünewald, MD, PhD, Technical University of Munich

BALLROOM 1

10:45–11:05 AM
Fanzor is a Eukaryotic Programmable RNA-guided Endonuclease
Makoto Saito, MIT/Broad Institute

11:05–11:25 AM
Programmable Multi-kilobase RNA Editing Using CRISPR-mediated Trans-splicing

11:25–11:45 AM
Computational Design of Sequence-specific DNA-binding Proteins
Cameron Glasscock, PhD, University of Washington

11:45–11:50 AM
CLOSING REMARKS

* Workshops can be added to your current registration for an additional fee. Update your online registration or stop by the Registration Desk for assistance.

TUESDAY, MAY 07

WORKSHOP*

Advancements in Immune Effector Cell Therapies: Innovations and Future Prospects

Co-chairs: Aude Chapuis, MD, Fred Hutchinson Cancer Center and Justin Eyquem, PhD, UCSF

BALLROOM 2

8:00–8:05 AM — **OPENING REMARKS**

8:05–8:29 AM — **Unlocking the Full Potential of T cells with CRISPR Screening**
Julia Carnevale, MD, UCSF

8:29–8:53 AM — **iPSC-derived CAR T Cells**
Sjoukje van Der Stegen, PhD, Memorial Sloan Kettering Cancer Center

8:53–9:17 AM — **Pooled CRISPR Knockin Screens: Reprogramming Therapeutic T Cells**
Franziska Blaeschke, MD, PhD, German Cancer Research Center (DKFZ)

9:17–9:41 AM — **GD2 CAR Clinical Trial**
Francesca Del Bufalo, MD, PhD, Bambino Gesù Chidren's Hospital

9:41–10:05 AM — **T-cell Signaling Biology Inspires Unique CAR T-cell Engineering**
Robbie Majzner, MD, Dana Farber Cancer Institute

10:05–10:20 AM — **BREAK**

10:20–10:44 AM — **Targeting Aberrant Glycosylation in Tumors**
Avery Posey, PhD, UPenn

10:44–11:08 AM — **Synthetic Biology Beyond Logic Gauging-T Leukemia Genes**
Kole Roybal, PhD, UCSF

TUESDAY, MAY 07

WORKSHOP (CONTINUED)

Advancements in Immune Effector Cell Therapies: Innovations and Future Prospects

Co-chairs: Aude Chapuis, MD, Fred Hutchinson Cancer Center and Justin Eyquem, PhD, UCSF

BALLROOM 2

11:08–11:32 AM

Augmenting CAR T Cell Fitness Through Memory Reprogramming
Evan Weber, PhD, Children's Hospital of Philadelphia

11:32–11:56 AM

TCR Gene Therapy Targeting Recurrently Mutated "Public" Neoantigens
Christopher Klebanoff, MD, Memorial Sloan Kettering Cancer Center

11:56 AM–12:00 PM

CLOSING REMARKS

* Workshops can be added to your current registration for an additional fee. Update your online registration or stop by the Registration Desk for assistance.

TUESDAY, MAY 07

WORKSHOP*

AAV Manufacturing for CMC Success: Translating Preclinical Gene Therapy Products to Clinical Success

Co-chairs: Adam Cockrell, PhD, Solid Biosciences and
Nathalie Clement, PhD, Siren Biotechnology

BALLROOM 3

8:00–8:05 AM
OPENING REMARKS

8:05–8:25 AM
AAV Manufacturing Now and Then: The Role of Academia in Shaping Today's Industry Platforms
Johannes Van Der Loo, PhD, Children's Hospital of Philadelphia

8:25–8:45 AM
Preparing for CMC Success: How Early CMC Decision Drives Your Pace and Success to the Clinic
David Desmuke, PhD, Forge Biologics

8:45–9:05 AM
How to Design Phase-Appropriate Successful IND-Enabling Tox Studies for an AAV DP
Kirstin Coleman, University of Florida

9:05–9:40 AM
Panel Discussion
Johannes Van Der Loo, PhD
David Desmuke, PhD
Kirstin Coleman

9:40–10:00 AM
BREAK

10:00–10:20 AM
CMC Considerations for Adeno-Associated Virus (AAV)-Vector Based Gene Therapies – A CBER Perspective
Bo Liang, PhD, FDA CBER OTAT

10:20–10:40 AM
Successful Manufacturing Platforms and Strategies to Support Clinical Development Up To BLA Filing
Matthias Hebben, PhD, Complement Therapeutics

TUESDAY, MAY 07

WORKSHOP (CONTINUED)

AAV Manufacturing for CMC Success: Translating Preclinical Gene Therapy Products to Clinical Success

Co-chairs: Adam Cockrell, PhD, Solid Biosciences and
Nathalie Clement, PhD, Siren Biotechnology

BALLROOM 3

10:40–11:00 AM

The Future: Moving Toxicology Studies from the Animal to *In Vitro* Analysis
Lauren Kelly, Siren Biotechnology

11:00–11:20 AM

Using A.I. to Shape Early Design of the Drug Product and the Manufacturing Process
Claire Aldridge, PhD

11:20–11:55 AM

Panel Discussion
Bo Liang, PhD
Claire Aldridge, PhD
Lauren Kelly
Matthias Hebben, PhD

11:55 AM–12:00 PM

CLOSING REMARKS

* Workshops can be added to your current registration for an additional fee. Update your online registration or stop by the Registration Desk for assistance.

TUESDAY, MAY 07

WORKSHOP*

Creating an Analytical Roadmap for Gene & Cell Therapies

Co-chairs: Daniel Leblanc, Ensoma and John P. Tomtishen, Cellares

BALLROOM 4

8:00–8:25 AM

Developing Your Control Strategy for Genetically-modified Cell Therapies - Navigating the Youthful River
Sunetra Biswas, PhD, Kyverna Therapeutics

8:25–8:50 AM

Understanding the Differences Between Release Testing and Characterization Assays
Sam Mallonee, BioMarin Pharmaceuticals

8:50–9:15 AM

Next Gen Sequencing for Testing and Characterization: Exploring the Transition Away from *In Vivo* Methods
Dwight Baker, Resilience

9:15–9:45 AM

Panel Discussion
Sunetra Biswas, PhD
Sam Mallonee
Dwight Baker

10:15–10:40 AM

FDA Programs to Advance Personalized Medicine: Advanced Manufacturing Technologies Designation Program
Ingrid Markovic, PhD, FDA

10:40–11:05 AM

The Role of AI in Analytics and Data Collection
David Del Bourgo, WhiteLab Genomics

11:05–11:30 AM

Adaptive T Cell Processing Through Integrated Process Analytical Technologies
Ivie Aifuwa, PhD, Bristol Myers Squibb

TUESDAY, MAY 07

WORKSHOP (CONTINUED)

Creating an Analytical Roadmap for Gene & Cell Therapies

Co-chairs: Daniel Leblanc, Ensoma and John P. Tomtishen, Cellares

BALLROOM 4

11:30 AM–12:00 PM

Panel Discussion
Ingrid Markovic, PhD
David Del Bourgo
Ivie Aifuwa, PhD

* Workshops can be added to your current registration for an additional fee. Update your online registration or stop by the Registration Desk for assistance.

TUESDAY, MAY 07

WORKSHOP*

Why Do Rare Disease Clinical Trials Often Miss the Mark? Best Practices and Approaches to Optimize Design of Rare Disease Clinical Trials

Co-chairs: Joseph Hacia, PhD, University of Southern California and Samuel Hughes, St. Jude Children's Research Hospital

ROOM 307-308

8:05–8:30 AM

Clinical Trial Enrollment - Approaches to Optimize Patient Recruitment and Develop Patient Registries
Nancy Braverman, MD, McGill University

8:30–8:55 AM

Clinical Trial Enrollment - Strategies to Address Barriers in Recruiting
Maria Santaella, PhD, National Bleeding Disorders Foundation

8:55–9:15 AM

Panel Discussion
Maria Santaella, PhD
Nancy Braverman, MD

9:30–9:55 AM

Clinical Trial Endpoints - Approaches to Optimize Natural History Studies and Engage Patient Advocacy Groups
Erika Augustine, MD, Kennedy Krieger Institute

9:55–10:25 AM

Clinical Trial Endpoints - Strategies to Maximize Clinically Relevant and Patient Driven Outcomes
Audrey Thurm, PhD, NIMH and Cristan Farmer, PhD, NIMH

10:25–10:45 AM

Panel Discussion
Audrey Thurm, PhD
Cristan Farmer, PhD
Erika Augustine, MD

TUESDAY, MAY 07

WORKSHOP (CONTINUED)

Why Do Rare Disease Clinical Trials Often Miss the Mark? Best Practices and Approaches to Optimize Design of Rare Disease Clinical Trials

Co-chairs: Joseph Hacia, PhD, University of Southern California and Samuel Hughes, St. Jude Children's Research Hospital

ROOM 307-308

11:00–11:30 AM

Clinical Trials in Rare Diseases - Strategies for Resource Management
Souad Messahel, PhD, Elpida Therapeutics

11:30 AM–12:00 PM

Panel Discussion
Audrey Thurm, PhD
Erika Augustine, MD
Maria Santaella, PhD
Nancy Braverman, MD
Souad Messahel, PhD

This workshop is co-hosted with NORD.

NORD® National Organization for Rare Disorders

* Workshops can be added to your current registration for an additional fee. Update your online registration or stop by the Registration Desk for assistance.

TUESDAY, MAY 07

WORKSHOP*

Revolutionizing Care: Gene Therapy Unveiled for Clinicians

Eligible for CME (In-person or Livestream Only)

Chair: Kimberly Goodspeed, MD, UT Southwestern Medical Center

ROOM 309-310

8:00–8:35 AM
A Gene Therapy Overview
Phillip Tai, PhD, UMass Chan Medical School

8:35–9:10 AM
Clinical Trial Considerations in AAV Gene Therapy
Benjamin Samelson-Jones, MD, PhD, Children's Hospital of Philadelphia

9:10–9:45 AM
Delivery After Approval
Susan Matesanz, MD, Children's Hospital of Philadelphia

9:45–10:15 AM
BREAK

10:15–10:50 AM
Systemic Toxicity and AAV
Barry Byrne, MD, PhD, University of Florida

10:50–11:25 AM
Adeno-Associated Virus Vectors for Central Nervous System Gene Therapy
Juliette Hordeaux, DVM, PhD, UPenn

11:25 AM–12:00 PM
Transgene Immune Responses
Julie Crudele, PhD, University of Washington

Accreditation Council for Continuing Medical Education through the joint providership of the UMass Chan Medical School and ASGCT. The UMass Chan Medical School is accredited by the Accreditation Council for Continuing Medical Education (ACCME) to provide continuing medical education for physicians.

The UMass Chan Medical School designates this live activity for a maximum of 4 *AMA PRA Category 1 Credit(s)*™. Physicians should claim only credit commensurate with the extent of their participation in the activity. Jointly provided by the University of Massachusetts Chan Medical School and ASGCT.

UMass Chan MEDICAL SCHOOL | Office of Continuing Medical Education

* Workshops can be added to your current registration for an additional fee. Update your online registration or stop by the Registration Desk for assistance.

TUESDAY, MAY 07

ORAL ABSTRACT SESSION

New Technologies for Gene Targeting and Gene Correction

Co-chairs: David Segal, PhD, UC Davis Genome Center and
Samuele Ferrari, PhD, SR-Tiget

BALLROOM 1

1:30–1:45 PM | **11**

Efficacy and Integration of a Non-Viral ABCA4 Transposon in Treating Stargardt Disease: Evidence from Mice and Primate Studies
Michelle LeBlanc, PhD, SalioGen Therapeutics

1:45–2:00 PM | **12**

RNA-Based Gene Writer and Lipid Nanoparticle (LNP) Delivery Enables Generation of Functional Chimeric Antigen Receptor (CAR) T Cells with *In Vitro* and *In Vivo* Anti-Tumor Activity and T Cell-Specific Genome Engineering *In Vivo*
Kartika Venugopal, Tessera Therapeutics

2:00–2:15 PM | **13**

Language Models Generate Novel Genome Editors from Scratch
Peter Cameron, PhD, Profluent Bio

2:15–2:30 PM | **14**

Retron Mediated Exon-Sized Genome Insertion Using an All- RNA System
Inna Shcherbakova, PhD, ReNAgade Therapeutics

2:30–2:45 PM | **15**

Reduction in Triglycerides through a Novel Ultracompact CRISPR System: Efficacy in Mouse Models and NHP Studies
Lucas Harrington, Mammoth Biosciences

2:45–3:00 PM | **16**

Selective Repression of C9ORF72 Repeat Expansion-Containing Sense and Antisense Transcripts in an ALS Mouse Model
Claudia Huichalaf, PhD, Alexion AstraZeneca Rare Disease

3:00–3:15 PM | **17**

Systematic Discovery, *In Vivo* Delivery, and DNA Repair Mechanism of Single-Strand Annealing Protein for Precision Integration of Large DNA Sequences
Le Cong, PhD, Stanford University

TUESDAY, MAY 07

ORAL ABSTRACT SESSION

AAV Vectors - Preclinical and Proof-of-Concept: Therapy Focus

Co-chairs: Nicolas Wein, PhD, Center for Gene Therapy - Nationwide Children's Hospital and Nicole Paulk, PhD, Siren Biotechnology

BALLROOM 2

1:30–1:45 PM | **18**

Breaking Ground in CMT1B Treatment: AAV9-Mediated Dual RNAi and Gene Replacement Therapy Targeting Schwann Cells Improves Myelination and Peripheral Nerve Function in Mice
Daniella Munezero, PhD, Nationwide Children's Hospital

1:45–2:00 PM | **19**

GABA Selective AAV-Mediated Gene Therapy Provides Durable Seizure Protection in Multiple Refractory Epilepsy Models
Brooke Babineau, PhD, Encoded Therapeutics

2:00–2:15 PM | **20**

Curable Hyperactive Behaviors and Serum Biomarkers upon Gene Replacement Therapy in Succinic Semialdehyde Dehydrogenase Deficiency in Mice
Henry Lee, PhD, Boston Children's Hospital

2:15–2:30 PM | **21**

From Darkness to Light: CIB2 Gene Delivery Rescues Age-Related Macular Degeneration-Like Phenotype in a Preclinical Model
Sehar Riaz, University of Maryland, School of Medicine

2:30–2:45 PM | **22**

Prevention of High-Risk Allogeneic Corneal Transplant Rejection by AAV-Mediated Transfer of Novel Anti-Inflammatory Immunomodulator
Tomoko Hasegawa, MD, PhD, University of North Carolina at Chapel Hill

2:45–3:00 PM | **23**

Neonatal AAV9 Gene Therapy Prevents Hepatic Mitochondrial Dysfunction in a Mouse Model of DGUOK Deficiency
Nandaki Keshavan, PhD, University College London

TUESDAY, MAY 07

ORAL ABSTRACT SESSION (CONTINUED)

AAV Vectors - Preclinical and Proof-of-Concept: Therapy Focus

Co-chairs: Nicolas Wein, PhD, Center for Gene Therapy - Nationwide Children's Hospital and Nicole Paulk, PhD, Siren Biotechnology

BALLROOM 2

3:00–3:15 PM | **24**

AAV9 Delivered Artificial microRNAs Effectively Improves GFAP Pathology and Motor Impairment in an Alexander Disease Rat Model

Wassamon Boonying, PhD, UMass Chan Medical School

TUESDAY, MAY 07

ORAL ABSTRACT SESSION

AAV Manufacturing I

Co-chairs: Francesca Vitelli, PhD, Intellia Therapeutics and
Scott Harper, PhD, The Ohio State University & Nationwide Children's Hospital

BALLROOM 3

1:30–1:45 PM | **25**

Production of Different AAV Serotypes Using a Novel Plant-Based Viral Vector Manufacturing Platform
Pranav Mathur, DVM, PhD, Cirsium Biosciences

1:45–2:00 PM | **26**

A Novel Proviral Plasmid Reduces Cross-Packaging and ITR Promoter Activity in AAV Vector Preparations
Pranali Mistry, Nationwide Children's Hospital

2:00–2:15 PM | **27**

Removal of Empty and Partial AAV Capsids Using Cu Ions and Oversaturated Loading on Multicolumn AEX Chromatography
Ohnmar Khanal, PhD, Spark Therapeutics

2:15–2:30 PM | **28**

Novel Mechanism to Increase rAAV Yield through Blocking rAAV Transduction of the Manufacturing Hek293 Cells During rAAV Production
Xiaofei E, PhD, Solid Biosciences

2:30–2:45 PM | **29**

Enhancing Product Quality: Utilizing Salt-Tolerant Anion Exchange Chromatography in Flow-Through Mode as a Polishing Step for Advanced Process-Related Impurity Removal
Johanna Weizenegger, Ascend

2:45–3:00 PM | **30**

Optimizing Affinity Chromatography Purification for Enhanced Quality and Stability of Adeno-Associated Viral Vectors (rAAVv)
Thuy Linh Do, Roche

3:00–3:15 PM | **31**

Not Created Equal: Impact of Deamidation on Capsid Heterogeneity and Anion Exchange Chromatography (AEX) Performance
Alex Meola, Oxford Biomedica (US) LLC

TUESDAY, MAY 07

ORAL ABSTRACT SESSION

Neurologic Diseases I

Co-chairs: Patricia Musolino, MD, PhD, Massachusetts General Hospital and Steven Gray, PhD, UT Southwestern

BALLROOM 4

1:30–1:45 PM | **32**

Promoter Influences Acute Liver Toxicity and Long-Term Hepatic Genotoxicity in rAAV SMA Gene Therapy in Mice
Xiupeng Chen, PhD, UMass Chan Medical School

1:45–2:00 PM | **33**

Intra-CSF AAV9-GBA Delivery Produces Species and Route of Administration Differences in Safety and Efficacy
Kelly Glajch, PhD, Biogen

2:00–2:15 PM | **34**

AAV1.NT-3 Gene Therapy Improves Function, Electrophysiology, and Histopathology in Sh3tc2$^{-/-}$Mouse, a CMT4C Model
Burcak Ozes, PhD, Abigail Wexner Research Institute, Nationwide Children's Hospital, Columbus

2:15–2:30 PM | **35**

AAV9-rMOCS1 Gene Therapy, a Bicistronic Gene Delivery, Fully Rescues Neonatally Lethal Mouse Model of Molybdenum Cofactor Deficiency Type A
Claire Shamber, Massachusetts General Hospital

2:30–2:45 PM | **36**

Developing an AAV-Based Gene Replacement Therapy for Mitochondrial Alanyl-tRNA Synthetase 2 (AARS2) Leukodystrophy
Ruxiao Xing, UMass Chan Medical School

2:45–3:00 PM | **37**

Extracellular Vesicles as the Underlying Mechanism for Cross-Correction with rAAV-Based Gene Therapy for GM3 Synthase Deficiency
Sophia Liu, UMass Chan Medical School

TUESDAY, MAY 07

ORAL ABSTRACT SESSION (CONTINUED)

Neurologic Diseases I

Co-chairs: Patricia Musolino, MD, PhD, Massachusetts General Hospital and Steven Gray, PhD, UT Southwestern

BALLROOM 4

3:00–3:15 PM **38**

AAV Gene Therapy Corrects Neurological Phenotypes with Clinically Relevant Doses in a Mouse Model of STXBP1-Related Developmental and Epileptic Encephalopathy

Wu Chen, PhD, Baylor College of Medicine

TUESDAY, MAY 07

ORAL ABSTRACT SESSION

Genetically Modified Immune Cells for Malignant and Non-Malignant Diseases

Co-chairs: Donald Kohn, MD, UCLA and Tiffany Chen, PhD, GentiBio

ROOM 307-308

1:30–1:45 PM 39
An Allogeneic Engineered Tissue Treg Approach for the Treatment of Acute Ischemic and Inflammatory Diseases
Payam Zarin, PhD, GentiBio

1:45–2:00 PM 40
Myelin-Specific Engineered Treg Exhibit Therapeutic Benefit in a Murine Model of Multiple Sclerosis
Travis Drow, Seattle Children's Research Institute

2:00–2:15 PM 41
Development of Chimeric Antigen Receptor Macrophages Targeting Amyloid-β in Alzheimer's Disease
Mara Davis, UPenn

2:15–2:30 PM 42
Base Editor Mediated CAR Integration with Simultaneous Multiplex Knockout for Enhanced Cancer Immunotherapies
Joseph Skeate, PhD, University of Minnesota - Moriarity Lab

2:30–2:45 PM 43
Peptide-MHC-Targeted Retroviruses Expand and Deliver Therapeutic Cargoes to Rare Populations of Anti-Tumor T Cells
Ellen Xu, Massachusetts Institute of Technology

2:45–3:00 PM 44
T Cell Engineering Using V(D)J Recombination
Adi Barzel, PhD, Tel Aviv University

3:00–3:15 PM 45
Novel α3-MICA/B-Specific CAR T-Cell Immunotherapy Demonstrates Ubiquitous Targeting of Cancer Cells and Resistance to Immune-Surveillance Evasion
Alex Garcia, PhD, Fate Therapeutics Inc.

TUESDAY, MAY 07

ORAL ABSTRACT SESSION

Lysosomal Storage Disorders

Co-chairs: Brian Bigger, PhD, University of Edinburgh and Vinod Prasad, MD, Duke University Medical Center

ROOM 309-310

1:30–1:45 PM | **46**

Hematopoietic Stem Cell Gene Therapy for Hurler Syndrome: Interim Skeletal Outcome and Skeletal Cross-Correction Mechanisms
Maria Ester Bernardo, MD, PhD, SR-Tiget

1:45–2:00 PM | **47**

Adeno-Associated Virus-Based Gene Therapy Delivering Combinations of Two Growth-Associated Genes
Shunji Tomatsu, MD, PhD, Nemours/Alfred I. DuPont Hospital for Children

2:00–2:15 PM | **48**

CAMPSIITE™ Phase I/II/III: An Interim Clinical Study Update of RGX-121, an Investigational Gene Therapy for the Treatment of Neuronopathic Mucopolysaccharidosis Type II (MPS II)
Laura Pisani, MD, REGENXBIO Inc.

2:15–2:30 PM | **49**

Rescue of Lysosomal Acid Lipase Deficiency in Mice by AAV Liver Gene Transfer
Marine Laurent, Genethon

2:30–2:45 PM | **50**

AAV-Ep+ in Preclinical Studies for Late Infantile Neuronal Ceroid Lipofuscinosis
Luis Tecedor, PhD, Children's Hospital of Philadelphia

2:45–3:00 PM | **51**

Long-Term Haematopoietic Stem Cell Gene Therapy in Mucopolysaccharidosis IIIB Mice Corrects Disease with No Evidence of Insertional Mutagenesis Despite High Vector Copy Numbers
Brian Bigger, PhD, University of Edinburgh

TUESDAY, MAY 07

ORAL ABSTRACT SESSION (CONTINUED)

Lysosomal Storage Disorders

Co-chairs: Brian Bigger, PhD, University of Edinburgh and Vinod Prasad, MD, Duke University Medical Center

ROOM 309-310

3:00–3:15 PM | 52

From Mouse to Sheep: Developing an AAV Gene Therapy for Sialidosis

Jillian Gallagher, UMass Chan Medical School

TUESDAY, MAY 07

GENERAL SESSION

Founders, Catalyst, & Mendell Award Symposium

Co-Chairs: Jeffrey S. Chamberlain, PhD, University of Washington, and Paula Cannon, PhD, USC

HALL A-B

3:45–3:50 PM
Welcome
David Barrett, JD, ASGCT CEO

3:50–3:55 PM
In Memoriam: Nicholas Muzyczka and Kenneth Burns

3:55–4:27 PM
Founders Award Keynote
Katherine High, MD, Rockefeller University

4:27–4:59 PM
Catalyst Award Keynote
Maritza McIntyre, PhD, Advanced Therapies Partners LLC

4:59–5:30 PM
Mendell Award Keynote
John Tisdale, MD, National Heart, Lung, and Blood Institute (NHLBI)

WEDNESDAY, MAY 08

ORAL ABSTRACT SESSION

Clinical Trials Spotlight Symposium

Co-chairs: Isabelle Riviere, PhD, Takeda and John Gray, PhD

BALLROOM 1

8:00–8:15 AM | **4**

Gene Therapy for Adult and Pediatric Patients with Severe Pyruvate Kinase Deficiency: Results from a Global Study of RP-L301
Julián Sevilla, Hematol y Hemoterapia, Fundación Invest Bioméd, Hosp Infantil Univ Niño Jesús (HIUNJ)

8:15–8:30 AM | **5**

Personalized Neoantigen DNA Vaccine (GNOS-PV02) and Pembrolizumab Develop Antitumor Poly-Functional Neoantigen-Specific CD4+ and CD8+ Effector T Cells in Advanced Hepatocellular Carcinoma Therapy
Renzo Perales, PhD, Geneos Therapeutics

8:30–8:45 AM | **6**

Allogeneic NKT Cells Expressing a CD19-Specific CAR in Patients with Relapsed or Refractory B cell Malignancies: An Interim Analysis
Leonid Metelitsa, MD, PhD, Baylor College of Medicine

8:45–9:00 AM | **7**

Reprogramming of the Tumor Microenvironment in Glioblastoma Multiforme by Transplantation of Genetically-Engineered Hematopoietic Stem Cells
Bernhard Gentner, MD, PhD, CHUV

9:00–9:15 AM | **8**

Long Term Safety and Integration Site Analysis Over a Large Cohort of Patients Treated with T Cells Modified by Lentiviral or Gammaretroviral Gene Addition
Frederic Bushman, PhD, UPenn

WEDNESDAY, MAY 08

ORAL ABSTRACT SESSION (CONTINUED)

Clinical Trials Spotlight Symposium

Co-chairs: Isabelle Riviere, PhD, Takeda Pharmaceuticals and John Gray, PhD

BALLROOM 1

9:15–9:30 AM | **9**

Safety and Efficacy of AB-1002 Gene Therapy in Patients with Advanced Heart Failure: Results from an Ongoing Phase 1 Clinical Trial

Luke Roberts, PhD, Asklepios BioPharmaceutical, Inc

9:30–9:45 AM | **10**

Intracochlear Administration of DB-OTO Gene Therapy in Pediatric Patients with Profound Hearing Loss Due to Otoferlin Mutations: The CHORD Phase 1/2 Open-Label Trial

Lawrence Lustig, MD, Columbia University Medical Center

WEDNESDAY, MAY 08

EDUCATION SESSION

Clinical Translation of Gene and Cell Therapies (Organized by the Education Committee)

Co-chairs: Julia Davydova, MD, PhD, University of Minnesota and
Satiro De Oliveira, MD, UCLA

BALLROOM 2

8:00–8:10 AM
Opening Remarks/Session Introduction

8:10–8:30 AM
Gene-edited Minipigs - A More Predictive Preclinical Model System
Chris Rogers, PhD, Exemplar Genetics

8:30–8:50 AM
Clinical Translation of Gene Therapies in Cancer
Yuman Fong, MD, City of Hope Medical Center

8:50–9:10 AM
Navigating the Translational Development Pipeline of Novel Cellular Therapeutics
John Wagner, MD, University of Minnesota

9:10–9:45 AM
Panel Discussion
Yuman Fong, MD
John Wagner, MD
Chris Rogers, PhD

WEDNESDAY, MAY 08

SCIENTIFIC SYMPOSIUM

Frontline of Gene and Cell Therapy Research in Japan (Organized by the Japanese Society of Gene and Cell Therapy)

Co-chairs: Ko Mitani, PhD, Saitama Medical University and
Masato Yamamoto, MD, PhD, University of Minnesota

BALLROOM 3

8:00–8:26 AM

Gene Therapy for Brain Tumors Using Genome-Edited iPS Cells
Masahiro Toda, MD, PhD, Keio University

8:26–8:52 AM

Novel approaches of Genome Editing For Treatment and Diagnosis of Genetic Diseases
Tsukasa Ohmori, MD, PhD, Jichi Medical University

8:52–9:18 AM

Gene Edited and Engineered Stem Cell Mediated Immunotherapy for Brain Metastatic Tumors
Nobuhiko Kanaya, MD, PhD, Okayama University Graduate School of Medicine, Dentistry and Pharmaceutical Sciences

9:18–9:44 AM

Next-Generation Vaccinia Viral Platform for Oncolytic Virotherapy
Takafumi Nakamura, PhD, Tottori University

WEDNESDAY, MAY 08

SCIENTIFIC SYMPOSIUM

From the Clinic Back to the Bench: Solving Old Problems with New Technologies (Organized by the Neurologic and Ophthalmic Gene and Cell Therapy Committee)

Co-chairs: Eva Andres-Mateos, PhD, Atsena Therapeutics and
Heather Gray-Edwards, DVM, PhD, UMass Chan Medical Center

BALLROOM 4

8:00–8:26 AM

Optimizing Subretinal Gene Therapy: Surgical Techniques and Capsid Design
Christine Kay, MD, Vitreoretinal Associates

8:26–8:52 AM

Post-approval Experience in Clinical Implementation for CNS Gene Therapies
Kevin Strauss, MD, Clinic for Special Children

8:52–9:18 AM

Beyond AAV, Gene Editing of HSV in Patients with Herpetic Stromal Keratitis
Yuija Cai, PhD, Shanghai Jiao Tong University

9:19–9:45 AM

From Cystinosis to Friedreich's Ataxia and Alzheimer's Disease: Lessons Learned from a Rare Metabolic Disease Using *Ex Vivo* Gene Therapy
Stephanie Cherqui, PhD, UCSD

WEDNESDAY, MAY 08

SCIENTIFIC SYMPOSIUM

Prospects for Prenatal Gene and Cell Therapy (Organized by the Prenatal Gene and Cell Therapy Committee)

Co-chairs: Agnieszka Czechowicz, MD, PhD, Stanford School of Medicine and William Peranteau, MD, Children's Hospital of Philadelphia

ROOM 307-308

8:00–8:26 AM

Prenatal Therapies - How Far We've Come and the Challenges That Remain
Alice Tarantal, PhD, UC Davis

8:26–8:52 AM

Ethics of Prenatal Gene and Cell Therapy
Julia Brown, PhD, UCSF

8:52–9:18 AM

Preclinical Advances in Prenatal Gene Therapy
Simon Waddington, PhD, University College London

9:18–9:44 AM

Phase 1 Clinical Trial of In Utero ERT and Preclinical Work on Gene Therapy/Editing
Tippi MacKenzie, MD, UCSF

WEDNESDAY, MAY 08

SCIENTIFIC SYMPOSIUM

The Impact of Generative Artificial Intelligence (AI) on CGT: Best Practices and Real-World Applications for Communicators (Organized by the Communications Committee)

Co-chairs: Catherine Gillespie, Baylor College of Medicine and Christopher Leidli

ROOM 318-323

8:00–8:25 AM
Generative AI Progress and Applications for CGT
David Cheng, Arbor Biotechnologies

8:25–8:50 AM
Proven AI Tools for Real-World CGT Communications Applications
Priyanka Shah, Elephant Head Communications

8:50–9:15 AM
AI Ethics Considerations When Publishing CGT Research
Rory Bricker-Anthony, PhD, ASGCT

9:15–9:45 AM
Panel Discussion
Priyanka Shah
Rory Bricker-Anthony, PhD

WEDNESDAY, MAY 08

SCIENTIFIC SYMPOSIUM

Research Readiness for Rare Diseases (Organized by the Patient Outreach Committee)

Co-chairs: Allison Bradbury, PhD, Nationwide Children's Hospital and Kimberly Goodspeed, MD, Ultragenyx

ROOM 339-342

8:00–8:26 AM

The Four Pillars of Research Readiness
Leah Schust Myers, Executive Director of FamilieSCN2A Foundation

8:26–8:52 AM

Basic Science Of Understanding Your Disease Biology and Physiology
Michael Boland, PhD, UPenn

8:52–9:18 AM

Innovative Tools and Strategies to Make Each Step in the Translational Process More Effective and Efficient
Joni Rutter, PhD, NCATS-NIH

9:18–9:44 AM

Clinical Trial Design and Participation In Clinical Trials with Investigational Drugs
Richard Finkel, MD, St. Jude Children's Research Hospital

WEDNESDAY, MAY 08

FIRESIDE CHAT

Fireside Chat: Regulating Cell and Gene Therapies from IND to BLA

Chair: Kristin Van Goor, PhD, Takeda Pharmaceuticals

ROOM 314-317

8:00–9:45 AM

Regulating CGTs from IND to BLA
Peter Marks, MD, PhD, FDA

WEDNESDAY, MAY 08

SPONSORED SYMPOSIUM

ROOM 337-338

8:00–8:30 AM

SARTORIUS BIA SEPARATIONS:
From Process Development To Manufacturing - How To Successfully Optimize And Scale Up The AAV Enrichment Step On Novel CIMmultus QA HR Line

ROOM 309-310

8:30–9:30 AM

FORTREA:
So, You're Planning A First-In-Human Clinical Trial? Patient-Centric Considerations For Cell & Gene Therapy Studies. Regulatory And Operational Considerations And Strategies For Pediatric And Rare Disease Populations, Diversity, Equity, And Inclusion, And Long-Term Follow Up

ROOM 324-326

8:30–9:30 AM

MYRTELLE, INC.:
Update on Myrtelle's Clinical Trial and Future Directions for Oligodendrocyte Targeting AAVs

ROOM 327-329

8:30–9:30 AM

PAREXEL:
Analytical strategies and evidentiary requirements for developing value demonstration in support of commercializing cell and gene therapies in rare disease and beyond

WEDNESDAY, MAY 08

GENERAL SESSION

Presidential Symposium

Co-Chairs: Jeffrey S. Chamberlain, PhD, University of Washington, and Paula M. Cannon, PhD, USC

Presidential Symposium Sponsored By

ANEMOCYTE
Manufacturing Biotech Innovation

HALL A-B

10:15–10:45 AM

Mechanistic Insights and Therapeutic Approaches to Restore Muscle Function in Muscular Dystrophy
Kevin Campbell, PhD, University of Iowa

10:45–11:15 AM

Improving Human Cardiomyocyte Therapy with Genome Editing and Metabolic Reprogramming
Charles Murry, MD, PhD, UW Medicine

11:15–11:30 AM 1

Atidarsagene Autotemcel (Hematopoietic Stem Cell GeneTherapy) Preserves Cognitive and Motor Development in Metachromatic Leukodystrophy with up to 12 Years Follow-Up
Alessandro Aiuti, MD, PhD, SR-Tiget; Vita Salute San Raffaele University

11:30–11:45 AM 2

***In Vivo* Generation of Both CAR T Cells and CAR NK Cells Using a CD7 Targeted Lentiviral Vector**
Karla Mullen, PhD, Interius BioTherapeutics

11:45 AM–12:00 PM 3

A Recombinant Human Bocavirus Vector Delivers Therapeutic Levels of CTFR to Cystic Fibrosis Primary Human Airway Cells and Transduces Airway Epithelium *In Vivo*
Jeff Moffit, PhD, Carbon Biosciences

WEDNESDAY, MAY 08

POSTER VIEWING

Wednesday Posters 436 – 926 and Late-Breaking 15-25

EXHIBIT HALL

12:00–1:30 PM POSTER VIEWING

5:30–7:00 PM POSTER RECEPTION

WEDNESDAY, MAY 08

SPONSORED SYMPOSIUM

EXHIBIT HALL PRESENTATION THEATER

12:15–1:15 PM

KRIYA THERAPEUTICS:
Advancing Gene Therapy for Common Diseases: From Concept to Reality

ROOM 309-310

12:15–1:15 PM

OXFORD BIOMEDICA:
Accelerating Gene Therapy: Enhancing Viral Vector Manufacturing for Rapid Patient Impact

ROOM 324-326

12:15–1:15 PM

SIREN BIOTECHNOLOGY:
Universal AAV Immuno-Gene Therapy for Cancer

ROOM 327-329

12:15–1:15 PM

SAREPTA THERAPEUTICS:
A Path Forward: Building Upon Existing AAV Platforms for Rare Diseases

WEDNESDAY, MAY 08

ORAL ABSTRACT SESSION

Epigenetic Editing and RNA Editing

Co-chairs: Annarita Miccio, PhD, Imagine Institute and
Kyle Fink, PhD, UC Davis

BALLROOM 1

1:30–1:45 PM | **53**

A High-Fidelity CRISPR-Cas13 System Improves Abnormalities Associated with C9ORF72-Linked ALS/FTD
Tristan McCallister, University of Illinois Urbana Champaign

1:45–2:00 PM | **54**

Do Some Loci Have a Propensity to Retain Ectopic Methylation?
Henriette O'Geen, PhD, UC Davis Genome Center

2:00–2:15 PM | **55**

Engineered Extrachromosomal Genetic Technologies for Persistent and Tunable Control of Therapeutic Human Cell Types
Daniel Brenner, Rice University

2:15–2:30 PM | **56**

Compact Epigenetic Modulators for CRISPR Mediated Persistent Gene Activation
Dan Hart, PhD, Epicrispr Biotechnologies

2:30–2:45 PM | **57**

>95% Targeted RNA Editing in Brain with High Durability and Specificity Enabled by Systemic Injection of AAV-Packaged ADAR Guide RNAs
Alison VanSchoiack, PhD, Shape Therapeutics, Inc.

2:45–3:00 PM | **58**

Durable and Specific Silencing of Therapeutically Relevant Genes Using Epigenetic Editors is Reversible *In Vivo*
Aron Jaffe, Chroma Medicine

3:00–3:15 PM | **59**

Predicting CRISPR-Cas13 On-Target Efficiencies and Intrinsic RNA Off-Targets
Wei Li, PhD, Children's National Hospital

WEDNESDAY, MAY 08

ORAL ABSTRACT SESSION

AAV Manufacturing II

Co-chairs: Johannes Van Der Loo, PhD, Children's Hospital of Philadelphia and Melanie Sorensen, Cytiva

BALLROOM 3

1:30–1:45 PM | **60**

pAAVing the Way to High Titres: Plasmid Ratio Optimisation for AAV Production Utilising the Ambr 15 Stirred-Tank Bioreactor
Sandeep Mangrati, University College London

1:45–2:00 PM | **61**

Producing High-Purity rAAV Vectors by Recombination-Dependent Minicircle Dual Transfection
Hao Liu, PhD, Horae Gene Therapy Center, UMass Chan Medical School

2:00–2:15 PM | **62**

Elucidating the Function of Adenovirus Late Gene Products and Impact on AAV Production
Hao Pan, PhD, Predevelopment, Genomic Medicine Unit CMC, Sanofi

2:15–2:30 PM | **63**

Process Development of HEK293-Based rAAV2 Manufacturing Platform
Alexander Burns, Keck Graduate Institute

2:30–2:45 PM | **64**

Transforming AAV Vector Production with the TruStableTM AAV Producer Cell Line
Sandhya Pande, Shape Tx

2:45–3:00 PM | **65**

Developability Assessment of Novel AAV Capsids and Payloads at Early Preclinical Stage to Enable Development of Gene Therapies
Matteo Placidi, PhD, Voyager Therapeutics

3:00–3:15 PM | **66**

Comparative Analysis of Sterile Grade Filters in Adeno-Associated (AAV) Manufacturing: Accelerating First-in-Human AAV Therapeutic Production with Quality by Design Principles
Angela N. Johnson, PhD, Cytiva

WEDNESDAY, MAY 08

ORAL ABSTRACT SESSION

AAV Vector Biology and Development I

Co-chairs: Philip Leopold, PhD, Weill Cornell Medicine and
Zhenwei Song, PhD, University of North Carolina at Chapel Hill

BALLROOM 4

1:30–1:45 PM | **67**

Novel AAV8 and AAVrh39 ITRs and ITR-Proximal Regions Significantly Enhance Transgene Expression through Enhancer/Promoter-Like Activities
Suk Namkung, Horae Gene Therapy Center, UMass Chan Medical School

1:45–2:00 PM | **68**

A Genome-Wide CRISPR/Cas9 Screen Identifies an Essential Role of SLC35A1 in rAAV Transduction
Xiujuan Zhang, PhD, University of Kansas Medical Center

2:00–2:15 PM | **69**

Screening for Cellular Factors Underlying Mechanisms of AAV Tropism
Mark Kay, MD, PhD, Stanford University School of Medicine

2:15–2:30 PM | **70**

Consequences of Human Genetic Variations of the Adeno-Associated Virus Receptor (AAVR) Gene (KIAA0319L) on AAV-Mediated Gene Transfer
Jenifer Vasquez, Weill Cornell Medical College

2:30–2:45 PM | **71**

Epigenetic Blueprint: Understanding How the AAV Capsid Influences the Vector Epigenome
Eirini Vamva, PhD, Stanford University Laboratory for Cell & Gene Medicine

2:45–3:00 PM | **72**

Lack of the Ability to Bind Heparin and a Y-to-F Mutation are Sufficient for AAP-Independent AAV2 Capsid Assembly
Anusha Sairavi, Oregon Health and Science University

WEDNESDAY, MAY 08

ORAL ABSTRACT SESSION (CONTINUED)

AAV Vector Biology and Development I

Co-chairs: Philip Leopold, PhD, Weill Cornell Medicine and
Zhenwei Song, PhD, University of North Carolina at Chapel Hill

BALLROOM 4

3:00–3:15 PM | **73**

Specific Di-Nucleotide Pattern in Adeno-Associated Virus Genomes
Conradin Baumgartl, Universitätsklinikum Heidelberg

WEDNESDAY, MAY 08

ORAL ABSTRACT SESSION

Next Generation Gene & Cell Therapies for Heart, Lung, and Kidney Diseases

Co-chairs: Ashley Cooney, PhD, University of Iowa and
Katherine Excoffon, PhD, Spirovant Sciences, Inc

ROOM 314-317

1:30–1:45 PM | 74
Rescue of Lethal SP-B Deficiency in a Murine Model Using Lentiviral Vector-Mediated Gene Therapy
Kamran Miah, PhD, University of Oxford

1:45–2:00 PM | 75
Aerosolized Gene and Oligonucleotide Therapy Targeting microRNA-224 Ameliorates Pulmonary Hypertension by Orchestrating the BMP Pathway
Olympia Bikou, MD, LMU University Hospital

2:00–2:15 PM | 76
AAV2-GEC: Advancing Gene Delivery for Precision Treatment of Antibody-Mediated Kidney Diseases
Shuya Liu, MD, University Medical Center Hamburg-Eppendorf

2:15–2:30 PM | 77
Adoptive Transfer of Allogeneic Murine Engineered T Regulatory Cells Ameliorates Disease in Models of Acute Kidney Injury in Mice
Maegan Hoover, GentiBio

2:30–2:45 PM | 78
Addressing a Critical Limitation of First-Generation Dystrophin-Mimetics: AAV-Nanotrophins Provide Superior Cardioprotection and Circumvent Junctional Fragmentation *In Vivo*
Coral Kasden, UPenn

2:45–3:00 PM | 79
AAV FGF21 Gene Therapy as a Variant Agnostic Treatment for Arrhythmogenic Cardiomyopathy
Noah Davidsohn, PhD, Rejuvenate Bio

3:00–3:15 PM | 80
Increasing Saline Tonicity Enhances Airway Gene Transfer
Ashley Cooney, PhD, University of Iowa

WEDNESDAY, MAY 08

ORAL ABSTRACT SESSION

Ophthalmic and Auditory: Delivery Innovations

Co-chairs: Devin McDougald, PhD, Biogen,
Killian Hanlon, PhD, Harvard Medical School, and
Yonghong Chen, PhD, The Children's Hospital of Philadelphia

ROOM 318-323

1:30–1:45 PM | **81**
Optimization of Viral Gene Therapy for Inherited Retinal Diseases Using Fibrin-Mediated Delivery
Aubrey Berger, Mayo Clinic

1:45–2:00 PM | **82**
Suprachoroidal Injection of Poly (Beta-Amino Ester) Nanoparticles Enables Safe, Efficacious, and Durable Gene Delivery Across Multiple Species
Jordan Green, PhD, Johns Hopkins University

2:00–2:15 PM | **83**
Efficient Spiral Ganglion Neuron Transduction with a Novel Capsid-Engineered AAV Vector for Improved Cochlear Implant Outcomes in Hearing Loss
Jennifer Marx, Hannover Medical School

2:15–2:30 PM | **84**
Optimizing Ocular Gene Therapy: Harnessing the Potential of the Proteolipid Vehicle Nucleic Acid Delivery Platform
Douglas Brown, PhD, Ento Pharmaceuticals

2:30–2:45 PM | **85**
Cochlear Gene Therapy Delivery Innovations, AAV Capsid Variants and Cell Type-Specific Regulatory Elements to Facilitate CSF-Mediated Administration
Paul Ranum, PhD, Latus Bio

2:45–3:00 PM | **86**
Identification of Novel Ligand-Conjugated AAV Vectors with Enhanced Properties for Suprachoroidal Gene Delivery
Gaelle Lefevre, PhD, Coave Therapeutics

WEDNESDAY, MAY 08

ORAL ABSTRACT SESSION (CONTINUED)

Ophthalmic and Auditory: Delivery Innovations

Co-chairs: Devin McDougald, PhD, Biogen and
Killian Hanlon, PhD, Harvard Medical School

ROOM 318-323

3:00–3:15 PM | 87

Development of an *In Vivo* Non-Viral Ocular Editing Platform and Application to Potential Treatments for Glaucoma

Mary-Lee Dequeant, PhD, CRISPR Therapeutics

WEDNESDAY, MAY 08

ORAL ABSTRACT SESSION

CAR T and Other Genetically Modified Immune Cells

Co-chairs: M Graça Almeida-Porada, MD, PhD, Wake Forest School of Medicine and Satiro De Oliveira, MD, UCLA

ROOM 339-342

1:30–1:45 PM | **88**
Development of a FAP-CAR T Cell Protocol to Reduce Skeletal Muscle Fibrosis in a Murine Model of Duchenne Muscular Dystrophy
Anne Galy, PhD, Genethon

1:45–2:00 PM | **89**
Genetic Depletion of TIGIT Augments CAR-NK Cell Effector Function in the Solid Tumor Microenvironment
Ishwar Navin, Baylor College of Medicine

2:00–2:15 PM | **90**
HLA-A2 CAR/IL-2-CISC Engineered Treg Exhibit Robust *In Vitro* and *In Vivo* Efficacy
Subhash Tripathi, PhD, Seattle Children's Research Institute

2:15–2:30 PM | **91**
Anti-B7-H3 Chimeric Antigen Receptor NK Cells Suppress the Growth of Atypical Teratoid / Rhabdoid Tumor Orthotopic Xenografts
Jun Choe, Johns Hopkins University School of Medicine

2:30–2:45 PM | **92**
An Immune Synapse-Stabilizing Receptor Enhances Sensitivity of CAR- and TCR-Mediated Tumor Targeting
Chiou-Tsun Tsai, PhD, Baylor College of Medicine

2:45–3:00 PM | **93**
Transduction with Bicistronic CD20 CAR with NIS Reporter Allows PET/CT Visualization of CAR-T Cell Trafficking *In Vivo*
Brynn Duncan, MD, NIH

3:00–3:15 PM | **94**
Chimeric Cytokine Receptors Induce Antigen-Specific Activation in Natural Killer Cells Against Acute Myeloid Leukemia
Natalie Holl, Johns Hopkins University School of Medicine

WEDNESDAY, MAY 08

SCIENTIFIC SYMPOSIUM

Novel Nucleic Acid and Cell-based Vaccines for Cancer (Organized by the Infectious Diseases and Vaccines Committee)

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

BALLROOM 2

1:30–1:55 PM

Personalized mRNA Vaccines for Pancreatic Cancer
Pablo Guasp Baratech, PhD, Memorial Sloan Kettering Cancer Center

1:55–2:20 PM

Therapeutic DNA Vaccines for Cancer
Mary (Nora) Disis, MD, University of Washington

2:20–2:45 PM

Combinatorial Approach to Cancer Immunotherapy of Novel High-Impact Treatments (Cell-Based Approach)
Eli Gilboa, PhD, University of Miami

2:45–3:15 PM

Panel Discussion
Pablo Guasp Baratech, PhD
Mary (Nora) Disis, MD
Eli Gilboa, PhD

WEDNESDAY, MAY 08

SCIENTIFIC SYMPOSIUM

BGTC Annual Meeting 2024

ROOM 307-308

1:30–1:40 PM

State of the BGTC
Courtney Silverthorn, PhD, FINH

1:40–1:55 PM

Adapting the Regulatory Environment
Peter Marks, MD, PhD, FDA

1:55–2:50 PM

Panel Discussion
Adrian McKemey, PhD
Dominique Pichard, MD
Sharon King
Terry Pirovolakis

2:50–3:20 PM

BGTC Coordination Center Update
Amritha Jaishankar, IQVIA
Carmen Sivakumaren, PhD, IQVIA
Jenny Fam, IQVIA
Philip Brooks, PhD, NIH / NCATS
Timothy Miller

WEDNESDAY, MAY 08

SCIENTIFIC SYMPOSIUM (CONTINUED)

BGTC Annual Meeting 2024

ROOM 307-308

3:30–4:00 PM

BGTC Regulatory Playbook User Engagement
Amritha Jaishankar, IQVIA
Carmen Sivakumaren, PhD, IQVIA
Jean Dehdashti, RAC
Jenny Fam, IQVIA
Jim Noll
Philip Brooks, PhD, NIH / NCATS
Timothy Miller

4:00–4:45 PM

Panel Discussion
Edward Neilan, MD, PhD
Philip Brooks, PhD

4:45–5:30 PM

Stable Propagation of AAV2 Inverted Terminal Repeat Elements in Plasmid Vectors
Fred Bunz, Johns Hopkins University School of Medicine

WEDNESDAY, MAY 08

SPONSORED SYMPOSIUM

ROOM 337-338

1:30–2:00 PM

AGC BIOLOGICS:
Designing process and analytical validation and characterization studies for EU and FDA approval

ROOM 337-338

2:30–3:00 PM

TERUMO BLOOD AND CELL TECHNOLOGIES:
Achieving Consistency in Fill & Finish for Cell Therapies

ROOM 337-338

3:45–4:15 PM

LABCORP:
To Administer or Not to Administer? Benefits and Challenges to Incorporating of Immunosuppressive Regimens During Nonclinical Safety Evaluations

ROOM 337-338

4:45–5:15 PM

BIO-TECHNE:
Novel RNAscope™ Multi-omics Spatial Approach to Characterize Biodistribution and Safety of Cell and Gene Therapies

WEDNESDAY, MAY 08

TOOLS AND TECHNOLOGY FORUM

Tools and Technology Forum 1

Chair: Kaye Spratt, PhD

EXHIBIT HALL PRESENTATION THEATER

2:00–2:15 PM

FORGE BIOLOGICS:
Improving Analytical Tools for AAV Characterization

2:15–2:30 PM

VIVEBIOTECH:
A lentiviral-vector-specialized company developing and GMP-manufacturing lentivectors with a very well-stablished, scalable, plug-and-play platform that delivers lentiviruses in a timely and regulatory-compliant manner from scratch to commercial-scale. Due to relevant bioprocess development and virology expertise, VIVEbiotech releases high-titer, pure and cost-effective lentivirus that can be administered both *ex-vivo* and *in-vivo*

2:30–2:45 PM

MILLIPORESIGMA:
How to Boost AAV Titer with Feed Supplementation in both HEK293 and Sf-9 Platforms

2:45–3:00 PM

UNCHAINED LABS:
Next level AAV and lentivirus biophysical characterization with Stunner and Leprechaun

3:00–3:15 PM

ARTICZYMES TECHNOLOGIES:
Clearing DNA from Viral Vectors with Salt Active Nucleases: Why Salt is a Hidden Catalyst to Bioprocessing Optimization

WEDNESDAY, MAY 08

ORAL ABSTRACT SESSION

Base Editing and Prime Editing I

Co-chairs: Jerry Chan, KK Women's and Children's Hospital / Duke NUS Medical School and Toni Cathomen, PhD, Medical Center - University of Freiburg

BALLROOM 1

3:45–4:00 PM | **95**

Nucleotide Metabolism Constrains Prime Editing in Hematopoietic Stem and Progenitor Cells
Sébastien Levesque, PhD, Boston Children's Hospital

4:00–4:15 PM | **96**

Prime Editors Precisely Correct Pathogenic RHO Mutations and Preserve Photoreceptors *In Vivo*
Deepak Reyon, PhD, Prime Medicine

4:15–4:30 PM | **97**

In Utero and Postnatal Base Editing for a Metabolic Liver Disease in Mice and Nonhuman Primates
Ana Maria Dumitru, MD, PhD, Children's Hospital of Philadelphia

4:30–4:45 PM | **98**

The Impact of Base Editing on Rhesus Macaque Hematopoietic Stem Cell Engraftment and *In Vivo* Clonal Behavior
Ashley Gin, BSAG, National Heart, Lung, and Blood Institute

4:45–5:00 PM | **99**

Base Editors Provoke Non-Predictable Chromosomal Translocations and Off-Target Editing
Toni Cathomen, PhD, Medical Center - University of Freiburg

5:00–5:15 PM | **100**

Epitope Editing Combined with Extended Schedule Anti-KIT Antibody Treatment Enhances Immune-Based *In Vivo* Selection of Multiplex Gene-Engineered Cells
Gabriele Casirati, MD, PhD, Boston Children's Hospital / Dana Farber Cancer Institute

5:15–5:30 PM | **101**

Development of a Prime Edited CD34+ Cell Drug Product for the Treatment of P47phox Chronic Granulomatous Disease
Jennifer Gori, PhD, Prime Medicine, Inc.

WEDNESDAY, MAY 08

EDUCATION SESSION

Trending Topics in Gene and Cell Therapies (Organized by the Education Committee)

Co-chairs: Christopher LaRocca, MD, University of Minnesota and Donald Kohn, MD, UCLA

BALLROOM 2

3:45–3:55 PM — **Opening Remarks**

3:55–4:25 PM — **Viral and Non-viral Delivery Methods for *In Vivo* Editing of Hematopoietic Cells**
Annarita Miccio, INSERM

4:25–4:55 PM — **Lipid Nanoparticles for *In Vivo* Gene Editing**
Yizhou Dong, PhD, Icahn School of Medicine at Mount Sinai

4:55–5:25 PM — **Genome Editing for T/NK cell therapy**
Dimitrios Wagner, MD, PhD, Center for Cell and Gene Therapy

5:25–5:30 PM — **Closing Remarks**

WEDNESDAY, MAY 08

ORAL ABSTRACT SESSION

AAV Analytical Methods

Co-chairs: J. Fraser Wright, PhD, Stanford School of Medicine and Pooja Agarwal, PhD, Biomarin Pharmaceutical

BALLROOM 3

3:45–4:00 PM **102**

Three-Dimensional Linkage Analysis with Digital PCR for Recombinant Adeno-Associated Virus Characterization Beyond Genomic Titer: Integrity and Identity
Tam Duong, PhD, Lonza Houston Inc.

4:00–4:15 PM **103**

Quantification of pH Induced AAV9 Capsid Alterations and TLR9 Interactions by Charge Detection Mass Spectrometry
Li Lin, UC Berkeley

4:15–4:30 PM **104**

Detection, Biophysical Characterization, and *In Vitro* Potency Impact of Distinct Empty/Light Capsid Populations in Recombinant AAV Preparations
Surya Simha Addepalli, BioMarin Pharmaceuticals Pharmaceutical

4:30–4:45 PM **105**

Analytical Technologies in Development of Potency Assay for Gene Therapies
Shiqian Zhu, PhD, BioAgilytix

4:45–5:00 PM **106**

Quantification of Full and Empty Particles of Adeno-Associated Virus Vectors via a Novel Dual Fluorescence-Linked Immunosorbent Assay
Sereirath Soth, Osaka University

5:00–5:15 PM **107**

Analysis of Heterogeneous, Unpurified Gene Therapy Formulations by High Throughput Charge Detection Mass Spectrometry
Benjamin Draper, PhD, Megadalton Solutions, Inc.

WEDNESDAY, MAY 08

ORAL ABSTRACT SESSION (CONTINUED)

AAV Analytical Methods

Co-chairs: J. Fraser Wright, PhD, Stanford School of Medicine and Pooja Agarwal, PhD, Biomarin Pharmaceutical

BALLROOM 3

5:15–5:30 PM | **108**

Rapid Optimization of AAV Production: Integrating Custom Design of Experiment, Cutting-Edge Production and Analytical Methods in Advancement of Gene Therapies

Tricia Jennings, Advanced Medicine Partners (AMP)

WEDNESDAY, MAY 08

ORAL ABSTRACT SESSION

Neurologic Diseases II

Co-chairs: Casey Maguire, PhD, Mass General Research Institute and Heather Gray-Edwards, DVM, PhD, UMass Chan Medical School

BALLROOM 4

3:45–4:00 PM | **109**

Gene Replacement of Human EPM2A and EPM2B as a Potential Therapy for Fatal Childhood Neurodegenrative Disease, Lafora
Esther Alao, UT Southwestern Medical Center

4:00–4:15 PM | **110**

CasRm-Mediated PTBP1 Knockdown Improves Motor Functions in Parkinson' Disease Mouse and Non-Human Primate Models
Shimin Wang, PhD, Reforgene Medicine

4:15–4:30 PM | **111**

Unveiling the Therapeutic Potential of Targeted AAV-Mediated Glycogen Clearance in the Central Nervous System in a Glycogen Storage Disease IV Mouse Model
Su Jin Choi, PhD, Duke University Medical Center

4:30–4:45 PM | **112**

hfCas12Max-Mediated Gene Editing Therapy for Amyotrophic Lateral Sclerosis
Dong Yang, HuidaGene Therapeutics Co., Ltd.

4:45–5:00 PM | **113**

All-in-One Adeno-Associated Virus Delivery for Therapeutic miR-21 Editing by saCas9 *In Vivo*
Lisa Nieland, Massachusetts General Hospital

5:00–5:15 PM | **114**

Chemogenetics for the Treatment of Spasticity
Nicholas Boulis, MD, Emory University School of Medicine

5:15–5:30 PM | **115**

Delivery of scAAV9.co GM2A for Phenotypic Correction of GM2 Gangliosidosis AB-Variant, in the Newly Characterized Gm2a/Neu3 Double Knockout Mouse Model
Camilyn Cheng, Queen's University

WEDNESDAY, MAY 08

ORAL ABSTRACT SESSION

Breaking Barriers to the CNS via AAV Capsid Engineering

Co-chairs: Li Ou, PhD, Avirmax, Inc. and
Melissa Kotterman, PhD, Iris Medicine

ROOM 309-310

3:45–4:00 PM | **116**

Human Blood-Brain Barrier Receptor-Guided Rapid Evolution of AAVs for Brain Gene Therapy
Changfan Lin, PhD, Caltech

4:00–4:15 PM | **117**

Identification and Characterization of STAC-BBB, an Engineered AAV Capsid That Exhibits Widespread Transduction of the Central Nervous System in Cynomolgus Macaques
Matthew Tiffany, PhD, Sangamo Therapeutics, Inc.

4:15–4:30 PM | **118**

Antibody-Based AAV Retargeting to Transferrin Receptor Mediates Efficient Blood Brain Barrier Crossing and *In Vivo* Gene Delivery to the CNS in Mice and Non-Human Primates
Kalyani Nambiar, PhD, Regeneron Pharmaceuticals, Inc.

4:30–4:45 PM | **119**

Continued Directed Evolution of VCAP-101 and VCAP-102 Identifies Second Generation Capsid Variants with Increased Brain Tropism and in Non-Human Primates and Mice
Tyler Moyer, PhD, Voyager Therapeutics

4:45–5:00 PM | **120**

Second Generation AAV Capsids Reprogrammed to Bind Human Transferrin Receptor are Targeted to the Brain and De-Targeted from the Liver in Human TFRC Knock-In Mice
Ken Chan, PhD, Broad Institute of MIT and Harvard

5:00–5:15 PM | **121**

Engineering Viral Vectors for Acoustically Targeted Gene Delivery to the Brain across Species
Hongyi Li, Caltech

WEDNESDAY, MAY 08

ORAL ABSTRACT SESSION (CONTINUED)

Breaking Barriers to the CNS via AAV Capsid Engineering

Co-chairs: Li Ou, PhD, Avirmax, Inc. and
Melissa Kotterman, PhD, Iris Medicine

ROOM 309-310

5:15–5:30 PM | 122

Directed Evolution of AAV9 Libraries in Non-Human Primates Identifies a Capsid Family with Enhanced Central Nervous System Tropism and Liver De-Targeting Following Systemic Delivery
Xiaojing Shi, PhD, Capsida Biotherapeutics, Inc.

WEDNESDAY, MAY 08

ORAL ABSTRACT SESSION

Strategies and Technologies for Advanced CMC

Co-chairs: Angela Johnson, Northeastern University; Cytiva and Sixuan Li, PhD, Johns Hopkins University

ROOM 314-317

3:45–4:02 PM | **123**

FDA and NIST Collaboration to Evaluate Assays and Control Materials for Characterizing Animal Biotechnology Products Generated by Genome Editing

Patricia Kiesler, PhD, NIST

4:02–4:19 PM | **124**

Purification Process Optimization of mRNA Lipid Nanoparticles via Microfluidic Platform to Improve Payload Distribution and Transfection

Xiang Liu, PhD, Johns Hopkins University

4:19–4:36 PM | **125**

Recombinant Nuclease Cas9 for Therapeutic Genome-Editing - The GMP Manufacturer´s Point of View

Hans Huber, PhD, Biomay

4:36–4:53 PM | **126**

Addressing the Impact of RM Critical Quality Attributes and Variability on Complex Biologic Manufacturing

Nandu Deorkar, PhD, Avantor

4:53–5:10 PM | **127**

Validation of Rapid Microbial Testing in an Academic GMP Cell Therapy Facility

Daniel Kota, PhD, Houston Methodist Research Institute

5:10–5:27 PM | **128**

Systematic Analysis of Primary Field Data from Biologics Therapy Regulatory Non-Approvals: Why Aren't We Talking About FDA Rejections?

Limin Wang, PhD, Cytiva Global Regulatory Strategy

WEDNESDAY, MAY 08

ORAL ABSTRACT SESSION

Lipid Nanoparticles

Co-chairs: David Dean, PhD, University of Rochester and
Chun-Ye Chen, PhD, Seattle Children's Hospital

ROOM 318-323

3:45–4:00 PM | **129**
PET Imaging of *In Situ*-Engineered CAR-T Cells in B Cell Lymphoma with ^{68}Ga-PSMA-11
Nisi Zhang, PhD, Stanford University

4:00–4:15 PM | **130**
COATSOME® SS Series: Highly Versatile Ionizable Lipid as Vital Component of Lipid Nanoparticle for Gene Therapy and Vaccine Application
Ryosuke Fukuda, PhD, NOF Corporation

4:15–4:30 PM | **131**
Map the Formulation Space of Lipid Nanoparticles (LNPs) Using Microfluidic Assembly in the Intraperitoneal (IP) Administration of RNA Therapeutics
Farhana Islam, University of Nebraska Medical Center

4:30–4:45 PM | **132**
***In Vivo* RNA Delivery to T Cells and Hematopoietic Stem Cells in Humanized Mice and Non-Human Primates Using Targeting Lipid Nanoparticles**
Rahul Palchaudhuri, PhD, Tessera Therapeutics

4:45–5:00 PM | **133**
Machine Learning-Guided Optimization of Lipid Nanoparticle Composition for B Cell Transfection
Wu Han Toh, Johns Hopkins University

5:00–5:15 PM | **134**
Formulation Optimized Circular RNA-LNP Vaccines Elicit Enhanced Immune Responses Against SARS-CoV-2
Edo Kon, PhD, RiboX Therapeutics (Israel) Ltd

WEDNESDAY, MAY 08

ORAL ABSTRACT SESSION (CONTINUED)

Lipid Nanoparticles

Co-chairs: Carol Miao, PhD, Seattle Children's Research Institute and
John Lueck, PhD, University of Rochester

ROOM 318-323

5:15–5:30 PM | 135

Development of a Novel "CLAMP" Platform for Scalable Preparation of Antibody-Conjugated Lipid Nanoparticles for T Cell-Targeted mRNA Delivery

Xi Zhu, PhD, Shanghai Vitalgen Biopharma

WEDNESDAY, MAY 08

ORAL ABSTRACT SESSION

Genetically Modified Immune Cells for AML and Solid Tumors

Co-chairs: Agnieszka Czechowicz, MD, PhD, Stanford School of Medicine and M. Paulina Velasquez, MD, St Jude Children's Research Hospital

ROOM 339-342

3:45–4:00 PM **136**

Logic Gated CAR T Cells Targeting Acute Myeloid Leukemia Effectively Clear Leukemia While Mitigating Skin and Neuronal Toxicity

Phoebe Duong, PharmD, University of Colorado, Anschutz Medical Campus

4:00–4:15 PM **137**

Development of Extracellularly Linked Concatemeric Trivalent Cytokine (ELeCTriC) Chimeric Antigen Receptor (CAR) T-Cells as Dual Conditioning and Immunotherapeutic Agents

Quenton Bubb, Stanford University

4:15–4:30 PM **138**

Developing Adoptive Cell Therapy Strategies to Leverage Myeloid Immunity Against AML

Shannon Oda, PhD, Seattle Children's Research Institute

4:30–4:45 PM **139**

***Ex Vivo* and *In Vivo* Generation of Dual Specific CAR T Cells for Combination CAR T/Oncolytic Virus Therapy**

Richard Vile, PhD, Mayo Clinic

4:45–5:00 PM **140**

ROR1 CAR Armored with Dominant Negative TGF-Beta Receptor II Demonstrates Enhanced Efficacy in Xenograft Models of Pancreatic Cancer

Bal Krishna Chand Thakuri, PhD, Lentigen Technology, Inc., a Miltenyi Biotec Company

5:00–5:15 PM **141**

A Phase 1 Dose Escalation Study of a Novel Coupled CAR T Cell Therapy, GCC19CART, for Patients with Metastatic Colorectal Cancer

Victor Lu, PhD, Innovative Cellular Therapeutics

WEDNESDAY, MAY 08

ORAL ABSTRACT SESSION (CONTINUED)

Genetically Modified Immune Cells for AML and Solid Tumors

Co-chairs: Agnieszka Czechowicz, MD, PhD, Stanford School of Medicine and M. Paulina Velasquez, MD, St Jude Children's Research Hospital

ROOM 339-342

5:15–5:30 PM | 142

Targeting AML with Multi-Antigen Specific T Cells Engineered to Express a CAR Specific for SSX2 Peptide 41-49

Gloria Pezzella, Children's National Hospital

WEDNESDAY, MAY 08

SCIENTIFIC SYMPOSIUM

Stem Cells in 3D (Stem Cells to Model Organs, Organoids as Therapy) (Organized by the Stem Cell Committee)

Co-chairs: April Pyle, PhD, UCLA and
David Mack, PhD, University of Washington

ROOM 324-326

3:45–4:11 PM

Brain Organoids
Alysson Muotri, PhD, UCSD

4:11–4:37 PM

Engineering Human Skeletal Muscle for Advanced Modelling of Neuromuscular Diseases and Therapeutics
Francesco Saverio Tedesco, MD, PhD, University College London & The Francis Crick Institute

4:37–5:03 PM

Hematopoietic Stem Cell Organoids
David Scadden, MD, Massachusetts General Hospital

5:03–5:29 PM

Modeling Sensory Development and Disease with Organoids
Karl Koehler, Boston Children's Hospital-Harvard Medical School

WEDNESDAY, MAY 08

SCIENTIFIC SYMPOSIUM

Upregulating Gene Expression with Oligonucleotide Therapeutics (Organized by the Oligonucleotide and RNAi Therapeutics Committee)

Co-chairs: Nizar Saad, PhD, Nationwide Children's Hospital and Parimal Pande, PhD

ROOM 327-329

3:45–4:11 PM

RNA Activation with Small Activating RNA (saRNA) for Therapy in Common and Rare Genetic Diseases
Nagy Habib, Imperial College of London

4:11–4:37 PM

RNA Amplifiers
Alla Sigova, CAMP4 Therapeutics

4:37–5:03 PM

Deep Screening of Splicing-Regulatory Elements for Oligonucleotide Therapeutics
Chaolin Zhang, Columbia University

5:03–5:29 PM

Splicing Modulation: ASO for Dravet Syndrome (Phase I/II)
Isabel Aznarez, Stoke Therapeutics

WEDNESDAY, MAY 08

TOOLS AND TECHNOLOGY FORUM

Tools and Technology Forum 2

Chair: Kaye Spratt, PhD

EXHIBIT HALL PRESENTATION THEATER

3:45–4:00 PM

BIO-TECHNE:
Advancements in GMP and Closed System Reagents for Immune Cell Therapy Manufacturing

4:00–4:15 PM

SCALEREADY:
Revolutionizing CAR-T Cell Production: Can we make CAR-Ts like we make CARS?

4:15–4:30 PM

AZENTA LIFE SCIENCES:
Advancing AAV and Lentiviral Therapy Development with Streamlined Production and Product Characterization

4:30–4:45 PM

WATERS CORPORATION:
Advancing Size Exclusion Chromatography of mRNA, LNPs and mRNA for Improved Potency and Safety-Indicating Measurements

4:45–5:00 PM

WATERS / WYATT TECHNOLOGY:
Enhancing Gene Delivery Analysis Using Light Scattering

5:00–5:15 PM

REFEYN:
Assessing viral vectors purity and stability with mass photometry technologies

5:15–5:30 PM

WUXI ADVANCED THERAPIES:
Discover TESSA® Technology for Scalable, Versatile, Economical AAV Manufacture

THURSDAY, MAY 09

SCIENTIFIC SYMPOSIUM

Personalized or 'Off the Shelf' Cancer Immunotherapies? Novel Approaches to Cancer Immunotherapies (Organized by the Cancer Gene and Cell Therapy Committee)

Co-chairs: Bernhard Gentner, MD, SR-Tiget and
Christopher LaRocca, MD, University of Minnesota

BALLROOM 1

8:00–8:26 AM
Engineered Dendritic Cells for Cancer Immunotherapy
Michelle De Palma, PhD, EPFL

8:26–8:52 AM
Third Party T-cells
Catherine Bollard, MD, Children's National Medical Center/George Washington University

8:52–9:18 AM
Oncolytic Vectors for Pediatric Brain Tumors
Marta Alonso, PhD, University Hospital of Navarra

9:18–9:44 AM
CAR-T Trials
Waseem Qasim, PhD, University College London

THURSDAY, MAY 09

SCIENTIFIC SYMPOSIUM

Searching for Goldilocks – Scaling for AAV Clinical Dose Prediction (Organized by the Translational Science Committee)

Co-chairs: Jessica Lynch, PhD, Johnson&Johnson and
Nicole Paulk, PhD, Siren Biotechnology

BALLROOM 4

8:00–8:25 AM

Dose Scaling From Preclinical Models to FIH for Local CNS AAV Gene Therapies
Stephanie Tagliatela, PhD, Encoded Therapeutics

8:25–8:50 AM

Dosing Considerations for Ocular AAV Gene Therapies
Prathap Nagaraja Shastri, PhD, Johnson&Johnson

8:50–9:15 AM

Dose Scaling From Preclinical Models to FIH for Systemic AAV Gene Therapies
Jordan Shin, MD, PhD, LEXEO

9:15–9:45 AM

Panel Discussion
Jordan Shin, MD, PhD
Stephanie Tagliatela, PhD
Prathap Nagaraja Shastri, PhD

THURSDAY, MAY 09

SCIENTIFIC SYMPOSIUM

Lessons Learned from Clinical Efficacy and Safety of Gene and Cell Therapies (Organized by the Gene & Cell Therapy of Genetic and Metabolic Diseases Committee)

Co-chairs: Alessio Cantore, PhD, SR-Tiget and Hongju Wu, PhD, Tulane University

ROOM 307-308

8:00–8:26 AM

Hemophilia Gene Therapy Follow Up, Factor 8 and 9, Dose-Response, Efficacy, Durability
Lindsey George, MD, Perelman School of Medicine, UPenn

8:26–8:52 AM

Molecular Forms of AAV in Primate Liver and Long-term Expression
Jenny Greig, PhD, UPenn

8:52–9:18 AM

Adverse Events With High Doses Systemic of AAV
Chamindra Laverty, MD, UCSD

9:18–9:44 AM

Hematopoietic Stem Cell Gene Therapy for Lysosomal Storage Disorders
Alessandro Aiuti, MD, PhD, SR-Tiget

THURSDAY, MAY 09

SCIENTIFIC SYMPOSIUM

Innovations in the Targeted Delivery of Cell and Gene Therapies to the Lung and GI Tract (Organized by the Respiratory and GI Tract Gene and Cell Therapy Committee)

Co-chairs: Amy Ryan, PhD, USC and Roland Kolbeck, PhD, Spirovant Sciences

ROOM 318-323

8:00–8:26 AM

Novel Lung Targeting Cell Penetrating Peptides as Vectors for Delivery of Therapeutics (Specific Lung Basal Cell Targeting From Systemic Circulation)
Maliha Zahid, PhD, Mayo Clinic

8:26–8:52 AM

Engraftment of Autologous Epithelial Cells to the Trachea
Robert Hynds, PhD, University College London

8:52–9:18 AM

Anti-CD20 Permits Secondary Lung Gene Transfer
Jay Kolls, MD, Tulane University

9:18–9:44 AM

Restoring Macrophage Immune Functions by Transplantation of Gene-modified HSCs: A Therapeutic Approach to NOD2 Crohn's Disease
Pervinder Sagoo, PhD, Orchard Therapeutics

THURSDAY, MAY 09

EDUCATION SESSION

Gene Editing Technologies (Organized by the Education Committee)

Co-chairs: Dimitrios Wagner, MD, PhD, Center for Cell and Gene Therapy and Nuria Morral, PhD, Indiana University School of Medicine

BALLROOM 2

8:00–8:10 AM — **Opening Remarks**

8:10–8:40 AM — **How Confident Are You? Expanded Results From the First NIST Genome Editing Consortium Interlab Stud**
Samantha Maragh, PhD, NIST

8:40–9:10 AM — **Gene Editing for Cystic Fibrosis**
Anna Cereseto, University of Trento

9:10–9:40 AM — **Gene Editing for Hereditary Deafness**
David Corey, PhD, Harvard Medical School

9:10–9:40 AM — **Closing Remarks**

THURSDAY, MAY 09

FIRESIDE CHAT

Fireside Chat: Global Regulatory Convergence

Chair: Jennifer Wellman, Akouos

ROOM 314-317

8:00–9:45 AM

Global Regulatory Convergence – ANVISA, Brazil
Francielli Melo, PhD

Global Regulatory Convergence – US, FDA
Peter Marks, MD, PhD

Global Regulatory Convergence – PMDA, Japan
Yoshiaki Maruyama, PhD

THURSDAY, MAY 09

ORAL ABSTRACT SESSION

Late-Breaking Abstracts I

Co-chairs: Alberto Auricchio, MD, PhD, FONDAZIONE TELETHON and Eugenio Montini, PhD, SR-Tiget, Milan, Italy

BALLROOM 3

8:00–8:15 AM
LBA-1

AAV1-hOTOF Gene Therapy for Children with Autosomal Recessive Deafness 9
Yilai Shu, MD, PhD, Institutes of Biomedical Science, Fudan University

8:15–8:30 AM
LBA-2

Results from GALILEO-1, A First-in-Human Clinical Trial of FLT201, an AAV-Gene Therapy, in Adults with Gaucher Disease Type 1
Ozlem Goker-Alpan, MD, Lysosomal and Rare Disorders Research & Treatment Center

8:30–8:45 AM
LBA-3

Multi-Year Enzyme Expression in Mucopolysaccharidosis Type VI Patients After Liver-Directed Gene Therapy
Nicola Brunetti-Pierri, MD, Telethon Institute of Genetics and Medicine

8:45–9:00 AM
LBA-4

Development and Translation of a Novel CRISPR Genome Editing Therapy to Induce Fetal Hemoglobin for Sickle Cell Disease
Varun Katta, Masters, St. Jude Children's Research Hospital

9:00–9:15 AM
LBA-5

A Phase 1 Clinical Trial of High Dose AAV1.SERCA2a in Patients with Heart Failure: Modulation of SERCA2a of Intra-myocytic Calcium trafficking in Heart Failure with Reduced Ejection Fraction (MUSIC-HFrEF)
Jeffrey Rudy, Sardocor Corp

THURSDAY, MAY 09

ORAL ABSTRACT SESSION (CONTINUED)

Late-Breaking Abstracts I

Co-chairs: Alberto Auricchio, MD, PhD, FONDAZIONE TELETHON and Eugenio Montini, PhD, SR-Tiget, Milan, Italy

BALLROOM 3

9:15–9:30 AM
LBA-6

Harnessing Chromatin Architecture and Post-Transcriptional Regulation to Benefit the Safety of the Lentiviral Vector Platform
Monica Volpin, PhD, SR-Tiget, IRCCS San Raffaele Hospital, Italy

9:30–9:45 AM
LBA-7

The AMETHYST (Advanced Mesenchymal Enhanced cell THerapY for SepTic) Trial: A First-in-Human, Dose Escalation Phase 1 Safety Trial of Genetically Enhanced MSCs (GEM00220) Appeared Safe and Well Tolerated in Patients with Septic Shock
Michael Callahan, MD, Massachusetts General Hospital

THURSDAY, MAY 09

AWARDS

Career Development & DEI Awardee Presentations I

Chair: Kyle Cromer, PhD, UCSF

ROOM 339-342

8:00–8:26 AM

Assessing the Potential of Adeno-Associated Virus (AAV) Inverted Terminal Repeats (ITRs) to Selectively Induce Cancer Stem Cell Toxicity

Jacquelyn J. Bower, UNC Chapel Hill

8:52–9:18 AM

Antisense Oligonucleotide Neonatal Therapy for Osteogenesis Imperfecta

Liubin Yang, MD, PhD, Baylor College of Medicine

9:18–9:44 AM

Membrane Engineering of exoAAVs for Shifting Their Organ Tropism

Miguel Santoscoy, Harvard Medical School

THURSDAY, MAY 09

SPONSORED SYMPOSIUM

ROOM 337-338

8:00–8:30 AM

POLYPLUS:
In 2024, what tools are available to improve AAV productivity and quality

ROOM 309-310

8:30–9:30 AM

MAXCYTE
Overcoming Challenges in Translating Research Development to a Scalable Clinical Manufacturing Process

ROOM 324-326

8:30–9:30 AM

BIO-TECHNE:
Protein Analytics Strategies for Gene Therapy - from Viral Vector Characterization to Clinical Trials

ROOM 327-329

8:30–9:30 AM

RESILIENCE:
Strategies for Autologous Cell Therapy Success: From >$100K/batch to <$30K/batch

THURSDAY, MAY 09

GENERAL SESSION

George Stamatoyannopoulos Memorial Lecture, Featuring the Outstanding Achievement Award Presentation

Co-Chairs: Jeffrey S. Chamberlain, PhD, University of Washington and Terence R. Flotte, MD, UMass Chan Medical School

George Stamatoyannopoulos Memorial Lecture Sponsored By

Spark™ THERAPEUTICS

HALL A-B

10:15–10:25 AM

Announcing the Winners of Excellence in Research Awards, George Stamatoyannopoulos Mentorship Award, and Mavis Agbandje-McKenna Scholarship

10:25–10:55 AM

Unlocking the Power of AAVs for Brain Gene Therapies
Beverly Davidson, PhD, Children's Hospital of Philadelphia

10:55–11:25 AM

Engineering CAR T-Cells with Novel Receptor Architectures
Philip Gregory, DPhil, Regeneron

11:30 AM–12:00 PM

Outstanding Achievement Award Keynote
David R. Liu, PhD, Broad Institute

THURSDAY, MAY 09

POSTER VIEWING

Thursday Posters 927 – 1418 and Late-Breaking 26 – 36

EXHIBIT HALL

12:00–1:30 PM POSTER VIEWING

5:30–7:00 PM POSTER RECEPTION

THURSDAY, MAY 09

SPONSORED SYMPOSIUM

EXHIBIT HALL PRESENTATION THEATER

12:15–1:15 PM

PFIZER:
Progresses and Challenges in Genetic Medicine

ROOM 309-310

12:15–1:15 PM

DYNO THERAPEUTICS:
AAV Capsid Design in the Era of Artificial Intelligence

ROOM 324-326

12:15–1:15 PM

VERTEX PHARMACEUTICALS:
Harnessing the Potential of Stem Cells: Emerging Therapeutic Approaches

ROOM 327-329

12:15–1:15 PM

CYTIVA:
The future of viral vector manufacturing: Is it time for cell lines to take center stage?

THURSDAY, MAY 09

ORAL ABSTRACT SESSION

In Vivo Gene Therapy Clinical Trials

Co-chairs: Christian Buchholz, PhD, Paul-Ehrlich-Institut and
Diana Bharucha-Goebel, MD, NIH/NINDS and Children's National Hospital

BALLROOM 1

1:30–1:45 PM **143**

Long-Term Efficacy and Safety in Adults with Glycogen Storage Disease Type IA (GSD IA) from a Phase 1/2 Clinical Trial and Long-Term Follow-Up Study of DTX401, an AAV8-Mediated, Liver-Directed Gene Therapy
John Mitchell, Montreal Children's Hospital

1:45–2:00 PM **145**

VGN-R08b Gene Therapy for Neuronopathic Gaucher Disease
Huiwen Zhang, Xinhua Hospital Affiliated to Shanghai Jiao Tong University School of Medicine

2:00–2:15 PM **146**

Interim Review of Safety, Tolerability, and Efficacy From a First-In-Human Phase 1/2a Clinical Study of ICM-203, an Intra-Articular, AAV Gene Therapy for Osteoarthritis
Alison Heald, MD, ICM Co.

2:15–2:30 PM **147**

Demonstration of Nuclease-Free Genome Editing (GeneRide™) in Pediatric Patients with Methylmalonic Acidemia
Marie Payton, Alexion AstraZeneca Rare Disease

2:30–2:45 PM **148**

Phase 1/2 Trial of Combined Intrathalamic/Intracisternal/Intrathecal Gene Therapy for Tay-Sachs and Sandhoff Diseases
Terence Flotte, MD, UMass Chan Medical School

2:45–3:00 PM **149**

Subretinal Gene Therapy in Patients with Bietti Crystalline Dystrophy: Preliminary Results of a Phase 1/2 Clinical Trial
Shiyi Yin, PhD, Beijing Tongren Hospital, Capital Medical University

THURSDAY, MAY 09

ORAL ABSTRACT SESSION

Gene Disruption and Excision

Co-chairs: Caner Gunaydin, PhD, Weill Cornell Medical College and Jonathan Levy, PhD, Prime Medicine

BALLROOM 3

1:30–1:47 PM | **150**

Identification and Engineering of ABR-004, a Compact, High-Fidelity Nuclease for Therapeutic Gene Editing
Jeffrey Haswell, PhD, Arbor Biotechnologies

1:47–2:04 PM | **151**

Toward an *In Vivo* Approach to Knock-Out the HIV Co-Receptor CCR5 in Hematopoietic Stem Cells Using HDAd Vectors Expressing Base Editors
Anna Anderson, University of Washington

2:04–2:21 PM | **152**

Targeting the Hepatitis B Virus cccDNA via the LNP-Delivered hfCas12Max Nuclease to Eliminate Hepatitis B Virus *In Vitro* and *In Vivo*
Zikang Wang, HuidaGene Therapeutics Co., Ltd.

2:21–2:38 PM | **153**

Non-Viral Gene Editing with Dual Guide RNAs for Chronic Hepatitis B Infection
Ryo Takeuchi, Excision BioTherapeutics

2:38–2:55 PM | **154**

A Comparison Study Between Clustered Regularly Interspaced Short Palindromic Repeats (CRISPR) Guide RNAs and Discovery of LgRNA-33 as an Anti-HBV Agent of Multiple Mechanisms of Action (MOA)
Minghong Zhong, PhD, GeneLancet Biosciences, Inc.

2:55–3:12 PM | **155**

Engineering CasX to Create a Gene Editor with Potent Activity in Non-Human Primates
Addison Wright, Scribe Therapeutics Inc

THURSDAY, MAY 09

ORAL ABSTRACT SESSION

New Avenues for Viral Vectors

Co-chairs: Masataka Suzuki, PhD, Baylor College of Medicine and Patricia Devaux, PhD, Mayo Clinic

BALLROOM 4

1:30–1:45 PM | **156**

HDAd6/3+-cxcr4 Vectors: A Safe and Efficient Vector Platform for *In Vivo* HSC Transduction in Rhesus Macaques
Chang Li, PhD, University of Washington

1:45–2:00 PM | **157**

Foamy Viral Gene Therapy Using a Foamy Viral Vector and Strategies to Induce Transgene Tolerance
Sanal MG, PhD, Institute of Liver and Biliary Sciences

2:00–2:15 PM | **158**

A Novel Method for Generating Unprecedented Unbiased, Ultra-High-Sequence Diversity Libraries of Plasmids and Recombinant Viruses (With Validation) for High-Throughput Screening, Data for Machine Learning (AI) and Targeted Therapy
Praveensingh Hajeri, PhD, University of Minnesota

2:30–2:45 PM | **160**

Delivery of Large Gene Circuits *In Vivo* Using an Engineered Baculovirus Vector for Multifactorial Control of Therapeutic Gene Expression
Lucas Brown, Rice University

2:45–3:00 PM | **161**

Defining the Role of Viral and Cellular Insulators in Promoting Durable HSV-1 Vector Mediated Transgene Expression in Muscle and Brain
Selene Ingusci, PhD, University of Pittsburgh

3:00–3:15 PM | **162**

Adenoviral DNA Replication of HC-AdV Genomes
Jonas Kolibius, University of Zurich

THURSDAY, MAY 09

ORAL ABSTRACT SESSION

Gene Transfer and Genome Editing for Inherited Metabolic Disorders

Co-chairs: Andrés Muro, PhD, International Centre for Genetic Engineering & Biotechnology (ICGEB) and Randy Chandler, PhD, NIH

ROOM 307-308

1:30–1:45 PM **163**

Long-Term Efficacy, Safety, and Extrahepatic Benefit of Liver-Directed Lentiviral Gene Therapy in a Methylmalonic Acidemia Mouse Model

Elena Barbon, PhD, SR-Tiget, IRCCS San Raffaele Scientific Institute

1:45–2:00 PM **164**

Efficient Gene Therapy via Retrograde Injection into the Ductal Systems of Liver and Pancreas

Amita Tiyaboonchai, PhD, Oregon Health and Science University

2:00–2:15 PM **165**

Development of ABO-101, a Novel Gene Editing Therapy for Primary Hyperoxaluria Type 1

Tia DiTommaso, Arbor Biotechnologies

2:15–2:30 PM **166**

Ubiquitous Promoter in AAV9 GAMT Gene Therapy Yields Lower Levels of Guanidinoacetic Acid in the Brain

Puja Patel, UCLA School of Medicine

2:30–2:45 PM **167**

***In Vitro* and *In Vivo* Efficacy of Single-Strand and Self-Complementary AAV9 Vectors to Treat Isolated Cobalamin B Type Methylmalonic Acidemia**

Karenna Choi, BA, NHGRI/NIH

2:45–3:00 PM **168**

Hypomorphic Model of MMAB Deficiency: Resistance to Lethality from Dietary Challenge Conferred by Systemic AAV Gene Therapy

John Selser, NHGRI

THURSDAY, MAY 09

ORAL ABSTRACT SESSION (CONTINUED)

Gene Transfer and Genome Editing for Inherited Metabolic Disorders

Co-chairs: Andrés Muro, PhD, International Centre for Genetic Engineering & Biotechnology (ICGEB) and Randy Chandler, PhD, NIH

ROOM 307-308

3:00–3:15 PM | 169

***In Vivo* Correction of Metabolic Pathogenic Variants via Base Editing and Prime Editing: Toward the Equitable Treatment of Hepatic Inborn Errors of Metabolism**

Xiao Wang, PhD, UPenn

THURSDAY, MAY 09

ORAL ABSTRACT SESSION

Correction of Genetic Disorders of the Blood and Immune System

Co-chairs: Anne Galy, PhD, Inserm and
Ngoc Tung Tran, PhD, Indiana University School of Medicine

ROOM 314-317

1:30–1:45 PM | **170**
Highly Specific Non-Viral Gene Editing with P-KLKB1-101 for Hereditary Angioedema
Oscar Alvarez, PhD, Poseida Therapeutics

1:45–2:00 PM | **171**
Base Editing of BCL11A Cis-Regulatory Regions to Treat Beta-Hemoglobinopathies
Letizia Fontana, Imagine Institute

2:00–2:15 PM | **172**
A Novel Model of X-Linked Sideroblastic Anemia (Alas2-KO, XLSA) with Ring Sideroblasts, Severe Anemia, and Iron Overload is Rescued by Lentiviral Gene Transfer
Carlo Castruccio Castracani, PharmD, PhD, The Children's Hospital of Philadelphia

2:15–2:30 PM | **173**
Development of Best-in-Class Gene Editing Therapy for β-Hemoglobinopathies Using Innovative Transformer Base Editor (tBE)
Bingbing Wu, CorrectSequence Therapeutics Co., Ltd

2:30–2:45 PM | **174**
A Non-Genotoxic Conditioning Regimen Based on a Combination of Monoclonal Antibodies and Hematopoietic Stem and Progenitors Cell Mobilizers Enhances Engraftment Levels and Theraputic Efficacy
Isabel Ojeda Pérez, PhD, CIEMAT

2:45–3:00 PM | **175**
Hematopoietic Stem Cell Gene Therapy for the Treatment of X-Linked Agammaglobulinemia
Christopher Luthers, UCLA

3:00–3:15 PM | **176**
Lipid Nanoparticle Delivery of Circular mRNA Improves Hematopoietic Function in Fanconi Anemia HSPCs
Sarah Adams, Children's Hospital of Philadelphia

THURSDAY, MAY 09

ORAL ABSTRACT SESSION

Ophthalmic and Auditory: Disease Focus

Co-chairs: Luk Vandenberghe, PhD, Harvard and
Paul Ranum, PhD, Children's Hospital of Philadelphia

ROOM 318-323

1:30–1:45 PM **177**

Gene Therapy for Clarin-2, a New Deafness Gene in Humans and Mice
Clara Mendia, Institut de l'Audition / Institut Pasteur

1:45–2:00 PM **178**

***In-Vitro* and *In-Vivo* Studies of HG202 as a CRISPR/Cas13 RNA Targeting Therapy for Macular Degeneration**
Weiwei Wu, HuidaGene Therapeutics Co., Ltd.

2:00–2:15 PM **179**

AAV-Mediated RhoA Knockdown as a Potential Gene Therapy for Glaucoma
Tapan Sharma, PhD, UMass Chan Medical School

2:15–2:30 PM **180**

AAV-Mediated Delivery of a Novel CasX-Editor Molecule Achieves Allele-Specific and Potent Editing of P23H Rhodopsin in a Mouse Model of Retinitis Pigmentosa
Cécile Fortuny, PhD, Scribe Therapeutics Inc

2:30–2:45 PM **181**

LX103 a Novel Recombinant AAV Gene Therapy for Intravitreal Treatment of X Linked Juvenile Retinoschisis
Zhuoyu Ni, PhD, Innostellar Biotherapeutics

2:45–3:00 PM **182**

AAV Vectorized Antibody Fragment-Mediated Dual Complement Pathway Inhibition for the Treatment of AMD
Suzanne Jacobs, PhD, Sanofi

3:00–3:15 PM **183**

Pre-Clinical Study of RM-101, a Novel AAV-Based Gene Therapy for USH2A-Related Retinitis Pigmentosa
Hui Xu, PhD, Reforgene Medicine

THURSDAY, MAY 09

SPONSORED SYMPOSIUM

ROOM 337-338

1:30–2:00 PM

FORGE BIOLOGICS:
Forge's discovery and modification of genetic elements for enhanced productivity and efficacy of AAV-based gene therapies

ROOM 337-338

2:30–3:00 PM

CATALENT CELL & GENE THERAPIES:
Accelerate Your AAV: Catalent's UpTempo Platform Delivers Quality, Yield, and Speed

ROOM 337-338

3:45–4:15 PM

MILLIPORESIGMA:
Maximizing production and quality of lentiviral vectors for *ex-vivo* gene therapies

ROOM 337-338

4:45–5:15 PM

CYTIVA:
Ex Vivo Engineering of T cells and Hematopoietic Stem Cells Using RNA-Lipid Nanoparticles for Cell and Gene Therapies

THURSDAY, MAY 09

SCIENTIFIC SYMPOSIUM

Gene and Cell Therapy in Australia

BALLROOM 2

1:30–1:51 PM

Gene Therapy for Genetic Diseases of Childhood; The Australian Landscape
Ian Alexander, MD, PhD, Sydney Children's Hospitals Network

1:51–2:12 PM

Viral Vector Development in Australia
Leszek Lisowski, PhD, Children's Medical Research Institute

2:33–2:54 PM

Using Gene Therapy Technologies To Study and Treat Heritable and and Acquired Muscle Diseases
Paul Gregorevic, The University of Melbourne

2:54–3:15 PM

Gene Therapy for Eye Disorders
Livia Carvalho, PhD, University of Melbourne

THURSDAY, MAY 09

SCIENTIFIC SYMPOSIUM

How to Develop Gene and Cell Therapies with a DEI Forward Approach (Organized by the DEI Committee)

Co-chairs: Christina Fuentes, PhD, Dark Horse Consulting Group and Jorge Santiago-Ortiz, PhD, Apertura Gene Therapy

ROOM 339-342

1:30–2:00 PM

Round Table: A Historical Perspective
Maritza McIntyre, PhD, Advanced Therapies Partners and S. Kaye Spratt, PhD, Spratt Advanced Gene and Cell Therapy Regulatory Consulting, LLC

2:00–2:25 PM

Incorporating Principles of DEI into Grant Review: Perspectives from a Funding Agency
Hayley Lam, PhD, CIRM

2:25–2:50 PM

Regulatory and Clinical Research Strategies on How To Develop Gene and Cell Therapies with a DEI Forward Approach
Isaac Rodriguez-Chavez, PhD, MHS, MS, 4Biosolutions Consulting

2:50–3:15 PM

Panel Discussion
Maritza McIntyre, PhD
S. Kaye Spratt, PhD
Hayley Lam, PhD
Isaac Rodriguez-Chavez, PhD, MHS, MS

THURSDAY, MAY 09

TOOLS AND TECHNOLOGY FORUM

Tools and Technology Forum 3

Chair: Jim Wang, PhD, Regeneron Pharmaceuticals

EXHIBIT HALL PRESENTATON THEATER

2:00–2:15 PM

STEMCELL TECHNOLOGIES:
New GMP Reagents for Reliable Cell Activation and Expansion in T Cell Therapy Development and Manufacturing

2:15–2:30 PM

GENEVOYAGER:
Introduction to Genevoyager's One-Bac 4.0 System: Addressing the Bottlenecks and Challenges of Adeno-Associated Virus (AAV) Manufacturing

2:30–2:45 PM

TOUCHLIGHT DNA SERVICES:
Touchlight doggybone DNA - In 15 minutes Learn How We are Enabling the Gene Therapy Revolution

2:45–3:00 PM

ANDELYN BIOSCIENCES:
Andelyn's AAV Curator™ Platform: Enabling Gene Therapy Program Success Through Data-Driven and Experience-Based Optimization-By-Design™

3:00–3:15 PM

BIO-RAD LABORATORIES:
Accelerating the Evaluation of Empty and Full Capsids: Utilizing Droplet Digital PCR for Increased Speed and Reliability

THURSDAY, MAY 09

SCIENTIFIC SYMPOSIUM

Enabling Analytics for Decentralized and Point of Care Manufacturing (Organized by the CMC Committee)

Co-chairs: Katy Spink, PhD, Dark Horse Consulting Group and
Sadik Kassim, PhD, Danaher Life Sciences

BALLROOM 1

3:45–4:05 PM

Defining the Issues: FDA's Approach to Centralized, Decentralized, and Point of Care Manufacturing for CGTs
Kimberly Schultz, PhD, FDA

4:05–4:25 PM

The Importance of Analytics for CGT Manufacturing Models and How to Enable Them
Therese Choquette, PhD, Tigen Pharma

4:25–4:45 PM

Enabling Distributive Point of Care Manufacturing of CAR-T and Other Gene-modified Cell Therapies Within an Academic Medical Center Network
Rimas Orentas, PhD, Caring Cross

4:45–5:05 PM

What Are Regulatory Expectations for Analytical Testing of Bedside Cell Therapies: The Lupagen Experience Across Several Jurisdictions
David Peritt, PhD, Lupagen

5:05–5:30 PM

Panel Discussion
Kimberly Schultz, PhD
Therese Choquette, PhD
Rimas Orentas, PhD
David Peritt, PhD

THURSDAY, MAY 09

SCIENTIFIC SYMPOSIUM

Lipid Nanoparticles (LNPs): From Basic Development to the Treatment of Human Diseases (Organized by the Nanoagents and Synthetic Forumulations Committee)

Co-chairs: Ramesh Rajagopal, PhD, University of Oklahoma Health Sciences and Roman Bogorad, PhD, NewCo

ROOM 327-329

3:45–4:11 PM

Development of Nanoparticles for Extrahepatic Targeting
Jacob Witten, MIT

4:11–4:37 PM

Bioinspired Artificial Exosomes Based on Lipidnanoparticles Carrying let-7b-5p Promoteangiogenesis *In Vitro* and *In Vivo*
Costanza Emanueli, PhD, Imperial College London National Heart & Lung Institute

4:37–5:03 PM

Transcytosis in Tumor Delivery of RNA
Song Li, MD, PhD, University of Pittsburgh School of Pharmacy

5:03–5:29 PM

Messenger RNA Therapies in Metabolic Diseases
Gerard Vockley, MD, PhD, University of Pittsburgh

THURSDAY, MAY 09

EDUCATION SESSION

Education and Regulations in Gene and Cell Therapy (Organized by the Education Committee)

Co-chairs: Juliana Alvarez Argote, MD, Medical College of Wisconsin and Mary Kathryn McKenna, PhD, Baylor College of Medicine

BALLROOM 2

3:45–3:55 PM — **Opening Remarks**

3:55–4:25 PM — **Fundamentals of the FDA Regulatory Framework for Gene Therapy**
Daniela Drago, PhD, NDA Partners

4:25–4:55 PM — **Cell and Gene Therapies CMC Aspects**
Tim Olson, MD, PhD, Children's Hospital of Philadelphia

4:55–5:25 PM — **Pre-Clinical Aspects of Cell and Gene Therapy**
Elvira Argus, PhD, FDA, CBER

4:25–5:30 PM — **Closing Remarks**

THURSDAY, MAY 09

TOOLS AND TECHNOLOGY FORUM

Tools and Technology Forum 4

Chair: Jim Wang, PhD, Regeneron Pharmaceuticals

EXHIBIT HALL PRESENTATION THEATER

3:45–4:00 PM

CATALENT CELL & GENE THERAPIES:
Development of UpTempo CAR-T Platform and iPSC Platform to Speed Your Path to Patient for Your Autologous and Allogeneic Cell Therapies

4:00–4:15 PM

PLASMIDFACTORY GMBH:
Presenting an AAV ITR Healthpack: Maintaining ITR integrity in AAV transfer plasmids

4:15–4:30 PM

SCIEX:
Multiple quality attribute analysis of the mRNA and mRNA-lipid nanoparticle (mRNA-LNP) by capillary electrophoresis (CE)

4:30–4:45 PM

EMIT IMAGING, INC.:
Cryo-Fluorescence Tomography: Transformative 3D Imaging to Monitor Gene and Cell Therapies

4:45–5:00 PM

ANEMOCYTE:
Manufacturing Biotech Innovation: shaping tomorrow's therapies

5:00–5:15 PM

TEKNOVA:
The absence of highly purified viral vectors for bioproduction remains a barrier to the broader market adoption of gene therapies. Teknova delivers customer-specified and proprietary reagents to support gene therapy developers from early-stage research and DOEs, to process development and clinical manufacturing, reducing their time to market.

5:15–5:30 PM

XIOGENIX:
Disrupt. Create. Advance. Revolutionizing Fill & Finish

THURSDAY, MAY 09

ORAL ABSTRACT SESSION

Targeted Gene Insertion

Co-chairs: Morgan Maeder, PhD, Chroma Medicine and
Shengdar Tsai, PhD, St. Jude Children's Research Hospital

BALLROOM 3

3:45–4:00 PM | **191**

Efficient and Versatile Programmable Large-Gene Integration by Evolved Recombinases and Prime Editing
Xin Gao, PhD, Broad Institute of MIT and Harvard

4:00–4:15 PM | **192**

Directed Evolution of Bxb1 for the Development of Modular Integrases (MInts)
Sebastian Arangundy Franklin, Sangamo Therapeutics

4:15–4:30 PM | **193**

Large Serine Integrase Off-Target Discovery and Validation for Therapeutic Human Genome Editing
Dane Hazelbaker, Tome Biosciences

4:30–4:45 PM | **194**

***In Vivo* Expansion of Gene-Targeted Hepatocytes through Transient Inhibition of an Essential Gene**
Marco De Giorgi, PhD, Baylor College of Medicine

4:45–5:00 PM | **195**

Programmable Genomic Integration in Induced Pluripotent Stem Cells and Hematopoietic Stem and Progenitor Cells
Ravindra Amunugama, Tome Biosciences

5:00–5:15 PM | **196**

Internal Base Modifications Enable Highly Efficient Gene Insertion in Primary Cells Using Single Stranded DNA
Sriram Vaidyanathan, PhD, Nationwide Children's Hospital

5:15–5:30 PM | **197**

Targeted Gene Insertion of Vectorized Monoclonal Antibodies in Non-Human Primates Overcomes AAV Genome Silencing in the Liver and Supports High, Sustained *In Vivo* Expression of Functional Antibodies
Rachel Sattler, Regeneron Pharmaceuticals, Inc.

THURSDAY, MAY 09

ORAL ABSTRACT SESSION

Emerging Viral Vectors

Co-chairs: Sébastien Levesque, PhD, UAMS and
Masato Yamamoto, MD, PhD, University of Minnesota

BALLROOM 4

3:45–4:00 PM | **198**
Lentiviral Gene Therapy to the Liver for Homozygous Familial Hypercholesterolemia (FH): Assessing the Best Vector Design
Cesare Canepari, SR-Tiget

4:00–4:15 PM | **199**
Alpha-Retrovirus-Based Virus-Like Particles for Efficient Gene Editor Delivery into Hematopoietic Stem Cells
Denise Klatt, PhD, Boston Children's Hospital, Harvard Medical School

4:15–4:30 PM | **200**
Structure Derived Insights into the Similarities and Differences in Coagulation Factor Binding to Species C and Species D Adenoviruses
Michael Barry, PhD, Mayo Clinic

4:30–4:45 PM | **201**
Rational Design of a Detargeted Vesiculovirus Fusogen to Enable Targeted *In Vivo* Gene Delivery
Ronnie Russell, PhD, Interius BioTherapeutics

4:45–5:00 PM | **202**
Engineering Single-Cycle Measles Vector for CRISPR/Cas9 Gene Editing
Patricia Devaux, PhD, Mayo Clinic

5:00–5:15 PM | **203**
Anellovectors, a Gene Delivery Platform Based on Commensal Human Anelloviruses, Have the Potential to Evade the Immune System and Deliver DNA Payloads to a Broad Range of Tissues in a Redosable Manner
Chris Wright, Ring Therapeutics

5:15–5:30 PM | **204**
Novel Self-Replicating RNA Vectors Broaden Therapeutic Window and Expand Use Outside of Vaccines
Parinaz Aliahmad, Replicate Bioscience, Inc

THURSDAY, MAY 09

ORAL ABSTRACT SESSION

CAR T-cell Therapies

Co-chairs: Charles Gersbach, PhD, Duke University and
Sarwish Rafiq, PhD, Emory University

ROOM 307-308

3:45–4:00 PM **184**
Innovative Applications of CAR T-Cell Therapy in Organ Transplant Patients
Hong Qin, Mayo Clinic - Jacksonville

4:00–4:15 PM **185**
Discovering Novel Transcriptional Regulators of T Cell Exhaustion for Epigenetic Reversal of T Cell Dysfunction
Christian McRoberts Amador, Duke University

4:15–4:30 PM **186**
T-Cell Epigenetic Reprogramming with TGIF2LX-Overexpression Enhances Adoptive Cell Therapy
Tomer Rotstein, Duke University

4:30–4:45 PM **187**
Identification of Exhaustion-Associated Genes by Non Viral Editing of Human CD8+ T Cells
Brandon Simone, PhD, UPenn

4:45–5:00 PM **188**
CRISPR Engineering of Armoured CAR T Cells Enables Tumour-Restricted Payload Delivery with Enhanced Safety and Efficacy
Amanda Chen, The Peter MacCallum Cancer Centre

5:00–5:15 PM **189**
Preclinical Specificity and Potency Evaluation of a Novel CD19-Specific KIR-CAR T Cell Therapy (SynKIR-310)
Nora Yucel, PhD, Verismo Therapeutics

5:15–5:30 PM **190**
***In Vivo* Generation of αCD19-CAR T Cells Using a Novel LV-based Platform Successfully Clears Advanced NALM-6 Tumor without Noticeable Toxicity**
Karina Krotova, PhD, Imanis Life Sciences

THURSDAY, MAY 09

ORAL ABSTRACT SESSION

Developing the Next Generation of Muscle-Targeted Gene Delivery Vehicles via AAV Capsid Engineering

Co-chairs: Mathieu Nonnenmacher, PhD, Voyager Therapeutics and Nathalie Clement, PhD, Siren Biotechnology

ROOM 309-310

3:45–4:00 PM **212**
An AI-Generated Novel AAV Variant Targeting Pan-Muscle and Sparing from the Liver in NHP
Lijia Ma, Westlake University

4:15–4:30 PM **214**
Directed Evolution of Novel MyoAAV Capsid Variants Enabling Effective Systemic Muscle Transduction While De-Targeting the Liver in Non-Human Primates
Sharif Tabebordbar, Kate Therapeutics

4:30–4:45 PM **215**
Identification of Natural Human-Derived AAV2 Variants with Muscle Tropic Properties in Non-Human Primate Screens
Fang Zhang, UMass Chan Medical School

4:45–5:00 PM **216**
Novel Neurotropic and Myotropic AAV Capsids for Translational Gene Therapy Applications
Irvin Garza, UT Southwestern Medical Center

5:00–5:15 PM **217**
An AAV Capsid Programmed to Bind Human and Macaque Receptors Mediates Enhanced Gene Delivery to the CNS and Skeletal Muscle *In Vivo*
Jason Wu, PhD, Broad Institute of MIT and Harvard

5:15–5:30 PM **218**
Retargeting of AAV Using Bispecific Antibodies
Sven Moller-Tank, Regeneron

THURSDAY, MAY 09

ORAL ABSTRACT SESSION

Exosomes, Virus like Particles, and LNPs

Co-chairs: Camila Hochman-Mendez, PhD, Texas Heart Institute and Lakshmi Raj, PhD, Novartis

ROOM 314-317

3:45–4:00 PM **219**

Durable DNA-Based Gene Therapy Using the Non-Viral Fusogenix Proteolipid Vehicle Platform
Arun Raturi, PhD, Entos Pharmaceuticals

4:00–4:15 PM **220**

Engineered Virus-Like Particles for Transient Delivery of Prime Editor Ribonucleoprotein Complexes *In Vivo*
Meirui An, Broad Institute

4:15–4:30 PM **221**

New Biological Insights into Extracellular Vesicle Associated AAV Help Improve Vector Yield and Provides Rational for Intraluminal AAV Packing
Casey Maguire, PhD, Massachusetts General Hospital

4:30–4:45 PM **222**

Development of a First-in-Class Adjustable Dose Gene Therapy Platform and its Application to the Treatment of Type 2 Diabetes and Obesity
Alexei Goraltchouk, Remedium Bio, Inc.

4:45–5:00 PM **223**

Exosome-Mediated Intracellular Delivery of Arginase 1: A Potential Application for the Treatment of Arginase 1 Deficiency
Li-En Hsieh, PhD, Capricor Therapeutics

5:00–5:15 PM **224**

Engineering Endogenous Loading Methods for Therapeutic RNA Cargo into Extracellular Vesicles
Steven Jay, University of Maryland

5:15–5:30 PM **225**

Utilizing the Endogenous NF1 Promoter for Widespread Cell Specific Expression
Miguel Sena-Esteves, PhD, UMass Chan Medical School

THURSDAY, MAY 09

ORAL ABSTRACT SESSION

Cancer Immunotherapy and Cancer Vaccines

Co-chairs: Eric Smith, MD, PhD, Dana Farber Cancer Institute and Maksim Mamonkin, PhD, Baylor College of Medicine

ROOM 318-323

3:45–4:00 PM **226**

Interferon Alpha Gene Therapy Alters Tumor Metabolism in Bladder Cancer
Sharada Mokkapati, PhD, UT MD Anderson Cancer Center

4:00–4:15 PM **227**

Targeting AML with Naturally Occurring CD7 Negative T Cells Expressing CD7 CAR
Unmesha Thanekar, St. Jude Children's Research Hospital

4:15–4:30 PM **228**

A mRNA Lipid Nanoparticle Incorporated Nanofiber-Hydrogel Composite Generates a Local Immunostimulatory Niche for Cancer Immunotherapy
Yining Zhu, Johns Hopkins University

4:30–4:45 PM **229**

Targeting EphA3 on Glioblastoma Stem Cells with CART Cell Therapy to Overcome GBM Recurrence
Ekene Ogbodo, PhD, Mayo Clinic

4:45–5:00 PM **230**

Combining the Anti-Viral and Anti-Tumor CD8+ T Cell Response Facilitates Immune Checkpoint Blockade Therapy and Oncolytic Virotherapy
Richard Vile, PhD, Mayo Clinic

5:00–5:15 PM **231**

Adenovirus Capsid-Based Anti-Fentanyl Vaccine Protects Against Fentanyl-Induced Cardio-Respiratory Depression
Stephen Kaminksy, Weill Cornell Medical College

THURSDAY, MAY 09

ORAL ABSTRACT SESSION (CONTINUED)

Cancer Immunotherapy and Cancer Vaccines

Co-chairs: Eric Smith, MD, PhD, Dana Farber Cancer Institute and Maksim Mamonkin, PhD, Baylor College of Medicine

ROOM 318-323

5:15–5:30 PM | 232

The Novel Autologous AML Vaccine, TriLeukeVax, Stimulates Robust Anti-Leukemic Patient T Cell Responses

Jacob Du, UCSF

THURSDAY, MAY 09

ORAL ABSTRACT SESSION

Liver Genetic Diseases

Co-chairs: Gloria Gonzalez-Aseguinolaza, PhD, Vivet Therapeutics and Sandeep Kumar, PhD, Indiana University

ROOM 324-326

3:45–4:00 PM **205**

Correction of Ornithine Transcarbamylase Deficiency Using a Mutation-Independent CRISPR-Cas9-Mediated Targeted Integration Approach with Restoration of Liver-Wide Metabolic Zonation
Samantha Ginn, PhD, Children's Medical Research Institute

4:00–4:15 PM **206**

Safe and Effective Liver Directed AAV-Mediated Homology Independent Targeted Integration in Mouse Models of Inherited Diseases
Federica Esposito, TIGEM

4:15–4:30 PM **207**

Correlation of Antigen Expression with Epigenetic Modifications After rAAV Delivery of a Hyperactive Human Factor IX Variant in Mice and Rhesus Macaques
Mark Kay, MD, PhD, Stanford School of Medicine

4:30–4:45 PM **208**

Knock-Out, Tissue Specific Transgenic, and Knock-In Mouse Models of PCCB Deficiency Recapitulate of the Clinical Spectrum of Propionic Acidemia and Enable the First Successful Proof-of-Principial Gene Therapy Studies
Randy Chandler, PhD, NIH

4:45–5:00 PM **209**

***In Vivo* Lentiviral Gene Therapy for Arc Syndrome**
Andrei Claudiu Cozmescu, PhD, University College London

5:00–5:15 PM **210**

Sustained FVIII Expression with a Tolerable, Titratable, Fully Non-Viral Gene Therapy for Hemophilia A
Brian Truong, PhD, Poseida Therapeutics

THURSDAY, MAY 09

ORAL ABSTRACT SESSION (CONTINUED)

Liver Genetic Diseases

Co-chairs: Gloria Gonzalez-Aseguinolaza, PhD, Vivet Therapeutics and Sandeep Kumar, PhD, Indiana University

ROOM 324-326

5:15–5:30 PM | **211**

Pursuing Optimal Therapeutic Conditions through *In Vivo* Lentiviral Vector Gene Therapy for Primary Hyperoxaluria Type 1

Andrea Molinos Vicente, Biomedical Innovation Unit, CIEMAT, CIBERER

THURSDAY, MAY 09

AWARDS

Career Development & DEI Awardee Presentations II

Chair: Blythe Sather, PhD, Tune Therapeutics

ROOM 339-342

3:45–4:11 PM

Readily Programmable RNA Interference in Extracellular Vesicles for Biomedical Applications
Tristan Scott, PhD, City of Hope

4:11–4:37 PM

Repeat AAV Dosing and Overcoming Preexisting Immunity Through TLR9 Inhibition
Martin Kang, PhD, The Medical University of South Carolina

4:37–5:03 PM

Engineering Super-Suppressor Tr1 Cells
Alma-Martina Cepika, MD, PhD, Stanford University School of Medicine

5:03–5:29 PM

Oncolytic HSV Therapy: Beyond the Role of Notch
Karina Vazquez-Arreguin, Augusta University - Georgia Cancer Center

FRIDAY, MAY 10

ORAL ABSTRACT SESSION

Late-Breaking Abstracts II

Co-chairs: Kah-Whye Peng, PhD, Mayo Clinic and
Rachel Bailey, PhD, UT Southwestern Medical Center

BALLROOM 4

8:00–8:15 AM
LBA-8

REKLAIM, A Phase I/II Clinical Trial Using a Novel Immune Modulation Strategy for Systemic Administration of FBX-101 (AAVrh10.GALC) After Umbilical Cord Blood Transplantation for the Treatment of Infantile Krabbe Disease
Maria Escolar, MD, Forge Biologics

8:15–8:30 AM
LBA-9

Therapeutic Vaccination for the Elimination of HPV16+ High Grade Cervical Dysplasia: A Phase I Clinical Trial of the DNA Vaccine pNGVL4aCRTE6E7L2, Administered via the TriGrid™ Electroporation Device
Richard Roden, PhD, Johns Hopkins University

8:30–8:45 AM
LBA-10

Interim Safety and Efficacy of Anti-CD70 CAR-NKT (CGC729) for Patients with Refractory Metastatic Renal Cell Carcinoma
Dingwei Ye, PhD, Fudan University Shanghai Cancer Center

8:45–9:00 AM
LBA-11

Academic BCMA-CART Cells HBI0101, a Promising Approach for the Treatment of LC Amyloidosis
Miri Assayag, PhD, Hadassah Medical Center

9:00–9:15 AM
LBA-12

Oncolytic Adenovirus ORCA-010 Induces Systemic Tumor-Specific T cell Responses and Activation of the Tumor Microenvironment in Prostate Cancer: A Phase I/IIa Clinical Trial
Reza Nadafi, PhD, ORCA Therapeutics

FRIDAY, MAY 10

ORAL ABSTRACT SESSION (CONTINUED)

Late-Breaking Abstracts II

Co-chairs: Kah-Whye Peng, PhD, Mayo Clinic and
Rachel Bailey, PhD, UT Southwestern Medical Center

BALLROOM 4

9:15–9:30 AM
LBA-13

Updated Clinical Results of Metabolically Armored CD19 CAR-T Cells for Safe and Effective Treatment of Relapsed or Refractory B-Cell Hematological Malignancies at Extremely Low Doses
Min Gao, Leman Biotech Co.

9:30–9:45 AM
LBA-14

Allograft Overexpression of Modified PD-L1 Confers Tolerance after Heart Transplantation in a Murine Model
Krish Dewan, MD, Duke University Medical Center

FRIDAY, MAY 10

ORAL ABSTRACT SESSION

Pharmacology Toxicology Studies and Analytics Assay Development

Co-chairs: Eva Andres-Mateos, PhD, Atsena Therapeutics and
Julia Davydova, MD, PhD, University of Minnesota

ROOM 339-342

8:00–8:15 AM **233**

CAR T-Cell-Derived TNF Drives Cytokine Release Syndrome and Enhances M1 Macrophage Differentiation
Pieter Lindenbergh, Memorial Sloan Kettering Cancer Center

8:15–8:30 AM **234**

Identification of Cellular Factors that Contribute to Peripheral Blood-Derived CAR-NK Cell Mediated Inflammatory Toxicities
Supreet Khanal, PhD, FDA

8:30–8:45 AM **235**

Pharmacokinetics and Vector Shedding in NHPs Following a Single Intravenous Infusion of a CD20-Targeted Engineered Lentiviral Vector
Elaine Youngman, Interius BioTherapeutics

8:45–9:00 AM **236**

Characterization of Preclinical Models for Systemically Delivered Vectors Based on Human Adenoviruses HAdV-B3, HAdV-B11, HAdV-C5, HAdV-C6, and HAdV-D26
Margarita Romanenko, PhD, University of Minnesota

9:00–9:15 AM **237**

Potency Assay Enabling Both *Ex Vivo* and *In Vivo* Genome Editing Therapeutics for Sickle Cell Disease
Utku Goreke, PhD, Innovative Genomics Institute, UC Berkeley

9:15–9:30 AM **238**

Association Between Potency Critical Quality Attributes and Clinical Efficacy Across Lentiviral Vector Cell and Gene Therapy Products
Ilya Shestopalov, PhD, bluebird bio

FRIDAY, MAY 10

ORAL ABSTRACT SESSION (CONTINUED)

Pharmacology Toxicology Studies and Analytics Assay Development

Co-chairs: Eva Andres-Mateos, PhD, Atsena Therapeutics and
Julia Davydova, MD, PhD, University of Minnesota

ROOM 339-342

9:30–9:45 AM 239

Transcriptional Changes in Non-Human Primate Tissues After Intrathecal Delivery of Serotype 9 Adeno-Associated Viral Vector: Insights into Organ Toxicities

Fumiaki Aihara, Novartis

FRIDAY, MAY 10

SCIENTIFIC SYMPOSIUM

Therapeutic Vaccines for Persistent Viral Infections (Organized by the Infectious Diseases and Vaccines Committee)

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

BALLROOM 1

8:00–8:25 AM

Approaches to HIV Vaccines, Overview of Genetic Based HIV Vaccines
Gary Kobinger, PhD, UTMB

8:25–8:50 AM

Adenoviral Based HPV Vaccines
Mohsen Mohammadi, The Wistar Institute

8:50–9:15 AM

Vaccines as Boosting Agents for Engineered Lymphocytes
Christopher Peterson, PhD, Fred Hutchinson Cancer Center

9:15–9:45 AM

Panel Discussion
Gary Kobinger, PhD
Mohsen Mohammadi
Christopher Peterson, PhD

FRIDAY, MAY 10

SCIENTIFIC SYMPOSIUM

Advances in Genome Editing: *In Vivo* Small Edits and the Promise of Large Insertions (Organized by the Genome Editing Committee)

Co-chairs: Janice Chen, PhD, Mammoth Biosciences and
Le Cong, PhD, Stanford University

BALLROOM 2

8:00–8:26 AM

Integrase-mediated Programmable Genomic Integration (I-PGI)
Rahul Kakkar, MD, Tome Biosciences

8:26–8:52 AM

Precision Genome Editing for Treatment of Genetic Neurological Diseases
Mandana Arbab, PhD, Harvard Medical School and Boston Children's Hospital

8:52–9:18 AM

DNA Polymerase Editors (DPEs)
Erik Sontheimer, PhD, UMass Chan Medical School

9:18–9:44 AM

EBT-101: First-in-human Clinical Trial of Systemic CRISPR-Cas9 Multiplex Targeting of Latent HIV
Rachel Presti, MD, PhD, Washington University

FRIDAY, MAY 10

SCIENTIFIC SYMPOSIUM

Immune Suppression 2.0: Next Generation Therapeutics for Gene Therapy (Organized by the Immune Responses Committee)

Co-chairs: David Markusic, PhD, Spark Therapeutics and
Melissa Spencer, PhD, UCLA

BALLROOM 3

8:00–8:26 AM

Overview of Current Immune Suppression
Shari Gordon, PhD, Asklepious Biopharmaceutical

8:26–8:52 AM

Drugs to Address Complement Activation/ Up and Coming Treatments to Block
Genevieve Laforet, MD, PhD, Aspa Therapeutics

8:52–9:18 AM

Clinical Trials-Using Drugs that Suppress Immunity to Improve Safety and Efficacy of AAV-Mediated Gene Therapy
Barry Byrne, MD, PhD, University of Florida

9:18–9:44 AM

Preclinical Highlight of Up and Coming Approaches
Moanaro Biswas, PhD, Indiana University

FRIDAY, MAY 10

SCIENTIFIC SYMPOSIUM

Collaboration Models for Global Gene Therapy (Organized by the Global Outreach Committee)

Co-chairs: Liudmila Cebotaru, MD, JD, Johns Hopkins and
Meisam Naeimi Kararoudi, DVM, PhD, Nationwide Children's Hospital and The Ohio State University

ROOM 307-308

8:00–8:05 AM

Announcement of Global Outreach Abstract Travel Award
Jayandharan Rao, PhD, Institute of Technology, Kanpur, India

8:05–8:15 AM

Global Gene Therapy Training: ASGCT Partnership in Tanzania
Kenneth Cornetta, MD, Indiana University

8:15–8:35 AM

Industry Partnership: Hemophilia A Gene Therapy Trial in South Africa
Johnny Mahlangu, MBBCh, University of Witwatersrand

8:35–8:55 AM

Local Innovation: Indigenous CAR-T Cell Therapy Trial in India
Hasmukh Jain, MD, DM, Tata Memorial Centre

8:55–9:15 AM

Modular Academic Collaboration for Rapid Deployment of Genetic Therapy: Bio-Manguinhos in Brazil
Carla Wolanski, Institute of Technology on Immunobiologicals (Bio-Manguinhos)/Oswaldo Cruz Foundation (Fiocruz)

9:15–9:45 AM

Panel Discussion
Kenneth Cornetta, MD
Johnny Mahlangu, MBBCh
Hasmukh Jain, MD, DM
Carla Wolanski

FRIDAY, MAY 10

SCIENTIFIC SYMPOSIUM

2nd Annual 5-Minute Thesis Challenge

Co-chairs: Li Ou, PhD, University of Minnesota and
Sarwish Rafiq, PhD, Emory University

ROOM 318-323

8:00–9:45 AM

JUDGES:

Alim Ladha, PHD, MPM Capital
Kathleen Flynn, National Tay-Sachs and Allied Diseases Association
John Vroom, Co-Founder & CBO at Kano Therapeutics

PARTICIPANTS:

8:10–8:25 AM

Durable HTT Silencing Using Non-evolved dCas9 Epigenome Editors in Patient-derived Cells
Jennifer Waldo, UC Davis

8:25–8:40 AM

Efficient Dual-AAV platforms for Large Transgene Delivery
Mariana Ferreira, IBET

8:40–8:55 AM

Targeting Underlying Disease Mechanisms to Execute an Efficacious and Translatable AAV-mediated Gene Supplementation Strategy for Vanishing White Matter Disease
Jessica Herstine

8:55–9:10 AM

The mitoDdCBE System as a Mitochondrial Gene Therapy Approach
Jose Domingo Barrera-Paez, PhD

9:10–9:25 AM

Purification of Viral Vectors Using Continuous Aqueous Two-Phase Extraction
Natalie Nold, Michigan Technological University

FRIDAY, MAY 10

FIRESIDE CHAT

Fireside Chat: Supporting Translational Science and Transformative Therapies

Chair: Jeffrey Chamberlain, PhD, University of Washington

ROOM 314-317

8:00–9:45 AM

Supporting Translational Science and Transformative Therapies at NINDS
Amir Tamiz, PhD, National Institute of Neurological Disorders and Stroke

Supporting Translational Science and Transformative Therapies at ARPA-H
Amy Jenkins, PhD, Advanced Research Projects Agency for Health

Supporting Translational Science and Transformative Therapies at NHLBI
Gary Gibbons, MD, National Heart, Lung, and Blood Institute

Supporting Translational Science and Transformative Therapies at NCATS
Joni Rutter, PhD, National Center for Advancing Translational Sciences

FRIDAY, MAY 10

SPONSORED SYMPOSIUM

ROOM 337-338

8:00–8:30 AM

THERMO FISHER SCIENTIFIC:
Revolutionizing gene therapy services: A synergistic approach to analytics and scaling up through CDMO-CRO collaboration

FRIDAY, MAY 10

GENERAL SESSION

OUTSTANDING NEW INVESTIGATOR SYMPOSIUM

Co-Chairs: Paula Cannon, PhD, USC and Terence R. Flotte, MD, UMass Chan Medical School

HALL A-B

10:15–10:37 AM

Announcing the Recipients of the Career Development Awards, Sonia Skarlatos Public Service Award, and the Exemplary Service Award

10:37–10:57 AM

Advanced Genetic Engineering of Hematopoiesis for the Treatment of Human Diseases
Pietro Genovese, PhD, Dana-Farber Cancer Institute

10:58–11:18 AM

Cell Type-programmable Delivery to Enable Genome Engineering *In Vivo*
Jenny Hamilton, PhD, UC Berkeley + Azalea Therapeutics

11:19–11:39 AM

Non-Genotoxic Conditioning and Innovative New Therapies for Fanconi Anemia
Agnieszka Czechowicz, MD, PhD, Stanford Medicine

11:40 AM–12:00 PM

Developing Highly Potent and Safe Muscle Gene Therapy Candidates by Combining Effective Liver De-targeted MyoAAV Capsids and Cell Type Specific Regulatory Elements
Sharif Tabebordbar, PhD, Kate Therapeutics

FRIDAY, MAY 10

POSTER VIEWING

FRIDAY POSTERS 1419 – 1908

EXHIBIT HALL

12:00–1:30 PM POSTER VIEWING

5:30–7:00 PM POSTER RECEPTION

FRIDAY, MAY 10

SPONSORED SYMPOSIUM

ROOM 309-310

12:15–1:15 PM

VIRALGEN VECTOR CORE:
Cell and Gene Therapy Approaches for Parkinson's Disease

FRIDAY, MAY 10

SCIENTIFIC SYMPOSIUM

The Role of Government in Ensuring Equity of Access to CGTs (Organized by the Government Relations Committee)

Co-chairs: Christina Markus, JD, King & Spalding and
Scott McGoohan, JD, Vertex Pharmaceuticals

ROOM 318-323

1:30–1:50 PM

Assessing Lifetime Value for CGTs: Case Study on Sickle Cell Disease
Andrew Campbell, MD, Children's National Hospital

1:50–2:10 PM

Building Health System Readiness for Transformative High-cost, High-value Therapies
Navneet Majhail, MD, Sarah Cannon Cancer Institute

2:10–2:30 PM

Models to Support Patient Access: Lessons From the Past, Current Efforts, and Ideas for the Future
Erin Estey Hertzog, JD, Foley Hoag

2:30–2:50 PM

Publicly-funded Research and Private Development: Intellectual Property Considerations for Genetic Medicines
Becky Kaufman, JD, The Ohio State University

2:50–3:15 PM

Panel Discussion
Andrew Campbell, MD
Becky Kaufman, JD
Erin Estey Hertzog, JD
Navneet Majhail, MD

FRIDAY, MAY 10

SCIENTIFIC SYMPOSIUM

You Have a Great Technology...Now What? (Organized by the New Investigator Committee)

Co-chairs: Jonathan Brunger, PhD, Vanderbilt and
Kah-Whye Peng, PhD, Mayo Clinic

ROOM 339-342

1:30–1:40 PM	**Opening Remarks**
1:40–2:10 PM	**Young Investigator to Industry; Academics Identifying Partners Within Industry; Sponsored Research Programs, Licensing Their Drugs, etc.** Shannon Boye, PhD, University of Florida
2:10–2:40 PM	**Established Investigator to Industry; Pitching to Investors; Structure; What Term Sheet looks like; How much involvement; VC** Claire Aldridge, PhD, Form Bio
2:40–3:10 PM	**Balancing Careers in Academia and Industry to Accelerate the Development of *In Vivo* Targeted Genetic Medicines** Stephen Russell, MD, PhD, Vyriad, Inc.
3:10–3:15 PM	**Closing Remarks**

FRIDAY, MAY 10

ORAL ABSTRACT SESSION

Cell Therapy and Cell-Based Gene Therapy Trials

Co-chairs: Giuliana Ferrari, PhD, SR-Tiget, Fondazione S. Raffaele and John Tisdale, MD, National Heart, Lung, and Blood Institute (NHLBI)

BALLROOM 1

1:30–1:45 PM **240**

FVIII Expression through Autologous CD34+ Hematopoietic Stem Cells Transduced with a Lentiviral Vector with a Novel F8 Transgene for Gene Therapy of Hemophilia A - A First-in-Human Clinical Trial
Alok Srivastava, MD, Christian Medical College Vellore

1:45–2:00 PM **241**

Investigation of mRNA Cell Therapy as a Treatment for Autoimmune Disease in Patients with Myastenia Gravis
Christopher Jewell, PhD, Cartesian Therapeutics

2:00–2:15 PM **242**

Safety, Tolerability, and Clinical Assessment of Bemdaneprocel for Parkinson's Disease: Results up to 18 Months from a Phase 1 Study
Claire Henchcliffe, MD, DPhil, UC Irvine

2:15–2:30 PM **243**

Eighteen-Year Follow-Up of GD2 Chimeric Antigen Receptor-Cytotoxic T Lymphocyte Therapy in Patients with Neuroblastoma
Che-Hsing Li, MD, Baylor College of Medicine–Texas Children's Hospital–Houston Methodist Hospital

2:30–2:45 PM **244**

Efficacy and Safety of BRL-101, CRISPR-Cas9-Mediated Gene Editing of The BCL11A Enhancer in Transfusion-Dependent β-Thalassemia
Biao Zheng, MD, PhD, BRL Medicine Inc.

2:45–3:00 PM **245**

Lentiviral-Mediated Gene Therapy (RP-L102) for Fanconi Anemia [Group A] is Associated with Polyclonal Integration Patterns in the Absence of Conditioning.
Agnieszka Czechowicz, MD, PhD, Stanford University

FRIDAY, MAY 10

ORAL ABSTRACT SESSION (CONTINUED)

Cell Therapy and Cell-Based Gene Therapy Trials

Co-chairs: Giuliana Ferrari, PhD, SR-Tiget, Fondazione S. Raffaele and John Tisdale, MD, National Heart, Lung, and Blood Institute (NHLBI)

BALLROOM 1

3:00–3:15 PM **246**

Autologous *Ex-Vivo* Lentiviral Gene Therapy for Pediatric Patients with Severe Leukocyte Adhesion Deficiency-I Provides Sustained Efficacy with a Favorable Safety Profile

Donald Kohn, MD, UCLA

FRIDAY, MAY 10

ORAL ABSTRACT SESSION

AAV Vectors - Preclinical and Proof-of-Concept: Technology Focus

Co-chairs: Alisha Gruntman, DVM, PhD, UMass Chan Medical School and Luis Tecedor, PhD, Children's Hospital of Philadelphia

BALLROOM 2

1:30–1:45 PM | **247**

S/MAR-Containing AAV Vectors Result in an Increase in AAV Episomes & a Reduction in AAV Integration Sites in a Mouse Model with a High Rate of Hepatocyte Proliferation
Andrea Llanos-Ardaiz, Vivet Therapeutics

1:45–2:00 PM | **248**

A Durable Gene Therapy with a Robust AAV-LNP Delivery System Allowing for a Reduced AAV Dose
Matthew Nitzahn, Poseida Therapeutics

2:00–2:15 PM | **249**

Novel Micro-Dystrophin Constructs for DMD Gene Therapy
Ruchita Selot, PhD, Grow Lab Narayana Netralaya Foundation

2:15–2:30 PM | **251**

***In Situ* Biodistribution and Localization of Bidridistrogene Xeboparvovec (SRP-9003) in LGMD2E/R4 Mice**
Stephen Baine, Sarepta Therapeutics, Inc.

2:30–2:45 PM | **252**

Transgene Protein Evolution as a Novel Strategy for Next-Gen Gene Therapy in Canavan Disease
Sarah Foley, South East Technological University

2:45–3:00 PM | **253**

Antibody Gene Therapy for Rabies Encephalitis
Jyoti Yadav, Auburn University

FRIDAY, MAY 10

ORAL ABSTRACT SESSION

On- and Off-Target Method Development

Co-chairs: Douglas Smith, PhD, SeQure DX and
Stephen Hart, PhD, UCL Great Ormond Street Institute of Child Health

BALLROOM 3

1:30–1:45 PM **254**
Precise Measurement of CRISPR Genome-Editing Outcomes through Single-Cell DNA Sequencing
Ayal Hendel, PhD, The Mina and Everard Goodman Faculty of Life Sciences, Bar-Ilan University

1:45–2:00 PM **255**
Heterogeneous On-Target Genomic Rearrangements Occur upon Long Range Gene Editing in T-Cells
Daniele Canarutto, MD, PhD, SR-Tiget

2:00–2:15 PM **256**
Selecting Highly Conserved and Specific Guide RNAs for CRISPR/CasX-Mediated Gene Editing of the HSV-1 Genome
Meltem Isik, PhD, Excision BioTherapeutics

2:15–2:30 PM **257**
In Silico Prediction Tool for Meganuclease Off-Target Sites
Zhe Zhang, UPenn

2:30–2:45 PM **258**
Validating EPI-321 Safety: A Comprehensive Off Target Characterization Platform for Epigenetic Gene Therapies
Linsin Smith, PhD, Epic Bio

2:45–3:00 PM **259**
Off-Target Analysis Shows Favorable Safety Profile of Prime Editing
Maria Collier, Prime Medicine, Inc.

3:00–3:15 PM **260**
Casmini-Tool: A Comprehensive Database for Efficient and Specific Guide RNA Design Using dCasMINI
Robin Yeo, PhD, EpiCRISPR Biotechnologies

FRIDAY, MAY 10

ORAL ABSTRACT SESSION

AAV Vector Biology and Development II

Co-chairs: Victoria Madigan, PhD, Massachusetts Institute of Technology and Miguel Chuapoco, PhD, Capsida Biotherapeutics

BALLROOM 4

1:30–1:45 PM **261**

Multi-Step Engineered Adeno-Associated Virus Enables Whole-Brain mRNA Delivery
Weiya Bai, HuidaGene Therapeutics Co., Ltd.

1:45–2:00 PM **262**

Optimization of RNA End Joining (REJ) to Safely and Efficiently Express Large Proteins
Ryan Hsu, Salk Institute & UC San Diego

2:00–2:15 PM **263**

Encapsidation of, and Transgene Expression from, Generation Z (Genz) Single-Stranded AAV Vectors is AAV Serotype Capsid-Specific
Arun Srivastava, PhD, University of Florida

2:15–2:30 PM **264**

Development of ITR-Modified AAVrh74 Vectors with Improved Transgene Expression in Primary Human Skeletal Muscle Cells *In Vitro* and in Murine Skeletal Muscles *In Vivo*
Jakob Shoti, University of Florida

2:30–2:45 PM **265**

Transcriptional Crosstalk Between AAV Genomes Depends on Concatemer Formation and Enables Cell Type-Targeted Delivery of Oversized Cargo
Gerard Coughlin, Caltech

2:45–3:00 PM **266**

Newly Identified AAV Binders: Blood-Brain-Barrier Transcytosis Mediator LRP6 for Engineered AAVs and Immune Cytokine IL3 for AAV9-Host Interaction
Seongmin Jang, PhD, Caltech

FRIDAY, MAY 10

ORAL ABSTRACT SESSION (CONTINUED)

AAV Vector Biology and Development II

Co-chairs: Victoria Madigan, PhD, Massachusetts Institute of Technology and Miguel Chuapoco, PhD, Capsida Biotherapeutics

BALLROOM 4

3:00–3:15 PM | **267**

Creation of an AAV-Based Replication Origin for Novel AAV Vectors That Cannot Be Replicated in Nature

Leah Whitfield, University of North Carolina at Chapel Hill

FRIDAY, MAY 10

ORAL ABSTRACT SESSION

Neurologic Diseases III

Co-chairs: Juliette Hordeaux, DVM, PhD, UPenn and
Nettie Pyne, Nationwide Children's Hospital

ROOM 307-308

1:30–1:45 PM | **268**

AAV-Based Prion Protein-Lowering Gene Therapy for CJD
Qingzhong Kong, PhD, Case Western Reserve University

1:45–2:00 PM | **270**

Development of an Adeno-Associated Virus Gene Therapy for the Treatment of CDKL5 Deficiency Disorder
Janine Lamonica, UPenn

2:00–2:15 PM | **271**

Validation of an AAV Gene Therapy Designed to Complement the Loss of TDP-43 Splicing Repression for ALS-FTD
Aswathy Peethambaran Mallika, PhD, Johns Hopkins University School of Medicine

2:30–2:45 PM | **272**

ATAD1 Gene Rescue of Zellweger Spectrum Disorders via Targeting of Shared Common Mechanism
Joshua Bonkowski, MD, PhD, University of Utah

2:30–2:45 PM | **273**

AAV-Based Anti-RAN Antibody Therapy for C9orf72 ALS FTD
Lisa Romano, PhD, University of Florida, Center for NeuroGenetics and the Genetics Institute

2:45–3:00 PM | **274**

Systemic AAV Gene Therapy with CNS-Targeted Engineered Capsids Achieves Significant GCase Activity Increases in the Primate Brain to Support the Potential Treatment of GBA-PD
Nicholas Flytzanis, PhD, Capsida Biotherapeutics, Inc.

FRIDAY, MAY 10

SPONSORED SYMPOSIUM

ROOM 337-338

1:30–2:00 PM

GENSCRIPT USA INC.:
An adjustable dose gene therapy platform technology - Prometheus™

ROOM 337-338

2:30–3:00 PM

AAVNERGENE:
AAVone: An All-in-One Single-Plasmid System for Efficient and Streamlined Production of High-Quality AAV Vectors

ROOM 337-338

3:45–4:15 PM

SYNEOS HEALTH:
Unlocking the Promise of Genetic Medicines: From Clinical to Commercial Development

ROOM 337-338

4:45–5:15 PM

CITELINE:
How Cell & Gene Therapy Clinical Trial Design Impacts Commercial Access

FRIDAY, MAY 10

TOOLS AND TECHNOLOGY FORUM

Tools and Technology Forum 5

Chair: Daniel Kavanagh, PhD, WCG

EXHIBIT HALL PRESENTATION THEATER

2:00–2:15 PM

CURI BIO:
Building Functional, Reproducible, and Clinically-Relevant Human 3D Engineered Muscle Tissues and Neuromuscular Junctions with the Mantarray Platform

2:15–2:30 PM

TAKARA BIO USA:
Dissolvable Microfluidics to Enhance Viral Transduction Efficiencies

2:30–2:45 PM

SYNTHEGO:
Enabling GMP Production of sgRNA for CRISPR-based Cell and Gene Therapies

2:45–3:00 PM

DIGITAL BIOLOGY:
Light-directed Fixed Tissue Sequencing: Partnering for Precision CGT Optimization

3:00–3:15 PM

RECIBIOPHARM:
Transforming Gene Therapy Manufacturing - Introducing a flexible, robust and scalable next generation rAAV production platform

FRIDAY, MAY 10

TOOLS AND TECHNOLOGY FORUM

Tools and Technology Forum 6

Chair: Daniel Kavanagh, PhD, WCG

EXHIBIT HALL PRESENTATION THEATER

3:45–4:00 PM

CELLFE:
A Novel, Microfluidics Approach to Advance Innovative Cell Therapy Manufacturing

4:00–4:15 PM

QIAGEN:
Ensuring Genome Integrity Of Recombinant AAV Vectors Using Digital PCR

4:15–4:30 PM

QUTEM AB

4:30–4:45 PM

EUROFINS BIOPHARMA PRODUCT TESTING:
From Days to Hours: The Dynamic Shift in Biological Testing

4:45–5:00 PM

VIRALGEN VECTOR CORE

FRIDAY, MAY 10

ORAL ABSTRACT SESSION

Oligonucleotide Therapeutics

Co-chairs: Afrooz Rashnonejad, PhD, Center for Gene Therapy at Abigale Wexner Research Institute of Nationwide Children's Hospital and Isabel Aznarez, PhD, Stoke Therapeutics

BALLROOM 1

3:45–4:00 PM | **331**

Prenatal Intra-Amniotic Administration of an Antisense Oligonucleotide Achieves Widespread Biodistribution with Therapeutic Levels in Fetal Lambs
Beltran Borges, MD, UCSF

4:00–4:15 PM | **332**

Targeting NLRP3 Splicing Variants with Antisense Oligonucleotides to Control Pathological Inflammation
Roni Klein, Rosalind Franklin University of Medicine and Science

4:15–4:30 PM | **333**

First in Class ASO Targeting IGHMBP2 Cryptic Splice Variant: Efficacy and Safety
Sandra Smieszek, PhD, Vanda Pharmaceuticals Inc.

4:30–4:45 PM | **334**

Potent, Durable mRNA Knockdown in Extrahepatic Tissues Using siRNAs with Novel Phosphoryl Guanidine Backbone Variants
Wei Liu, Wave Life Sciences

4:45–5:00 PM | **335**

miRNA Site-Blocking Oligonucleotides as a Novel Therapeutic Strategy for STXBP1 Encephalopathy
Alex Felix, UPenn

5:00–5:15 PM | **336**

Modulation of Somatic Repeat Expansion with Small Interfering RNAs
Jillian Belgrad, UMass Chan Medical School

5:15–5:30 PM | **337**

Therapeutic Oligonucleotides Induce Acute Toxicity in CNS, Preventable by the Addition of Divalent Cations to Formulation
Rachael Miller, UMass Chan Medical School

FRIDAY, MAY 10

ORAL ABSTRACT SESSION

Novel Production Platforms

Co-chairs: Nidhi Kotecha, PhD, University of Colorado, Denver and Hai-Quan Mao, PhD, Johns Hopkins University

BALLROOM 2

3:45–4:00 PM | **282**
Healthy Donor vs Patient Manufactured Autologous Deltex DRI Product; Immunophenotyping Gene Expression
Mariska Ter Haak, IN8bio

4:00–4:15 PM | **283**
Enhancing Cell Transfection Efficiency via Modulation of Extracellular Fluid Viscosity
Jingyao Ma, Johns Hopkins University

4:15–4:30 PM | **284**
A Short, Simple, and Cost-Effective CAR-T Cell Manufacturing Process
Ying Xiong, PhD, Caring Cross, Inc.

4:30–4:45 PM | **285**
An Efficient Way to Generate Virus- and Factor-Free Human iPSCs Using S/MAR DNA Vectors
Anna Hartley, German Cancer Research Centre

4:45–5:00 PM | **286**
Establishment of Automated Point-of-Care Manufacturing of ΔNPM1 TCR-Engineered T Cells for AML Therapy
Ian Johnston, PhD, Miltenyi Biotec B.V. & Co. KG

5:00–5:15 PM | **287**
Engineering Inducible Signaling Receptors for Erythropoietin-Free Erythropoiesis
Aadit Shah, Stanford University School of Medicine

5:15–5:30 PM | **288**
Non-Viral Engineering of Primary Human Macrophages Using a cGMP-Compliant Electroporation Platform
Ashley Strickland, PhD, MaxCyte Inc.

FRIDAY, MAY 10

ORAL ABSTRACT SESSION

AAV Manufacturing III

Co-chairs: Barbara Sullivan, PhD, Ultragenyx and
Liujiang Song, PhD, Asklepios BioPharmaceutical, Inc.

BALLROOM 3

3:45–4:00 PM **289**

Optimizing for > 95 % rAAV Empty Capsid Removal at Process Scale with Anion Exchange Chromatography Using a Mechanistic Model
Vijesh Kumar, PhD, Spark Therapeutics

4:00–4:15 PM **290**

A Proprietary HEK293 AAV Production System Can Achieve Greater Than 50% Full Capsids with Greater Than 1e15vg/L at Harvest Enabling Scalable Chromatography-Based Polishing with High Yield and Purity
Matt Edwards, Affinia Therapeutics

4:15–4:30 PM **291**

Identification and Control of AAV Charge Heterogeneity through Optimized Bioreactor Operation
Thomas Thiers, Oxford Biomedica (US) LLC

4:30–4:45 PM **292**

Next Generation Adeno Helper Plasmids and Helper Stable Cell Lines for Efficient AAV Vector Production
Jiten Doshi, PhD, Mass Eye and Ear Infirmary

4:45–5:00 PM **293**

Development of a Powerful AAV Production Platform by Leveraging Comprehensive Design-of-Experiment & High-Throughput Automated Bioreactors
Érica Schulze, PhD, Sartorius Stedim Biotech GmbH

5:00–5:15 PM **294**

Short-Term and Large-Scale Production of High-Quality Recombinant Adeno-Associated Virus Using a Zonal Ultracentrifugation
Mikako Wada, PhD, The University of Tokyo

5:15–5:30 PM **295**

Generation of a Stable High-Titer Production Cell Line for Therapeutic AAV Vectors
Stefan Seeber, PhD, Roche Diagnostics GmbH

FRIDAY, MAY 10

ORAL ABSTRACT SESSION

AAV Vectors - Non-Human Primates and Large Animal Models

Co-chairs: Amanda Gross, PhD, Auburn University and
Russ Addis, PhD, NeuExcell Therapeutics

BALLROOM 4

3:45–4:00 PM **296**
Neuron Regeneration Drives Functional Recovery from Severe Stroke Following Intracranial Administration of NXL-001 Gene Therapy in Non-Human Primates
Anna Kabanova, PhD, NeuExcell Therapeutics

4:00–4:15 PM **297**
AAV Intein Retinal Gene Therapy for Stargardt Disease is Effective and Safe in Large Animal Models
Ivana Trapani, Telethon Institute of Genetics and Medicine

4:15–4:30 PM **298**
Tissue De-Targeting Abrogates Hepatotoxicity and Complement-Related Thrombotic Complications Associated with High-Dose AAV Gene Therapies
Andrew Baik, PhD, Regeneron Pharmaceuticals

4:30–4:45 PM **299**
Toxicity Profile of Clade F Vectors Administered Intravenously in Nonhuman Primates
Juliette Hordeaux, DVM, PhD, UPenn

4:45–5:00 PM **300**
AAV-ARSA Mediated Gene Replacement for the Treatment of Metachromatic Leukodystrophy
Shyam Ramachandran, PhD, Sanofi

5:00–5:15 PM **301**
Applying Artificial Intelligence to Multi-Property Optimization of AAV Capsids for Neuronal Gene Delivery
Mugdha Deshpande, Dyno Therapeutics

5:15–5:30 PM **302**
Improving the Expression of AAV-Delivered Anti-HIV Broadly Neutralizing Antibodies in Nonhuman Primates
Michael Kuipa, Emory National Primate Research Center

FRIDAY, MAY 10

ORAL ABSTRACT SESSION

Neurologic Diseases IV

Co-chairs: Alvin Luk, PhD, Huidagene Therapeutics Inc and
Lisa Stanek, PhD, Affinia Therapeutics

ROOM 307-308

3:45–4:00 PM | **303**

Optimized Artificial miRNAs Delivered by rAAV9 Dramatically Improve Survival, Respiratory and Motor Functions of SOD1^{G93A}-ALS Mice
Fang Wan, PhD, UMass Chan Medical School

4:00–4:15 PM | **304**

Reduction of Atxn2, a Therapeutic Target for Sporadic ALS, in Non-Human Primates Using a Novel, Intravenously Delivered AAV Capsid
Giridhar Murlidharan, PhD, Affinia Therapeutics

4:15–4:30 PM | **305**

Development of Tau Isoform-Specific Reduction Therapy
Rachel Bailey, PhD, UT Southwestern Medical Center

4:30–4:45 PM | **306**

Towards an AAV-Based Gene Therapy Strategy for Hereditary Spastic Paraplegia Type 52 (SPG52)
Laura Rodriguez-Estevez, Universitat Autònoma de Barcelona (UAB)

4:45–5:00 PM | **307**

Establishment of Gene Therapy Drug Development Assays Confirms Antiepileptic Activity of an AAV Encoding Neuropeptide Y (NPY) and Y2 Receptor (Y2R)
Barbara Terzic, PhD, Spark Therapeutics

5:00–5:15 PM | **308**

Long-Term Safety and Feasibility of a Cas13-Based RNA Editing Therapy for MECP2 Duplication Syndrome in Humanized MECP2 Transgenic Mice
Dong Yang, HuidaGene Therapeutics Co., Ltd.

FRIDAY, MAY 10

ORAL ABSTRACT SESSION (CONTINUED)

Neurologic Diseases IV

Co-chairs: Alvin Luk, PhD, Huidagene Therapeutics Inc and
Lisa Stanek, PhD, Affinia Therapeutics

ROOM 307-308

5:15–5:30 PM | 309

AI Enabled ASO Design Can Lead to Rapid Initiation of Treatment for an Ultra-Rare Disorder Leading to Allele Selective Knockdown of a Toxic Protein and Consequent Clinical Improvement
David Dimmock, MD, Creyon Bio

FRIDAY, MAY 10

ORAL ABSTRACT SESSION

AAV Capsid Engineering: Multilevel Approaches for Enhanced AAV Delivery

Co-chairs: Adam Schieferecke, PhD, UC Berkeley and
Jingxuan "Mimi" Guo, PhD, UC Berkeley

ROOM 309-310

3:45–4:00 PM | **310**

Identification of AAV Capsid with Improved Tropism to Macular Retina Through Multi-Species Screening in Mice, Rabbits, Pigs, and Monkeys
Li Ou, PhD, Avirmax, Inc.

4:00–4:15 PM | **311**

AAV Capsid Selection at Spatial and Single-Cell Resolution in Non-Human Primate Retina
Ashley Robbins, UPenn

4:15–4:30 PM | **312**

Identification of Novel AAV3B and AAV8 Variants with Superior Retina Transduction Profiles Following Suprachoroidal Delivery in Multiple Large Animal Models
April Giles, PhD, REGENXBIO Inc.

4:30–4:45 PM | **313**

Reprogramming Adeno-Associated Virus Tropism via Displayed Peptides Tiling Receptor-Ligands in Mice and Non-Human Primates
Andrew Portell, UCSD

4:45–5:00 PM | **314**

Improvement of Liver Tropic AAV Capsids: Neutralizing Antibody-Evading Variants of sL65 Which Demonstrate Cross-Species Transduction
Jing Liao, Alexion Genomic Medicine, Astra Zeneca Rare Disease

5:00–5:15 PM | **315**

Enhanced Precision for *In Vivo* Gene Delivery into Lymphocytes Through Mono- and Bispecific DART-AAVs
Luca Zinser, Paul-Ehrlich-Institut

FRIDAY, MAY 10

ORAL ABSTRACT SESSION (CONTINUED)

AAV Capsid Engineering: Multilevel Approaches for Enhanced AAV Delivery

Co-chairs: Adam Schieferecke, PhD, UC Berkeley and
Jingxuan "Mimi" Guo, PhD, UC Berkeley

ROOM 309-310

5:15–5:30 PM | **316**

Engineered Adeno Associated Virus Capsids for Packaging Proteins
Ryan Sorensen, University of Minnesota

FRIDAY, MAY 10

ORAL ABSTRACT SESSION

Physical Delivery and Novel Approaches

Co-chairs: Geeta Palsule, PhD, Rutgers University and
Nizar Saad, PhD, The Ohio State University and Nationwide Children's Hospital

ROOM 314-317

1:30–1:45 PM | **317**

Single-Particle Spectroscopic Hydrodynamic Chromatography Reveals Heterogeneous RNA Loading and Size Correlations in Lipid Nanoparticles
Sixuan Li, PhD, Johns Hopkins University

1:45–2:00 PM | **318**

Engineered Staphylococcus Epidermidis as a Protein Delivery System for Treating Skin Diseases
Travis Whitfill, Azitra Inc.

2:00–2:15 PM | **319**

Lipid Nanocrystal Delivery of siRNA: Dynamics of Uptake in Innate Immune Cells in Human Blood and Visualization of Small Oligonucleotide Delivery in Cell Culture
James Ferguson, MD, Matinas BioPharma

2:15–2:30 PM | **320**

Improving Nonviral Gene Expression in T Cells via Inhibition of the Restrictosome
Eric Warga, Villanova University

2:30–2:45 PM | **321**

Megakaryocyte-Derived Extracellular Vesicles Deliver Gene Therapy Cargo That Targets Bone Marrow and Evades the Liver *In Vivo*
Laura Goldberg, MD, PhD, STRM.BIO

2:45–3:00 PM | **322**

Ultrasound-Facilitated Brain Genome Editing
Yeh-Hsing Lao, University at Buffalo, The State University of New York

3:00–3:15 PM | **323**

Exploring mRNA Expression Dynamics in Mammalian Cells through Live Imaging of Single-Cells
Judith Müller, Ludwig-Maximilians-University

FRIDAY, MAY 10

ORAL ABSTRACT SESSION

Targeted Gene and Cell Therapy I

Co-chairs: Balveen Kaur, PhD, Augusta University and
Blake Aftab, PhD, Atara Biotherapeutics, Inc.

ROOM 318-323

3:45–4:00 PM | **418**
Development of Inhalable Polymeric Nanoemulsions for Delivery of Gene Silencing Therapeutics in Lung Tumors
Kasturi Siddhanta, University of Nebraska Medical Center

4:00–4:15 PM | **325**
Protection of CD33 Modified HSC Progeny from CD33-Directed CAR T Cells in Nonhuman Primates
Nicholas Petty, Fred Hutchinson Cancer Center

4:15–4:30 PM | **326**
Chimeric Antigen Receptor Discovery Through T Cell Display
Joseph Muldoon, PhD, UCSF

4:30–4:45 PM | **327**
"Off-the-Shelf" Combination CAdVEC and CAR-NK Cell Immunotherapy for Pancreatic Ductal Adenocarcinoma
Greyson Biegert, Baylor College of Medicine

4:45–5:00 PM | **328**
Selective Support of CAR-T Cell Therapies by Cis-Targeted IL2
Sara Sleiman, MD, Perelman School of Medicine, UPenn

5:00–5:15 PM | **329**
ADI-270: An Armored Allogeneic Anti-CD70 CAR γδ T Cell Therapy Designed for Multiple Solid and Hematological Cancer Indications
Shon Green, PhD, Adicet Bio

5:15–5:30 PM | **330**
Discovery of Novel CARs for Solid Tumors Using Senti REVEAL™, a Massively Parallel Technology Platform Comprising Pooled Screening, Machine Learning, and Lab Automation
Nicholas Frankel, PhD, Senti Biosciences

FRIDAY, MAY 10

ORAL ABSTRACT SESSION

Advancements in Technologies for *In Vivo* Gene Therapies

Co-chairs: Axel Schambach, MD, PhD, University of Michigan and Megan Hoban, PhD, ReNAgade Therapeutics

ROOM 324-326

3:45–4:00 PM | **275**

***In Vivo* Application of BaEVRLess-Pseudotyped Lentiviral Vectors for Hematopoietic Stem Cell Gene Therapy**
Denise Klatt, PhD, Boston Children's Hospital, Harvard Medical School

4:00–4:15 PM | **276**

LNP-Based Delivery of CRISPR/Cas12a for the Potential Treatment of Myocilin-Associated Glaucoma
Heather MacLeod, PhD, Editas Medicine

4:15–4:30 PM | **277**

G-CSF-Free HSC Mobilization with WU-106/AMD3100 Allows for Safe and Efficient *In Vivo* HSC Prime Editing of the Sickle Cell Disease Mutation in a Mouse Model
Chang Li, PhD, University of Washington

4:30–4:45 PM | **278**

Cas12a CRISPR Hybrid RNA-DNA (chRDNA)-Mediated *In Vivo* Genome-Editing Technology for Efficient and Functional Hepatic Gene Disruption
Meghdad Rahdar, PhD, Caribou Biosciences

4:45–5:00 PM | **280**

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

5:00–5:15 PM | **281**

The Development of a Staphylococcus epidermidis Strain Expressing LEKTI-D6 (ATR12-351) for Netherton Syndrome
Mary Spellman, MD, Azitra Inc.

FRIDAY, MAY 10

ORAL ABSTRACT SESSION

Nonviral Methods for Delivering Nucleic Acids

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

ROOM 327-329

3:45–4:00 PM **338**
A Novel High-Throughput Screen to Identify Factors Controlling CRISPR-Mediated Non-Viral Genome Editing
Shivani Saxena, PhD, University of Wisconsin-Madison

4:00–4:15 PM **339**
Development of Envelope NanoBodies for Guided *In Vivo* Cell Engineering
Jesus Beltran, PhD, UCSD

4:15–4:30 PM **340**
An Optimized PBAE Nanoparticle Formulation Allows for Targeted Gene Delivery to the Lungs Following Systemic Injection
Erin Kavanagh, Johns Hopkins School of Medicine

4:30–4:45 PM **341**
High-Throughput Screening Platform Identifies Nanoparticles Capable of Delivering 19.5 kbp Plasmid to Neuronal Cells *In Vitro* and *In Vivo*
Cherry Gupta, PhD, Battelle Memorial Institute

4:45–5:00 PM **342**
Mechanistic Investigation of Cas9 Affinity for CRISPR-AuNP Utilizing Direct RNA Surface Assembly
Daniel Lane, PhD, Fred Hutchinson Cancer Center

5:00–5:15 PM **343**
Peptide-Based Nanoplatform for Cas9 RNP Delivery and Gene Editing
Joseph Lavalla, Clemson University

5:15–5:30 PM **344**
Novel Non-Viral Delivery Vehicles That Distribute to the Sciatic Nerve and Brain Identified via Iterative Design-Build-Test-Learn Polymer Nanoparticle Discovery Platform
Kenneth Sims, PhD, Battelle Memorial Institute

FRIDAY, MAY 10

ORAL ABSTRACT SESSION

AAV Vector Integration

Co-chairs: Nadia Sellami, PhD, PacBio and
Paul Valdmanis, PhD, University of Washington

ROOM 339-342

3:45–4:00 PM **345**

The Genomic Architecture of 24 Carcinogenic rAAV Integrations in Mice: Implications for Human Gene Therapy
Randy Chandler, PhD, NIH

4:00–4:15 PM **346**

Comprehensive Preclinical Integration Site Analyses of SPK-3006, a Liver-Mediated AAV Gene Therapy for Pompe Disease Reveals a Low Theoretical Risk for Insertional Mutagenesis
Ali Nowrouzi, PhD, Spark Therapeutics Inc

4:15–4:30 PM **347**

Predicting Truncation Events in AAV Vector Genome Designs Using Deep Learning
Sandhiya Ravi, PhD, UMass Chan Medical School

4:30–4:45 PM **348**

Characterization of AAV Vectors in Tissues Transduced with Conventional and Novel ITRs Show Differences in ITR Recombination, Episome Structures, and Transgene Expression
Suk Namkung, UMass Chan Medical School

4:45–5:00 PM **349**

In Silico Reconstruction of Genotoxic AAV Integration Events from a Tiling Array Dataset: Proof of Concept and Validation
Tyra Wolfsberg, PhD, NIH/NHGRI

5:00–5:15 PM **350**

Characterization of AAV Integrations in Preclinical Models of Gene Therapy Using RAAVioli Pipeline with Long and Short Sequencing Reads
Carlo Cipriani, SR-Tiget, IRCCS San Raffaele Scientific Institute

FRIDAY, MAY 10

ORAL ABSTRACT SESSION (CONTINUED)

AAV Vector Integration

Co-chairs: Nadia Sellami, PhD, PacBio and
Paul Valdmanis, PhD, University of Washington

ROOM 339-342

5:15–5:30 PM | 351

A Highly Potent Engineered AAV Capsid, STAC-150, Enables High-Throughput AAV Production and Arrayed Epigenetic Regulator Screening Directly in Cultured Neurons

Patrick Dunn, Sangamo Therapeutics, Inc.

SATURDAY, MAY 11

ORAL ABSTRACT SESSION

AAV Vectors - Immune Modulation

Co-chairs: Helena Costa Verdana, PhD, Nationwide Children's Hospital and Manish Muhuri, PhD, Voyager Therapeutics, Inc.

BALLROOM 2

8:00–8:15 AM **352**

C3 Complement Inhibitor Limits Transaminitis and Immune Response Following Liver Targeted Adeno-Associated Virus (AAV) Gene Transfer (GT) in Non-Human Primates (NHPs)
Anna Majowicz, PhD, Spark Therapeutics

8:15–8:30 AM **353**

Orthogonal B Cell and Plasma Cell Immunosuppression Strategies Prevent and Suppress High-Titer Antibody Immunity to Enable AAV Vector Re-Dosing
Nicholas Giovannone, PhD, Regeneron Pharmaceuticals

8:30–8:45 AM **354**

Vectorized Antibody-Mediated Anti-Eosinophil Gene Therapy
Maria Gioulvanidou, MD, Weill Cornell Medical College

8:45–9:00 AM **355**

Modulating the AAV-Specific Immune Response: A Comparison of T Cell Co-Stimulation Blockade with Rapamycin Plus B Cell Depletion
Shari Gordon, PhD, AskBio

9:00–9:15 AM **356**

Vectorized Expression of a Pan-Filovirus Bispecific Monoclonal Antibody in a Murine Model for Protection Against Ebola and Marburg Virus Challenge
Elena Campbell, PhD, University of Guelph

9:15–9:30 AM **357**

Evaluating the Effects of IdeS-Generated Anti-AAV IgG Fragments on AAV Gene Delivery
Saghana Muraleetharan, Spark Therapeutics

9:30–9:45 AM **358**

IgG Enzymatic Cleavage Prevents Uptake of AAV and Activation of Phagocytic Immune Cells in Seropositive Human Donors
Blake Rust, PhD, Asklepios BioPharmaceutical, Inc.

SATURDAY, MAY 11

ORAL ABSTRACT SESSION

Oncolytic Viruses

Co-chairs: Paola Grandi, PhD, CG Oncology and
Richard Vile, PhD, Mayo Clinic

ROOM 327-329

8:00–8:15 AM **359**

Additional Expression of T Cell Engager in Clinically Tested Oncolytic Adeno-Immunotherapy Redirects Tumor-Infiltrated, Irrelevant T Cells Against Cancer Cells, Enhancing Anti-Tumor Immunity
Daisuke Morita, MD, PhD, Baylor College of Medicine

8:15–8:30 AM **360**

Repurposing the Selective Serotonin-Reuptake Inhibitor (SSRI) Paroxetine to Combat Systemic Immunosuppression in an Orthotopic Syngeneic Murine Glioblastoma Model Treated with Oncolytic MV-s-NAP-uPA Based Immunovirotherapy
Georgios Stergiopoulos, MD, Mayo Clinic

8:30–8:45 AM **361**

The Clinical Oncolytic Virotherapy, VET3-TGI, Displays Potent Therapeutic Activity in Multiple Mouse Tumor Models through Blocking TGF-Beta and Augmenting Type-1 Immune Response
Steve Thorne, PhD, Kalivir Immunotherapeutics

8:45–9:00 AM **362**

Where's Waldo: Finding Tumor Antigens in the Circus of the Immune Response to Oncolytic Viruses
Benjamin Kendall, Mayo Clinic

9:00–9:15 AM **363**

Anti-Tumor Immune Responses of Oncolytic Vaccinia Virus are Synergistically Enhanced by Induction of Cell-Cell Fusion and Delivery of Multiple Immunomodulators
Motomu Nakatake, PhD, Tottori University

SATURDAY, MAY 11

ORAL ABSTRACT SESSION (CONTINUED)

Oncolytic Viruses

Co-chairs: Paola Grandi, PhD, CG Oncology and
Richard Vile, PhD, Mayo Clinic

ROOM 327-329

9:15–9:30 AM **364**

Oncolytic Adenovirus Armed with a Type I Interferon Exerts Systemic Antitumor Immunity in Immunocompetent Replication-Permissive Models
Kazuho Inoko, MD, PhD, University of Minnesota

9:30–9:45 AM **365**

Novel Vesiculovirus Library Characterization for Repeat Dosing Strategies
Natalie Elliott, Mayo Clinic

SATURDAY, MAY 11

SCIENTIFIC SYMPOSIUM

Recent Advancements That Expand Viral Vector Delivery Applications (Organized by the Viral Gene Transfer Vectors Committee)

Co-chairs: Dirk Grimm, PhD, Heidelberg University Hospital and Masato Yamamoto, MD, PhD, University of Minnesota

BALLROOM 1

8:00–8:26 AM

Lentivirus-derived Nanoparticles for Delivery of Gene Editing Systems
Jacob Mikkelsen, PhD, Aarhus University

8:26–8:52 AM

Engineering Oncolytic Viruses for Therapeutic Applications
Paola Grandi, PhD, CG Oncology

8:52–9:18 AM

AAV Vector Integration
Jenny Greig, PhD, UPenn

9:18–9:44 AM

AAV Genome Engineering to Accommodate Oversized Transgenes and Regulatory Elements
Shannon Boye, PhD, University of Florida

SATURDAY, MAY 11

SCIENTIFIC SYMPOSIUM

Moving from *Ex Vivo* to *In Vivo*: Challenges and Advances in *In Vivo* Gene Therapy for Hematological Disorders (Organized by the Hematologic and Immunologic Gene and Cell Therapy Committee)

Co-chairs: Vinod Prasad, MD, Duke University Medical Center and
Reza Shahbazi, PhD, Indiana University School of Medicine

BALLROOM 3

8:00–8:15 AM

Brief Overview On Why *In Vivo* Applications are Necessary (Challenges of *Ex Vivo* Therapies, Economics and Equitable Therapies, Quality of Products Needed for *Ex Vivo* vs *In Vivo* Applications)
Hans-Peter Kiem, MD, PhD, Fred Hutchinson Cancer Center

8:15–8:35 AM

Possible Immune Responses to *In Vivo* Delivery Approaches for Hematologic Disorders and Ways to Overcome Them
Michela Milani, PhD, SR-Tiget

8:35–8:55 AM

Targeting and Redirection of Hematopoietic Stem Cells
Laura Breda, PhD, Children's Hospital of Philadelphia

8:55–9:15 AM

***In Vivo* Generation of anti-BCMA CAR T Cells for the Treatment of Multiple Myeloma**
Shannon Contrastano, PhD, kelonia Therapeutics

9:15–9:45 AM

Panel Discussion
Hans-Peter Kiem, MD, PhD
Laura Breda, PhD
Michela Milani, PhD
Shannon Contrastano, PhD

SATURDAY, MAY 11

SCIENTIFIC SYMPOSIUM

Novel Approaches for Musculoskeletal Diseases (Organized by the Musculoskeletal Gene and Cell Therapy Committee)

Co-chairs: Niclas Bengtsson, PhD, University of Washington and
Nizar Saad, PhD, The Ohio State University and Nationwide Children's Hospital

BALLROOM 4

8:00–8:26 AM

Enveloped Viruses Pseudotyped with Mammalian Myogenic Cell Fusogens Target Skeletal Muscle for Gene Delivery
Sajedah Hindi, PhD, Cincinnati Children's hospital

8:26–8:52 AM

Fast, Multiplexable and Efficient Somatic Gene Deletions in Adult Mouse Skeletal Muscle Fibers Using AAV-CRISPR/Cas9
Markus Rüegg, Biozentrum, University of Basel

8:52–9:18 AM

Single Nuclei Transcriptomics of Muscle Reveals Intra-Muscular Cell Dynamics Linked to Dystrophin Loss and Rescue
Carrie Miceli, PhD, UCLA

9:18–9:44 AM

T Cell Responses and Other Therapies
Francesco Muntoni, MD, University College London

SATURDAY, MAY 11

SCIENTIFIC SYMPOSIUM

Unending Experiments: Ethical Challenges and Responsibilities for Gene Therapy's Road Ahead (Organized by the Ethics Committee)

Chair: Krishanu Saha, PhD, University of Wisconsin-Madison

ROOM 309-310

8:00–8:25 AM

Sickle Cell Research and Therapeutic Innovation
Matthew Porteus, MD, PhD, Stanford University

8:25–8:50 AM

CAR T Innovation: Keeping Access Front and Center
Bruce Levine, PhD, UPenn

8:50–9:15 AM

Patient Advocacy Perspective on DMD
Pat Furlong, Parent Project Muscular Dystrophy

9:15–9:45 AM

Panel Discussion
Bruce Levine, PhD
Matthew Porteus, MD, PhD
Pat Furlong

SATURDAY, MAY 11

SCIENTIFIC SYMPOSIUM

Targeting Specific Cell Types with Nonviral Delivery (Organized by the Nonviral Therapeutic Delivery Committee)

Co-chairs: Jonathan Hoggatt, PhD, Moderna Therapeutics and
Lauren Woodard, PhD, Vanderbilt University Medical Center

ROOM 314-317

8:00–8:35 AM

Arc Protein Nanoparticles to Target Neurons
Jason Shepherd, PhD, University of Utah School of Medicine

8:35–9:10 AM

Highly Specific, *In Vivo* Delivery to T-cells with Cell-Targeted Lipid Nanoparticles
Nathaniel Silver, PhD, Generation Bio

9:10–9:45 AM

siRNA Multiple Myeloma and/or Placenta Delivery
Michael Mitchell, PhD, UPenn

SATURDAY, MAY 11

SCIENTIFIC SYMPOSIUM

Companion Diagnostics for AAV-based Gene Therapies (Organized by the Regulatory Affairs Committee)

Co-chairs: Aron Stein, PhD, Intellia Therapeutics and
Keith Wonnacott, PhD, Lexeo Therapeutics

ROOM 318-323

8:00–8:20 AM

Regulatory Pathways for the Development of CDx AAV Antibody Assays: From Investigational Device Use to a Gene Therapy CDx
Natasha Thorne, PhD, FDA

8:20–8:40 AM

Technical and Regulatory Perspectives on CDx
Kelly Gordon, PhD, Boudicca Dx.

8:40–9:00 AM

The CDx Development Process: Clinical Trial Criteria and Considerations for a Global Regulatory Approach
Chelsea Welch, ARUP Laboratories

9:00–9:20 AM

Application of a Total Antibody (TAb) Assay as a CDx for AAV5 Gene Therapy
Greg de Hart, PhD, BioMarin Pharmaceuticals

9:20–9:45 AM

Panel Discussion
Natasha Thorne, PhD, FDA
Kelly Gordon, PhD, Boudicca Dx
Chelsea Welch, ARUP Laboratories
Gregory de Hart, PhD, BioMarin Pharmaceuticals

SATURDAY, MAY 11

SCIENTIFIC SYMPOSIUM

Pressing Clinical Challenges in Gene Therapy (Organized by the Bio-Industry Committee)

Co-chairs: Jim Wang, PhD, Regeneron Pharmaceuticals and Kaye Spratt, PhD

ROOM 324-326

8:00–8:25 AM
Choice of Jurisdiction for FIH Trials
Christopher Novashinski, RareMoon

8:25–8:50 AM
Safety and the Need for Better Data Sharing Around Safety in Gene rx Trials
Genevieve Laforet, MD, PhD, Aspa Therapeutics

8:50–9:15 AM
Comparing and Contrasting Viral and Non-Viral Delivery
Luigi Naldini, PhD, SR-Tiget

9:15–9:45 AM
Panel Discussion
Christopher Novashinski
Genevieve Laforet, MD, PhD
Luigi Naldini, PhD

SATURDAY, MAY 11

SCIENTIFIC SYMPOSIUM

Innovations in Cardiac Gene and Cell Therapy (Organized by the Cardiovascular Gene and Cell Therapy Committee)

Co-chairs: Laura Richman, DVM, PhD, Affinia Theraputics and
Michael Regnier, PhD, University of Washington

ROOM 339-342

8:00–8:26 AM

Engineering Quiescent-yet-excitable Cardiomyocytes for Cardiac Regeneration Therapy
Silvia Marchiano, PharmD, PhD, University of Washington School of Medicine

8:26–8:52 AM

***Ex Vivo* Gene Therapy for Heart Transplant**
Dawn Bowles, PhD, Duke University

8:52–9:18 AM

IPS Cardiomyocytes, Large Animal Data
Sara Vasconcelos, PhD, University of Toronto

9:18–9:44 AM

Machine-learning Guided Rational Design of Cardiotropic Capsids That Detarget Liver and DRG
Sherry Cao, PhD, Affinia Therapeutics

SATURDAY, MAY 11

SCIENTIFIC SYMPOSIUM

Gene Therapy and Genome Editing in Europe (Organized by the European Society of Gene and Cell Therapy)

Co-chairs: Axel Schambach, MD, PhD, Hannover Medical School and Gloria Gonzalez-Aseguinolaza, PhD, CIMA of the University of Navarra

ROOM 324-326

10:15–10:41 AM
ExpEditing AAV Gene Therapy
Alberto Auricchio, MD, PhD, FONDAZIONE TELETHON

10:41–11:07 AM
Genome Editing of T cells for Cancer Immunotherapy
Chiara Bonini, MD, Università Vita Salute San Raffaele

11:07–11:33 AM
Engineering CRISPR Technologies for Application in Cardiology
Julian Grünewald, MD, PhD, Technical University of Munich

11:33–11:59 AM
Advances of Hematopoietic Stem Cell Gene Therapy
Juan Bueren, PhD, CIEMAT

SATURDAY, MAY 11

ORAL ABSTRACT SESSION

Other Cellular and Regenerative Therapies

Co-chairs: David Young, MD, PhD, NIH and
Saad Kenderian, MB, ChB, Mayo Clinic

BALLROOM 1

10:15 AM–10:30 AM | **366**
Abeta-Responsive Cells as a Therapy in Alzheimer's Disease
Madeline Spetz, Vanderbilt University

10:30 AM–10:45 AM | **367**
Improvement of Disease Phenotype in Beta-Thalassemic Mice with a Novel Radiation-Free Conditioning Regimen of Pro-Apoptotic mRNA
Laura Breda, PhD, Children's Hospital of Philadelphia

10:45 AM–11:00 AM | **368**
A Gene Agnostic Stem Cell Therapy for Mid-Stage Retinitis Pigmentosa Treatment
Deepti SIngh, PhD, Ingel Therapeutics

11:00 AM–11:15 AM | **369**
REGEN-HEPS: Expandable and Functional Hepatocytes Derived from Primary Cells
Arnav Chhabra, PhD, Satellite Biosciences

11:15 AM–11:30 AM | **370**
A Synthetic Receptor Platform to Program Mesenchymal Stromal Cells for the Detection and Treatment of Osteoarthritis
Bonnie Walton, Vanderbilt University

11:30 AM–11:45 AM | **371**
Genetic Modification of Vascularized Composite Allografts via *Ex Vivo* Machine Perfusion
Raphaela Bento, Massachusetts General Hospital, Harvard Medical School

11:45 AM–12:00 PM | **372**
Nongenotoxic CD45-ADC-Based Conditioning Maintains Potent Suppression of Plasma Viremia in Nonhuman Primate Model of HIV
Jason Murray, PhD, Fred Hutchinson Cancer Center

SATURDAY, MAY 11

ORAL ABSTRACT SESSION

Novel Immune Effector Cell Manufacturing

Co-chairs: Ian Johnston, PhD, Miltenyi Biotec B.V. & Co. KG and Rimas Orentas, PhD, Caring Cross, Inc.

BALLROOM 2

10:15 AM–10:30 AM | **373**

Single-Chain Variable Fragment Affinity Tuning Can Optimize Anti-AML CAR-NK Cell Functionality
Ruyan Rahnama, Johns Hopkins School of Medicine

10:30 AM–10:45 AM | **374**

iMSCs Derived from mRNA-Engineered B2M-KO iPSCs Exhibit Enhanced Immunosuppressive Activity and Stealthing Features
Raven Hinkel, Factor Bioscience

10:45 AM–11:00 AM | **375**

Massively Parallel CAR-T Cell Phenotyping Enables Identification of High Efficiency Candidates
Gary Schroth, PhD, Cellanome

11:00 AM–11:15 AM | **376**

Chimeric Antigen Receptor Engineered Tregs with Donor Specificity and Enhanced Functional Stability for the Induction of Liver Transplantation Tolerance
Madhav Kishore, Quell Therapeutics

11:15-11:30 AM | **376**

NK510, a Small Genetic Edit, a Giant Leap for NK Cells
Tianhong Xu, Base Therapeutics

11:30 AM–11:45 AM | **378**

G Protein-Coupled Receptor Class C Group 5 Member D (Gprc5d) and B-Cell Maturation Antigen (BCMA) Bi-Specific Dual Chimeric Antigen Receptors (CARS) Effectively Address Antigen Escape and Tumor Heterogeneity Challenge in Multiple Myeloma (MM)
Chia-Wei Chang, PhD, Overland Pharmaceuticals (CY) Inc

11:45 AM–12:00 PM | **379**

A Same-Day Manufacturing Platform Leveraging an RNA-Based Lipid Nanoparticle (LNP) Gene Writer System to Generate Chimeric Antigen Receptor (CAR) T Cells
Kassi Stein, PhD, Tessera Therapeutics

SATURDAY, MAY 11

ORAL ABSTRACT SESSION

Base Editing and Prime Editing II

Co-chairs: Jakob Haldrup Jensen, PhD, University of Oxford and Scot Wolfe, PhD, UMass Chan Medical School

BALLROOM 3

10:15 AM–10:30 AM | **380**
Addressing the dNTP Bottleneck Restricting Prime Editing Activity
Scot Wolfe, PhD, UMass Chan Medical School

10:30 AM–10:45 AM | **381**
Characterization of Guide RNA Site Consistency Across Ancestries and the Potential for Off-Target Editing with the Clinical-Stage Base Editing Medicine, VERVE-101
Joseph Biedenkapp, PhD, Verve Therapeutics

10:45 AM–11:00 AM | **382**
The mitoDdCBE System as a Mitochondrial Gene Therapy Approach
Jose Domingo Barrera-Paez, University of Miami

11:00 AM–11:15 AM | **383**
Engineered IscB-ωRNA System with Expanded Target Range for Base Editing
Qingquan Xiao, HuidaGene Therapeutics Co., Ltd.

11:15 AM–11:30 AM | **384**
Development of Deaminase-Free Base Editors by Engineering DNA Glycosylases
Huawei Tong, HuidaGene Therapeutics Co., Ltd.

11:30 AM–11:45 AM | **385**
Systemic Delivery of LNP mRNA In Utero Permits Editing in a Mouse Model of Spinal Muscular Atrophy (SMA)
Nicole Kus, MD, Children's Hospital of Philadelphia

11:45 AM–12:00 PM | **386**
Traceless Delivery of Genome Editors
Jakob Haldrup Jensen, PhD, University of Oxford

SATURDAY, MAY 11

ORAL ABSTRACT SESSION

AAV Vector Biology and Development III

Co-chairs: Dawn Bowles, PhD, Duke University and
Xiaojing Shi, PhD, Capsida Biotherapeutics

BALLROOM 4

10:15 AM–10:30 AM | **387**
Analyzing and Improving AAV Vector Design with Long Read Sequencing and Analysis
Amicia Elliott, PhD, Form Bio

10:30 AM–10:45 AM | **388**
Unwanted Concatemeric Knock-Ins Occur Frequently with Cas9/AAV-Mediated Gene-Editing: Detection and Prevention
Fabian Suchy, PhD, Stanford University

10:45 AM–11:00 AM | **389**
Molecular Origami: AAV Genome Recombination Patterns Mediated by ITRs in the Liver
Liujiang Song, PhD, AskBio

11:00 AM–11:15 AM | **390**
Orthogonal Characterization of rAAV Genomes Reveals Vector Attributes That Drive Dimer Encapsidation and ITR Repair
Mitchell Yip, UMass Chan Medical School

11:15 AM–11:30 AM | **391**
An Evolved AAV Variant Enables *In Vivo* CAR-T Cell Generation, Tumor Cytotoxicity, and B Cell Elimination
Lijia Ma, PhD, Westlake University

11:30 AM–11:45 AM | **392**
Deep Learning and RNN-Enhanced Models for Tissue-Dependent Codon Optimization
Tapan Sharma, PhD, UMass Chan Medical School

11:45 AM–12:00 PM | **393**
A Strong and Compact Bidirectional Neuronal Promoter for AAV-Mediated and CNS-Directed Delivery of Dual Transgenes
Xiupeng Chen, UMass Chan Medical School

SATURDAY, MAY 11

ORAL ABSTRACT SESSION

Musculo-Skeletal Diseases

Co-chairs: Carsten Bonnemann, MD, NINDS/NIH, Chunping Qiao, PhD, Novartis Gene Therapies, and Markus Ruegg, PhD, Biozentrum, University of Basel

ROOM 307-308

10:15 AM–10:30 AM | **394**

Ten-Year Durability of AAV8-MTM1 Gene Transfer in a Canine Model of X-Linked Myotubular Myopathy
David Mack, PhD, University of Washington

10:30 AM–10:45 AM | **395**

RGX-202, an Investigational Gene Therapy for the Treatment of Duchenne Muscular Dystrophy: Interim Clinical Data
Jahannaz Dastgir, DO, REGENXBIO Inc.

10:45 AM–11:00 AM | **396**

Sustained Clinical Effects of Single-Dose Intraarticular PCRX201 Injection in Moderate-to-Severe Osteoarthritis of the Knee
Emily Walsh, PhD, Tremont Therapeutics Consulting

11:00 AM–11:15 AM | **397**

Correction of Myotonia and Reduction of Toxic DMPK Foci After Treatment of the HSALR Mouse Model of Myotonic Dystrophy Type 1 Using an Adeno-Associated Virus Delivering U7snRNA Interfering with CTG Repeat Expansion
Nicolas Wein, PhD, Abigail Wexner Research Institute at Nationwide Children's Hospital

11:15 AM–11:30 AM | **398**

CSF Delivery of INS1201 AAV9-Micro-Dystrophin Demonstrates Efficacy in a Mouse Model of DMD
Gretchen Thomsen, Insmed Gene Therapy

11:30 AM–11:45 AM | **399**

Evaluation of Novel Muscle-Specific Promoters in Mice and Non-Human Primates
Randolph Qian, PhD, REGENXBIO Inc.

SATURDAY, MAY 11

ORAL ABSTRACT SESSION (CONTINUED)

Musculo-Skeletal Diseases

Co-chairs: Carsten Bonnemann, MD, NINDS/NIH and
Chunping Qiao, PhD, Novartis Gene Therapies

ROOM 307-308

11:45 AM–12:00 PM | 400

An Optogenetics-Based Gene Therapy for Obstructive Sleep Apnea
Fiona Knapman, NeuRA

SATURDAY, MAY 11

ORAL ABSTRACT SESSION

Molecular and Cellular Methods and Technologies

Co-chairs: Quanyi (Charlie) Li, PhD, Spark Therapeutics and
Daniela Cesana, PhD, SR-Tiget

ROOM 309-310

10:15 AM–10:30 AM | **401**

Long-Term Tracking of Hematopoietic Clonal Dynamics and Mutations in Nonhuman Primate Post-Autologous Lentiviral-Barcoded Hematopoietic Stem and Progenitor Cell Transplantation
Rohan Hosuru, NIH

10:30 AM–10:45 AM | **402**

A Genotoxicity Mouse Model of HSPC Gene Therapy to Identify Early Biomarkers in the Interaction between Senescent Cells and the Immune System
Cristina Colleoni, SR-Tiget, IRCCS San Raffaele Scientific Institute

10:45 AM–11:00 AM | **403**

Exploring Novel Mechanisms of Alzheimer's Disease Pathophysiology through High-Dimension Transcriptomic Technology
Marie Johns, University of British Columbia

11:00 AM–11:15 AM | **404**

Stable Treatment of Infant Mice with Pompe Disease by Direct Transduction of Muscle
Kate Ilich, MD, Duke University

11:15 AM–11:30 AM | **405**

Epigenomic Fingerprinting of Primary Immune Cells Defines Unique Cell Identities
Andrea Johnstone, PhD, EpiCypher

11:30 AM–11:45 AM | **406**

Characterizing Insertional Oncogenic Risk of a Novel CD8-Targeted Fusosome for *In Vivo* CAR T Cell Generation
Christina Chaivorapol, PhD, Sana Biotechnology

SATURDAY, MAY 11

ORAL ABSTRACT SESSION (CONTINUED)

Molecular and Cellular Methods and Technologies

Co-chairs: Quanyi (Charlie) Li, PhD, Spark Therapeutics and
Daniela Cesana, PhD, SR-Tiget

ROOM 309-310

11:45 AM–12:00 PM | 407

Somatic Mutation Tracking in Hematopoietic Stem Cell Gene Therapy Reveals Absence of Clonal Hematopoiesis

Francesco Gazzo, SR-Tiget, IRCCS San Raffaele Scientific Institute

SATURDAY, MAY 11

ORAL ABSTRACT SESSION

Vector Product Engineering, Development, and Manufacturing (excluding AAV)

Co-chairs: Meisam Naeimi Kararoudi, DVM, PhD, Nationwide Children's Hospital and Raquel Martin Ibanez, PhD, Stanford University School of Medicine

ROOM 314-317

10:15 AM–10:30 AM	408	**Single and Low Dose Self-Replicating RNA Vaccine Provides Effective Immune Protection Against Rabies in Healthy Volunteers** Zelanna Goldberg, MD, Replicate Bioscience Inc
10:30 AM–10:45 AM	409	**Kinetics-Based Assembly of Shelf-Stable PEI/DNA Particles for Viral Vector Production** Jinghan Lin, Johns Hopkins University
10:45 AM–11:00 AM	410	**Machine-Designed Synthetic 3′ UTRs Significantly Increase mRNA Stability** Elise Flynn, PhD, Patch Biosciences
11:00 AM–11:15 AM	411	**Generation of Off-the-Shelf CAR Gamma Delta T Cells Using CRISPR and AAV, a New Era in Cell Therapy** Genesis Snyder, Nationwide Children's Hospital
11:15 AM–11:30 AM	412	**Lentiviral Vector Packaging and Producer Cell Lines Yield Titres Equivalent to the Industry-Standard Four-Plasmid Process** Qian Liu, PhD, OXGENE
11:30 AM–11:45 AM	413	**Establishment of a Scalable Stable Lentivirus Manufacturing Process in Suspension Perfusion Mode** Holger Laux, CSL Behring Innovation GmbH
11:45 AM–12:00 PM	414	**Stable Lentiviral Producer and Packaging Cell Lines Achieve > 1E8 TU/mL** Adam Carcella, Asimov

SATURDAY, MAY 11

ORAL ABSTRACT SESSION

Targeted Gene and Cell Therapy II

Co-chairs: Kah-Whye Peng, PhD, Mayo Clinic and
Stephen Russell, MD, PhD, Vyriad

ROOM 318-323

10:15 AM–10:30 AM | **415**

KRAS Mutant Gene Editing Abolishes Tumor Growth *In Vivo* and Overcomes Acquired Resistance to $KRAS^{G12C}$ and $KRAS^{G12D}$ Small Molecule Inhibitors
Neil Desai, PhD, AADIGEN

10:30 AM–10:45 AM | **416**

A High-Avidity, CD8-Co-Receptor-Independent, HPV-Specific TCR Coupled with a TGF-β Chimeric Switch Receptor Enhances Polyfunctional Profiles and Anti-Tumor Activities in HPV-16/52-Associated Cancers
Susanne Wilde, PhD, SCG Cell Therapy

10:45 AM–11:00 AM | **417**

Hostile Takeover: Diagnosing Cancer from within Using a Cancer-Activated Genetic Construct as a Novel Imaging Platform for NSCLC Detection
David Suhy, PhD, Earli, Inc

11:00 AM–11:15 AM | **324**

Healthy Donor-Derived Unedited CD7.CAR T-Cells for Improved Therapy of T-Cell Malignancies
Daniil Shmidt, MD, Baylor College of Medicine

11:15 AM–11:30 AM | **419**

Discovery of Tumor Reactive TCRs and Their Cognate Antigenic Targets via High-Throughput Functional Screening
Candace Perullo, TScan Therapeutics

11:30 AM–11:45 AM | **420**

CRISPR-Cas9 Genetic Targeting of Metastatic Pancreatic Cancer
Kirsten Bowland, PhD, Johns Hopkins University School of Medicine

SATURDAY, MAY 11

ORAL ABSTRACT SESSION (CONTINUED)

Targeted Gene and Cell Therapy II

Co-chairs: Kah-Whye Peng, PhD, Mayo Clinic and
Stephen Russell, MD, PhD, Vyriad

ROOM 318-323

11:45 AM–12:00 PM | 421

CRISPR-Cas9 for Selective Targeting of Somatic Mutations in Pancreatic Cancers: A Novel Cancer Gene Therapy Approach
Selina Shiqing Teh, PhD, Johns Hopkins University School of Medicine

SATURDAY, MAY 11

ORAL ABSTRACT SESSION

Nonviral Approaches for Therapeutic Applications

Co-chairs: Chantal Pichon, PhD, Centre de Biophysique Moléculaire- CNRS and Stefan Radtke, PhD, Fred Hutchinson Cancer Center

ROOM 327-329

10:15 AM–10:30 AM	422	**Optimization of Fluoroscopy Guided Ultrasound Mediated Gene Delivery in Canines for Sustained FVIII Expression** Carol Miao, PhD, Seattle Children's Research Institute
10:30 AM–10:45 AM	423	**Intrathecal Administration of Lipid Nanoparticles to Silence Pain-Associated Genes by CRISPR Interference** Thomas Payne, Monash Institute of Pharmaceutical Sciences
10:45 AM–11:00 AM	424	**Focused Ultrasound- and Long-Circulating Nanoparticle-Mediated Nucleic Acid Delivery and Genome Editing in the Brain** Gijung Kwak, PhD, University of Maryland School of Medicine
11:00 AM–11:15 AM	425	**Supramolecular Assembly of Polycation/mRNA Nanoparticles and *In Vivo* Monocyte Programming** Leonardo Cheng, Johns Hopkins University
11:15 AM–11:30 AM	426	**Non-Viral Targeted Laser Delivery of Multi-Characteristic Opsin Genes for Treatment of Geographic Atrophy** Samarendra Mohanty, Nanoscope Therapeutics Inc
11:30 AM–11:45 AM	427	**Unraveling a Multifactorial Host Immune Response to Intramuscular Electrotransfer of DNA-Encoded Antibody Therapy** Kevin Hollevoet, KU Leuven, University of Leuven
11:45 AM–12:00 PM	428	**Sustained Endogenous FVIII Expression in Hemophilia A Mice is Achieved through *In Vivo* CRISPR/Cas9 mRNA LNP Gene Editing** Chun-Yu Chen, PhD, Seattle Children's Research Institute

SATURDAY, MAY 11

ORAL ABSTRACT SESSION

Challenges to Immunological Responses to Therapeutic Interventions

Co-chairs: Roberto Calcedo, PhD, Affinia Therapeutics and
Ying Kai Chan, PhD, Harvard University

ROOM 339-342

10:15 AM–10:30 AM | **429**

Intricate Play Between Different Cell Types is Critical for CD8⁺ T Cell Response Against AAV Encoded Transgene Product in Liver
Sandeep Kumar, PhD, Indiana University

10:30 AM–10:45 AM | **430**

Immune Modulation via Expansion of Treg Cells by THOR-834 Leads to Enhanced rAAV Transgene Expression
Lavesh Gwalani, PhD, Sanofi

10:45 AM–11:00 AM | **431**

Single-Cell and TCR Analysis from Duchenne Muscular Dystrophy Patients Treated with AAV9 Mini-Dystrophin Reveals an Expansion of Human Herpesvirus-Specific T-Cells in a Patient That Exhibited Thrombotic Microangiopathy
Mark Brimble, PhD, St Jude Children's Research Hospital

11:00 AM–11:15 AM | **432**

Adeno-Associated Virus Serotype 8 Efficiently Transduces Multiple Antigen-Presenting Cells in the Liver
Di Cao, Herman B Wells Center for Pediatric Research, Indiana University

11:15 AM–11:30 AM | **433**

Artificial miRNA Delivered through AAV Induces Dorsal Root Ganglion Toxicity in Cynomolgus Macaques and C57BL6 Mice
Shih-Ching Joyce Lo, PhD, Biogen

11:30 AM–11:45 AM | **434**

Understanding the Immune Response to Central Nervous System-Directed Adeno-Associated Virus Vectors
Ashley Harkins, UMass Chan Medical School

SATURDAY, MAY 11

ORAL ABSTRACT SESSION (CONTINUED)

Challenges to Immunological Responses to Therapeutic Interventions

Co-chairs: Roberto Calcedo, PhD, Affinia Therapeutics and
Ying Kai Chan, PhD, Harvard University

ROOM 339-342

11:45 AM–12:00 PM **435**

***In Vivo* Delivery and Protection by Engineered Synthetic DNA-Encoded Influenza H1HA Head-Directed Monoclonal Antibodies in Mice**

Abigail Rose Trachtman, DVM, PhD, The Wistar Institute


28TH ANNUAL MEETING
ASGCT
NEW ORLEANS
MAY 13–17, 2025
SEE YOU
NEXT YEAR!