

AMERICAN SOCIETY OF
GENE & CELL THERAPY

23rd Annual Meeting

FINAL PROGRAM GUIDE

VIRTUAL MEETING
FORMAT



MAY
12-15,
2020





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- Immunotherapy for GD2 diffuse intrinsic pontine glioma (DIPG)
- Gene therapy for Fanconi anemia and cerebral adrenoleukodystrophy
- Antibody-based conditioning to replace chemotherapy in stem cell transplantation
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A WELCOME FROM GUANGPING GAO, PHD



Dear Colleagues,

On behalf of the American Society of Gene & Cell Therapy (ASGCT), it is my pleasure to welcome you to our first-ever virtual ASGCT Annual Meeting. I am honored to join thousands of our colleagues from around the globe who believe in our Society's mission of advancing knowledge, awareness, and education to expand the discovery and clinical application of gene and cell therapies for alleviation of human disease.

I'm especially grateful this year that we're able to come together (separately) to share valuable research in our field and learn from each other in this virtual environment. All of the material this week will be available to view online and I hope you enjoy all that we have to offer.

I would like to extend a warm welcome to Carl June, M.D. and Feng Zhang, Ph.D., who will present the George Stamatoyannopoulos Memorial Lecture on Wednesday and the Presidential Symposium on Thursday, respectively. I would also like to take this opportunity to congratulate Drs. Mavis Agbandje-McKenna, Daniel Bauer, James Dahlman, Viviana Gradinaru, Shengdar Tsai, and Peter Marks on the awards they will so deservedly receive.

This scientific and educational program reflects the wonderful advancements that have occurred in gene and cell therapy over the past year, and I remain humbled by enthusiasm of our invited speakers in delivering their expertise in this unprecedented environment.

In addition, we are thrilled to share more new abstracts than we ever have before. This incredible level of work demonstrates the advances across the breadth of our field that will be presented virtually as oral presentations or poster sessions.

Be sure to visit our virtual Exhibit Hall to find out about the products and services offered by our partner companies, via conversations at their virtual booths and more formal presentations in the Tools & Technologies Forum.

Finally, I would like to thank everyone involved in the planning and execution of our Annual Meeting, especially the Program Committee, Scientific and Education Committees, abstract reviewers, staff, and all of our volunteers. Their sustained support, dedication, and hard work throughout the year now comes to fruition in what is sure to be a spectacular meeting.

Sincerely,

A handwritten signature in black ink, appearing to be 'G. Gao', written in a cursive style.

Guangping Gao, Ph.D. *President, ASGCT*

ATTEND THE 23RD ANNUAL VIRTUAL MEETING

- Live-stream event coverage
- Attend the virtual career fair
- Visit the virtual exhibit hall

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App support:
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support@tripbuildermedia.com

GENERAL MEETING INFORMATION

American Society of Gene & Cell Therapy

MISSION AND VISION

The mission of ASGCT is to advance knowledge, awareness, and education leading to the discovery and clinical application of genetic and cellular therapies to alleviate human disease.

ASGCT's strategic vision is to be a catalyst for bringing together scientists, physicians, patient advocates, and other stakeholders to transform the practice of medicine by incorporating the use of genetic and cellular therapies to control and cure human disease.

ABSTRACT VOLUME - MOLECULAR THERAPY

All abstracts accepted for presentation at the ASGCT 23rd Annual Meeting have been published in the May supplement of *Molecular Therapy*. Attendees are able to access a copy of the supplement online at the *Molecular Therapy* website, or have access to the abstracts through the Mobile App.

CONTINUING MEDICAL EDUCATION

The ASGCT Board of Directors decided that the Society will not offer CME credit for the 23rd Annual Meeting.

DISCLOSURE

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

EDUCATIONAL METHODS AND MATERIALS

Lectures, Case Presentations, Panel Discussions, Question and Answer Sessions, Audio/Video Presentations, Abstracts, Posters.

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.

GENERAL MEETING INFORMATION

EVALUATION METHOD

Evaluation by questionnaire will address program content, presentation, and possible bias.

NEEDS

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

TARGET AUDIENCE

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

DATES

The ASGCT 23rd Annual Meeting will begin on the morning of Tuesday, May 12, 2020 and continue through 12 pm on Friday, May 15, 2020. Exhibits will be open 24/7, Monday, May 11 through Friday, May 15.

MOBILE APP

The full ASGCT 23rd Annual Meeting program, including session schedules, faculty, exhibits, sponsors and abstracts, is available through the ASGCT Mobile App which can be accessed by searching "ASGCT" on the Apple App Store or Google Play Store to download.

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PROGRAM COMMITTEE & ABSTRACT PLANNING COMMITTEE

2020 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 23rd Annual Meeting.

CHAIR

Guangping Gao, PhD – *University of Massachusetts Medical School*

MEMBERS

Jennifer E. Adair, PhD – *Fred Hutchinson Cancer Research Center*

Aravind Asokan, PhD – *Duke University School of Medicine*

Paula M. Cannon, PhD – *University of Southern California*

Terence R. Flotte, MD – *University of Massachusetts Medical School*

2020 Abstract Planning Committee

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

PRESIDENT

Guangping Gao, PhD – *University of Massachusetts Medical School*

ASGCT PRESIDENT-ELECT

Stephen J. Russell, MD, PhD – *Mayo Clinic*

ASGCT SECRETARY AND ABSTRACT CHAIR

Terence R. Flotte, MD – *University of Massachusetts Medical School*

ASGCT PROGRAM COMMITTEE MEMBERS

Aravind Asokan, PhD – *Duke University School of Medicine*

Paula M. Cannon, PhD – *University of Southern California*

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Please join us at our scientific oral presentations:

“Gb3 substrate in endothelial cells of renal peritubular capillaries was reduced in a previously untreated classic Fabry male patient treated with AVR-RD-01 investigational lentiviral gene therapy”

AVROBIO-sponsored Phase 2 trial of AVR-RD-01

Birgitte Volck, M.D., Ph.D., president of Research & Development, AVROBIO

Wednesday, May 13, 2020, 5 – 5:15 p.m. ET

“Hematopoietic stem cell gene therapy for cystinosis: Initial results from a Phase I/II clinical trial”

Collaborator-sponsored Phase 1/2 trial of AVR-RD-04*

Stephanie Cherqui, Ph.D., associate professor, University of California, San Diego

Wednesday, May 13, 2020, 4:30 – 4:45 p.m. ET

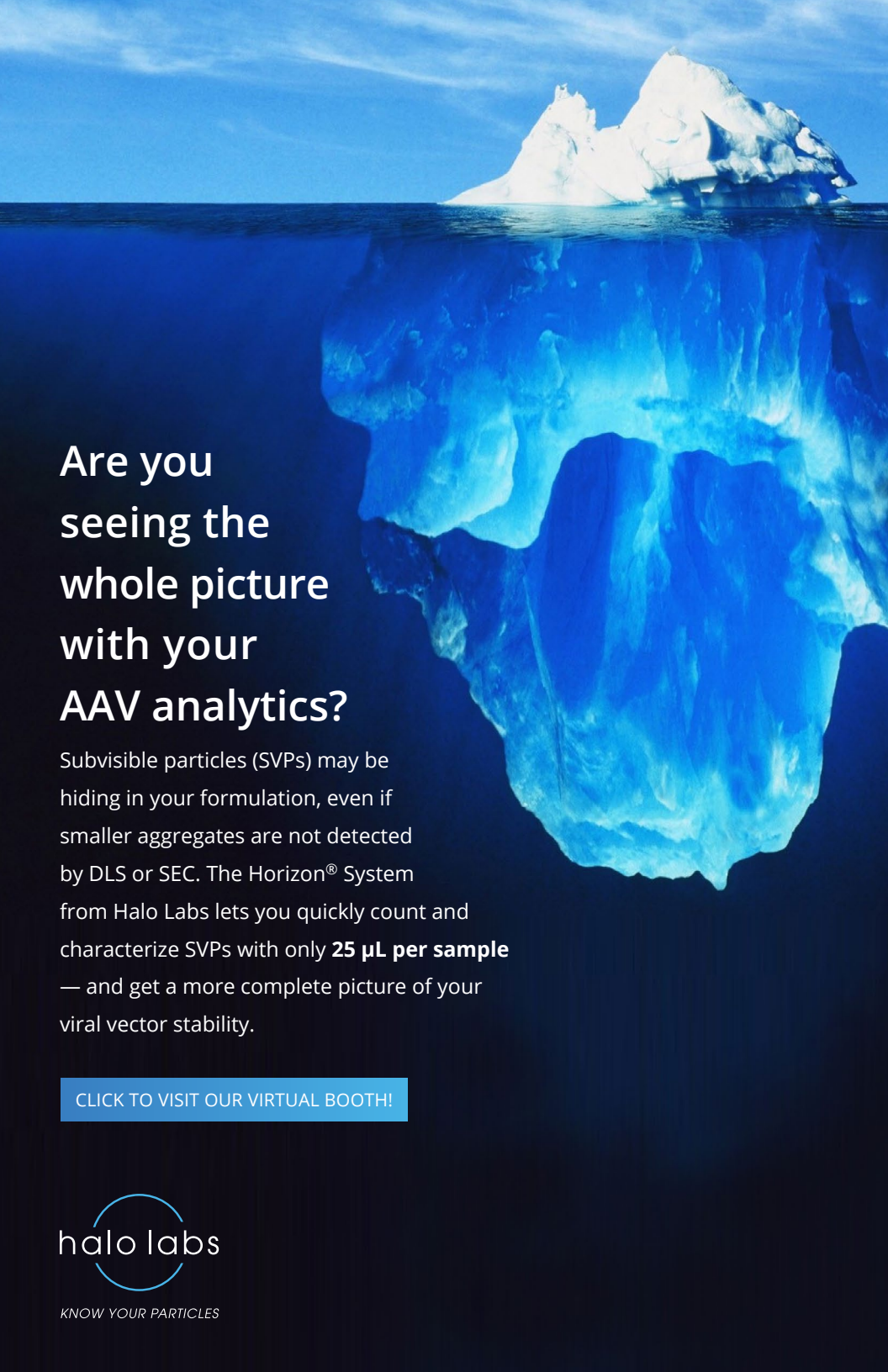
**The Phase 1/2 clinical trial is funded by grants to UCSD from the California Institute for Regenerative Medicine (CIRM) and the Cystinosis Research Foundation (CRF).*

“Hematopoietic stem cell gene therapy corrects neuromuscular manifestations in preclinical study of Pompe mice”

AVROBIO-sponsored Phase 1/2 trial of AVR-RD-03

Niek van Til, Ph.D., senior director, AVROBIO

Thursday, May 14, 2020, 4:30 – 4:45 p.m. ET



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FROM BENCH TO BEDSIDE: UNCOVERING THE POTENTIAL OF GENE THERAPY

PTC Therapeutics Symposium at the 23rd Annual Meeting of the American Society of Gene & Cell Therapy (ASGCT)

Join our expert Faculty for an interactive discussion on hot topics and key challenges associated with the constantly evolving field of gene therapy. In a peer-to-peer setting, we will share key insights gathered through our experience in this space while illustrating our commitment to finding innovative treatment options for patients with rare diseases.

We look forward to connecting with you.

Thursday, May 14 2020
12:00–1:30pm EDT

Please find our symposium
webinar by following this link:
asgct.org/2020

Led by:

Mark Pykett

Chief Scientific Officer
PTC Therapeutics

Timothy Feyma

Co-Director, Complex
Movement Disorder Clinic
Gillette Children's Specialty
Healthcare
St Paul, Minnesota

Time	Topic	Speaker
12:00–12:05	Welcome and Introduction	Mark Pykett, Chair
12:05–12:40	Overview of PTC Therapeutics' Gene Therapy Programs	Mark Pykett
12:40–13:10	Gene Therapy for Neurological Disease: Clinical Outcomes in AADC Deficiency	Timothy Feyma
13:10–13:25	Interactive Q&A	All (Moderated by Chair)
13:25–13:30	Adjourn	Mark Pykett

ABSTRACT REVIEWERS

Thank you to our 2020 abstract reviewers for their time and expertise!

RNA VIRUS VECTORS

Brian W. Bigger, PhD – *University of Manchester*

Bobby Gaspar, MD, PhD – *UCL Great Ormond Street Institute of Child Health*

Carol H. Miao, PhD – *University of Washington*

Axel Schambach, MD, PhD – *Hannover Medical School*

Kshitiz Singh, MBBS, MMST – *Children's Hospital of Philadelphia*

Andrew C. Wilber, PhD – *SIU School of Medicine*

AAV VECTORS - VIROLOGY AND VECTOROLOGY

Aravind Asokan, PhD – *Duke University School of Medicine*

John A. Chiorini, PhD – *NIDCR/National Institutes of Health*

Beverly L. Davidson, PhD – *Children's Hospital of Philadelphia*

Federico Mingozzi, PhD – *Spark Therapeutics*

Luk H. Vandenberghe, PhD – *Massachusetts Eye and Ear/Broad Institute/Harvard*

Jun Xie, PhD – *University of Massachusetts Medical School*

AAV VECTORS - PRECLINICAL AND PROOF-OF-CONCEPT STUDIES

Shannon E. Boye, PhD – *University of Florida*

Allison M. Bradbury, PhD – *Nationwide Children's Hospital*

Vania Broccoli, PhD – *Ospedale San Raffaele*

Miguel Sena-Esteves, PhD – *University of Massachusetts Medical School*

Phillip WL Tai, PhD – *University of Massachusetts, Medical School*

Jagdeep S. Walia, MBBS, FRCPC, FCCMG – *Queen's University*

AAV VECTORS - CLINICAL STUDIES

Carsten G. Bonnemann, MD – *NINDS/NIH*

Florian Eichler, MD – *Massachusetts General Hospital*

Steven J. Gray, PhD – *University of Texas Southwestern*

Daniel M. Lipinski, MSc, DPhil – *Medical College of Wisconsin*

Jerry R. Mendell, MD – *Gene Therapy Center Nationwide*

Amit Nathwani, MD, PhD – *Freeline Therapeutics*

GENE TARGETING AND GENE CORRECTION

Paula M. Cannon, PhD – *University of Southern California*

Benjamin P. Kleinstiver, PhD – *Massachusetts General Hospital and Harvard Medical School*

Punam Malik, MD – *Cincinnati Children's Hospital Medical Center*

Shondra M. Miller, PhD – *St. Jude Children's Research Hospital*

David J. Segal, PhD – *UC Davis Genome Center*

Scott A. Wolfe, PhD – *University of Massachusetts Medical School*



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Catarina Flyborg
Vice President
Cell & Gene Therapy,
Cytiva

Learn about advances in
AAV production at our industry
symposium 13 May 2020 at 12:00 pm.

ABSTRACT REVIEWERS

OLIGONUCLEOTIDE THERAPEUTICS

Annemieke M. Aartsma-Rus, PhD – *Leiden University Medical Center*
Mark A. Behlke, MD, PhD – *Integrated DNA Technologies*
Si-ping Han, PhD – *Beckman Research Institute, City of Hope*
Mark A. Kay, MD, PhD – *Stanford University School of Medicine*
Anastasia Khvorova, PhD – *University of Massachusetts Medical School*
Xuehai Liang, PhD – *Ionis Pharmaceuticals, Inc.*

SYNTHETIC/MOLECULAR CONJUGATES AND PHYSICAL METHODS FOR DELIVERY OF GENE THERAPEUTICS

David A. Dean, PhD – *University of Rochester*
Dexi Liu, PhD – *University of Georgia College of Pharmacy*
Angela K. Pannier, PhD – *University of Nebraska-Lincoln*
Kevin G. Rice, PhD – *University of Iowa College of Pharmacy*
Daniel Siegwart, PhD – *UT Southwestern Medical Center*
Matthew H. Wilson, MD, PhD – *Vanderbilt University*
Assem G. Ziady, PhD – *Cincinnati Children's Hospital Medical Center*



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ABSTRACT REVIEWERS

METABOLIC, STORAGE, ENDOCRINE, LIVER AND GASTROINTESTINAL DISEASES

Nicola Brunetti-Pierri, MD – *Telethon Institute of Genetics & Medicine*

Cary O. Harding, MD – *Oregon Health & Sciences University*

Dwight Koeberl, MD, PhD – *Duke University Medical Center*

Gerald S. Lipshutz, MD – *David Geffen School of Medicine at UCLA*

Pasquale Piccolo, PhD – *Telethon Institute of Genetics and Medicine*

Charles P. Venditti, MD, PhD – *National Human Genome Research Institute*

CARDIOVASCULAR AND PULMONARY DISEASES

Uta Griesenbach, PhD – *Imperial College Faculty of Medicine*

Costanza Emanuelli, PhD, FAHA – *Imperial College of London National Heart & Lung Institute*

Judith S. Greengard, PhD – *Adverum Biotechnologies*

Xiao Xiao, PhD – *University of North Carolina at Chapel Hill*

David W. Parsons, PhD – *Women's & Children's Hospital*

Patrick L. Sinn, PhD – *University of Iowa*



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RON

Hemophilia B patient in AMT-061 gene therapy trial

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NEUROLOGIC DISEASES

Rebecca Ahrens-Nicklas, MD, PhD – *Children's Hospital of Philadelphia*

Rita Batista, PhD – *University of Massachusetts Medical School*

Paul D. Gamlin, PhD – *University of Alabama at Birmingham*

Gwladys Gernoux, PhD – *University of Nantes*

Heather L. Gray-Edwards, DVM, PhD – *University of Massachusetts Medical School*

Dan Wang, PhD – *University of Massachusetts Medical School*

MUSCULO-SKELETAL DISEASES

Jeffrey S. Chamberlain, PhD – *University of Washington School of Medicine*

Dongsheng Duan, PhD – *University of Missouri School of Medicine*

Paul Gregorevic, PhD – *The University of Melbourne*

Scott Q. Harper, PhD – *Ohio State University & Nationwide Children's Hospital*

Rita Perlingeiro, PhD – *University of Minnesota*

Kathryn R. Wagner, MD, PhD – *The Johns Hopkins School of Medicine*



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CANCER - IMMUNOTHERAPY, CANCER VACCINES

Chiara Bonini, MD – *Università Vita Salute San Raffaele*

Maria G. Castro, PhD – *University of Michigan Medical School*

Monica Casucci, MSc – *San Raffaele Scientific Institute*

Conrad Russell Y. Cruz, MD, PhD – *Children's National Hospital*

Paola Grandi, PhD – *University of Pittsburgh*

CANCER - ONCOLYTIC VIRUSES

Evanthia Galanis, MD, DSc – *Mayo Clinic*

Karin Jooss, PhD – *Gritstone Oncology, Inc.*

David H. Kirn, MD – *4D Molecular Therapeutics*

Melissa A. Kotterman, PhD – *4D Molecular Therapeutics*

Liliana Maruri Avidal, PhD – *Ignite Immunotherapy*

Stephen J. Russell, MD, PhD – *Mayo Clinic*



4DMT

PRECISION-GUIDED GENE THERAPY

ORAL PRESENTATIONS

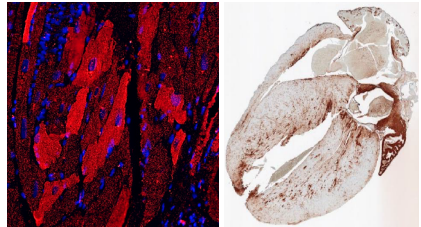
A Highly-Evolved Novel AAV Gene Therapy Directly Addresses Fabry Disease Pathology In Vivo by Cell Autonomous Expression in the Heart and Other Target Organs

Date & Time: Tuesday May 12, 2020
5:15 PM - 5:30 PM ET

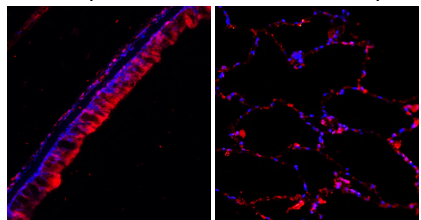
Directed Evolution of AAV Targeting Lung Epithelia Using Aerosol Delivery Identifies 4D-A101, a Variant Demonstrating Robust Gene Delivery in Non-Human Primates

Date & Time: Friday May 15, 2020
11:00 AM – 11:15 AM ET

Robust transgene payload expression in heart tissue from NHP and Fabry knockout mice



4D-A101 payload expression in primate upper airway and alveoli after aerosol delivery



ABSTRACT REVIEWERS

CANCER - TARGETED GENE AND CELL THERAPY

Prasad S. Adusumilli, MD – *Memorial Sloan Kettering Cancer Center*

Catherine M. Bollard, MD – *Children's National Medical Center*

Marco L. Davila, MD, PhD – *H. Lee Moffitt Cancer Center and Research Institute*

Saad Kenderian, MD – *Mayo Clinic*

Michael C. Milone, MD PhD – *Hospital of the University of Pennsylvania*

Yiping Yang, MD, PhD – *Ohio State University*

HEMATOLOGIC AND IMMUNOLOGIC DISEASES

Daniel E. Bauer, MD, PhD – *Boston Children's Hospital/Harvard Medical School*

Cynthia Dunbar, MD – *NIH/NHLBI Translational Stem Cell Biology Branch*

Lindsey A. George, MD – *The Children's Hospital of Philadelphia*

Megan D. Hoban, PhD – *bluebird bio*

Jean-Yves Metais, PhD – *St. Jude Children's Research Hospital*

Emmanuelle Six, PhD – *Imagine Institute*

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ABSTRACT REVIEWERS

IMMUNOLOGICAL ASPECTS OF GENE THERAPY AND VACCINES

Andrea Annoni, PhD – *San Raffaele Telethon Inst. for Gene Therapy*

Roberto Calcedo, PhD – *Affinia Therapeutics*

Ying Kai Chan, PhD – *Harvard*

Roland W. Herzog, PhD – *Indiana University*

Allison M. Keeler-Klunk, PhD – *University of Massachusetts Medical School*

Masataka Suzuki, PhD – *Baylor College of Medicine*

CELL THERAPIES

Jennifer E. Adair, PhD – *Fred Hutchinson Cancer Research Center*

M Graça D. Almeida-Porada, MD, PhD – *Wake Forest School of Medicine*

Paul Gadue, PhD – *Children's Hospital of Philadelphia*

Donald B. Kohn, MD – *University of California, Los Angeles*

Eirini P. Papapetrou, MD, PhD – *Icahn School of Medicine at Mount Sinai*

Alice F. Tarantal, PhD – *University of California, Davis*

VECTOR AND CELL ENGINEERING, PRODUCTION OR MANUFACTURING

Matthias Hebben, PhD – *LogicBio Therapeutics*

Robert M. Kotin, PhD – *University of Massachusetts Medical School*

Maritza C. McIntyre, PhD – *Advanced Therapies Partners, LLC*

Isabelle Riviere, PhD – *Memorial Sloan Kettering Cancer Center*

Johannes C.M. van Der Loo, PhD – *Children's Hospital of Philadelphia*

J. Fraser Wright, PhD – *Stanford School of Medicine*

PHARMACOLOGY/TOXICOLOGY STUDIES OR ASSAY DEVELOPMENT

Eva Andres-Mateos, PhD – *Akouos, Inc.*

Linda B. Couto, PhD – *Akouos, Inc.*

David J. Dismuke, PhD – *StrideBio*

Virginia A. Haurigot, PhD – *Spark Therapeutics*

Jakob Reiser, PhD – *Food and Drug Administration*

Abraham Scaria, PhD – *Iveric Bio*

Join the ESGCT e-School...

...live and online every Wednesday at **14:00 CEST**.

This weekly programme of FREE 30 minute virtual lectures is designed especially for students and Early Career Researchers. The e-School has been created in lieu of the 2020 ESGCT Spring School in Athens, which has now been postponed to March 2021.

Cutting-edge updates on SARS CoV2

The ESGCT e-Seminars series is a programme of FREE online specialist lectures. This topical series focuses and provides cutting-edge updates on SARS CoV2. Five talks are currently confirmed, and this weekly series begins on Wednesday 20 May 2020 at **15:00 CEST**.

Athens Spring School 2021

New dates: 17-19 March 2021

28th Congress

in collaboration with BSGCT:
New dates coming shortly.



FACULTY LIST

Annemieke Aartsma-Rus, PhD
Leiden University Medical Center
Leiden, Netherlands

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Seattle, WA

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San Francisco, CA

Nadav Ahituv, PhD
University of California, San Francisco
San Francisco, CA

Nabil Ahmed, MD
Baylor College of Medicine
Houston, TX

Alessandro Aiuti, MD, PhD
IRCCS San Raffaele Scientific Institute;
San Raffaele Telethon Institute for
Gene Therapy (SR-Tiget); Vita-Salute
San Raffaele University
Milan, Italy

Juliana Alvarez, MD
Medical College of Wisconsin
Milwaukee, WI

Eduard Ayuso, DVM, PhD
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Nantes, France

John Bell, PhD
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Maria Bernardo, MD, PhD
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Milan, Italy

Antonio Bertoletti, MD
Duke-Nus Medical School
Singapore

Michael R. Betts, PhD
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Kapil Bharti, PhD
NEI/NIH
Bethesda, MD

Diana Bharucha-Goebel, MD
NIH & Children's National Health
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Michael Brehm, PhD
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Malcolm Brenner, PhD
Baylor College of Medicine
Houston, TX

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Milano, Italy

PJ Brooks, PhD

National Center for Advancing
Translational Sciences, NIH
Bethesda, MD

Christine Brown, PhD

City of Hope National Medical Center
Duarte, CA

Christian Buchholz, PhD

Paul-Ehrlich-Institut
Langen, Germany

Hildegard Büning, PhD

Hannover Medical School
Hanover, Germany

Barry J. Byrne, MD, PhD

University of Florida College of
Medicine
Gainesville, FL

Roberto Calcedo, PhD

University of Pennsylvania
Philadelphia, PA

Jose Cancelas, PhD

University of Cincinnati Hoxworth
Blood Center
Cincinnati, OH

Nathalie Cartier-Lacave, PhD

INSERM
Paris, France

Toni Cathomen, PhD

Medical Center - University of
Freiburg
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Beckman Research Institute of City
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Assem G Ziady, PhD

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John Anthony Zuris, PhD

Editas Medicine
Cambridge, MA

ASGCT AWARD RECIPIENTS

Congratulations to the following individuals for receiving an ASGCT Award.

OUTSTANDING NEW INVESTIGATOR AWARDS

Daniel Bauer, MD, PhD

Principal Investigator and Staff Physician, Dana-Farber/Boston Children's Cancer and Blood Disorders Center, Assistant Professor in Pediatrics, Harvard Medical School

James Dahlman, PhD

Assistant Professor, Wallace H. Coulter Department of Biomedical Engineering at Georgia Tech and Emory University

Viviana Gradinaru, PhD

Professor of Neuroscience and Biological Engineering, California Institute of Technology

Shengdar Tsai, PhD

Assistant Member in the Department of Hematology, St. Jude Children's Research Hospital

The Outstanding New Investigator are sponsored by



OUTSTANDING ACHIEVEMENT AWARD

Mavis Agbandje-McKenna, PhD

Professor of Biochemistry and Molecular Biology, University of Florida College of Medicine

The Outstanding Achievement Award is sponsored by



SONIA SKARLATOS PUBLIC SERVICE AWARD

Peter Marks, MD, PhD

Director of the Center for Biologics Evaluation and Research (CBER), U.S. Food and Drug Administration

ASGCT AWARD RECIPIENTS

EXCELLENCE IN RESEARCH AWARDS

The Excellence in Research Awards will be presented on Wednesday, May 13 during the George Stamatoyannopoulos Memorial Lecture.

The Excellence in Research Awards are sponsored by



The Meritorious Abstract Travel Awards and Underrepresented Minority Travel Awards are sponsored by



Funding for this program was made possible (in part) by (5 R13 TR 002411 - 02) from the National Center for Advancing Translational Sciences (NCATS). The views expressed in written program materials or publications and by speakers and moderators do not necessarily reflect the official policies of the Department of Health and Human Services; nor does mention by trade names, commercial practices, or organizations imply endorsement by the U.S. Government.



SCHEDULE AT A GLANCE

All times are listed in Eastern Daylight Time (EDT UTC -4).

TUESDAY, MAY 12, 2020

Open 24 Hours Online	Exhibit Hall Open <i>Exhibitors Available 8:00 am - 6:30 pm</i>
Open 24 Hours Online	Virtual Career Fair
8:00 am – 9:45 am	Scientific Symposia Session I
10:15 am – 12:00 pm	Oral Abstract Session I
12:00 pm – 1:30 pm	Lunch Break
12:00 pm – 1:30 pm	Tools and Technologies Forum I
1:30 pm – 3:15 pm	Education Session I
3:30 pm – 4:45 pm	Tools and Technologies Forum II
3:30 pm – 4:45 pm	Oral Abstract Session II

WEDNESDAY, MAY 13, 2020



Open 24 Hours Online	Exhibit Hall Open <i>Exhibitors Available 8:00 am - 6:30 pm</i>
Open 24 Hours Online	Virtual Career Fair
8:00 am – 9:45 am	Scientific Symposia Session II
8:00 am – 9:45 am	Education Session II
10:15 am – 12:00 pm	George Stamatoyannopoulos Memorial Lecture & Presentation of the Excellence in Research Awards <i>Sponsored by</i> 
12:00 pm – 1:30 pm	Lunch Break
12:00 pm – 1:30 pm	Tools and Technologies Form III
12:00 pm – 1:30 pm	Industry Sponsored Symposia - <i>Sponsored by Cytiva, formerly known as GE Healthcare Life Sciences, Sarepta Therapeutics, Pall Biotech</i>
1:30 pm – 3:00 pm	Outstanding New Investigator Symposium <i>Sponsored by</i> 
3:30 pm – 4:45 pm	Tools and Technologies Forum IV
3:30 pm – 4:45 pm	Oral Abstract Session III

Eastern Time Zone (EDT UTC -4)

SCHEDULE AT A GLANCE

All times are listed in Eastern Daylight Time (EDT UTC -4).

THURSDAY, MAY 14, 2020

Open 24 Hours Online	Exhibit Hall Open <i>Exhibitors Available 8:00 am - 6:30 pm</i>
Open 24 Hours Online	Virtual Career Fair
8:00 am – 9:45 am	Scientific Symposia Session IV
10:15 am – 12:00 am	Outstanding Achievement Award Lecture & Sonia Skarlatos Public Service Award Presentation <i>Sponsored by</i> 
12:00 pm – 1:30 pm	Lunch Break
12:00 pm – 1:30 pm	Tools and Technologies Forum V
12:00 pm – 1:30 pm	Industry Sponsored Symposia - <i>Sponsored by Terumo BCT, FUJIFILM Diosynth Biotechnologies, PTC Therapeutics, GenScript USA Inc.</i>
1:30 pm – 3:15 pm	Presidential Symposium & Presentation of the Top Abstracts <i>Sponsored by</i> 
3:45 pm – 5:30 pm	Oral Abstract Session IV

FRIDAY, MAY 15, 2020

Open 24 Hours Online	Exhibit Hall Open <i>Exhibitors Available 8:00 am - 12:00 pm</i>
Open 24 Hours Online	Virtual Career Fair
7:30 am - 8:00 am	ASGCT Business Meeting
8:00 am – 9:45 am	Scientific Symposia Session V
9:45 am – 10:15 am	Break
10:15 am - 12:00 pm	Oral Abstract Session V
12:00 pm	Annual Meeting Concludes

Eastern Time Zone (EDT UTC -4)

PROGRAM SCHEDULE

TUESDAY, MAY 12

All times are listed in Eastern Daylight Time (EDT UTC -4).

SCIENTIFIC SYMPOSIUM 100

8:00 AM - 9:45 AM

Room: Ballroom B

**Developing New Therapies for Children with Metabolic Disorders -
Organized by the Gene & Cell Therapy of Genetic and Metabolic Diseases
Committee**

CO-CHAIRS: Paris Margaritis, DPhil and Dao Pan, PhD

SPEAKERS

8:00 AM – 8:26 AM

Petr Ilyinskii, PhD. Selecta Biosciences, Watertown, MA

ImmTOR Immune Tolerance

8:26 AM – 8:52 AM

Lili Wang, PhD. University of Pennsylvania, Philadelphia, PA

mRNA Therapy and Gene Editing for OTC Deficiency

8:52 AM – 9:18 AM

Andrés F. Muro, PhD. International Centre for Genetic Engineering and
Biotechnology (ICGEB), Trieste, Italy

Promoterless Gene Editing in Crigler-Najjar

9:18 AM – 9:45 AM

Gerald Lipshutz, MD. David Geffen School of Medicine at UCLA, Los
Angeles, CA

mRNA Therapy in Arginase Deficiency

SCIENTIFIC SYMPOSIUM 101

8:00 AM - 9:45 AM

Room: Ballroom A

**Advances in Allogeneic and Off-the-Shelf Hematopoietic Cell Therapies -
Organized by the Hematologic and Immunologic Gene and Cell Therapy
Committee**

CO-CHAIRS: David Markusic, PhD and Jayandharan Rao, PhD

SPEAKERS

8:00 AM – 8:26 AM

Barbra J. Sasu, PhD. Allogene, South San Francisco, CA

Allogeneic CART Cells for the Treatment of Cancer

PROGRAM SCHEDULE

TUESDAY, MAY 12

All times are listed in Eastern Daylight Time (EDT UTC -4).

8:26 AM – 8:52 AM

Agnieszka Czechowicz, MD, PhD. Stanford University School of Medicine, Stanford, CA

Improving HSCT Transplantation by Novel Conditioning - How it Applies to Allogeneic HSCT

8:52 AM – 9:18 AM

Branden Moriarity, PhD. University of Minnesota, Minneapolis, MN

Engineering NK Cells and Monocytes for Research and Therapies

9:18 AM – 9:45 AM

Gay M. Crooks, MD. University of California Los Angeles, Los Angeles, CA

From Pluripotent Stem Cells to Engineered T Cells

SCIENTIFIC SYMPOSIUM 102

8:00 AM – 9:45 AM

Room: 302

Growing Gene and Cell Therapy (GGACT): An NCATS-funded Collaborative Effort to Support Development of Academic Clinical Trials for Rare Diseases

CO-CHAIRS: Jose Cancelas, PhD and Donald Kohn, MD

SPEAKERS

8:00 AM – 8:25 AM

David A. Wilcox, PhD. Department of Pediatrics, Medical College of Wisconsin, Milwaukee, WI; Children's Research Institute, Children's Hospital of Wisconsin, Milwaukee, WI

Pilot and Feasibility Study of Hematopoietic Stem Cell Gene Transfer to Utilize Platelet-Derived Factor VIII for Hemophilia A: Transduction Process Development Speakers

8:25 AM – 8:50 AM

Bruce Trapnell, MD. Cincinnati Children's Hospital Medical Center, Cincinnati, OH

Pulmonary Transplantation of Autologous CD34+ Cell Derived Macrophages for Hereditary Pulmonary Alveolar Proteinosis

8:50 AM – 9:15 AM

Jianping Huang, MD, PhD. University of Florida, Gainesville, FL

Clinical Development of CART Cell Therapy Targeting CD70 in Glioblastoma

PROGRAM SCHEDULE

TUESDAY, MAY 12

All times are listed in Eastern Daylight Time (EDT UTC -4).

9:15 AM – 9:45 AM

David Wald, PhD, MD. Case Western Reserve University, Cleveland, OH
NK Cell Therapy for AML

SCIENTIFIC SYMPOSIUM 103

8:00 AM - 9:45 AM

Room: Ballroom C

New Developments in Particles, Transfer Methods and Vectors for Gene Delivery - Organized by the Physical Delivery, Therapeutics & Vector Development Committee

CO-CHAIRS: Carol Miao, PhD and Matthew Wilson, PhD

SPEAKERS

8:00 AM – 8:26 AM

Jennifer Adair, PhD. Fred Hutchinson Cancer Research Center, Seattle, WA
Nanoformulations to Deliver CRISPR Gene Editing

8:26 AM – 8:52 AM

Gabriele Thumann, MD. University of Geneva, Geneva, Switzerland
Gene Transfer to the Eye Using Transposons

8:52 AM – 9:18 AM

Mariola Fotin-Mleczek, PhD. CureVac AG, Tübingen, Germany
RNA Delivery as a Vaccine

9:18 AM – 9:45 AM

Richard Price, PhD. University of Virginia, Charlottesville, VA
To the Blood-Brain Barrier and Beyond: MRI-Guided Gene Delivery with Focused Ultrasound

PROGRAM SCHEDULE

TUESDAY, MAY 12

All times are listed in Eastern Daylight Time (EDT UTC -4).

SCIENTIFIC SYMPOSIUM 104

8:00 AM - 9:45 AM

Room: 312

Pharmacovigilance and Long-Term Follow-Up of Gene Therapies in the Post-Approval World - Organized by the Regulatory Affairs Committee

CO-CHAIRS: Marcela Maus, PhD and Kaye Spratt, PhD

SPEAKERS

8:00 AM – 8:20 AM

Kerry Welsh, MD, PhD. Food and Drug Administration, Silver Spring, MD

Collection and Use of Long-Term Follow-Up/Pharmacovigilance Data in the Post-Market Setting

8:20 AM – 8:40 AM

Kenneth Cornetta, MD. Indiana University, Indianapolis, IN

Long-Term Results of Replication-Competent Vector Testing

8:40 AM – 9:00 AM

Marcelo Pasquini, MD. Medical College of Wisconsin, Milwaukee, WI

CIBMTR's Registry Collection of LTFU Data for CAR T-Cell Therapies

9:00 AM – 9:20 AM

Anne-Virginie Eggimann, MSc. bluebird bio, Cambridge, MA

Challenges Associated With Long-Term Follow-Up of Gene Therapies

9:20-9:45 AM

Panel Discussion

SCIENTIFIC SYMPOSIUM 105

8:00 AM - 9:45 AM

Room: 304 & 306

Successes in Gene Therapy for Musculoskeletal Disease - Organized by the Musculo-Skeletal Gene & Cell Therapy Committee

CO-CHAIRS: Claudia Harper, DABT and Rita Perlingeiro, PhD

SPEAKERS

8:00 AM – 8:26 AM

Xiao Xiao, PhD. University of North Carolina at Chapel Hill, Chapel Hill, NC

DMD Mechanisms and Therapy Development

PROGRAM SCHEDULE

TUESDAY, MAY 12

All times are listed in Eastern Daylight Time (EDT UTC -4).

8:26 AM – 8:52 AM

Jeffrey Chamberlain, PhD. University of Washington School of Medicine, Seattle, WA

Development and Delivery of Microdystrophins

8:52 AM – 9:18 AM

Jerry R. Mendell, MD. Gene Therapy Center Nationwide, Columbus, OH

Clinical Development of Gene Therapy Vector Applications for the Muscular Dystrophies

9:18 AM – 9:45 AM

Laurie Goodrich, DVM, PhD. Colorado State University College of Veterinary Medicine, Fort Collins, CO

AAV Gene Deliver for Bone Disease and Stem Cell Therapy

SCIENTIFIC SYMPOSIUM 106

8:00 AM - 9:45 AM

Room: Auditorium

The Golden Age of Gene Therapy: Past, Present and Future Perspectives

CO-CHAIRS: Jean Bennett, MD, PhD and Malcolm Brenner, PhD

SPEAKERS

8:00 AM – 8:26 AM

Jude Samulski, PhD. UNC Gene Therapy Vector Core, Chapel Hill, NC

Adeno-associated Virus Evolution, from “Almost A Virus” to “An Amazing Vector”

8:26 AM – 8:52 AM

Michel Sadelain, MD, PhD. Memorial Sloan Kettering Cancer Center, New York, NY

Cancer Immunotherapy Revolution and Where It Is Going

8:52 AM – 9:18 AM

Marina Cavazzana, MD, PhD. Assistance Publique-Hôpitaux de Paris, Paris, France

Clinical Breakthroughs in HSC-based Gene Therapy

9:18 AM – 9:45 AM

David Liu, PhD. Harvard University and HHMI, Cambridge, MA

Base Editing and Prime Editing: Genome Editing without Double-strand Breaks

PROGRAM SCHEDULE

TUESDAY, MAY 12

All times are listed in Eastern Daylight Time (EDT UTC -4).

SCIENTIFIC SYMPOSIUM 107

8:00 AM - 9:45 AM

Room: 302

Advances in Therapeutic RNAi - Organized by the Oligonucleotide and RNAi Therapeutics Committee

CO-CHAIRS: Christian Mueller, PhD and Shen Shen, PhD

SPEAKERS

8:00 AM – 8:26 AM

Anastasia Khvorova, PhD. University of Massachusetts Medical School, Worcester, MA

siRNAs That Cross the Blood Brain Barrier

8:26 AM – 8:52 AM

Christian Mueller, PhD. University of Massachusetts Medical School, Worcester, MA, Sanofi Genzyme, Framingham, MA

Suppression of Mutant SOD1 in Amyotrophic Lateral Sclerosis with an Intrathecally Administered AAVmiR

8:52 AM – 9:18 AM

Jae-Hyuck Shim, PhD. University of Massachusetts Medical School, Worcester, MA

Bone-targeting AAV-mediated Gene Silencing for Osteoporosis Therapy

9:18 AM – 9:45 AM

Omar Khwaja, MD, PhD. Voyager, Oberwil, Switzerland

Development of AAV-based RNAi Therapeutics for Huntington Disease

BREAK

9:45 AM - 10:15 AM

PROGRAM SCHEDULE

TUESDAY, MAY 12

All times are listed in Eastern Daylight Time (EDT UTC -4).

ORAL ABSTRACT SESSION 110

10:15 AM - 12:00 PM

Room: Auditorium

Gene Therapy for the Special Senses

Chair: Paul Gamlin, PhD

10:15 AM – 10:30 AM

1: Retinal Gene Therapy in X-Linked Retinitis Pigmentosa Caused by Mutations in *RPGR*: Results at 6 Months in a First in Human Clinical Trial
Jasmina Cahajic-Kapetanovic, University of Oxford, Oxford, United Kingdom

10:30 AM – 10:45 AM

2: Therapeutic Efficacy of ARCUS Meganuclease Gene Editing - Arrest of Rod Degeneration and Restoration of Rod Function in a Transgenic Pig Model of Autosomal Dominant Retinitis Pigmentosa
Maureen McCall, University of Louisville, Louisville, KY, United States

10:45 AM – 11:00 AM

3: Gene Therapy in a Novel Large Animal Model of Stargardt Disease
Ivana Trapani, Telethon Institute of Genetics and Medicine (TIGEM), Pozzuoli, Italy

11:00 AM – 11:15 AM

4: Preclinical Testing of AAV9-PHP.B for Transgene Expression in the Non-Human Primate Cochlea
Maryna Ivanchenko, Harvard Medical School, Boston, MA, United States

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PROGRAM SCHEDULE

TUESDAY, MAY 12

All times are listed in Eastern Daylight Time (EDT UTC -4).

11:15 AM – 11:30 AM

5: Dual AAV Delivery of Otoferlin Durably Rescues Hearing in Congenitally Deaf Preclinical Models

Jonathon Whitton, Decibel Therapeutics, Boston, MA, United States

11:30 AM – 11:45 AM

6: Optimizing the Safety of Dual AAV-Based Treatments for MYO7A Usher Syndrome (USH1B) in *Myo7a*^{-/-} Mice

Kaitlyn Calabro, University of Florida, Gainesville, FL, United States

11:45 AM – 12:00 PM

7: Multi-Center Blinded Preclinical Efficacy Study Shows Significant Differences in Promoter Performance for Gene Replacement Therapy of SMARD1/CMT2S

Kathrin Meyer, Nationwide Children's Hospital, Columbus, OH, United States

ORAL ABSTRACT SESSION 111

10:15 AM - 12:00 PM

Room: Ballroom A

Genome Editing: Preclinical & Rare Disease

Chair: Scot Wolfe, PhD

10:15 AM – 10:30 AM

8: Clonal Tracking Uncovers Barriers and Validates New Strategies to Enhance Gene Editing in Human Hematopoietic Stem Cells

Aurelien Jacob, San Raffaele Telethon Institute for Gene Therapy, IRCCS San Raffaele Scientific Institute, Milan, Italy

10:30 AM – 10:45 AM

9: Therapeutic Base Editing of Human Hematopoietic Stem Cells

Jing Zeng, Boston Children's Hospital, Boston, MA, United States

10:45 AM – 11:00 AM

10: Expansion with E478 Significantly Increases the Rate of CRISPR-Mediated Homology Directed Repair (HDR) and Improves Engraftment of Human Hematopoietic Stem Cells

Kevin Goncalves, Magenta Therapeutics, Cambridge, MA, United States

11:00 AM – 11:15 AM

11: Development of AAV-Based CRISPR/Cas9 Therapies for Correcting Duchenne Muscular Dystrophy by Targeted Genomic Integration

Adrian Oliver, Duke University, Durham, NC, United States

PROGRAM SCHEDULE

TUESDAY, MAY 12

All times are listed in Eastern Daylight Time (EDT UTC -4).

11:15 AM – 11:30 AM

12: Regulated Allele Specific Gene Editing for Huntington's Disease
Alex Mas Monteys, University of Pennsylvania/CHOP, Philadelphia, PA, United States

11:30 AM – 11:45 AM

13: Engraftment and Persistence of HDR-Edited Hematopoietic Stem and Progenitor Cells in Nonhuman Primates
Christopher Peterson, Fred Hutchinson Cancer Research Center, Seattle, WA, United States

11:45 AM – 12:00 PM

14: Highly Efficient Base Correction of Adult Dystrophic Mice Using iABE-NG
Renzhi Han, The Ohio State University, Columbus, OH, United States

ORAL ABSTRACT SESSION 112

10:15 AM - 11:45 AM

Room: Ballroom B

Cancer - Targeted Gene and Cell Therapy

Chair: Michael Milone, PhD

10:15 AM – 10:30 AM

15: Defining the Role of Spatial and Molecular Adaption of Glioblastoma to Personalized Neural Stem Cell Therapy Using Integrated *In Vivo* and *Ex Vivo* Models

Andrew Satterlee, University of North Carolina at Chapel Hill, Chapel Hill, NC, United States

10:30 AM – 10:45 AM

16: Transforming Challenges into Opportunities: The T Cell Exhaustion Signature Can Guide the Identification of Patient-Derived, Tumor-Reactive TCRs and Promote TCR Gene Editing for AML

Francesco Manfredi, Vita-Salute San Raffaele University, Milan, Italy

10:45 AM – 11:00 AM

17: Tumor-Selective Gene Circuits Enable Highly Specific Localized Cancer Immunotherapy

Russell Gordley, Senti Biosciences, South San Francisco, CA, United States

PROGRAM SCHEDULE

TUESDAY, MAY 12

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11:00 AM – 11:15 AM

18: MyD88/CD40 (MC) Enhances Chimeric Antigen Receptor Natural Killer (CAR-NK) Cell Proliferation, Cytokine Release and Anti-Tumor Efficacy Against BCMA⁺ Tumors

J. Henri Bayle, Bellicum Pharmaceuticals, Houston, TX, United States

11:15 AM - 11:30 AM

21: Tumor-Tropic Liposome-Mediated Therapeutic Delivery of mRNA for T Cell Malignancies

Shoji Saito, Shinshu University School of Medicine, Matsumoto, Japan

11:30 AM – 11:45 AM

20: Nanoparticles for Targeted Theranostic Gene Delivery to Hepatocellular Carcinoma

Hannah Vaughan, Johns Hopkins, BALTIMORE, MD, United States

ORAL ABSTRACT SESSION 113

10:15 AM - 12:00 PM

Room: Ballroom C

Advances in AAV Vector Engineering

Chair: Jun Xie, PhD

10:15 AM – 10:30 AM

22: Evolution and Investigation of Engineered AAV Capsids Exhibiting Enhanced Transduction of the Central Nervous System with or without Murine Strain Specificity

Sripriya Ravindra Kumar, California Institute of Technology, Pasadena, CA, United States

Congratulations to ASGCT's Newly Elected Directors and Officers

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PROGRAM SCHEDULE

TUESDAY, MAY 12

All times are listed in Eastern Daylight Time (EDT UTC -4).

10:30 AM – 10:45 AM

23: Engineering and Evaluation of Novel AAV Serotypes for Gene Delivery to the Central Nervous System

David Ojala, Sangamo Therapeutics, Richmond, CA, United States

10:45 AM – 11:00 AM

24: Cross-Species Evolution of Synthetic AAV Strains for Clinical Translation

Trevor Gonzalez, Duke University School of Medicine, Durham, NC, United States

11:00 AM – 11:15 AM

25: A Highly Efficient Dual AAV Technology for Therapeutic (epi) Genome Editing Applications

Lisa Riedmayr, Ludwig-Maximilians-University Munich, Munich, Germany

11:15 AM – 11:30 AM

26: A Novel AAV9 Variant Exhibiting Strong Preference for Neuronal Transduction in Mice and Non-Human Primates Following Systemic Administration

Samuel Huang, Oregon Health & Science University, Portland, OR, United States

11:30 AM – 11:45 AM

27: Sequencing of Barcoded AAV Libraries Enables Reprogramming of AAV Capsids with Respect to Liver Tropism

Eric Zinn, Grousbeck Gene Therapy Center, Mass Eye and Ear, Harvard Medical School & The Broad Institute of Harvard and MIT, Boston, MA, United States

11:45 AM – 12:00 PM

28: Deamidation of Adeno-Associated Virus Capsid Impacts Transduction Activity in AAV9

Rosemary Meggersee, University of Pennsylvania, Perelman School of Medicine, Philadelphia, PA, United States

PROGRAM SCHEDULE

TUESDAY, MAY 12

All times are listed in Eastern Daylight Time (EDT UTC -4).

ORAL ABSTRACT SESSION 114

10:30 AM - 12:00 PM

Room: 302

CAR T-Cell Therapies I

Chair: Jen Adair, PhD

10:30 AM – 10:45 AM

30: CT-0508 is an Anti-HER2 Chimeric Antigen Receptor (CAR) Macrophage that Promotes a Pro-Inflammatory Solid Tumor Microenvironment and Eliminates Cancer Cells via Phagocytosis

Michael Klichinsky, Carisma Therapeutics, Philadelphia, PA, United States

10:45 AM – 11:00 AM

31: Mechanisms of Adoptive T Cell Micropharmacies

Christopher Bourne, Weill Cornell Medicine, New York, NY, United States

11:00 AM – 11:15 AM

32: CAR T-Cells Targeting the EDB Splice Variant of Fibronectin Have Potent Anti-Tumor and Anti-Vasculature Activity in Preclinical Solid Tumor Models

Jessica Wagner, St. Jude Children's Research Hospital, Memphis, TN, United States

11:15 AM – 11:30 AM

33: Pooled Knock-In Targeting for Genome Engineering of Cellular Immunotherapies

Theodore Roth, UCSF, San Francisco, CA, United States

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PROGRAM SCHEDULE

TUESDAY, MAY 12

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11:30 AM – 11:45 AM

34: G-CSF Mobilized Apheresis as an Alternative Source of CART Cells
Katherine Cummins, The University of Pennsylvania, Perelman School of Medicine, Philadelphia, PA, United States

11:45 AM – 12:00 PM

35: Enhanced tgTCR T Cell Product Attributes Through Process Improvement of CRISPR/Cas9 Engineering
Aaron Prodeus, Intellia Therapeutics, Cambridge, MA, United States

ORAL ABSTRACT SESSION 115

10:15 AM - 12:00 PM

Room: 304 & 306

Oligonucleotide Therapies for Genetic Diseases

Chair: Anastasia Khvorova, PhD

10:15 AM – 10:30 AM

36: A Novel Oligonucleotide-Based RNA Base Editing Therapeutic Approach for the Treatment of Hurler Syndrome
Pengfei Yuan, EdiGene, Inc., Beijing, China

10:30 AM – 10:45 AM

37: Targeting DNA Damage Response Genes with Oligonucleotides for Therapeutic Modulation of Somatic Instability at Disease Gene Repeats
Brian Bettencourt, Triplet Therapeutics, Cambridge, MA, United States

10:45 AM – 11:00 AM

38: SINEUPs: A New Antisense, Long Non-Coding RNA-Based Platform to Increase Endogenous Protein Levels for Therapy
Stefano Espinoza, Istituto Italiano di Tecnologia, Genova, Italy

11:00 AM – 11:15 AM

39: Restoration of Full-Length Dystrophin Expression in the Dup2 Mouse Induced by Systemic Delivery of a Peptide-Conjugated Morpholino Oligomer
Liubov Gushchina, Abigail Wexner Research Institute at Nationwide Children's Hospital, Columbus, OH, United States

11:15 AM – 11:30 AM

40: A Novel Lentiviral Vector for Gene Therapy of β -Hemoglobinopathies: Co-Expression of a Potent Anti-Sickling Transgene and a microRNA Downregulating *BCL11A*
Mégane Brusson, Imagine Institute, INSERM UMR1163, Paris, France

PROGRAM SCHEDULE

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All times are listed in Eastern Daylight Time (EDT UTC -4).

11:30 AM – 11:45 AM

41: Engineering Backsplicing Introns for Robust Expression of Synthetic Circular RNAs from AAV Vectors

Rita M. Reganck, Duke University, Carrboro, NC, United States

11:45 AM – 12:00 PM

42: Delivery of mRNA and CRISPR in CNS with a Novel Polymer Nanoparticle

Kunwoo Lee, GenEdit, South San Francisco, CA, United States

ORAL ABSTRACT SESSION 116

10:15 AM - 12:00 PM

Room: 309

Immune Responses Against AAV Vectors

Chair: Roberto Calcedo, PhD

10:15 AM – 10:30 AM

43: IL-1R/MyD88-Dependent CD8+ T Cell Responses to Hepatic AAV Gene Transfer

Sandeep Kumar, Indiana University, Indianapolis, IN, United States

10:30 AM – 10:45 AM

44: A Vector Independent Method of Neutralizing Antibody Evasion Potently Protects AAV for Efficient Gene Delivery

Charles Askew, University of North Carolina, Chapel Hill, Chapel Hill, NC, United States

10:45 AM – 11:00 AM

45: Effect of CpG Depletion on Anti-Capsid CD8+T Cell Priming to AAV Gene Therapy

Thais Bertolini, Indiana University School of Medicine, Indianapolis, IN, United States

11:00 AM – 11:15 AM

46: Transgene-Specific T Cell Immunomodulation is Achieved in Mice by a Subretinal Co-Injection of the AAV Gene Transfer Vector with Peptides from the Transgene

Sylvain Fisson, Genethon, Evry, France

11:15 AM – 11:30 AM

47: Systemic Immunity Baseline Alterations in X Linked Retinoschisis: Do They Confound to AAV8-RS1 Gene Therapy Outcomes?

Alaknanda Mishra, UC Davis, Davis, CA, United States

PROGRAM SCHEDULE

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All times are listed in Eastern Daylight Time (EDT UTC -4).

11:30 AM – 11:45 AM

48: Elastin-Like Polypeptide Mediated AAV Delivery Improved Transduction Efficiency and Reduced Immune Response

Kai Wang, The University of North Carolina at Chapel Hill, Chapel Hill, NC, United States

11:45 AM – 12:00 PM

49: A Systematic Analysis of the Immunologic Effects of Intrathecal AAV9 Mediated Gene Transfer Targeting the Nervous System in Giant Axonal Neuropathy

Diana Bharucha-Goebel, NIH & Children's National Hospital, Bethesda, MD, United States

ORAL ABSTRACT SESSION 117

10:15 AM - 12:00 PM

Room: 310

Assay Development

Chair: Linda Couto, PhD

10:15 AM – 10:30 AM

50: Development of a Flexible In Situ Sequencing Method with Single-Cell Resolution

Jana Rajova, Lund University, Lund, Sweden

10:30 AM – 10:45 AM

51: Using Nanopore Sequencing for Quality Control Analysis of Viral Vectors for use as Gene Therapies

Carolyn Yrigollen, Children's Hospital of Philadelphia, Philadelphia, PA, United States

10:45 AM – 11:00 AM

52: Establishment of a Validated Assay for Clinical Vector Integration Site Analysis: S-EPTS/LM-PCR

Irene Gil-Farina, GeneWerk GmbH, Heidelberg, Germany

11:00 AM – 11:15 AM

53: Development of a Peptide Immunoaffinity LC-MS/MS Assay for Frataxin Transgene Protein Measurements after AAV9-FXN Treatment of the MCK Mouse Model of Friedreich Ataxia

Vahid Farrokhi, Pfizer Inc, Cambridge, MA, United States

PROGRAM SCHEDULE

TUESDAY, MAY 12

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11:15 AM – 11:30 AM

54: Development and Optimization of a New, Harmonized Droplet Digital PCR (ddPCR) Method for Pharmacokinetic (PK) Monitoring of Axicabtagene Ciloleucef (Axi-Cel) and Association with Outcomes in ZUMA-1

Vicki Plaks, Kite, a Gilead Company, Santa Monica, CA, United States

11:30 AM – 11:45 AM

55: Implementation of an Automated VCN Platform to Assess Integration of Lentiviral Vectors in a High-Throughput Capacity
Maple Gioia, bluebird bio, Cambridge, MA, United States

11:45 AM – 12:00 PM

56: Development of a Single-Cell Western Assay for Anti-Sickling Beta Globin Drug Products for Sickle Cell Disease

Paige Coles, bluebird bio, Cambridge, MA, United States

ORAL ABSTRACT SESSION 118

10:15 AM - 12:00 PM

Room: 311

Vector and Cell Engineering, Production or Manufacturing I

Chair: Robert Kotin, PhD

10:15 AM – 10:30 AM

57: Akt Inhibition Enhances Memory, Proliferation and Cytotoxicity in CD19 CART Cells

Vedika Mehra, UCL, London, United Kingdom

10:30 AM – 10:45 AM

58: A New Primary Human Muscle Stem Cell Product, PHSats

Janine Kieshauer, Experimental and Clinical Research Center, Berlin, Germany

10:45 AM – 11:00 AM

59: Novel Strategies to Enhance CAR-T Cell Production and Improve Quality During Manufacturing

Gauri Lamture, FDA, White Oak, MD, United States

11:00 AM – 11:15 AM

60: A Universal High Density Cell Respirator (HDCR) Bioreactor for Intensified Production of Gene Therapy Vectors

Colin Cook, Beckman Research Institute of the City of Hope, Duarte, CA, United States

PROGRAM SCHEDULE

TUESDAY, MAY 12

All times are listed in Eastern Daylight Time (EDT UTC -4).

11:15 AM – 11:30 AM

61: Acoustic Affinity Cell Selection: A Non-Paramagnetic Scalable Technology for T Cell Selection From Unprocessed Apheresis Products

Jack Saloio, MilliporeSigma, Wilbraham, MA, United States

11:30 AM – 11:45 AM

62: The Lisocabtagene Maraleucel (liso-cel) Manufacturing Process is Designed for Consistency, Purity, and Robustness Across B-Cell Malignancies

Jeffrey Teoh, Juno Therapeutics, a Bristol-Myers Squibb Company, Seattle, WA, United States

11:45 AM – 12:00 PM

63: Using Numerical Modelling (CFD) to Design and Improve a 3D Printed Atomizer for the Transfection of Cells with Virus

Justin O'Sullivan, APC Ltd, Dublin, Ireland

ORAL ABSTRACT SESSION 119

10:15 AM - 12:00 PM

Room: 312

CAR T-Cells Targeting Solid Tumors

Chair: Paola Grandi, PhD

10:15 AM – 10:30 AM

64: Combining De-Glycosylating Agents with CAR T Cells for Targeting Solid Tumors and Improving Therapeutic Index

Beatrice Greco, San Raffaele Hospital Scientific Institute, Milan, Italy

10:30 AM – 10:45 AM

65: Overcoming the Breast Tumor Microenvironment by Targeting MDSCs through CAR-T Cell Therapy

Saisha Nalawade PhD., Baylor College of Medicine, Houston, TX, United States

10:45 AM – 11:00 AM

66: Induction of Potent Systemic and Tissue Resident Immune Responses by ExoVACCTM: A Novel Exosome-Based Vaccine Platform

Nikki Ross, Codiak BioSciences, Cambridge, MA, United States

PROGRAM SCHEDULE

TUESDAY, MAY 12

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11:00 AM – 11:15 AM

67: Glypican-3-Specific CAR NKT Cells Armored with IL-15 Mediate Potent Anti-Tumor Response Against Hepatocellular Carcinoma
Rahamthulla Shaik, Baylor College of Medicine, Houston, TX, United States

11:15 AM – 11:30 AM

68: Preclinical Study of Oral Cancer Vaccine Using Recombinant *Bifidobacterium* Expressing WT1 Protein in Murine Bladder Cancer Model and Non-Human Primate
Koichi Kitagawa, Kobe University Graduate School of Science, Technology and Innovation, Kobe, Japan

11:30 AM – 11:45 AM

69: T Cells Redirected to the Pan-Cancer Target GRP78 Have Potent Antitumor Activity in Preclinical Models
Nikhil Hebbar, St. Jude Children's Research Hospital, Memphis, TN, United States

11:45 AM – 12:00 PM

70: Faster, Higher, Stronger: Optimizing Fuel Selection to Improve CAR-T Cell Metabolic Fitness in Solid Tumors
Roderick O'Connor, University of Pennsylvania, Philadelphia, PA, United States

BREAK

12:00 PM - 1:30 PM

Lunch Break

TOOLS AND TECHNOLOGIES FORUM

12:00 PM - 1:30 PM

Room: 210

Tools and Technologies Forum I

SPEAKERS:

12:00 PM - 12:15 PM

Guangnan Meng, PerkinElmer, Waltham, MA
AAV Purity Analysis Modernized for a New Decade

12:15 PM - 12:30 PM

Hongshan Li, PhD, ForteBio, Freemont, CA
AAV Quantification by Octet Using BLI Technology

PROGRAM SCHEDULE

TUESDAY, MAY 12

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12:30 PM - 12:45 PM

Rene Gantier. Repligen, Waltham, MA

Technology-Ready Processes for Gene Therapy Manufacturing 2.0

12:45 PM - 1:00 PM

Philippe Garabedian. MYRIADE, Paris, France

Characterize and Count Nanoparticles in a Single Drop and in Real Time

1:00 PM - 1:15 PM

Henry George. MilliporeSigma, Saint Louis, MO

VirusExpress™ Lentiviral Production Platform

1:15 PM - 1:30 PM

Peter Guterstam, PhD, Cytiva, Uppsala, Sweden

Fibro Chromatography to Revamp Productivity in Downstream AAV Processing

EDUCATION SESSION 120

1:30 PM - 3:15 PM

Room: 304 & 306

Synthetic Biology Meets Immunology: DNA and RNA Tools

CHAIR: David Weiner, PhD

SPEAKERS

1:30 PM – 2:05 PM

David Weiner, PhD. The Wistar Institute, Philadelphia, PA

**DNA Vaccines and Synthetic DNA Encoded Bifunctional Antibodies
BITES**

2:05 PM – 2:40 PM

Norbert Pardi, PhD. University of Pennsylvania, Philadelphia, PA

mRNA Vaccines - A Novel Vaccine Generation

2:40 PM – 3:15 PM

Eric Kelsic, PhD. Dyno Therapeutics, Cambridge, MA

AAV Gene Therapy and Capsid Engineering

PROGRAM SCHEDULE

TUESDAY, MAY 12

All times are listed in Eastern Daylight Time (EDT UTC -4).

EDUCATION SESSION 121

1:30 PM - 3:15 PM

Room: 312

“Off the Shelf” Cell Therapies

CO-CHAIRS: Catherine Bollard, MD and Rayne Rouce, MD

SPEAKERS

1:30 PM – 2:05 PM

Susan Prockop, MD. Memorial Sloan Kettering Cancer Center and Weill Cornell Medicine, New York, NY

Viral Specific T Cells - From Donor Derived to Off-the-Shelf Products

2:05 PM – 2:40 PM

Rayne Rouce, MD. Baylor College of Medicine, Houston, TX

NK Cells or iNKT Cells as a Platform for Off the Shelf Therapies

2:40 PM – 3:15 PM

Blake Aftab, PhD. Atara Biotherapeutics, Inc., Thousand Oaks, CA

Review of Clinical Stage Allogeneic T-Cell Therapies

EDUCATION SESSION 122

1:30 PM - 3:15 PM

Room: Auditorium

Adeno-associated Virus (AAV) Vectors - From Basic Biology to Vector Application

CO-CHAIRS: Hildegard Büning, PhD and Graeme Fielder, PhD

SPEAKERS

1:30 PM – 2:05 PM

Nicole K. Paulk, PhD. University of California San Francisco, San Francisco, CA

AAV Basics

2:05 PM – 2:40 PM

Eduard Ayuso, DVM, PhD. INSERM, University of Nantes, Nantes, France

Manufacturing AAV

2:40 PM – 3:15 PM

James Wilson, MD, PhD. University of Pennsylvania, Philadelphia, PA

Safety and AAV

PROGRAM SCHEDULE

TUESDAY, MAY 12

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EDUCATION SESSION 123

1:30 PM - 2:40 PM

Room: 311

Bioinformatics: Artificial Intelligence (AI) takes Gene Therapy to the Next Level

CO-CHAIRS: Paloma Giangrande, PhD and Thomas J. Cradick, PhD.

SPEAKERS

1:30 PM – 2:05 PM

Ryan Boudreau, PhD. University of Iowa, Iowa City, IA

Bioinformatics & Gene Therapy

2:05 PM – 2:40 PM

Andrew Giessel, PhD. Moderna Therapeutics, Cambridge, MA

Data Science and AI in mRNA Drug Development

EDUCATION SESSION 124

1:30 PM - 2:40 PM

Room: 302

Building Robust Animal Models for Preclinical Testing Gene and Immune Therapies for Humans

CO-CHAIRS: Avery Posey, PhD and Renata Stripecke, PhD

SPEAKERS

1:30 PM – 2:05 PM

Michael Brehm, PhD. University of Massachusetts Medical School, Worcester, MA

Human Immune Reconstitution in Mice for Modeling Diabetes

2:05 PM – 2:40 PM

Scott G. Kitchen, PhD. UCLA AIDS Institute, David Geffen School of Medicine at UCLA, Los Angeles, CA

Engineering CAR T-Cells and Cell-Based Vaccines against HIV in Mice and NHPs

PROGRAM SCHEDULE

TUESDAY, MAY 12

All times are listed in Eastern Daylight Time (EDT UTC -4).

EDUCATION SESSION 125

1:30 PM - 3:15 PM

Room: Ballroom B

Engineered T-Cell Therapies for Malignancies

CHAIR: Craig Sauter, MD

SPEAKERS

1:30 PM – 2:05 PM

Saar Gill, MD, PhD. University of Pennsylvania, Philadelphia, PA
New CAR Targets - AML Targets and Solid Tumor Targets

2:05 PM – 2:40 PM

Nabil Ahmed, MD. Baylor College of Medicine, Houston, TX
CAR T-cells to Overcome CD19 Escape in Acute B-lineage Leukemia

2:40 PM – 3:15 PM

Sandra P. D'Angelo, MD. Memorial Sloan Kettering Cancer Center, New York, NY
TCRs for Solid Organ Malignancies

EDUCATION SESSION 126

1:30 PM - 3:15 PM

Room: Ballroom A

Gene Editing with CRISPR/Cas9, Discovery to Application

CO-CHAIR: John Tisdale, MD

SPEAKERS

1:30 PM – 2:05 PM

Raed Ibraheim, University of Massachusetts Medical School, Worcester, MA
All-in-one Nme2Cas9 AAV Platforms for Segmental Deletions and Precise HDR-Based Editing

2:05 PM – 2:40 PM

Nadav Ahituv, PhD. University of California San Francisco, San Francisco, CA
CRISPR-mediated Activation of a Promoter or Enhancer to Rescue Obesity Caused by Haploinsufficiency

PROGRAM SCHEDULE

TUESDAY, MAY 12

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2:40 PM – 3:15 PM

Scot Wolfe, PhD. University of Massachusetts Medical School,
Worcester, MA

Structure-Guided Chemical Modification of Guide RNAs to Facilitate In Vivo Genome Editing by Cas9

EDUCATION SESSION 127

1:30 PM - 3:15 PM

Room: Ballroom C

Updates on Immunogenicity Against AAV and Cas9

SPEAKERS

1:30 PM – 2:05 PM

Federico Mingozzi, PhD. Spark Therapeutics, Philadelphia, PA

Adaptive Immunity to AAV

2:05 PM – 2:40 PM

Anna Kajaste-Rudnitski, PhD. SR-TIGET, Milano, Italy

Vector-host Interactions and Innate Immunity in Hematopoietic Stem Cell Gene Engineering

2:40 PM – 3:15 PM

Matthew Porteus, MD, PhD. Stanford University School of Medicine,
Stanford, CA

Immunity to CrisprCAS

BREAK

3:15 PM - 3:45 PM

TOOLS AND TECHNOLOGIES FORUM

3:30 PM - 4:45 PM

Room: 210

Tools and Technologies Forum II

SPEAKERS:

3:30 PM - 3:45 PM

Tim Schroeder. Repligen, Waltham, MA

Jana Langhoff. Tecan, Mannedorf, Switzerland

Automated High Throughput Viral Vector Purification to Streamline Downstream Process Development in Gene Therapy

PROGRAM SCHEDULE

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3:45 PM - 4:00 PM

Helen Gu. GenScript ProBio, Piscataway, NJ

Accelerate Gene and Cell Therapy Product Development

4:00 PM - 4:15 PM

Dr. Marco Schmeer. PlasmidFactory GmbH & Co. KG, Bielefeld, Germany

Minicircle DNA – New Tool for Cell Therapy

4:15 PM - 4:30 PM

Dana Holzinger, PhD. PROGEN Biotechnik GmbH, Heidelberg, Germany

AAV Antibodies: An Overview of Qualified Applications

4:30 PM - 4:45 PM

Dr. Wei Wang. GeneWerk GmbH, Heidelberg, Germany

Vector Safety in Gene and Immune Gene Therapies

ORAL ABSTRACT SESSION 130

3:45 PM - 5:30 PM

Room: Auditorium

New Gene Editing Technologies and Approaches

Chair: Shondra Pruett-Miller, PhD

3:45 PM – 4:00 PM

71: Programmable C-to-G Transversions in Human Cells with Engineered CRISPR Base Editors

Ibrahim Kurt, Massachusetts General Hospital, Charlestown, MA, United States

4:00 PM – 4:15 PM

72: Unconstrained Genome Targeting with Engineered Near-PAMless CRISPR-Cas Variants

Kathleen Christie, Massachusetts General Hospital, Boston, MA, United States

4:15 PM – 4:30 PM

73: Efficient Multiplex Base Editing at Single Target Sites with a Dual-Deaminase Base Editor

Julian Grünewald, MGH, Charlestown, MA, United States

PROGRAM SCHEDULE

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All times are listed in Eastern Daylight Time (EDT UTC -4).

4:30 PM – 4:45 PM

74: Rational Selection of CRISPR/Cas9 gRNAs to Maximize Homology-Directed Genome Editing

Kristina Tatioosian, University of Southern California, Los Angeles, CA, United States

4:45 PM – 5:00 PM

75: dCas13-Mediated Therapeutic RNA Base Editing for *In Vivo* Gene Therapy

Jiaming Wang, University of Massachusetts Medical School, Worcester, MA, United States

5:00 PM – 5:15 PM

76: CRISPR/Cas9-Mediated Gene Editing Corrects Disease-Causing Mutation in Crigler-Najjar Syndrome Mice

Giulia Bortolussi, ICGEB, Trieste, Italy

5:15 PM – 5:30 PM

77: Development of a CRISPR/Cas9 System for Mitochondrial Diseases

Michelle Ho, City of Hope, Duarte, CA, United States

ORAL ABSTRACT SESSION 131

3:45 PM - 5:30 PM

Room: Ballroom A

CART and Other Engineered T Cells Targeting Hematological Malignancies

Chair: Conrad Russell Cruz, MD, PhD

3:45 PM – 4:00 PM

78: Exploiting CRISPR-Genome Editing and WT1-Specific T Cell Receptors to Redirect T Lymphocytes Against Acute Myeloid Leukemia

Eliana Ruggiero, San Raffaele Scientific Institute, Milan, Italy

4:00 PM – 4:15 PM

79: Co-Targeting CD38 with a Chimeric Costimulatory Receptor Enhances Adoptive T Cell Therapy for Hematological Malignancies

Afroditi Katsarou, Amsterdam UMC, Location VUmc, Amsterdam, Netherlands

4:15 PM – 4:30 PM

80: Generation of Human Memory Stem T Cells Specific for Tumor Antigens and Resistant to Inhibitory Signals by Genome Editing

Beatrice Cianciotti, San Raffaele Scientific Institute, Milan, Italy

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4:30 PM – 4:45 PM

81: Pre-selected CART_{N/SCM} Outperform CART_{BULK} in Driving Tumor Eradication in the Absence of Cytokine Release Syndrome and Neurotoxicity

Claudia Mezzanotte, IRCSS San Raffaele Scientific Institute, Milan, Italy

4:45 PM – 5:00 PM

82: Therapeutic Efficacy of CAR-T Cells Targeting gp350 in a Humanized Mouse Model of Epstein Barr Virus-Induced Lymphoproliferation and Prospects for Clinical Trials

Renata Stripecke, Hannover Medical School, Hannover, Germany

5:00 PM – 5:15 PM

83: DNMT3A-Dependent Epigenetic Programs Constrain CART Cell Survival and Effector Function

Brooke Prinzing, St Jude Children's Research Hospital, Memphis, TN, United States

5:15 PM – 5:30 PM

84: Insufficient Activation Limits the Efficacy of CART Cell Therapy in Chronic Lymphocytic Leukemia

McKensie Collins, University of Pennsylvania, Philadelphia, PA, United States

ORAL ABSTRACT SESSION 132

3:45 PM - 5:30 PM

Room: Ballroom B

AAV Vector Biology: New Insights

Chair: Jay Chiorini, PhD

3:45 PM – 4:00PM

85: The Membrane-Associated Accessory Protein (MAAP) is Essential for Rapid Extracellular Secretion of Adeno-Associated Viruses

Zachary C. Elmore, Duke University School of Medicine, Durham, NC, United States

4:00 PM – 4:15 PM

86: Dependency of AAV Entry on GPR108 Maps to Motif on VP1u That is Transferable Across AAV Serotypes

Nerea Zabaleta, Grousbeck Gene Therapy Center, Mass Eye and Ear, Harvard Medical School & The Broad Institute of Harvard and MIT, Boston, MA, United States

PROGRAM SCHEDULE

TUESDAY, MAY 12

All times are listed in Eastern Daylight Time (EDT UTC -4).

4:15 PM – 4:30 PM

87: USP36, A Nucleolar Enriched Deubiquitinating Enzyme, Promotes AAV Vector Production by Optimally Stabilizing AAP Levels

Swapna Kollu, Oregon Health & Science University, Portland, OR, United States

4:30 PM – 4:45 PM

88: Improved Genome Packaging Efficiency of AAV Vectors Using Rep Hybrids

Mario Mietzsch, University of Florida, Gainesville, FL, United States

4:45 PM – 5:00 PM

89: The Primate Selective Transduction of rAAV-LK03 Vectors is Unrelated to Variation in Double-Stranded Viral Genome Formation in the Nucleus Between Species

Adriana Gonzalez Sandoval, Stanford University, Stanford, CA, United States

5:00 PM – 5:15 PM

90: AAPs Harbor Degrons, a Protein Self-Degradation Motif, Important for Regulating Stability of AAP in the Process of AAV Capsid Assembly

Anusha Sairavi, Oregon Health & Science University, Portland, OR, United States

5:15 PM – 5:30 PM

91: Cardiac Toxicity of AAV9 Vector Upon Heart-Specific Expression of an Immunogenic Non-Self Transgene

Andrew Park, AstraZeneca, Gaithersburg, MD, United States

ORAL ABSTRACT SESSION 133

3:45 PM - 5:15 PM

Room: Ballroom C

AAV Gene Delivery for CNS Disorders

Chair: Rita Batista, PhD

3:45 PM – 4:00 PM

92: A New Approach for Designing a Feedback-Enabled AAV Genome Improves Therapeutic Outcomes of MiniMeCP2 Gene Transfer in Mice Modeling Rett Syndrome (RTT)

Sarah Sinnett, UTSW, Dallas, TX, United States

PROGRAM SCHEDULE

TUESDAY, MAY 12

All times are listed in Eastern Daylight Time (EDT UTC -4).

4:00 PM – 4:15 PM

98: AAV9/hCDKL5 Delivery to Cerebrospinal Fluid of Juvenile CDKL5-Deficient Mice Improves Learning and Memory and Motor Function in Adult Mice

Maggie Wright, Ultragenyx Pharmaceutical, Novato, CA, United States

4:15 PM -4:30 PM

94: Gene Correction in Peripheral Organs May Contribute to the Treatment of Central Nervous System Diseases

Anoushka Lotun, University of Massachusetts Medical School, Worcester, MA, United States

4:30 PM – 4:45 PM

95: Evaluating the Efficacy and Safety of Cerebrospinal Fluid-Delivered Gene Therapy for Krabbe Disease in Murine and Canine Models

Juliette Hordeaux, University of Pennsylvania, Perelman School of Medicine, Philadelphia, PA, United States

4:45 PM – 5:00 PM

96: Developing a CDKL5 Gene-Therapy Vector for a Mouse Model of CDKL5-Deficiency Disorder

Ralf Schmid, University of Pennsylvania, Philadelphia, PA, United States

5:00 PM – 5:15 PM

97: Intrathecal Delivery of Human Bicistronic Hexosaminidase Vector (TGTX-101) to Correct Sandhoff Disease in a Murine Model: A Dosage Study

Alex Ryckman, Queen's University, Kingston, ON, Canada

ORAL ABSTRACT SESSION 134

3:45 PM - 5:00 PM

Room: 302

Oligonucleotide Therapies for Acquired Diseases

Chair: Annemieke Aartsma-Rus, PhD

3:45 PM – 4:00 PM

99: rAAV-Mediated Hepatocyte-Specific Expression of miR-375 Protects Against the Acetaminophen-Induced Acute Liver Failure in Mice

Yi Wang, Sichuan University, Chengdu, China

PROGRAM SCHEDULE

TUESDAY, MAY 12

All times are listed in Eastern Daylight Time (EDT UTC -4).

4:00 PM – 4:15 PM

100: Therapeutic saRNA targeting CEBPA in Myeloid Cells. A Potential Immunomodulatory switch for Anticancer Therapy

Vikash Reebye, Imperial College London, London, United Kingdom

4:15 PM – 4:30 PM

101: Targeted Systemic Delivery of Small Nucleic Acids to Metastatic and Brain-Localized Triple-Negative Breast Cancer by HER3-Homing Nano-Capsids

Felix Alonso-Valenteen, Cedars-Sinai Medical Center, Los Angeles, CA, United States

4:30 PM – 4:45 PM

104: A Novel Approach to Potentially Treat Influenza: Selective Induction of Apoptosis in Infected Cells by Hijacking the Virus Machinery

Serhat Gumrukcu, Seraph Research Institute, Los Angeles, CA, United States

4:45 PM – 5:00 PM

105: Systemic Senolysis in Naturally Aged Mice Using a Fusogenic FAST-LNP Gene Therapy Approach

John Lewis, University of Alberta, Edmonton, AB, Canada

ORAL ABSTRACT SESSION 135

3:45 PM - 5:30 PM

Room: 304 & 306

HSPC Gene Therapies for Blood and Immune Disorders

Chair: Cyndi Dunbar, MD

3:45 PM – 4:00 PM

106: Durable Therapeutic Restoration of Immunity in the Scid-X1 Canine Model via In-Vivo Delivery of Cocal Pseudotyped Lentivirus Vector Carrying Il2R- Γ c

Yogendra Rajawat, Fred Hutchinson Cancer Research Center, Seattle, WA, United States

PROGRAM SCHEDULE

TUESDAY, MAY 12

All times are listed in Eastern Daylight Time (EDT UTC -4).

4:00 PM – 4:15 PM

107: Efficient, Specific and Universal Therapeutic Gene Editing of ELANE For Severe Congenital Neutropenia in Human Hematopoietic Stem Cells

Shuquan Rao, Division of Hematology/Oncology, Boston Children's Hospital, Dana-Farber Cancer Institute, Harvard Stem Cell Institute, Broad Institute, Harvard Medical School, Boston, MA, United States

4:15 PM – 4:30 PM

108: A Genomic Editing-Based Therapeutic Approach for RAG2 Deficiency

Mara Pavel-Dinu, Stanford University, Stanford, CA, United States

4:30 PM – 4:45 PM

109: A Phase 1/2 Study of Lentiviral-Mediated Ex-Vivo Gene Therapy for Pediatric Patients with Severe Leukocyte Adhesion Deficiency-I (LAD-I): Initial Results from the First Treated Patient

Donald Kohn, University of California, Los Angeles, Los Angeles, CA, United States

4:45 PM – 5:00 PM

110: Updated Results of a European Gene Therapy Trial in Fanconi Anemia Patients, Subtype A

Juan Bueren, CIEMAT/CIBERER/Fund. Jiménez Díaz, Madrid, Spain

5:00 PM – 5:15 PM

111: Long-Term Follow-Up Study after Lentiviral Hematopoietic Stem/Progenitor Cell Gene Therapy for Wiskott-Aldrich Syndrome

Alessandra Magnani, Necker-Enfants Malades Hospital, Paris, France

5:15 PM – 5:30 PM

112: Results from a Phase I/II Clinical Trial for X Linked Chronic Granulomatous Disease (CGD): Possible Impact of Inflammation on Gene Therapy Efficacy

Emmanuelle Six, Imagine Institute, Paris, France

PROGRAM SCHEDULE

TUESDAY, MAY 12

All times are listed in Eastern Daylight Time (EDT UTC -4).

ORAL ABSTRACT SESSION 136

3:45 PM - 5:30 PM

Room: 309

Antiviral Immunotherapy

Chair: Masataka Suzuki, PhD

3:45 PM – 4:00 PM

113: In Situ Analysis of Follicular Targeting Antiviral CD4-MBL CAR/CXCR5 T Cells in SIV Infected Rhesus Macaques

Hadia Abdelaal, University of Minnesota, Saint Paul, MN, United States

4:00 PM – 4:15 PM

114: Targeting HIV Using Anti-HIV duoCAR-T Cell Therapy

Kim Anthony-Gonda, Lentigen, a Miltenyi Biotec Company, Gaithersburg, MD, United States

4:15 PM – 4:30 PM

115: Genome Editing the Immunoglobulin Locus with Single-Domain Antibodies to Create HIV-Specific B Cells

Geoffrey Rogers, University of Southern California, Los Angeles, CA, United States

4:30 PM – 4:45 PM

116: Lentiviral-Mediated Expression of Monoclonal Antibodies in the Lung to Protect Against Influenza

Yue Du, University of Oxford, Oxford, United Kingdom

4:45 PM – 5:00 PM

117: Multi-Virus Specific T Cells Dominate and Persist in the Patient Immune Repertoire Post Allogeneic Hematopoietic Stem Cell Transplant

Sarah Davies, NHLBI, Bethesda, MD, United States

5:00 PM – 5:15 PM

118: Protection Against Crimean-Congo Hemorrhagic Fever Virus in a Cynomolgus Macaque Disease Model by Genetic Vaccination

Matti Sallberg, Karolinska Institutet, Stockholm, Sweden

5:15 PM – 5:30 PM

119: Superior Hematopoietic Stem Cell based CAR-T Cell Therapy for HIV Infection

Anjie Zhen, UCLA, Los Angeles, CA, United States

PROGRAM SCHEDULE

TUESDAY, MAY 12

All times are listed in Eastern Daylight Time (EDT UTC -4).

ORAL ABSTRACT SESSION 137

3:45 PM - 5:30 PM

Room: 310

Hematopoietic Cell Therapies

Chair: Eirini Papapetrou, MD, PhD

3:45 PM – 4:00 PM

120: MGTA-456, A Cell Therapy Utilizing an Aryl Hydrocarbon Receptor Antagonist (AHRa) Culture, Promotes Expansion of CD34⁺CD90⁺Cord Blood (CB) Hematopoietic Stem Cells (HSC), Resulting in Rapid Hematopoietic Recovery, Uniform Engraftment and Better HLA Matched Grafts for Larger Recipients

John Wagner, University of Minnesota, Minneapolis, MN, United States

4:00 PM – 4:15 PM

121: Exchange of Alveolar Macrophages Restores Pulmonary Immunity by Niche Specific Adaption of *Ex Vivo* Generated Macrophages

Kathrin Haake, Medical School Hannover, Hannover, Germany

4:15 PM – 4:30 PM

122: Engineered B Cells Undergo Antigen Induced Activation to Allow Memory Retention, Class Switch Recombination and Clonal Selection in Mice

Alessio Nahmad, Tel Aviv University, Tel Aviv, Israel

4:30 PM – 4:45 PM

123: MGTA-145, in Combination with Plerixafor, Rapidly Mobilizes Large Numbers of HSCs in Humans That Can Be Gene Edited with CRISPR/Cas9 and Mediate Superior Engraftment to Standard-of-Care

Kevin Goncalves, Magenta Therapeutics, Cambridge, MA, United States

4:45 PM – 5:00 PM

124: "Cerberus" T Cells: A Single Glucocorticoid-Resistant T Cell Product to Simultaneously Target Multiple Pathogens in Immunocompromised Patients

Anastasia Papadopoulou, George Papanicolaou Hospital, Thessaloniki, Greece

5:00 PM – 5:15 PM

125: Restored Macrophage Function Ameliorates Disease Pathophysiology in a Mouse Model for Very Early Onset Inflammatory Bowel Disease (VEO-IBD)

Mania Ackermann, Hannover Medical School, Hannover, Germany

PROGRAM SCHEDULE

TUESDAY, MAY 12

All times are listed in Eastern Daylight Time (EDT UTC -4).

5:15 PM – 5:30 PM

126: Natural Killer Cells Generated from Human Induced Pluripotent Stem Cells Under Defined Conditions, Engineered for Immunotherapy of Solid Tumors

Kyle Lupo, Purdue University, West Lafayette, IN, United States

ORAL ABSTRACT SESSION 138

3:45 PM - 5:00 PM

Room: 311

Vector and Cell Engineering, Production or Manufacturing II

Chair: Matthias Hebben, PhD

3:45 PM – 4:00 PM

127: Enhanced Lentiviral Vector Transduction of CD34+ Hematopoietic Stem and Progenitor Cells by Targeting Anti-Viral Restriction Factors

Diego Leon-Rico, UCL GOS Institute of Child Health, London, United Kingdom

4:00 PM – 4:15 PM

128: Establishing cGMP Manufacturing of CRISPR/Cas9-Edited Human CART Cells

Xiuyan Wang, Memorial Sloan Kettering Cancer Center, New York, NY, United States

4:15 PM – 4:30 PM

132: Clinical Production of Ex-Vivo Gene Corrected Hematopoietic Stem and Progenitor Cells Using a cGMP-Compliant Semi-Closed Manufacturing Process

Premanjali Lahiri, Stanford University, Palo Alto, CA, United States

4:30 PM – 4:45 PM

130: Validation of cGMP-Generation of Genetically Engineered T Cells on the CliniMACS Prodigy™

Heather Daley, Dana-Farber Cancer Institute, Boston, MA, United States

4:45 PM – 5:00 PM

131: The Influence of Cytokines and Seeding-Density in a Scalable Feeder-Free System for Natural Killer Cell Expansion

Chris Johnson, Bio-Techne, Woburn, MA, United States

PROGRAM SCHEDULE

TUESDAY, MAY 12

All times are listed in Eastern Daylight Time (EDT UTC -4).

ORAL ABSTRACT SESSION 139

3:45 PM - 5:30 PM

Room: 312

AAV Vectors Preclinical and Proof-of-Concept Studies in Optimizing the Toolbox

Chair: Phillip Tai, PhD

3:45 PM – 4:00 PM

134: A Novel Lung Tropic AAV Vector Restores Surfactant Homeostasis and Improves Survival in a Mouse Model of Inherited Surfactant B Deficiency

Martin Kang, Ottawa Hospital Research Institute, Ottawa, ON, Canada

4:00 PM – 4:15 PM

135: Quantitative PET/CT Based Pharmacokinetic Study of AAV9 Administered to the Cerebrospinal Fluid of Non-Human Primates

Mikhail Papisov, Massachusetts General Hospital, Boston, MA, United States

4:15 PM – 4:30 PM

136: Building a Toolbox of Compact Enhancers for Cell Type-Specific Gene Expression from AAVs in the Primate Brain

John Mich, Allen Institute for Brain Science, Seattle, WA, United States

4:30 PM – 4:45 PM

137: Rapalog-Mediated Regulation of an AAV miRNA Targeting *Huntingtin* in a HD Transgenic Mouse Model

Rachael Miller, University of Massachusetts Medical School, Worcester, MA, United States

4:45 PM – 5:00 PM

138: A Discovery Platform that Screens Thousands of Gene Therapy Candidates in a Single Animal

Martin Borch Jensen, Gordian Biotechnology, San Francisco, CA, United States

5:00 PM – 5:15 PM

139: Combined Transgene and Intron-Derived miRNA Therapy for the Treatment of SCA1

Megan Keiser, The Children's Hospital of Philadelphia, Philadelphia, PA, United States

PROGRAM SCHEDULE

TUESDAY, MAY 12

All times are listed in Eastern Daylight Time (EDT UTC -4).

5:15 PM – 5:30 PM

140: A Highly-Evolved Novel AAV Gene Therapy Directly Addresses Fabry Disease Pathology In Vivo by Cell Autonomous Expression in the Heart and Other Target Organs

David Kirn, 4D Molecular Therapeutics, Emeryville, CA, United States

EXHIBIT HALL

Open 24 hours online

POSTER SESSION I

Open 24 hours on May 12

5:30 PM - 6:30 PM

Scheduled presenters requested to be available for live Q&A

PROGRAM SCHEDULE

WEDNESDAY, MAY 13

All times are listed in Eastern Daylight Time (EDT UTC -4).

EDUCATION SESSION 200

8:00 AM - 9:45 AM

Room: 302

Ins and Outs of Lentiviral Vectors: From Technology to Applications

CO-CHAIRS: Juliana Alvarez, MD and Punam Malik, MD

SPEAKERS

8:00 AM – 8:26 AM

Luigi Naldini, MD, PhD. San Raffaele Telethon Institute for Gene Therapy, Milan, Italy

Evolution and Application of Lentiviral Vectors to Correct Hematopoiesis

8:26 – 8:52 AM

Manfred Schmidt, PhD. GeneWerk GmbH, Heidelberg, Germany

Lentiviral Vectors - Safety and Integration Site Studies

8:52 AM – 9:18 AM

Bruce E. Torbett, PhD. The Scripps Research Institute, La Jolla, CA

Deciphering Cellular Pathways to Improve Lentiviral Vector Transduction

9:18 AM – 9:45 AM

Christian Buchholz, PhD. Paul-Ehrlich-Institut, Langen, Germany

Retargeting Lentiviral Vectors for In Vivo Applications

EDUCATION SESSION 201

8:00 AM - 9:20 AM

Room: 311

Career Development in Academia and Industry

CHAIR: Nuria Morral, PhD

SPEAKERS

8:00 AM – 8:20 AM

Andrew Wilber, PhD. SIU School of Medicine, Springfield, IL

Building a Gene Therapy Career in Academics

8:20 AM – 8:40 AM

Jennifer L. Gori, PhD. Obsidian Therapeutics, Inc., Cambridge, MA

Ensuring Long Term Career Success in the Gene and Cell Therapy Industry

PROGRAM SCHEDULE

WEDNESDAY, MAY 13

All times are listed in Eastern Daylight Time (EDT UTC -4).

8:40 AM – 9:00 AM

Jeffrey A. Medin, PhD. Medical College of Wisconsin, Milwaukee, WI
One PhD's Pathway

9:00 AM – 9:20 AM

Dirk Grimm, PhD. Heidelberg University Hospital, Heidelberg, Germany
Developing a Leadership Career in Gene Therapy

EDUCATION SESSION 202

8:00 AM - 9:45 AM

Room: 312

Strategies for Preparing Synthetic Vectors for Clinical Application

CHAIR: Chantal Pichon, PhD

SPEAKERS

8:00 AM – 8:35 AM

Raymond Schiffelers, PhD. University Medical Center Utrecht, Utrecht, Netherlands
Using Exosomes for Targeted RNA Delivery

8:35 AM – 9:10 AM

Assem G. Ziady, PhD. Cincinnati Children's Hospital Medical Center, Cincinnati, OH
Targeting DNA Nanoparticles for CF Gene Delivery

9:10 AM – 9:45 AM

Millicent Sullivan, PhD. University of Delaware, Newark, DE
Design of Non-viral Vectors for Clinical Applications

SCIENTIFIC SYMPOSIUM 210

8:00 AM - 9:20 AM

Room: Ballroom B

Cell-based Therapies for Eye and Nervous System Disorders: Translation from Research to Early Clinical Stages - Organized by the Neurologic & Ophthalmic Gene & Cell Therapy Committee

CO-CHAIRS: Paul Gamlin, PhD

SPEAKERS

8:00 AM – 8:26 AM

Sally Temple, PhD. Neural Stem Cell Institute, Rensselaer, NY
Overview of the Field - RPE Cell Therapy

PROGRAM SCHEDULE

WEDNESDAY, MAY 13

All times are listed in Eastern Daylight Time (EDT UTC -4).

8:26 AM – 8:52 AM

Penelope Hallett. McLean Hospital / Harvard Medical School, Belmont, MA
Neurologic Clinical: Autologous iPSC based Cell Therapy for Parkinson's Disease

8:52 AM – 9:18 AM

Kapil Bharti. National Eye Institute, National Institutes of Health, Bethesda, MD
Initiating a Phase I Clinical Trial for an Autologous IPS Cell Therapy for Macular Degeneration: From Bench-to-Bedside

SCIENTIFIC SYMPOSIUM 211

8:00 AM - 9:45 AM

Room: Ballroom A

Globalization Roadmap for Cell and Gene Therapies - Organized by the Commercialization Committee

CO-CHAIR: Debasish Roychowdhury, MD and Katy Spink, PhD

SPEAKERS

8:00 AM – 8:15 AM

Delfi Krishna, PhD. GlaxoSmithKline, Collegeville, PA
Assessing the Potential for Return on Investment of Cell and Gene Therapy Assets Across Global Markets

8:15 AM – 8:30 AM

John Doyle, PhD. Pfizer, New York, NY
Global Access/Pricing/Commercial Operations

POLICY  SUMMIT
American Society of Gene + Cell Therapy

Legislative | Regulatory | Ethical Issues

Sept 23-25, 2020

PROGRAM SCHEDULE

WEDNESDAY, MAY 13

All times are listed in Eastern Daylight Time (EDT UTC -4).

8:30 AM – 8:45 AM

Katherine Tsokas, JD. Janssen Pharmaceutical Companies of Johnson & Johnson, Spring House, PA

Global Regulatory Challenges and the Opportunities in Developing Cell and Gene Therapy Products

8:45 AM – 9:00 AM

Rogério Vivaldi, MD, MBA. Sigilon Therapeutics, Cambridge, MA

Patient Identification/Global Clinical Trial Design

9:00 AM – 9:45 AM

Panel Discussion

SCIENTIFIC SYMPOSIUM 212

8:00 AM - 9:45 AM

Room: Auditorium

Social, Ethical, and Scientific Perspectives on Germline Gene Editing - Organized by the Ethics and Government Relations Committees

CO-CHAIRS: Lauriel Earley, PhD and Timothy Hunt, JD

SPEAKERS

8:00 AM – 8:05 AM

Timothy Hunt, JD. Sudbury, MA

Introduction and Summary of the ASGCT Policy Summit on Germline Gene Editing

8:05 AM – 8:25 AM

Monica L. Weldon. Bridge the Gap - SYNGAP, Washington, DC

Germline Gene Editing: A Parent & Patient Advocate Perspective

8:25 AM – 8:45 AM

Victor J. Dzau, MD. National Academy of Medicine, Washington, DC

Germline Gene Editing: A Responsible Path Forward

8:45 AM – 9:05 AM

J. Keith Joung, MD, PhD. Massachusetts General Hospital, Charlestown, MA

Germline Gene Editing: The Scientific Case for a Moratorium

9:05 AM - 9:25 AM

Jeffrey Kahn, PhD. Johns Hopkins University, Baltimore, MD

Germline Gene Editing: A Bioethical Perspective

9:25 - 9:45 AM

Panel Discussion

PROGRAM SCHEDULE

WEDNESDAY, MAY 13

All times are listed in Eastern Daylight Time (EDT UTC -4).

SCIENTIFIC SYMPOSIUM 213

8:00 AM - 9:45 AM

Room: Ballroom C

Targeting the Lung: Vectors and Stem Cells - Organized by the Respiratory & GI Tract Gene & Cell Therapy Committee

CO-CHAIRS: Katherine Excoffon, PhD and Allison Keeler-Klunk, PhD

SPEAKERS

8:00 AM – 8:26 AM

Purushothama Rao Tata, MS, PhD. Duke University School of Medicine, Durham, NC

Lung Stem Cell-Trophic AAV Evolution for Targeted Regenerative Medicine

8:26 AM – 8:52 AM

Viviana Gradinaru, PhD. California Institute of Technology, Pasadena, CA

Systemic AAVs and Tissue Clearing for Targeting and Mapping Distinct Cell Types

8:52 AM – 9:18 AM

Matthew Porteus, MD, PhD. Stanford University School of Medicine, Stanford, CA

Gene Editing in Airway Stem Cells for CF

9:18 AM – 9:45 AM

Carlos G. Perez-Garcia, PhD. Arcturus Therapeutics, San Diego, CA

LUNAR-CF, An Aerosolized mRNA Therapy for CF Lung Disease

BREAK

9:45 AM - 10:15 AM

EXHIBIT HALL

Open 24 hours online

PLENARY SESSION

10:15 AM - 12:00 PM

Room: Auditorium

George Stamatoyannopoulos Memorial Lecture & Presentation of the Excellence in Research Awards

Sponsored by  **REGENXBIO**

PROGRAM SCHEDULE

WEDNESDAY, MAY 13

All times are listed in Eastern Daylight Time (EDT UTC -4).

BREAK

12:00 PM - 1:30 PM

LUNCH SESSION


12:00 PM - 1:30 PM

Transitioning from Academia to Industry

Morgan Maeder, PhD - Third Rock Ventures

Laura Sepp-Lorenzino, PhD - Intellia Therapeutics

Nick Restifo, MD - Lyell Immunopharma

Sponsored by  rocket
pharma

TOOLS AND TECHNOLOGIES FORUM

12:00 PM - 1:30 PM

Room: 210

Tools and Technologies Forum III

SPEAKERS:

12:00 PM - 12:15 PM

Jeffrey Hung, PhD. ViGene Biosciences, Rockville, MD

Scalable Upstream and Downstream Production for AAV, Lentivirus and Retrovirus

12:15 PM - 12:30 PM

Phil Morton. Albumedix Ltd., Nottingham, United Kingdom

Increasing Performance of Your Cells from Harvest to Administration – Addressing Logistical Challenges with an Optimized Cryopreservation Solution

12:30 PM - 12:45 PM

James Brown, PhD. Aldevron, Fargo, ND

12:45 PM - 1:00 PM

Johnny Truong. Benchling, San Francisco, CA

How Benchling Brings Life to Cell and Gene Therapy R&D

1:00 PM - 1:15 PM

Cedrick Rousseaux. Yposkesi, Corbeil-Essonnes, France

High Performing platform for the production of large-scale lentiviral vector in suspension

1:15 PM - 1:30 PM

Mathieu Porte, Polyplus-transfection, Illkirch, France

Next-Generation Transfection Reagent for Large Scale Manufacturing

PROGRAM SCHEDULE

WEDNESDAY, MAY 13

All times are listed in Eastern Daylight Time (EDT UTC -4).

INDUSTRY SPONSORED SYMPOSIUM

12:00 PM - 1:30 PM

Room: Ballroom A

Production of AAV Viral Vectors

Sponsored by  **cytiva**
Formerly GE Healthcare Life Sciences

PRESENTER: Dr. Mats Lundgren, Cytiva, formerly GE Healthcare Life Sciences

INDUSTRY SPONSORED SYMPOSIUM

12:00 PM - 1:30 PM

Room: Ballroom B

Development of Gene Transfer Therapies: Rational Design and Application to Duchenne Muscular Dystrophy and the LGMDs

Sponsored by  **SAREPTA**
THERAPEUTICS

12:00 PM - 12:30 PM

Rationale for Design of Sarepta's Gene Therapy Platform and Implications for Theoretical Effect

Louise Rodino-Klapac, PhD, Sarepta Therapeutics

12:30 PM - 1:00 PM

Translational Development of Sarepta's Gene Therapy Platform

Eric Pozsgai, PhD, Sarepta Therapeutics

1:00 PM - 1:30 PM

SRP-9001 Micro-dystrophin Clinical Updates

Louise Rodino-Klapac, PhD

CAREER DEVELOPMENT AWARDS



Providing \$300,000 in 2020 to ASGCT Members Developing Their Careers

Applications open June 1–August 1
asgct.org/CareerAwards

PROGRAM SCHEDULE

WEDNESDAY, MAY 13

All times are listed in Eastern Daylight Time (EDT UTC -4).

INDUSTRY SPONSORED SYMPOSIUM

12:00 PM - 1:30 PM

Room: Ballroom C

Current and Future Trends for Scalable Manufacturing of Viral Vectors for Cell and Gene Therapy

Sponsored by  Pall Biotech

12:00 PM - 12:05 PM

Introduction and Session Chair

Dr. Clive Glover, Director of Cell & Gene Therapy, Pall Biotech

12:05 PM - 12:30 PM

Implementation of iCELLis® Technology for the Manufacture of Lentivirus and AAV

Dr. Giuliana Vallanti, Development and Quality Control Director and Qualified Person, MolMed

12:30 PM - 12:55 PM

Single-Use Platform for Scalable Purification of a VSV-G Lentiviral Vector

Dr. Mark Schofield, Senior R&D Manager, Pall Biotech

12:55 PM - 1:20 PM

Speeding to the Clinic. From Contract Process Development to PODs

Jeremy Rautenbach, Global Product Manager for Integrated Solutions, Pall Biotech

Peter Makowenskyj, Director - Sales Engineering, G-CON Manufacturing

1:20 PM - 1:30 PM

Discussion and Summary

Dr. Clive Glover

PLENARY SESSION

1:30 PM - 3:00 PM

Room: Auditorium

Outstanding New Investigator Symposium

Sponsored by  Burroughs Wellcome Fund

BREAK

3:00 PM - 3:45 PM

PROGRAM SCHEDULE

WEDNESDAY, MAY 13

All times are listed in Eastern Daylight Time (EDT UTC -4).

TOOLS AND TECHNOLOGIES FORUM

3:30 PM - 4:45 PM

Room: 210

Tools and Technologies Forum IV

SPEAKERS:

3:30 PM - 3:45 PM

Steve Pincus, PhD. FUJIFILM Diosynth Biotechnologies, Morrisville, NC
A look at FUJIFILM Diosynth Development of an Advanced Gene Therapy Platform

3:45 PM – 4:00 PM

Alla Zilberman, PhD. Cygnus Technologies, Southport, NC
Analytics for bioprocess-related impurities in viral vector manufacturing

4:00 PM - 4:15 PM

Renee Tobias. Halo Labs, Burlingame, CA
Automated, Low-Volume Subvisible AAV Aggregate Analysis with the HORIZON System

4:15 PM - 4:30 PM

Kevin Lance, PhD. Unchained Labs, Pleasanton, CA
Unraveling the Uncoating, Capsid Stability, and Aggregation of AAV with High-Throughput Screening on Uncle

4:30 PM – 4:45 PM

Jayanthi Grebin. CPC - Colder Products Company, St. Paul, MN
Single-Use Connectors to Close Systems

ORAL ABSTRACT SESSION 250

3:45 PM - 5:30 PM

Room: Auditorium

CAR T-Cell Therapies II

Chair: Saad Kenderian, MB, ChB

3:45 PM – 4:00 PM

482: CD19-Positive Brain Pericytes as Targets of Immunotherapy-Associated Neurotoxicity
Denis Migliorini, University of Geneva, Geneva, Switzerland

PROGRAM SCHEDULE

WEDNESDAY, MAY 13

All times are listed in Eastern Daylight Time (EDT UTC -4).

4:00 PM – 4:15 PM

483: Cellular Delivery of Oncolytic Adenoviruses Utilizing Mesenchymal Stromal Cells (MSCs) in Combination with CAR-T Cell Therapy

Katie McKenna, Baylor College of Medicine, Houston, TX, United States

4:15 PM – 4:30 PM

484: Enhancing Tumor Directed T Cells with a Costimulatory CAR

Mara Cardenas, Baylor College of Medicine, Houston, TX, United States

4:30 PM – 4:45 PM

485: Multiplexed Cytidine Deamination Enables Generation of Fratricide-Resistant 'Universal T v T' Chimeric Antigen Receptor Cell Therapy

Christos Georgiadis, UCL, GOS Institute of Child Health, London, United Kingdom

4:45 PM – 5:00 PM

486: A Phase 1 Clinical Trial Using Armored GPC3-Car T Cells for Children with Relapsed/Refractory Liver Tumors

David Steffin, Baylor College of Medicine, Houston, TX, United States

5:00 PM – 5:15 PM

487: Automatic Neutralization of IL6 Storm and Blockade of IL1 Signaling During CART Therapy to Reduce Cytokine Toxicity and Minimize Neurotoxicity

Biliang Hu, Cellectis, Worcester, MA, United States

5:15 PM – 5:30 PM

488: Iberdomide Increases the Potency of the Anti-BCMA CAR T Cell Product Orvacabtagene Autoleucel (orva-cel)

Neha Soni, Juno Therapeutics, a Bristol-Myers Squibb Company, Seattle, WA, United States

PROGRAM SCHEDULE

WEDNESDAY, MAY 13

All times are listed in Eastern Daylight Time (EDT UTC -4).

ORAL ABSTRACT SESSION 251

3:45 PM - 5:15 PM

Room: Ballroom A

Preclinical Large Animal Studies for Neurologic Diseases

Chair: Heather Gray-Edwards, DVM, PhD

3:45 PM – 4:00 PM

489: Combination Hematopoietic Stem Cell Transplantation and Intravenous AAV Gene Therapy Corrects Neurologic Phenotype in Canine Krabbe Disease

Allison Bradbury, University of Pennsylvania, Philadelphia, PA, United States

4:00 PM – 4:15 PM

490: Method to Prevent AAV-Induced Dorsal Root Ganglia Toxicity & Axonopathy in Nonhuman Primates, and Insights into the Pathophysiology

Juliette Hordeaux, University of Pennsylvania, Philadelphia, PA, United States

4:15 PM – 4:30 PM

491: One-Time Intrathecal Administration of AAV5-miATXN3 in Non-Human Primates

Melvin Evers, uniQure, Amsterdam, Netherlands

4:30 PM – 4:45 PM

495: Effect of Administration Route and AAV Serotype for Treatment of Feline GM1 Gangliosidosis

Amanda Gross, Auburn University, Auburn, AL, United States

4:45 PM – 5:00 PM

493: Intravenous AAV Gene Therapy Improves Lifespan and Clinical Metrics in Feline Sandhoff Disease

Anne Maguire, Auburn University, Auburn, AL, United States

5:00 PM – 5:15 PM

494: Translating Gene Therapy for Spinocerebellar Ataxia Type 1: Unforeseen Consequences in Higher Mammals

Megan Keiser, The Children's Hospital of Philadelphia, Philadelphia, PA, United States

PROGRAM SCHEDULE

WEDNESDAY, MAY 13

All times are listed in Eastern Daylight Time (EDT UTC -4).

ORAL ABSTRACT SESSION 252

3:45 PM - 5:30 PM

Room: Ballroom B

Musculo-skeletal Diseases I

Chair: Rita Perlingeiro, PhD

3:45 PM – 4:00 PM

496: Progress Toward Translating AAV.CRISPR-Cas13 Therapy for FSHD

Afroz Rashnonejad, Abigail Wexner Research Institute at Nationwide Children's Hospital, Columbus, OH, United States

4:00 PM – 4:15 PM

497: CRISPR-Mediated Gene Correction in a Severe Humanized Mouse Model of Duchenne Muscular Dystrophy

Karen Bulaklak, Duke University, Durham, NC, United States

4:15 PM – 4:30 PM

498: A New Laminin α 2-Based Gene Therapy for Congenital Muscular Dystrophy 1A

Davin Packer, Nationwide Children's Hospital, Columbus, OH, United States

4:30 PM – 4:45 PM

499: AAV.CAPN3 Systemic Gene Therapy Improves the Phenotype in the Calpain3-Null Mouse Model for LGMD2A

Zarife Sahenk, The Abigail Wexner Research Institute, Nationwide Children's Hospital, Columbus, OH, United States

4:45 PM – 5:00 PM

500: SGT-001 Microdystrophin Gene Therapy for Duchenne Muscular Dystrophy

J Gonzalez, Solid Biosciences, Cambridge, MA, United States

5:00 PM – 5:15 PM

501: AAV PGC1-Alpha Gene Therapy Improves the Cardiac Function in a Mouse Model of Duchenne Muscular Dystrophy

Nalinda Wasala, University of Missouri, Columbia, MO, United States

5:15 PM – 5:30 PM

502: Assessment of Immunomodulatory Regimens that Allow Systemic Redosing of an AAV-CRISPR/Cas9 Therapy for Duchenne Muscular Dystrophy

Courtney Young, MyoGene Bio, Los Angeles, CA, United States

PROGRAM SCHEDULE

WEDNESDAY, MAY 13

All times are listed in Eastern Daylight Time (EDT UTC -4).

ORAL ABSTRACT SESSION 253

4:00 PM - 5:30 PM

Room: Ballroom C

AAV Vectors - Clinical Studies II

Chair: Carsten Bonnemann, MD

4:00 PM – 4:15 PM

504: ADVM-022 Intravitreal Gene Therapy for Neovascular AMD - Results from the Phase 1 OPTIC Study

Szilard Kiss, Weill Cornell University, New York, NY, United States

4:15 PM – 4:30 PM

505: AAV8 Gene Therapy as a Potential Treatment in Adults with Late-Onset OTC Deficiency: Results from a Phase 1/2 Clinical Trial

Wen-Hann Tan, Boston Children's Hospital, Harvard Medical School, Boston, MA, United States

4:30 PM – 4:45 PM

506: Optogenetics in the Clinic: PIONEER, a Phase 1/2a Gene Therapy Program for Non-Syndromic Retinitis Pigmentosa

Joseph Martel, University of Pittsburgh, Pittsburgh, PA, United States

4:45 PM – 5:00 PM

507: Improving the Quantification of rSIV.F/HN Vectors for Cystic Fibrosis Gene Therapy Using Droplet Digital PCR

Toby Gamlen, University of Oxford, Oxford, United Kingdom

5:00 PM – 5:15 PM

508: Engineering Viral Protein Ratios to Impact AAV Potency

Andrade Hendricks, Voyager Therapeutics, Cambridge, MA, United States

5:15 PM – 5:30 PM

509: Phase 1 Study of Gene Therapy in Late-Onset Pompe Disease: Analyses of Safety and Secondary Endpoints

Dwight Koeberl, Duke University School of Medicine, Durham, NC, United States

PROGRAM SCHEDULE

WEDNESDAY, MAY 13

All times are listed in Eastern Daylight Time (EDT UTC -4).

ORAL ABSTRACT SESSION 254

3:45 PM - 5:30 PM

Room: 302

Controlling AAV Gene Expression: Shifting Paradigms

Chair: Frederico Mingozzi, PhD

3:45 PM – 4:00 PM

510: A Reversible RNA On-Switch That Controls Expression of Aav-Delivered Transgenes In Vivo

Guocai Zhong, Shenzhen Bay Laboratory (SZBL), Shenzhen, China

4:00 PM – 4:15 PM

511: AAV Capsid-Promoter Interactions Determine CNS Cell Selective Gene Expression *In Vivo*

Sara Powell, Asklepios Biopharmaceutical, Inc., Durham, NC, United States

4:15 PM – 4:30 PM

512: The Genomic Architecture of a Genotoxic Recombinant AAV Integration in a Murine Hepatocellular Carcinoma

Randy Chandler, National Institutes of Health, Bethesda, MD, United States

4:30 PM – 4:45 PM

513: PESCA: A Scalable Platform for Regulatory Element Engineering of Viral Gene Therapy Vectors

Sinisa Hrvatin, Harvard Medical School, Boston, MA, United States

4:45 PM – 5:00 PM

514: Tailored AAV-Based Transgene Expression in the Inner Ear with Cell Type-Specific Promoters

Adam Palermo, Decibel Therapeutics, Boston, MA, United States

5:00 PM – 5:15 PM

515: Structure-Guided Rational Design of Adeno-Associated Viral Capsids with Expanded Sizes

Xiaozhe Ding, California Institute of Technology, Pasadena, CA, United States

5:15 PM – 5:30 PM

516: Engineering CpG-Free ITR for AAV Gene Therapy

Xiufang Pan, University of Missouri, Columbia, MO, United States

PROGRAM SCHEDULE

WEDNESDAY, MAY 13

All times are listed in Eastern Daylight Time (EDT UTC -4).

ORAL ABSTRACT SESSION 255

3:45 PM - 5:30 PM

Room: 304 & 306

Evaluating Genome Editing Activity and Precision

Chair: Shengdar Tsai, PhD

3:45 PM – 4:00 PM

517: Large-Scale CRISPR-Cas Genome-Wide Activity Profiling in Human Primary T-Cells Reveals Genetic and Epigenetic Determinants of Genome-Wide Nuclease Activity

Cicera Lazzarotto, St Jude Children's Research Hospital, Memphis, TN, United States

4:00 PM – 4:15 PM

518: Evaluation of the Long-term Effects of AAV-Meganuclease Genome Editing of PCSK9 in Macaque Liver

Lili Wang, University of Pennsylvania, Philadelphia, PA, United States

4:15 PM – 4:30 PM

519: Unintended Consequences of CRISPR/Cas9-Mediated Genome Editing for Treating Sickle Cell Disease

So Hyun Park, Rice University, Houston, TX, United States

4:30 PM – 4:45 PM

520: Muscle-Specific Editing in a Therapeutic Target of Duchenne Muscular Dystrophy Using Cas9 and miRNA-Repressible Anti-CRISPR Proteins

Jooyoung Lee, University of Massachusetts Medical School, Worcester, MA, United States

4:45 PM – 5:00 PM

521: Base Editing of Splice Sites Mediates High Efficiency Multiplex Disruption and Modulation of Genes in Primary Human T Cells and NK Cells

Mitchell Kluesner, University of Minnesota, Minneapolis, MN, United States

5:00 PM – 5:15 PM

522: High-Throughput Screen to Optimize gRNA Pairs for CRISPR-Cas9-Mediated Exon Deletion

Veronica Gough, Duke University, Durham, NC, United States

PROGRAM SCHEDULE

WEDNESDAY, MAY 13

All times are listed in Eastern Daylight Time (EDT UTC -4).

5:15 PM – 5:30 PM

523: A Dual AAV Vector System to Visualize Prime Editing in Mammalian Cells

Jonathan Lang, Children's Hospital of Philadelphia, Philadelphia, PA, United States

ORAL ABSTRACT SESSION 256

3:45 PM - 5:30 PM

Room: 309

Clinical Gene Therapies for Inborn Errors of Metabolism

Chair: Charles Venditti, MD, PhD

3:45 PM – 4:00 PM

524: Transpher A, an Open-Label, Multicenter, Single-Dose, Dose-Escalation, Phase 1/2 Clinical Trial of Gene Transfer of ABO-102 in Sanfilippo Syndrome Type A (Mucopolysaccharidosis IIIA): Safety, Tolerability, Biopotency and Neurocognitive Data

Kevin Flanigan, Nationwide Children's Hospital, Columbus, OH, United States

4:00 PM – 4:15 PM

525: Preliminary Outcomes of Haematopoietic Stem Cell Gene Therapy in a Patient with Mucopolysaccharidosis IIIA

Brian Bigger, University of Manchester, Manchester, United Kingdom

4:15 PM – 4:30 PM

526: Transpher B, an Open-Label, Multicenter, Single-Dose, Dose-Escalation, Phase 1/2 Clinical Trial of Gene Transfer of ABO-101 in Sanfilippo Syndrome Type B (Mucopolysaccharidosis IIIB)

Maria de Castro, Hospital Clinico Universitario de Santiago, Santiago De Compostela, Spain

4:30 PM – 4:45 PM

527: Hematopoietic Stem Cell Gene Therapy for Cystinosis: Initial Results from a Phase I/II Clinical Trial

Stephanie Cherqui, University of California, San Diego, La Jolla, CA, United States

PROGRAM SCHEDULE

WEDNESDAY, MAY 13

All times are listed in Eastern Daylight Time (EDT UTC -4).

4:45 PM – 5:00 PM

528: Pathophysiological Mechanisms of Bone Defects and Impact of Ex Vivo Hematopoietic Stem Cell Gene Therapy in Mucopolysaccharidosis Type I Hurler

Ludovica Santi, San Raffaele-Telethon Institute for Gene Therapy (SR-Tiget), Milan, Italy

5:00 PM – 5:15 PM

529: Gb3 Substrate in Endothelial Cells of Renal Peritubular Capillaries Was Reduced in a Previously Untreated Classic Fabry Male Patient Treated with AVR-RD-01 Investigational Lentiviral Gene Therapy

Birgitte Volck, AVROBIO, Inc., Cambridge, MA, United States

5:15 PM – 5:30 PM

530: Late Phases of Hematopoietic Reconstitution in Metachromatic Leukodystrophy Gene Therapy Patients Are Characterized by Lineage Commitment of Individual HSC Clones

Andrea Calabria, San Raffaele Telethon Institute for Gene Therapy, Milan, Italy

ORAL ABSTRACT SESSION 257

3:45 PM - 5:15 PM

Room: 310

Applications of Physical, Chemical and Exosomal Delivery

Chair: David Dean, PhD

3:45 PM – 4:00 PM

531: Induction of Long-Term Tolerance to a Specific Antigen Using Anti-CD3 Lipid Nanoparticles Following Gene Therapy

Chun-Yu Chen, Seattle Children's Hospital Research Institute, Seattle, WA, United States

4:00 PM – 4:15 PM

532: Fusion Gene-Based Therapy for Prevention and Treatment of Obesity and Obesity-Associated Metabolic Diseases

Yueze Yang, University of Georgia, Athens, GA, United States

4:15 PM – 4:30 PM

533: A Single Dose of Fast Half-Life CD117 Antibody Drug Conjugate Enables Hematopoietic Stem Cell Based Gene Therapy in Nonhuman Primates

Naoya Uchida, NIH, Bethesda, MD, United States

PROGRAM SCHEDULE

WEDNESDAY, MAY 13

All times are listed in Eastern Daylight Time (EDT UTC -4).

4:30 PM – 4:45 PM

534: Developing Nonviral Gene Therapies for Inherited Blinding Disorders Using CRISPR/Cas9 Genomic Editing and Human Pluripotent Stem Cells

Amr Abdeen, University of Wisconsin-Madison, Madison, WI, United States

4:45 PM – 5:00 PM

535: Building a Versatile and Clinically Translatable Platform for DNA-Based Antibody Therapeutics

Kevin Hollevoet, KU Leuven - University of Leuven, Leuven, Belgium

5:00 PM – 5:15 PM

537: Optimization of Lipid Nanoparticle Formulation Leads to Functional mRNA Delivery in Rat Lungs and New Opportunities for Gene Editing and Delivery

Kristina Friis, AstraZeneca, Gothenburg, Sweden

ORAL ABSTRACT SESSION 258

3:45 PM - 5:00 PM

Room: 311

Vector and Cell Engineering, Production or Manufacturing III

Chairs: Isabelle Riviere, PhD

3:45 PM – 4:00 PM

538: Generation of Immune Resistant, Stem Cell Targeting Lentivirus for Cystic Fibrosis Airway Gene Therapy

Juliette Delhove, University of Adelaide, Adelaide, Australia

4:00 PM – 4:15 PM

539: HeLa 3.0: CRISPR Knockout of Genes Modulating Titer in Established rAAV-Producing Cell Lines

Nicholas Richards, Ultragenyx Gene Therapy, Cambridge, MA, United States

4:15 PM – 4:30 PM

540: A Complete “Self-Repressing” Adenovirus System Enables Efficient Manufacture of Adeno-Associated Viral Vectors without Contamination by Adenovirus or Small Drugs

Weiheng Su, University of Oxford, Oxford, United Kingdom

4:30 PM – 4:45 PM

541: Massively Parallel Deep Diversification of AAV Capsid Proteins by Machine Learning

Sam Sinai, Dyno Therapeutics, Cambridge, MA, United States

PROGRAM SCHEDULE

WEDNESDAY, MAY 13

All times are listed in Eastern Daylight Time (EDT UTC -4).

4:45 PM – 5:00 PM

543: Characterization of Phenotypic and Genotypic Stability of rAAV Producing HeLa Cell Lines

Alix Eastham, Ultragenyx Gene Therapy, Cambridge, MA, United States

ORAL ABSTRACT SESSION 259

3:45 PM - 5:15 PM

Room: 312

HSPC Gene Therapies for Hemoglobin Disorders

Chair: Megan Hoban, PhD

3:45 PM – 4:00 PM

545: Durable Robust Fetal Globin Induction in Rhesus Monkeys Following *BCL11A* Erythroid Enhancer Edited Autologous Hematopoietic Stem Cell Transplant

Selami Demirci, NIH, Bethesda, MD, United States

4:00 PM – 4:15 PM

546: *In Vivo* HSC Gene Therapy for Hemoglobinopathies: A Proof of Concept Evaluation in Rhesus Macaques

Chang Li, University of Washington, Seattle, WA, United States

4:15 PM – 4:30 PM

547: Base Editing of Gamma Globin Gene Promoters Generates Durable Expression of Fetal Hemoglobin for the Treatment of Sickle Cell Disease

Adrian Rybak, Beam Therapeutics, Cambridge, MA, United States

4:30 PM – 4:45 PM

548: Gene Correction Using CRISPR/Cas9: IND-Enabling Studies to Support a Clinical Trial of a CRISPR/Homology-Directed Repair Treatment for Sickle Cell Disease

Mark DeWitt, University of California, Los Angeles, Los Angeles, CA, United States

4:45 PM – 5:00 PM

549: Editing the LRF Repressor Binding Site in the γ -Globin Promoters Induces Therapeutically Relevant Fetal Hemoglobin Levels for the Treatment of β -Hemoglobinopathies

Giacomo Frati, Imagine Institute, Paris, France

PROGRAM SCHEDULE

WEDNESDAY, MAY 13

All times are listed in Eastern Daylight Time (EDT UTC -4).

5:00 PM – 5:15 PM

551: Editing Hematopoietic Stem Cells at the FOXP3 Locus: A Gene Editing Approach to Treat IPEX Patients

Swati Singh, Seattle Children's Research Institute, Seattle, WA, United States

EXHIBIT HALL

Open 24 hours online

POSTER SESSION II

Open 24 hours on May 13

5:30 PM - 6:30 PM

Scheduled presenters requested to be available for live Q&A

PROGRAM SCHEDULE

THURSDAY, MAY 14

All times are listed in Eastern Daylight Time (EDT UTC -4).

SCIENTIFIC SYMPOSIUM 300

Organized by the American Society for Transplantation and Cellular Therapy

8:00 AM - 9:45 AM

Room: Ballroom A

Engineering Immunity: CARs for Non-B Cell Malignancies - Organized by the American Society for Transplantation and Cellular Therapy

SPEAKERS:

8:00AM – 8:26 AM

Yvonne Y. Chen, PhD. University of California Los Angeles, Los Angeles, CA
Engineering Next-Generation T Cells for Cancer Immunotherapy

8:26 AM – 8:52 AM

Aude Chapuis, PhD. Fred Hutchinson Cancer Research Center, Seattle, Washington

8:52 AM – 9:18 AM

Waseem Qasim, PhD. UC London, London UK
Extending Genome Edited CAR T Cells to Non-B cell Haematological Malignancies

9:18 AM – 9:45 AM

John DiPersio, MD, PhD. Washington University, St. Louis, MO
CART for T Cell Malignancies

SCIENTIFIC SYMPOSIUM 301

8:00 AM - 9:20 AM

Room: 304 & 306

Advancing Therapeutic Exosomes for the Treatment of Disease - Organized by the Nanoagents & Synthetic Formulations Committee

CO-CHAIRS: Angela Pannier, PhD and Rajagopal Ramesh, PhD

SPEAKERS

8:00 AM – 8:26 AM

Xandra Breakefield, PhD. Massachusetts General Hospital / Harvard Medical School, Charlestown, MA
Equipping Extracellular Vesicles with Functional Reporters

8:26 AM – 8:52 AM

Konstantin Konstantinov, PhD. Codiak BioSciences, Cambridge, MA
Towards Industrialization of the GMP Manufacture of Exosome-Based Biotherapeutics

PROGRAM SCHEDULE

THURSDAY, MAY 14

All times are listed in Eastern Daylight Time (EDT UTC -4).

8:52 AM – 9:18 AM

Susanne Gabrielsson, PhD. Karolinska Institute, Stockholm, Sweden
Exosomes for Cancer Therapy

SCIENTIFIC SYMPOSIUM 302

8:26 AM - 9:45 AM

Room: Ballroom C

Clearing the Path to an IND: How We Succeeded - Organized by the Translational Science Committee

CO-CHAIRS: Nicole Paulk, PhD and Johannes van Der Loo, PhD

SPEAKERS

8:26 AM – 8:52 AM

Miguel Sena-Esteves, PhD. University of Massachusetts Medical School, Worcester, MA

A DIY Approach to Bringing Academic AAV Gene Therapy Programs to First-in-Human Clinical Trials

8:52 AM – 9:18 AM

Francesca Vitelli, PhD. Lonza, Inc., Houston, TX

CDMO's Perspectives on Process Development for Viral Vectors

9:18 AM – 9:45 AM

Barry Byrne, MD, PhD. University of Florida College of Medicine, Gainesville, FL

IND Submission: Differences in Academia vs. Industry

SCIENTIFIC SYMPOSIUM 303

8:00 AM - 9:45 AM

Room: Auditorium

Latest Advances in Cancer Gene and Cellular Therapies - Organized by the Cancer Gene & Cell Therapy Committee

CHAIR: Paola Grandi, PhD

SPEAKERS

8:00 AM – 8:26 AM

Edward Stadtmauer, MD. University of Pennsylvania, Philadelphia, PA
CRISPR-engineered T Cells in Patients with Refractory Cancer

PROGRAM SCHEDULE

THURSDAY, MAY 14

All times are listed in Eastern Daylight Time (EDT UTC -4).

8:26 AM – 8:52 AM

Armand Keating, MD. University of Toronto, Toronto, ON, Canada
NK Cell Therapy with Off the Shelf™ Natural Killer Cells

8:52 AM – 9:18 AM

John Bell, PhD. Ottawa Hospital Research Institute, Ottawa, ON, Canada
Cancer Vaccines and Neo Antigens

9:18 AM 9:45 AM

Kah-Whye Peng, PhD. Mayo Clinic, Rochester, MN
Solid Tumor Oncolytic Viruses

SCIENTIFIC SYMPOSIUM 304

8:00 AM - 9:45 AM

Room: Ballroom B

**Vector Development, Clinical Implementation, and Corporate Connections
- Organized by the Viral Gene Transfer Vectors Committee**

CHAIR: Daniel M. Lipinski, DPhil

SPEAKERS

8:00 AM – 8:26 AM

Ben E. Deverman, PhD. Broad Institute of MIT and Harvard, Cambridge, MA
Engineering AAV Capsids for the Delivery of Gene Therapies to the CNS

8:26 AM – 8:52 AM

Joseph E. Rabinowitz, PhD. Pfizer, Inc., Cambridge, MA
Industry Perspectives of AAV Vectors

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PROGRAM SCHEDULE

THURSDAY, MAY 14

All times are listed in Eastern Daylight Time (EDT UTC -4).

8:52 AM – 9:18 AM

Maria-Grazia Roncarolo, MD. Stanford University School of Medicine,
Stanford, CA

Academic Perspectives of Lentiviral Vectors

9:18 AM – 9:45 AM

Philip D. Gregory, DPhil. bluebird bio, Cambridge, MA

Industry Perspectives of Lentiviral Vectors

NIH SESSION 310

8:00 AM - 9:45 AM

Room: 311

IND-Enabling NIH Resources to Advance Clinical and Translational Gene Therapy

CHAIR: Terence Flotte, MD

SPEAKERS

8:00 AM – 8:10 AM

Cheryl McDonald, MD. National Heart Lung and Blood Institute, NIH,
Bethesda, MD

Introduction and Overview of NIH Resource Programs

8:10 AM – 8:40 AM

David A. Wilcox, PhD. Department of Pediatrics, Medical College of
Wisconsin, Milwaukee, WI; Children's Research Institute, Children's
Hospital of Wisconsin, Milwaukee, WI

Platelet-Targeted Gene Therapy for Hemophilia A

8:40 AM – 9:10 AM

Barry J. Byrne, MD, PhD. University of Florida College of Medicine,
Gainesville, FL

Pathway for Investigator Initiated Studies to IND and Beyond

9:10 AM – 9:40 AM

Dwight Koeberl, MD, PhD. Duke University School of Medicine, Durham,
NC

Recombinant AAV Gene Therapy for Pompe Disease

9:40 AM – 9:45 AM

Terence Flotte, MD. University of Massachusetts Medical School,
Worcester, MA

Summary

PROGRAM SCHEDULE

THURSDAY, MAY 14

All times are listed in Eastern Daylight Time (EDT UTC -4).

EXHIBIT HALL

Open 24 hours online

BREAK

9:45 AM - 10:15 AM

PLENARY SESSION

10:15 AM - 12:00 PM

Room: Auditorium

Outstanding Achievement Award Lecture & Presentation & Sonia Skarlatos Public Service Award

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BREAK

12:00 PM - 1:30 PM

Lunch Break

LUNCH SESSION

12:00 PM - 1:30 PM


Navigating Career Paths for Trainees

Emmanuel Adu-Gyamfi, PhD - FDA

Kelly Moynihan, PhD - Third Rock Ventures

Xiuyan Wang, PhD - Memorial Sloan Kettering Cancer Center

Terry Flotte, MD - University of Massachusetts Medical School

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LUNCH SESSION

12:00 PM - 1:30 PM

Characterizing and Minimizing Genome Editing Off-Target Effects


CRISPR

Ayal Hendel, PhD - Bar Ilan University

TJ Cradick, PhD - CRISPR Therapeutics

Benjamin P. Kleinstiver, PhD - Massachusetts General Hospital and Harvard Medical School

Luca Pinello, PhD - Harvard Medical School

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PROGRAM SCHEDULE

THURSDAY, MAY 14

All times are listed in Eastern Daylight Time (EDT UTC -4).

TOOLS AND TECHNOLOGIES FORUM

12:00 PM - 1:30 PM

Room: 210

Tools and Technologies Forum V

SPEAKERS:

12:00 PM - 12:15 PM

Amanda Fentiman. STEMCELL Technologies, Vancouver, Canada
Optimized, Serum-Free, In Vitro Culture Conditions for CRISPR-Mediated Gene Editing of CD34+ Cells

12:15 PM - 12:30 PM

Emilie Vey, PhD. Ovizio Imaging Systems, Brussels, Belgium
Ivie Aifuwa, PhD. Bristol-Myers Squibb, Seattle, WA
Automated, Closed Loop, In-Line and Label-Free Monitoring of CAR-T Cells in a Production Process

12:30 PM - 12:45 PM

Seth Levy. Thermo Fisher Scientific, Alachua, FL
Documentation of Clonality as Part of the CMC Process in Viral Vector Production

12:45 PM - 1:00 PM

Rachel Legmann, PhD. Pall Biotech, Westborough, MA
AAV9 Viral Vector Large Scale Bioreactor Production: From Bench Top to Manufacturing

1:00 PM - 1:15 PM

Rob Durham, PhD. Gyros Protein Technologies, Uppsala, Sweden
Innovative Immunoassays: Boosting Workflow Efficiency and Data Quality in Bioprocessing and Immunogenicity

1:15 PM - 1:30 PM

Jian Chen, PhD. Celetrix Electroporation, Manassas, VA
Revisiting and Revamping Electroporation Concepts and Practices

PROGRAM SCHEDULE

THURSDAY, MAY 14

All times are listed in Eastern Daylight Time (EDT UTC -4).

INDUSTRY SPONSORED SYMPOSIUM

12:00 PM - 1:30 PM

Room: Ballroom A

Doing More with Less: Perspectives on Automating Manufacturing of Cell and Gene Therapy Products

Sponsored by



PRESENTERS: Gerhard Bauer, Professor, Director, UC Davis GMP Facility; Johnathon D. Anderson, PhD, Assistant Professor, UC Davis Institute for Regenerative Cures

INDUSTRY SPONSORED SYMPOSIUM

12:00 PM - 1:30 PM

Room: Ballroom B

Viral Safety: The Alpha and the Omega - Key considerations for developing and manufacturing safe products in a safe working environment when working with Advanced Viral Vectors

Sponsored by



MODERATOR: Steven Pincus, Head of Science and Innovation, FUJIFILM Diosynth Biotechnologies

PANELISTS: Akunna Iheanacho, PhD, Director of Research and Development, Texcell North America; Michael Mercaldi, Director of Purification Process Development, Homology Medicines; Daniel Strauss, Principal Scientist, Asahi Kasei Bioprocess America

INDUSTRY SPONSORED SYMPOSIUM

12:00 PM - 1:30 PM

Room: Ballroom C

From Bench to Bedside: Uncovering the Potential of Gene Therapy

Sponsored by



12:00 PM - 12:05 PM

Welcome and Introduction

Mark Pykett, Chair

PROGRAM SCHEDULE

THURSDAY, MAY 14

All times are listed in Eastern Daylight Time (EDT UTC -4).

12:05 PM - 12:40 PM

Overview of PTC Therapeutics' Gene Therapy Programs

Mark Pykett, Chief Scientific Officer, PTC Therapeutics

12:40 PM - 1:10 PM

Gene Therapy for Neurological Disease: Clinical Outcomes in AADC Deficiency

Timothy Feyma, Co-Director, Complex Movement Disorder Clinic, Gillette Children's Specialty Healthcare

1:10 PM - 1:25 PM

Interactive Q&A

Moderated by Chair

1:25-1:30

Adjourn

INDUSTRY SPONSORED SYMPOSIUM

12:00 PM - 1:30 PM

Room: 304/306

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12:00 PM - 12:30 PM

Characterization of AAV Provector Targeted Delivery through Scar Optimization

Susan Butler, Rice University

12:30 PM - 1:00 PM

AAV-Mediated CRISPR/Cas9 Delivery

Bence Gyogy, Institute of Molecular and Clinical Ophthalmology

1:00 PM - 1:30 PM

Precise, Non-Viral, CAR-T Engineering with CRISPR sgRNA and ssDNA

Dr. Lumeng Ye, GenScript USA

PRESIDENTIAL SYMPOSIUM

1:30 PM - 3:15 PM

Room: Auditorium

Presidential Symposium & Presentation of the Top Abstracts

Sponsored by  **Oxford Biomedica**

PROGRAM SCHEDULE

THURSDAY, MAY 14

All times are listed in Eastern Daylight Time (EDT UTC -4).

BREAK

3:15 PM - 3:45 PM

ORAL ABSTRACT SESSION 350

3:45 PM - 5:30 PM

Room: Auditorium

Viral Vector Development: RNA Virus Vectors

Chair: Carol Miao, PhD

3:45 PM – 4:00 PM

909: Human CD3 as a Target Receptor for Simultaneous Activation and Cell-Specific Transduction by Lentiviral Vectors

Annika Frank, Paul-Ehrlich-Institut, Langen, Germany

4:00 PM – 4:15 PM

910: Lentiviral Gene Therapy for p47phox Deficient Chronic Granulomatous Disease: One Step Closer to the Clinic

Andrea Schejtman, UCL Great Ormond Street Institute of Child Health, London, United Kingdom

4:15 PM – 4:30 PM

911: Investigating the Stability of Lentiviral Vector Targeted Liver Cells During Post-Natal Growth for In Vivo Gene Therapy Applications

Alessio Cantore, San Raffaele Telethon Institute for Gene Therapy, Milan, Italy

4:30 PM – 4:45 PM

912: Development of Cas9 Protein Delivery Non-Integrating Lentiviral Vectors for Gene Correction in Sickle Cell Disease

Naoya Uchida, NIH, Bethesda, MD, United States

4:45 PM – 5:00 PM

913: Cell Type Specific Co-Factors are Required for IFITM3 Antiviral Activity in Human Hematopoietic Stem and Progenitor Cells

Giulia Unali, San Raffaele Scientific Institute, TIGET, Milan, Italy

5:00 PM – 5:15 PM

914: Safety Evaluation of Intracerebral Injection of Lentiviral ABCD1 for X-ALD Gene Therapy

Yunyun Liu, UESTC, Chengdu, China

5:15 PM – 5:30 PM

915: Profiling of Encapsidated Producer Cell RNA in Lentiviral Vectors

Matteo Franco, GeneWerk GmbH, Heidelberg, Germany

PROGRAM SCHEDULE

THURSDAY, MAY 14

All times are listed in Eastern Daylight Time (EDT UTC -4).

ORAL ABSTRACT SESSION 351

3:45 PM - 5:30 PM

Room: Ballroom A

AAV Vector Development: Towards Translation

Chair: Luk Vandenberghe, PhD

3:45 PM – 4:00 PM

916: A Novel Approach for Rapid and Transient Depletion of Pre-Existing Neutralizing Antibodies Against AAV Vectors

Zachary Elmore, Duke University, Durham, NC, United States

4:00 PM – 4:15 PM

917: *In Situ* Detection of Adeno-Associated Virus Genomes with DNA SABER-FISH

Sean Wang, Harvard Medical School, Boston, MA, United States

4:15 PM – 4:30 PM

918: Long-Read Characterization of Adeno-Associated Virus Packaged DNA by Direct Nanopore Sequencing Reveals Genome Fusions and Recombination between Genomes and Producer Plasmids

Marco Radukic, Bielefeld University, Bielefeld, Germany

4:30 PM – 4:45 PM

919: Non-Stringent Requirement in AAV D-Element Leads to Erroneous Encapsulation of Non-Vector Sequences

Junping Zhang, Temple University, Philadelphia, PA, United States

4:45 PM – 5:00 PM

920: In-Depth Parallel Profiling of Tissue and Cell-Type Tropism of AAV Variants by Single-Cell RNA Sequencing

Michael Altermatt, California Institute of Technology, Pasadena, CA, United States

5:00 PM – 5:15 PM

921: Novel AAV Vector for Delivery of Transgenes to the Photoreceptors and RPE via Intravitreal Injection Enables Rescue of Multiple Models of Retinal Disease

Rajendra Kumar-Singh, Tufts University, Boston, MA, United States

PROGRAM SCHEDULE

THURSDAY, MAY 14

All times are listed in Eastern Daylight Time (EDT UTC -4).

5:15 PM – 5:30 PM

922: Brain Transduction Profiles of Novel Human-Derived Clade B and C Variants in Mice and Non-Human Primates

Phillip Tai, University of Massachusetts Medical School, Worcester, MA, United States

ORAL ABSTRACT SESSION 352

4:00 PM - 5:15 PM

Room: Ballroom B

Musculo-skeletal Diseases II

Chair: Scott Q. Harper, PhD

4:00 PM – 4:15 PM

924: A Universal Gene Correction Approach for FKRP Mutant Patient-Specific iPS Cells: Potential for Autologous Cell Therapy

Neha Dhoke, University Of Minnesota, Minneapolis, MN, United States

4:15 PM – 4:30 PM

925: Effective Pseudo-Exon Skipping of a COL6A1 Intronic Mutation in Cultured Muscle Interstitial Fibroblasts from a Novel Humanized Mouse Model

Véronique Bolduc, NINDS/NIH, Bethesda, MD, United States

4:30 PM – 4:45 PM

926: Hematopoietic Stem Cell Gene Therapy Corrects Neuromuscular Manifestations in Preclinical Study of Pompe Mice

Niek van Til, AVROBIO Inc, Cambridge, MA, United States

4:45 PM – 5:00 PM

929: Combined CNS and Systemic Directed Gene Therapy in a Mouse Model of Pompe Disease with Advanced Disease at Treatment

Juliette Hordeaux, University of Pennsylvania, Perelman School of Medicine, Philadelphia, PA, United States

5:00 PM – 5:15 PM

928: Follistatin Overexpression in *Sleeping Beauty*-Transposed, *In Vitro* Differentiated Human Plasmablasts: Enhancement of Muscle Mass and Strength in NSG Mice after Adoptive Transfer

Glen Granda, Immusoft, Seattle, WA, United States

PROGRAM SCHEDULE

THURSDAY, MAY 14

All times are listed in Eastern Daylight Time (EDT UTC -4).

ORAL ABSTRACT SESSION 353

3:45 PM - 5:30 PM

Room: Ballroom C

New Techniques in Gene Therapy for Neurological Disorders

Chair: Dan Wang, PhD

3:45 PM – 4:00 PM

930: Monosynaptic Tracing Induced by a Novel AAV Show Appropriate Circuit Integration of Human Dopamine Neurons (in a Rat Model of Parkinson's Disease)

Malin Akerblom, Lund University, Lund, Sweden

4:00 PM – 4:15 PM

931: Selective Intein Degradation for Safe AAV Mediated Large Gene Delivery

Patrizia Tornabene, TIGEM, Pozzuoli, Italy

4:15 PM – 4:30 PM

932: Allele-Specific AAV-Based Silencing of Mutant Ataxin-3 Alleviates Neuropathology and Motor Deficits in Spinocerebellar Ataxia Type 3

Rui Nobre, Center for Neuroscience and Cell Biology, Coimbra, Portugal

4:30 PM – 4:45 PM

933: CRISPR/Cas9-Mediated Excision of ALS-Causing Hexanucleotide Repeat Expansion in C9ORF72

Katharina Meijboom, UMASS Medical School, Worcester, MA, United States

4:45 PM – 5:00 PM

934: Gene Editing of the Ube3a Antisense-Transcript in an Angelman Syndrome Mouse Model

Ralf Schmid, University of Pennsylvania, Philadelphia, PA, United States

5:00 PM – 5:15 PM

935: Treatment of a Mouse Model of ALS by CRISPR Base Editing

Colin Lim, University of Illinois at Urbana-Champaign, Urbana, IL, United States

5:15 PM – 5:30 PM

936: Direct Vagus Nerve Injection of AAV9 as a Treatment Approach for Autonomic Dysfunction in Giant Axonal Neuropathy

Rachel Bailey, University of Texas Southwestern Medical Center, Dallas, TX, United States

PROGRAM SCHEDULE

THURSDAY, MAY 14

All times are listed in Eastern Daylight Time (EDT UTC -4).

ORAL ABSTRACT SESSION 354

3:45 PM - 5:30 PM

Room: 302

Gene Therapies for Hemophilia and Immune Disorders

Chair: Lindsey George, MD

3:45 PM – 4:00 PM

937: Modeling, Optimization and Comparative Efficacy of HSC- and T-cell Based Editing Strategies for Treating Hyper IgM Syndrome

Valentina Vavassori, SR-Tiget, Milan, Italy

4:00 PM – 4:15 PM

938: Production of Genetically Engineered T and NK Cells is Maintained in Humans by Common Long Term Lymphoid Progenitors 15 Years after Loss of Transplanted Hematopoietic Stem Cells

Natalia Izotova, University College of London, London, United Kingdom

4:15 PM – 4:30 PM

939: CRISPR/Cas9 Targeted *MAGT1* Gene Addition into XMEN Patient Hematopoietic Stem Cells and Peripheral Blood T Cells

Julie Brault, NIH, Bethesda, MD, United States

4:30 PM – 4:45 PM

940: AAV Integration Analysis after Long Term Follow Up in Hemophilia a Dogs Reveals the Genetic Consequences of AAV-Mediated Gene Correction

John Everett, Perelman School of Medicine, The University of Pennsylvania, Philadelphia, PA, United States

4:45 PM – 5:00 PM

941: First-In-Human Gene Therapy Study of AAVhu37 Capsid Vector Technology in Severe Hemophilia A: Safety and FVIII Activity Results

John Sheehan, University of Wisconsin–Madison, Madison, WI, United States

5:00 PM – 5:15 PM

942: Engineered Antigen-Expressing CAAR-T Cells Targeting Autoreactive B Cells to ADAMTS13 in the Context of Thrombotic Thrombocytopenic Purpura

Kalpna Parvathaneni, Perelman School of Medicine, Philadelphia, PA, United States

PROGRAM SCHEDULE

THURSDAY, MAY 14

All times are listed in Eastern Daylight Time (EDT UTC -4).

5:15 PM – 5:30 PM

943: AAV Mediated Expression of Activated Factor V (FVa) for Hemophilia

Junjiang Sun, University of North Carolina at Chapel Hill, Chapel Hill, NC, United States

ORAL ABSTRACT SESSION 355

3:45 PM - 5:30 PM

Room: 304 & 306

AAV Vectors Preclinical and Proof-of-Concept Studies in Systemic Diseases

Chair: Miguel Sena-Esteves, PhD

3:45 PM – 4:00 PM

944: PR001 Gene Therapy Increased GCase Activity and Ameliorated GBA1-Associated Disease Phenotypes

Patty Sheehan, Prevail Therapeutics, New York, NY, United States

4:00 PM – 4:15 PM

945: Developing AAV-Mediated Clinically Translatable Gene Therapy for Maple Syrup Urine Disease (MSUD) Caused by BCKDHA Mutations in a Bovine Model

Jiaming Wang, University of Massachusetts Medical School, Worcester, MA, United States

4:15 PM – 4:30 PM

946: A Novel NAGA Variant Designed to be Non-Immunogenic in Humans and Provide Broad Cross-Correction in Fabry Disease

Jolanda Liefhebber, uniQure Biopharma B.V., Amsterdam, Netherlands

4:30 PM – 4:45 PM

947: AAV Gene Immunotherapy Reverses MS-Like Disease Against Multiple Epitopes and in Genetically Diverse Mice

Brad Hoffman, University of Florida College of Medicine, Gainesville, FL, United States

4:45 PM – 5:00 PM

948: Preclinical Gene Replacement Therapy with a New scAAV9/SUMF1 Viral Vector for the Treatment of Multiple Sulfatase Deficiency

Maximiliano Presa, The Jackson Laboratory, Bar Harbor, ME, United States

PROGRAM SCHEDULE

THURSDAY, MAY 14

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5:00 PM – 5:15 PM

949: Efficacy and Safety of CSF- Delivered AVXS-401 in Mice and Non-Human Primates for the Treatment of Friedreich's Ataxia

Martin Fugere, AveXis Research and Development, San Diego, CA, United States

5:15 PM – 5:30 PM

950: Bicistronic AAV Gene Therapy for Tay-Sachs Disease

Toloo Taghian, University of Massachusetts Medical School, Worcester, MA, United States

ORAL ABSTRACT SESSION 356

3:45 PM - 5:30 PM

Room: 309

Tolerance and Vaccine Approaches

Chair: Allison Keeler-Klunk, PhD

3:45 PM -4:00 PM

951: Creating AAV Specific CAR T-Cells and CAR Regulatory T- Cells to Model and Mitigate AAV Immune Responses

Motahareh Arjomandnejad, University of Massachusetts Medical School, Worcester, MA, United States

4:00 PM – 4:15 PM

952: Combination of Rapamycin and Anti-IL-15 to Preserve Transgene Expression and Allow for Re-Administration in Hepatic AAV Gene Transfer

John Butterfield, University of Florida College of Medicine, Gainesville, FL, United States

4:15 PM – 4:30 PM

953: Robust Antigen-Specific Immune Tolerance Can be Achieved Through Platelet-Targeted Gene Therapy Even in a Primed Model via Peripheral Clonal Deletion and Treg Cell Induction

Qizhen Shi, Medical College of Wisconsin, Blood Research Institute, Children's Research Institute, Milwaukee, WI, United States

4:30 PM – 4:45 PM

954: Immunization with a Synthetic DNA Vaccine Encoding the OspA Antigen of *Borrelia burgdorferi* Provides Durable Immunity Against Lyme Disease

Trevor Smith, Inovio, San Diego, CA, United States

PROGRAM SCHEDULE

THURSDAY, MAY 14

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4:45 PM – 5:00 PM

955: Phase I-IIA Study of GLS-5300 Vaccine for Mers-Cov: Preliminary Results Demonstrate B and T Cell Immune Response Elicited in Lower-Dose 2- and 3-Vaccination Intradermally Administered Regimens
Christine Roberts, GeneOne Life Science, Seoul, Korea, Republic of

5:00 PM – 5:15 PM

956: Profiling the CD8 Response Following Liver Directed AAV Gene Therapy
Geoffrey Keeler, University of Florida, Gainesville, FL, United States

5:15 PM – 5:30 PM

957: Engineering of an Enhanced DNA-Based Immunotherapeutic Against *Neisseria gonorrhoeae*
Elizabeth Parzych, The Wistar Institute, Philadelphia, PA, United States

ORAL ABSTRACT SESSION 357

3:45 PM - 5:30 PM

Room: 310

Genome Editing in Inborn Errors of Metabolism

Chair: Dwight Koeberl, MD, PhD

3:45 PM – 4:00 PM

958: AAV-Mediated Homology-Independent Targeted Integration in Newborn Hepatocytes Corrects Mucopolysaccharidosis Type VI
Manel Llado Santaaularia, Fondazione Telethon, Roma, Italy

4:00 PM – 4:15 PM

959: Selective Expansion of Gene-Targeted Hepatocytes In Vivo Leads to Therapeutic Levels of Transgene Expression
Anne Vonada, Oregon Health and Science University, Portland, OR, United States

4:15 PM – 4:30 PM

960: *In vivo* Genome Editing at the *Albumin* Locus Corrects Two New Mouse Models of Methylmalonic Acidemia (MMA) Caused by Methylmalonyl-CoA Mutase Deficiency (Mmut)
Jessica Schneller, National Institutes of Health, Bethesda, MD, United States

PROGRAM SCHEDULE

THURSDAY, MAY 14

All times are listed in Eastern Daylight Time (EDT UTC -4).

4:30 PM – 4:45 PM

961: Genomic Instability and Tumorigenesis are Long-Term Effects of Therapeutic CRISPR/Cas9 Genome Editing in Hereditary Tyrosinemia Type I

Nika Furey, Duke University, Durham, NC, United States

4:45 PM – 5:00 PM

962: Crispr/Cas9-Mediated Genome Editing Z-Allele Mutation in Hepatocytes of Alpha-1 Antitrypsin Pizz Ferrets Using Adenovirus-Associated Virus

Xiaoming Liu, The University of Iowa, Iowa City, IA, United States

5:00 PM – 5:15 PM

963: Treatment of Juvenile Mice with Methylmalonic Acidemia (MMA) by Targeted Integration of *MMUT* into *Albumin* Using a Promoterless AAV Vector

Leah Venturoni, NIH, Bethesda, MD, United States

5:15 PM – 5:30 PM

964: *Ex Vivo* Editing of Human Hematopoietic Stem Cells for Erythroid-Specific Expression of Therapeutic Proteins

Giulia Pavani, Genethon, Evry, France

ORAL ABSTRACT SESSION 358

3:45 PM - 5:30 PM

Room: 311

Vector and Cell Engineering, Production or Manufacturing IV

Chair: Maritza Mcintyre, PhD

3:45 PM – 4:00 PM

965: Genome-Wide CRISPR Screen in HEK293 Identifies Putative Cellular Restriction Factors for AAV Manufacturing

Anna Maurer, Grousbeck Gene Therapy Center, Mass Eye and Ear, Harvard Medical School & The Broad Institute of Harvard and MIT, Boston, MA, United States

4:00 PM – 4:15 PM

966: CRISPR-Based High-Throughput Screening Reveals Host Factor SKA2 and ITPRIP Promoting AAV Manufacturing

Hyuncheol Lee, University of California, Berkeley, Berkeley, CA, United States

PROGRAM SCHEDULE

THURSDAY, MAY 14

All times are listed in Eastern Daylight Time (EDT UTC -4).

4:15 PM – 4:30 PM

967: CRISPR Screening Identifies Gene Knockouts That Improve Lentiviral Vector Titers in HEK293 Cells

Brian Iaffaldano, FDA, White Oak, MD, United States

4:30 PM – 4:45 PM

968: Novel Strategy for Generating rAAV Vectors Using Pseudorabies Virus

Oana Maier, University of Pennsylvania, Philadelphia, PA, United States

4:45 PM – 5:00 PM

969: Exploring Optimal REP Gene Expression Levels for the Generation of a Stable AAV Packaging Cell Line

Lovro Jalšić, National Research Council Canada, Montréal, QC, Canada

5:00 PM – 5:15 PM

970: rAAV for Tumor Therapy Using Transcriptional and Translational Control of Suicide Gene Expression Purified by a Newly Developed Affinity Chromatography Based on the PKD Domains of AAVR

Kathrin Teschner, Bielefeld University, Bielefeld, Germany

5:15 PM – 5:30 PM

971: Lac Repressor Inducible Control of AAV Capsid Protein Ratios and Decoupling VP1, VP2 from VP3 in the Sf9 insect cell/Baculovirus Expression Vector (BEV) Platform

Jeffrey Slack, Voyager Therapeutics, Cambridge, MA, United States

ORAL ABSTRACT SESSION 359

3:45 PM - 5:30 PM

Room: 312

Pharmacology/Toxicology Studies

Chair: Abraham Scaria, PhD

3:45 PM – 4:00 PM

972: Gene Transduction, Hematopoietic Stem Cells (HSCs) Engraftment, and Red Blood Cell (RBC) Physiology in Sickle Cell Disease (SCD) Gene Therapy

Francis Pierciey Jr., bluebird bio, Inc., Cambridge, MA, United States

4:00 PM – 4:15 PM

973: InGeTox - A Modular Vector Safety Prediction System

Marco Zahn, GeneWerk GmbH, Heidelberg, Germany

PROGRAM SCHEDULE

THURSDAY, MAY 14

All times are listed in Eastern Daylight Time (EDT UTC -4).

4:15 PM – 4:30 PM

974: A Single Administration of AAV5-hFIX in Newborn, Juvenile and Adult Mice Leads to Stable hFIX Expression up to 18 Months after Dosing

Lisa Spronck, UniQure Biopharma B.V., Amsterdam, Netherlands

4:30 PM – 4:45 PM

975: *In Vitro* Transformation Assays for Non-Clinical Safety Assessment of CRISPR/Cas9 Genome-Edited Cells

Myriam Lemmens, Novartis, Basel, Switzerland

4:45 PM – 5:00 PM

976: Preclinical Assessment of an In Vivo Lentiviral Vector Cure for Hereditary Tyrosinemia Type 1

Robert Kaiser, Children's Hospital Minnesota, Minneapolis, MN, United States

5:00 PM – 5:15 PM

977: Physiologically-Based Pharmacokinetic Modeling for Adeno-Associated Virus Gene Therapy

Nicholas van der Munnik, Biogen Inc., Cambridge, MA, United States

5:15 PM – 5:30 PM

978: XT-150 Human IL-10v Plasmid DNA: Results from 6-month GLP Safety Study and Efficacy After Intra-Articular Administration to Companion Dogs with OA

Raymond Chavez, Xalud Therapeutics, Inc., Berkeley, CA, United States

EXHIBIT HALL

Open 24 hours online

POSTER SESSION III

Open 24 hours on May 14

5:30 PM - 6:30 PM

Scheduled presenters requested to be available for live Q&A

PROGRAM SCHEDULE

FRIDAY, MAY 15

All times are listed in Eastern Daylight Time (EDT UTC -4).

ASGCT BUSINESS MEETING

7:30 AM - 8:00 AM

Room: 312

Business Meeting

ORAL ABSTRACT SESSION 399

8:00 AM - 9:45 AM

Room: 310

Clinical Trials Spotlight Symposium

Chairs: Aravind Asokan, PhD, and Jennifer Adair, PhD

8:00 AM - 8:15 AM

617. Safety and Tolerability of PF-06939926 in Ambulatory Boys with Duchenne Muscular Dystrophy: A Phase 1b Multicenter, Open-Label, Dose Ascending Study

Michael Binks, Pfizer Inc, Cambridge, MA, United States

8:15 AM – 8:30 AM

1298: Resolution of Sickle Cell Disease (SCD) Manifestations in Patients Treated with Lentiglobin Gene Therapy: Updated Results from the Phase 1/2 HGB-206 Group C Study

John Tisdale, NHLBI, National Institutes of Health, Bethesda, MD, United States

8:30 AM – 8:45 AM

1299: First-In-Human Gene Therapy for Tay-Sachs Disease: Report of Two Infants Treated on an Expanded Access Clinical Trial of rAAVrh8-HexA/HexB (AXO-AAV-GM2)

Terence Flotte, University of Massachusetts Medical School, Worcester, MA, United States

8:45 AM – 9:00 AM

1300: Lentiviral Gene Therapy with Autologous Hematopoietic Stem and Progenitor Cells (HSPCs) for the Treatment of Severe Combined Immune Deficiency Due to Adenosine Deaminase Deficiency (ADA-SCID): Two Year Follow-Up Results

Donald Kohn, University of California, Los Angeles, Los Angeles, CA, United States

PROGRAM SCHEDULE

FRIDAY, MAY 15

All times are listed in Eastern Daylight Time (EDT UTC -4).

9:00 AM – 9:15 AM

1301: Natural Killer T Cells Expressing a GD2-CAR and IL-15 for Children with Neuroblastoma - A First-In-Human Phase 1 Trial

Andras Heczey, Baylor College of Medicine, Houston, TX, United States

9:15 AM – 9:30 AM

1302: A Phase 2 Trial of MGTA-456 Cell Therapy Demonstrates Rapid and Durable Long-Term Improvement in Disease-Specific Outcomes in Inherited Metabolic Disease (IMD) Patients

John Wagner, University of Minnesota, Minneapolis, MN, United States

9:30 AM – 9:45 AM

1303: CoupledCAR™ Technology for Treating Thyroid and Colorectal Cancer

Lei Xiao, Innovative Cellular Therapeutics, Shanghai, China

SCIENTIFIC SYMPOSIUM 400

8:26 AM - 9:45 AM

Room: 311

Challenges and Future Directions in Cardiovascular Gene and Cell Therapy - Organized by the Cardiovascular Gene & Cell Therapy Committee

CHAIR: Costanza Emanuela, FAHA

SPEAKERS

8:26 AM – 8:52 AM

Luk H. Vandenberghe, PhD. Harvard, Broad Institute, Mass Eye and Ear, Boston, MA

Rational Design of AAV for Tissue Tropism and Pharmacological Control

8:52 AM – 9:18 AM

H. Kirk Hammond, MD. UCSD and VA San Diego, San Diego, CA

Urocortin 2 Gene Transfer: Effects on Age-Related Heart Failure with Preserved EF and Diabetes

9:18 AM – 9:45 AM

Duncan Stewart, MD, FRCPC. Ottawa Hospital Research Institute, Ottawa, ON, Canada

Angiogenic Cell Therapy for Pulmonary Arterial Hypertension: Go with the Flow!

PROGRAM SCHEDULE

FRIDAY, MAY 15

All times are listed in Eastern Daylight Time (EDT UTC -4).

SCIENTIFIC SYMPOSIUM 401

8:00 AM - 9:45 AM

Room: Ballroom A

Accessing Virus Vectors for Non-clinical and Clinical Gene Therapy Studies - Organized by the Bio-Industry Committee

CO-CHAIRS: Kinnari Patel, MBA and Richard Snyder, PhD

SPEAKERS

8:00 AM – 8:20 AM

J. Fraser Wright, PhD. Stanford, Philadelphia, PA

For-Profit and Non-Profit Cooperative Approaches

8:20 AM – 8:40 AM

PJ Brooks, PhD. NIH National Center for Advancing Translational Sciences, Bethesda, MD

NIH Government Initiatives

8:40 AM – 9:00 AM

Catherine Stehman-Breen, MD. Obsidian Therapeutics, Cambridge, MA

Contract Manufacturing, Consulting and Internal Corporate Efforts

9:00 - 9:45 AM

Panel Discussion

SCIENTIFIC SYMPOSIUM 402

8:00 AM - 9:45 AM

Room: 312

Challenges and Possible Solutions in Infectious Diseases - Organized by the Infectious Diseases and Vaccines Committee

CO-CHAIRS: Hildegund Ertl, MD and Matti Sallberg, DDS

SPEAKERS

8:00 AM – 8:26 AM

Hildegund CJ. Ertl, MD. Wistar Institute, University of Pennsylvania, Philadelphia, PA

Introductory Talk On What We Worry About in the Future and What We Need To Do.

PROGRAM SCHEDULE

FRIDAY, MAY 15

All times are listed in Eastern Daylight Time (EDT UTC -4).

8:26 AM - 8:52 AM

Scott G. Kitchen, PhD. UCLA AIDS Institute, David Geffen School of Medicine at UCLA, Los Angeles, CA

Adoptive Peripheral T Cell Versus Hematopoietic Stem Cell (HSC)-based HIV-specific CART Cell Therapy Approaches In Vivo

8:52 AM – 9:18 AM

Antonio Bertoletti, MD. Duke-Nus Medical School, Singapore

Challenges of CAR- and TCR-T Cell-based Therapy for Chronic Hepatitis B

9:18 AM – 9:45 AM

Ronald C. Desrosiers, PhD. University of Miami Miller School of Medicine, Miami, FL

Long-term AAV-mediated Delivery of Potent Broadly-Neutralizing Antibodies for the Treatment and Prevention of AIDS Virus Infection

SCIENTIFIC SYMPOSIUM 403

8:00 AM - 9:45 AM

Room: Ballroom C

Gene and Cell Therapeutic Strategies to Treat Disorders of the CNS – An International Perspective - Organized by the International Committee

CO-CHAIR: Anne Galy, PhD

SPEAKERS

8:00 AM - 8:35 AM

Nathalie Cartier-Lacave, PhD. INSERM, Paris, France

AAV-Based Strategies to Treat CNS Disorders: Focus on Alzheimer's Disease

8:35 AM - 9:10 AM

Gregory Stewart, PhD. Axovant, New York, NY

AXO-Lenti-PD: A Trans-Atlantic Collaboration on Gene Therapy for Parkinson's Disease

9:10 AM - 9:45 AM

Maria Bernardo, MD, PhD. San Raffaele Telethon Institute, Milan, Italy

HSC-Based Strategies to Treat CNS Disorders: Focus on Hurler/MPS1H

PROGRAM SCHEDULE

FRIDAY, MAY 15

All times are listed in Eastern Daylight Time (EDT UTC -4).

SCIENTIFIC SYMPOSIUM 404

8:00 AM - 9:45 AM

Room: Ballroom B

Gene Editing: Emerging New Technologies and Unbiased Assessment of On and Off Target Genome Alteration - Organized by the Genome Editing Committee

CHAIR: T.J. Cradick, PhD

SPEAKERS

8:00 AM – 8:26 AM

Samuel H. Sternberg, PhD. Columbia University, New York, NY

Transposon-encoded CRISPR-Cas Systems Direct RNA-guided DNA Integration

8:26 AM – 8:52 AM

Nicole Gaudelli, PhD. Beam Therapeutics, Cambridge, MA

ABE8: Superior Adenine Base Editors with Expanded Targeting Range and Therapeutic Applications

8:52 AM – 9:18 AM

Toni Cathomen, PhD. Medical Center - University of Freiburg, Freiburg, Germany

Large-scale Genomic Alterations upon Gene Editing

9:18 AM – 9:45 AM

John Anthony Zuris, PhD. Editas Medicine, Cambridge, MA

Highly Efficient Multi-Gene Knockout and Transgene Knock-in Using CRISPR-Cas12a in Induced Pluripotent Stem Cells for the Generation of Engineered Cell Immunotherapies

SCIENTIFIC SYMPOSIUM 405

8:00 AM - 9:45 AM

Room: 304 & 306

Stem Cell-derived Organoids - Organized by the Stem Cell Committee

CO-CHAIRS: Eirini Papapetrou, PhD and Bakhos Tannous, PhD

SPEAKERS

8:00 AM – 8:26 AM

Bakhos Tannous, PhD. Massachusetts General Hospital, Boston, MA

Patient-Derived Glioma Models: From Patients to Dish to Animals

PROGRAM SCHEDULE

FRIDAY, MAY 15

All times are listed in Eastern Daylight Time (EDT UTC -4).

8:26 AM – 8:52 AM

Giorgia Quadrato, PhD. University of Southern California, Los Angeles, CA
Modeling Human Brain Development and Disease at Single Cell Resolution with Organoids

8:52 AM – 9:18 AM

Ya-Wen Chen, PhD. University of Southern California, Los Angeles, CA
Generation of a Three-Dimensional Lung Organoid From Human Pluripotent Stem Cells and Its Applications

9:18 AM – 9:45 AM

Ryuji Morizane, MD, PhD. Massachusetts General Hospital, Boston, MA
Kidney Organoids for Disease Modeling

SCIENTIFIC SYMPOSIUM 406

8:00 AM - 9:20 AM

Room: Auditorium

The Long-term View: Extended follow up of Patients in Pivotal Gene Therapy Trials

SPEAKERS

8:00AM – 8:26 AM

Jerry Mendell, MD. Gene Therapy Center Nationwide, Columbus, OH
Onasemnogene Apeparovovec-xioi (ZOLGENSMA) in SMA 1 Patients

8:26 AM – 8:52 AM

Alessandro Aiuti, MD, PhD. IRCCS San Raffaele Scientific Institute for Gene Therapy, Milan, Italy
Long-term Outcomes of Strimvelis (Ex Vivo Gene Therapy for ADA-SCID)

8:52 AM – 9:18 AM

Richard A. Colvin, MD, PhD. bluebird bio, Cambridge, MA
Long-term Outcomes for the Treatment of Patients with Transfusion-dependent β -thalassemia with Betibeglogene Autotemcel (LentiGlobin) Gene Therapy

PROGRAM SCHEDULE

FRIDAY, MAY 15

All times are listed in Eastern Daylight Time (EDT UTC -4).

PLENARY SESSION 410

8:00 AM - 9:30 AM

Room: 309

2019 Career Development Award Recipients

SPEAKERS

Diana Bharucha-Goebel, MD. NIH & Children's National Health System, Bethesda, MD

A Systematic Clinical Analysis of the Immunologic Effects of Intrathecal AAV9 Mediated Gene Transfer Targeting the Central Nervous System

Benjamin Kleinstiver, PhD. Massachusetts General Hospital, Boston, MA

Enhancing the Efficacy and Safety of Cell Engineering with Novel CRISPR Enzymes

Nicole K. Paulk, PhD. University of California San Francisco, San Francisco, CA

Characterizing the Proteomic Landscape of rAAV Vectors for Human Liver Gene Therapies

Sandhya Sharma. Baylor College of Medicine, Houston, TX

Generation of Memory Cell Enriched, Broad Repertoire Antigen Specific T Cells for EBV Positive Malignancies

Katie M. Stiles. Weill Cornell Medical College, New York, NY

Modification of the AAV Expression Cassette to Enhance Transgene Translation

Jiehua Zhou, Ph.D.. Beckman Research Institute of City of Hope, Monrovia, CA

Exploring Functional RNAs: Gene Therapy & Beyond

BREAK

9:45 AM - 10:15 AM

PROGRAM SCHEDULE

FRIDAY, MAY 15

All times are listed in Eastern Daylight Time (EDT UTC -4).

ORAL ABSTRACT SESSION 420

10:15 AM - 12:00 PM

Room: Auditorium

AAV Vectors - Clinical Studies I

Chair: Steven Gray, PhD

10:15 AM – 10:30 AM

1305: RGX-314 Ocular Gene Therapy: Overview of Phase I/IIa Ongoing Trial for Neovascular Age-Related Macular Degeneration (nAMD) and Future Directions

Olivier Danos, REGENXBIO, Rockville, MD, United States

10:30 AM – 10:45 AM

1306: AAV8-Mediated Liver-Directed Gene Therapy as a Potential Therapeutic Option in Adults with Glycogen Storage Disease Type Ia (GSDIa): Results from a Phase 1/2 Clinical Trial

David Weinstein, University of Connecticut, Farmington, CT, United States

10:45 AM – 11:00 AM

1307: First China International Gene Therapy Study in Leber's Hereditary Optic Neuropathy

Alvin Luk, Neurophth Therapeutics Inc, Wuhan, China

11:00 AM – 11:15 AM

1308: A Systematic Review of Clinical Safety and Efficacy of AAV Gene Therapies

Dmitry Kuzmin, 4BIO Capital, London, United Kingdom

11:15 AM – 11:30 AM

1309: Clearance of Vector DNA from Bodily Fluids in Patients with Severe or Moderate-Severe Hemophilia B Following Systemic Administration of AAV5-hFIX and AAV5-hFIX Padua

Eileen Sawyer, UniQure Biopharma B.V., Lexington, MA, United States

11:30 AM – 11:45 AM

1310: Improved Motor Function in Children with AADC Deficiency Treated with Eladocagene Exuparvovec (PTC-AADC): Interim Findings from a Phase 2 Trial

Paul Wuh-Liang Hwu, National Taiwan University Hospital, Taipei, Taiwan

PROGRAM SCHEDULE

FRIDAY, MAY 15

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11:45 AM – 12:00 PM

1311: Improved Delivery in a New Clinical Trial of AAV2-BDNF Gene Therapy for Alzheimer's Disease

Michael Castle, University of California, San Diego, La Jolla, CA, United States

ORAL ABSTRACT SESSION 421

10:15 AM - 12:00 PM

Room: Ballroom A

AAV Vectors Preclinical and Proof-of-Concept Studies in CNS Disorders

Chair: Allison Bradbury, PhD

10:15 AM – 10:30 AM

1312: PR006, an AAV Gene Therapy Vector Expressing Progranulin, Improved FTD-GRN Phenotypes *In Vitro* and *In Vivo*

Laura Heckman, Prevail Therapeutics, New York, NY, United States

10:30 AM – 10:45 AM

1313: Gene Therapy for the Progressive Visual Pathology in Late Infantile Neuronal Ceroid Lipofuscinosis Patients Receiving Enzyme Replacement Therapy

Luis Tecedor, Childrens Hospital of Philadelphia, Philadelphia, PA, United States

10:45 AM – 11:00 AM

1314: A Transient Burst of Transgene Expression Promotes Regeneration of Mature Vestibular Hair Cells

Joseph Burns, Decibel Therapeutics, Boston, MA, United States

11:00 AM – 11:15 AM

1315: In Vivo Postnatal Base Editing Restores Sensory Transduction and Transiently Rescues Hearing in a Mouse Model of Recessive Deafness

Wei-Hsi (Ariel) Yeh, Merkin Institute of Transformative Technologies in Healthcare, Broad Institute of Harvard and MIT, Cambridge, MA, United States

11:15 AM – 11:30 AM

1316: Investigational AAV Gene Therapy LYS-GM101 for GM1-Gangliosidosis

Ralph Laufer, Lysogene, Neuilly sur Seine, France

PROGRAM SCHEDULE

FRIDAY, MAY 15

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11:30 AM – 11:45 AM

1317: Preclinical Results in Rodents Strongly Support Clinical Evaluation of scAAV9/MFSD8 as a Potential Gene Therapy for CLN7 Patients

Xin Chen, UTSW Medical Center, Dallas, TX, United States

11:45 AM – 12:00 PM

1318: Gene Therapy for Tuberous Sclerosis Complex Type 2 in a Mouse Model by Delivery of AAV9 Encoding a Condensed Form of Tuberin

Shilpa Prabhakar, Massachusetts General Hospital, Charlestown, MA, United States

ORAL ABSTRACT SESSION 422

10:15 AM - 11:30 AM

Room: Ballroom B

Gene Regulation and Delivery Technologies

Chair: Benjamin Kleinstiver, PhD

10:15 AM – 10:30 AM

1319: In Situ Gene Editing of HSPCs by TAGE, a New Biologics Class of Targeted Cas Ribonucleoproteins

Rina Mepani, Spotlight Therapeutics Inc., Hayward, CA, United States

10:30 AM – 10:45 AM

1320: Crispr Cas9 Mediated Gene Knockout of KLKB1 to Treat Hereditary Angioedema

Jessica Seitzer, Intellia Therapeutics Inc., Cambridge, MA, United States

10:45 AM – 11:00 AM

1321: Engineering the DNA-Binding Specificity of CTCF to Restore Enhancer-Mediated Gene Expression in Human Cells

Rebecca Cottman, Massachusetts General Hospital, Charlestown, MA, United States

11:00 AM – 11:15 AM

1322: In Vivo HSC Gene Therapy with Base Editors Allows for Efficient Reactivation of Fetal Gamma-Globin in Beta-YAC Mice

Chang Li, University of Washington, Seattle, WA, United States

11:15 AM – 11:30 AM

1324: AAV-CRISPR Gene Editing is Negated by Pre-Existing Immunity to Cas9

Ang Li, Rice University, Houston, TX, United States

PROGRAM SCHEDULE

FRIDAY, MAY 15

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ORAL ABSTRACT SESSION 423

10:15 AM - 11:45 AM

Room: Ballroom C

Physical, Chemical and Exosomal Gene Delivery

Chair: Matthew Wilson, MD, PhD

10:15 AM – 10:30 AM

1326: Assessment of Clinical Feasibility of Image-Guided Liver Specific Hydrodynamic Gene Transfer in Non-Human Primates

Kenya Kamimura, Niigata University, Niigata, Japan

10:30 AM – 10:45 AM

1327: A Novel Serum-Insensitive Fusogenic Polyvalent Cationic Lipid for *In Vivo* mRNA Delivery

Franklin Kostas, Novellus, Inc., Cambridge, MA, United States

10:45 AM – 11:00 AM

1328: Safe and Effective Delivery of Nucleic Acids Using LNPs Formulated with Fusion-Associated Small Transmembrane Proteins

Arun Raturi, Entos Pharmaceuticals, Edmonton, AB, Canada

11:00 AM – 11:15 AM

1329: LOX Biphasic Gene Electrotransfer Enhances Extruded Collagen Graft Integration Subcutaneously in a Small Animal Model

Anna Bulysheva, Old Dominion University, Norfolk, VA, United States

11:15 AM – 11:30 AM

1332: A Versatile Platform for Precision Exosome Engineering

Kevin Dooley, Codiak BioSciences, Cambridge, MA, United States

11:30 AM – 11:45 AM

1331: Engineering Exosomes with Altered Cellular Tropism for Targeted Payload Delivery *In Vivo*

Monique Kauke, Codiak BioSciences, Cambridge, MA, United States

PROGRAM SCHEDULE

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ORAL ABSTRACT SESSION 424

10:15 AM - 12:00 PM

Room: 302

Cardiovascular and Pulmonary Diseases

Chair: Uta Griesenbach, PhD

10:15 AM – 10:30 AM

1333: Lineage-Tracing Glandular Stem Cell Compartments in the Ferret Lung

Xiaoming Liu, The University of Iowa, Iowa City, IA, United States

10:30 AM – 10:45 AM

1334: Insertion of the CFTR cDNA in the Endogenous Locus in Airway Stem Cells Using CRISPR/Cas9 Restores CFTR Function to Wild-Type Levels in Differentiated Epithelia

Sriram Vaidyanathan, Stanford University, Stanford, CA, United States

10:45 AM – 11:00 AM

1335: A Novel AAV Variant that Exclusively Targets the Heart via a Potential Post-Entry Mechanism

Kei Adachi, Oregon Health & Science University, Portland, OR, United States

11:00 AM – 11:15 AM

1336: Directed Evolution of AAV Targeting Lung Epithelia Using Aerosol Delivery Identifies 4D-A101, a Variant Demonstrating Robust Gene Delivery in Non-Human Primates

Melissa Kotterman, 4D Molecular Therapeutics, Emeryville, CA, United States

11:15 AM – 11:30 AM

1337: Development of Receptor-Targeted Nanocomplexes for *In Vivo* Delivery of CRISPR/Cas9 as a Potential Therapy for Cystic Fibrosis

Amy Walker, UCL Great Ormond Street Institute of Child Health, London, United Kingdom

11:30 AM – 11:45 AM

1338: Liver-Directed Gene Therapy for Lung Disease in Alpha1 Antitrypsin Deficiency

Marina Zieger, University of Massachusetts Medical School, Worcester, MA, United States

PROGRAM SCHEDULE

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11:45 AM – 12:00 PM

1339: Improved Cardiac AAV Gene Transfer Efficacy in Pigs Using Temporal Mechanical Support

Kiyotake Ishikawa, Mount Sinai, New York, NY, United States

ORAL ABSTRACT SESSION 425

10:15 AM - 12:00 PM

Room: 304 & 306

Cancer Immunotherapy: Clinical Results

Chair: Monica Casucci, PhD

10:15 AM – 10:30 AM

1340: KTE-X19, an Anti-CD19 Chimeric Antigen Receptor (CAR) T Cell Therapy, in Patients (Pts) with Relapsed/Refractory Mantle Cell Lymphoma (R/R MCL): Results from Phase 2 of ZUMA-2

Michael Wang, MD Anderson Cancer Center, Houston, TX, United States

10:30 AM – 10:45 AM

1341: Exhaustion and Activation Status in Apheresed T Cells Correlate with Response to Anti-BCMA CAR T Cell Therapy in Myeloma

Iulian Pruteanu-Malinici, Novartis, Cambridge, MA, United States

10:45 AM – 11:00 AM

1342: Identification and Validation of Predictive Biomarkers to CD19- and BCMA-Specific CAR T-Cell Responses in Apheresed T-Cells

Iulian Pruteanu-Malinici, Novartis, Cambridge, MA, United States

11:00 AM – 11:15 AM

1343: Correlative Analysis from CRB-402: An Ongoing Phase 1 Clinical Study of bb21217 Anti-BCMA CAR T Cell Therapy

Olivia Finney, bluebird bio, Cambridge, MA, United States

11:15 AM – 11:30 AM

1344: A Comparison of 2-Year Outcomes in ZUMA-1 (Axicabtagene Ciloleucel; Axi-Cel) and SCHOLAR-1 in Patients (Pts) with Refractory Large B Cell Lymphoma (LBCL)

Sattva Neelapu, MD Anderson Cancer Center, Houston, TX, United States

PROGRAM SCHEDULE

FRIDAY, MAY 15

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11:30 AM – 11:45 AM

1345: Autologous CD34+-Enriched Hematopoietic Progenitor Cells Genetically Modified for Human Interferon-Alpha2 (Temferon), Rapidly Engraft and Mature in Patients with Glioblastoma Multiforme (TEM-GBM_001 Study)

Bernhard Gentner, IRCSS San Raffaele Scientific Institute, Milan, Italy

11:45 AM - 12:00 PM

1346. Earlier Steroid Use with Axicabtagene Ciloleucel (Axi-Cel) in Patients (Pts) with Relapsed/Refractory Large B Cell Lymphoma (R/R LBCL)

Marie Jose Kersten, on behalf of HOVON/LLPc (Lunenburg Lymphoma Phase I/II Consortium), Lunenburg, Netherlands

ORAL ABSTRACT SESSION 426

10:15 AM - 12:00 PM

Room: 309

Gene Therapy for Inborn Errors of Metabolism: New Approaches

Chair: Cary Harding, MD

10:15 AM – 10:30 AM

1347: ImmTOR™ Tolerogenic Nanoparticles Enable Gene Therapy of Mice with Maternally-Transferred Anti-AAV Antibodies in a Mouse Model of Methylmalonic Acidemia

Petr Ilyinskii, Selecta Biosciences, Watertown, MA, United States

10:30 AM – 10:45 AM

1348: The First Viable Mouse Model of Combined Methylmalonic Acidemia and Homocystinuria, *cb1C* Type Displays a Sustained Response to a Single Dose of AAV Gene Therapy and is Comparable to Chronic Hydroxocobalamin Administration

Stefanos Koutsoukos, National Human Genome Research Institute, Bethesda, MD, United States

10:45 AM – 11:00 AM

1349: Developing and Evaluating Second-Generation AAV Vectors for Liver-Directed Gene Therapy of Familial Hypercholesterolemia in Nonhuman Primates

Lili Wang, University of Pennsylvania, Philadelphia, PA, United States

PROGRAM SCHEDULE

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11:00 AM – 11:15 AM

1350: Intermittent mRNA Therapy for Arginase Deficiency Results in Prevention of Dysmyelination of the Corticospinal Tract

Suhail Khoja, University of California Los Angeles, Los Angeles, CA, United States

11:15 AM – 11:30 AM

1351: AAV Gene Therapy in Mucopolysaccharidosis IVA Murine Models

Shunji Tomatsu, Nemours/Alfred I. duPont Hospital for Children, Wilmington, DE, United States

11:30 AM – 11:45 AM

1352: Base Editing of an Allosteric Site within Serpina1 Yields Improved Phenotypes in Models of Alpha-1 Antitrypsin Deficiency

Vivek Chowdhary, UMass Medical School, Worcester, MA, United States

11:45 AM – 12:00 PM

1353: In Vivo Base Editing to Correct a Murine Model of Mucopolysaccharidosis Type IH

Mark Osborn, University of Minnesota, Minneapolis, MN, United States

ORAL ABSTRACT SESSION 427

10:15 AM - 12:00 PM

Room: 310

Technical Advances in Cell Therapies

Chair: Graca Almeida-Porada, MD, PhD

10:15 AM – 10:30 AM

1354: Genetically Corrected iPSC-Derived Neural Stem Cell Grafts Deliver Naglu-IGFII Fusion Protein to Affect CNS Disease in Sanfilippo B Mice

Yewande Pearse, The Lundquist Institute at Harbor-UCLA, Los Angeles, CA, United States

10:30 AM – 10:45 AM

1355: Sleeping Beauty IDUA Transposed Human Plasma Cells for Long-Term Treatment of an Immunodeficient Murine Model of Mucopolysaccharidosis Type I

Christiane Hampe, Immusoft, Seattle, WA, United States

10:45 AM – 11:00 AM

1356: Allotransplantation of Functional Retinal Ganglion Cells for Glaucoma

Julia Oswald, Schepens Eye Research Institute, Boston, MA, United States

PROGRAM SCHEDULE

FRIDAY, MAY 15

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11:00 AM – 11:15 AM

1357: Human Transcription Factor Library Based Cell Type Engineering
Volker Busskamp, University of Bonn, Bonn, Germany

11:15 AM – 11:30 AM

1358: Prenatal Therapy with Placental Cells Transduced with a Lentiviral Vector Encoding a Bioengineered fVIII Transgene Results in Sustained Therapeutic fVIII Levels and in Product Specific Tolerance
Martin Rodriguez, Wake Forest Institute for Regenerative Medicine, Winston Salem, NC, United States

11:30 AM – 11:45 AM

1359: Combined Gene and Cell Therapy for the Treatment of Hemophilia A within an Implantable Therapeutic Device
Alessia Cucci, Università del Piemonte Orientale, Novara, Italy

11:45 AM – 12:00 PM

1360: Privileged Cell-Material Interactions Through Engineered Synthetic Receptors
Jonathan Brunger, Vanderbilt University, Nashville, TN, United States

ORAL ABSTRACT SESSION 428

10:15 AM - 11:45 AM

Room: 311

Vector and Cell Engineering, Production or Manufacturing V

Chair: Johannes van der Loo, PhD

10:15 AM – 10:30 AM

1361: Single Molecule Real-Time Sequencing Reveals Higher Genome Heterogeneity in AAV Vectors Produced from Insect Cells Compared to Mammalian Cells
P Tai, University of Massachusetts, Medical School, Worcester, MA, United States

10:30 AM – 10:45 AM

1362: SSV-Seq 2.0, an Optimized PCR-Free Method for the High-Throughput Sequencing of Adeno Associated Viral Vector Genomes
Magalie Penaud-Budloo, University of Nantes, CHU of Nantes, INSERM UMR1089, Nantes, France

PROGRAM SCHEDULE

FRIDAY, MAY 15

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10:45 AM – 11:00 AM

1363: Characterizing Next-Generation Baculovirus Transduction Processes - A Quality by Design-Based Approach for AAV Manufacturing

Benjamin Blaha, uniQure, Amsterdam, Netherlands

11:00 AM – 11:15 AM

1364: Measuring the Concentration of Adeno-Associated Virus (AAV) with Multi-Angle Dynamic Light Scattering (MADLS) Using the New Zetasizer™ Ultra

Jonathan Mehtala, Malvern Panalytical, Westborough, MA, United States

11:15 AM - 11:30 AM

1367: Process Analytical Technology Strategy for Lentiviral Manufacture

Marc-Olivier Baradez, Cell and Gene Therapy Catapult, London, United Kingdom

11:30 AM – 11:45 AM

1366: Stability Under Stress of AT132, an AAV-Based Gene Therapy Drug Product for Systemic Administration to Patients Affected by X-Linked Myotubular Myopathy

Emdadul Haque, Audentes Therapeutics, South San Francisco, CA, United States

ORAL ABSTRACT SESSION 429

10:15 AM - 12:00 PM

Room: 312

Cancer - Oncolytic Viruses

Chair: Evanthia Galanis, MD

10:15 AM – 10:30 AM

1368: A Measles Virus Vector Pseudotyped with CD46-Retargeted Canine Distemper Envelope Achieves Oncolysis in the Presence of Measles-Immune Human Serum

Miguel Muñoz-Alía, Mayo Clinic, Rochester, MN, United States

10:30 AM – 10:45 AM

1369: ONCR-177, an Oncolytic HSV-1 Designed to Potently Activate Systemic Anti-Tumor Immunity

Christophe Queva, Oncorus, Inc., Cambridge, MA, United States

PROGRAM SCHEDULE

FRIDAY, MAY 15

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10:45 AM – 11:00 AM

1370: Phase I/II Trial of Adipose Tissue Derived Mesenchymal Stem Cell Delivery of a Measles Virus Strain Engineered to Express the Sodium Iodide Symporter in Ovarian Cancer Patients

Evanthia Galanis, Mayo Clinic, Rochester, MN, United States

11:00 AM – 11:15 AM

1371: A Novel ADP-Deleted, NIS-Expressing Adenovirus: Theranostic Applications and Clinical Development

Matthew Robertson, University of Minnesota, Minneapolis, MN, United States

11:15 AM – 11:30 AM

1372: Immunostimulatory Bacterial Antigen-Armed Oncolytic Measles Virotherapy Significantly Increases the Potency of aPD1 Checkpoint Therapy to Eradicate Glioblastoma

Eleni Panagioti, Mayo Clinic, Rochester, MN, United States

11:30 AM – 11:45 AM

1373: Single-Cell Transcriptomics Analysis of a Complete Response Phenotype in Mice with Disseminated Lung Cancer after Systemic Therapy with Oncolytic Adenovirus

Svetlana Atasheva, Emory University, Atlanta, GA, United States

11:45 AM – 12:00 PM

1374: Fusogenic Oncolytic Vaccinia Virus Changes the Tumor Immune Microenvironment by Cell Fusion and Enhances Systemic Anti-Tumor Immunity

Motomu Nakatake, Tottori University, Yonago, Japan

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The ESGCT is a non-profit organisation for educational and scientific purposes. The aim of ESGCT is to promote fundamental and clinical research in gene therapy, cell therapy and genetic vaccines by facilitating education, exchange of information and technology.

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