24th Annual Meeting
PROGRAM GUIDE

5.11–5.14 | 2021

#ASGCT21

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- Immunotherapy for GD2 diffuse intrinsic pontine glioma (DIPG)
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- Tr1 cells to suppress allogenic responses in stem cell transplantation
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A WELCOME FROM STEPHEN J. RUSSELL, M.D., PH.D.

Dear Colleagues,

On behalf of the American Society of Gene & Cell Therapy (ASGCT), it is my pleasure to welcome you to our second 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 Michel Sadelain, M.D., Ph.D., and Robert L. Martuza, M.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. Carl June, Michel Sadelain, Larry Corey, Kathleen Neuzil, Marcela Maus, Benjamin Kleinstiver, Natalia Gomez-Ospina, Annarita Miccio,and Jerry Mendell 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.

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,

Stephen J. Russell, M.D.
Ph.D. President, ASGCT

Mission + Vision

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

Abstract Publications

All abstracts accepted for presentation at the ASGCT 24th Annual Meeting have been published in the May supplement of Molecular Therapy, and are searchable for free on the Annual Meeting platform

Continuing Medical Education

The ASGCT Board of Directors decided that the Society will not offer CME credit for the 24th 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.

Education Methods + Materials

Pre-Meeting Workshops, Plenary Sessions, Scientific Symposia, Education Sessions, Networking Opportunities, Industry Sponsored Symposia, Oral Abstract Sessions, Digital Presentations, Exhibit Hall and Exhibitor Showcase, Tool and Technology Forums.

GENERAL MEETING INFORMATION

Educational Objectives

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

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

Evaluation Method

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

Needs

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

Target Audience

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

Dates

The ASGCT 24th Annual Meeting will begin on the morning of **Tuesday, May 11, 2021** and continue through 2 p.m. (ET) on **Friday, May 14, 2021.** Exhibits will be open 24/7, Tuesday, May 11 through Friday, May 14. All content will be available to registrants on-demand for 30 days following the conclusion of the meeting.

2021 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 24th Annual Meeting.

Chair

Stephen J. Russell, M.D., Ph.D. – *Mayo Clinic*

Members

Beverly L. Davidson, Ph.D. – *Children's Hospital of Philadelphia*
Helen Heslop, Ph.D. – *Baylor College of Medicine*
Hans-Peter Kiem – *Fred Hutchinson Cancer Research Center*

2021 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

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

ASGCT President Elect

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

ASGCT Secretary + Abstract Chair

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

ASGCT Program Committee Members

Helen Heslop, Ph.D. – *Baylor College of Medicine*
Hans-Peter Kiem – *Fred Hutchinson Cancer Research Center*

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JOIN US AT OUR ASGCT ANNUAL MEETING SHOWCASE SESSION

Trends and Challenges in Cell & Gene Therapy
Friday, May 14 • 10:45-11:30 am ET

Take a comprehensive look at the pipeline trends for in vivo and ex vivo cell and gene therapies, the top indications being targeted, the top companies involved, deal making and financing trends, and manufacturing, pricing and reimbursement challenges.

Presented by Ly Nguyen-Jatkoe, PhD
Executive Director, Americas
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ASGCT
AWARD RECIPIENTS

OUTSTANDING ACHIEVEMENT AWARD

Carl June, M.D.,
Richard W. Vague
Professor in Immunotherapy,
University of Pennsylvania

Michel Sadelain, M.D., Ph.D.,
Director, Center for Cell Engineering,
Memorial Sloan-Kettering Cancer Center

The Outstanding Achievement Award is Sponsored by:

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SONIA SKARLATOS PUBLIC SERVICE AWARD

Larry Corey, M.D.,
President and Director
Emeritus Fred Hutchinson Cancer Research Center

Kathleen Neuzil, M.D., MPH,
FIDSA, Professor,
University of Maryland

OUTSTANDING NEW INVESTIGATOR AWARDS

Marcela Maus, M.D., Ph.D.,
Director of Cellular Immunotherapy,
Massachusetts General Hospital

Benjamin Kleinstiver, Ph.D.,
Assistant Professor of Pathology,
Massachusetts General Hospital

Natalia Gomez-Ospina, M.D.,
Ph.D., Assistant Professor of Pediatrics,
Stanford University

Annarita Miccio, Ph.D., Lab
Director, Imagine Institute

The Outstanding New Investigator Award is Sponsored by:

BURROUGHS WELLCOME FUND

ASGCT AWARD RECIPIENTS

Congratulations to the Following Individuals for Receiving an ASGCT Award!

JERRY MENDELL AWARD FOR TRANSLATIONAL SCIENCE:

Jerry Mendell, M.D.,
Professor of Pediatrics and Neurology,
Nationwide Children's Hospital
Supported by Dr. Suku and Ann Nagendran

EXCELLENCE IN RESEARCH AWARDS

The Excellence in Research Awards will be presented on **Wednesday, May 12** during the George Stamatoyannopoulos Memorial Lecture.

The Excellence in Research Awards are sponsored by:

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10x Genomics
Pleasanton, CA
www.10xgenomics.com

10x Genomics builds solutions that match the complexity of biology, providing instruments, consumables, and software that enable fundamental discoveries across multiple research areas, including cancer, immunology, and neuroscience.

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www.absorption.com

We deliver a comprehensive range of non-clinical research services for all biomedical products with the highest data quality and integrity, focused on timely communication and exemplary customer service, to better predict human outcomes.

Agilent Technologies
Santa Clara, CA
www.agilent.com/en/products/genomics-agilent

Agilent offers a broad variety of high-quality workflow solutions for applications within genomics. Find everything you need to create your genomics workflow, from lab sample to library preparation, enrichment or hybridization, and more!

Aldevron
Fargo, ND
www.aldevron.com

Aldevron serves the biotechnology industry with custom production of nucleic acids and proteins. Aldevron-produced plasmids, RNA and gene editing enzymes are used in projects ranging from research grade to clinical trials to commercial applications.

ArcticZymes Technologies
Wayne, PA
www.arcticzymes.com

ArcticZymes develops and manufactures novel, GMP-ready nucleases tailor made for bioprocessing applications, which are proven to help achieve purer product and higher titers, at a reduced cost.

Beckman Coulter Life Sciences
Indianapolis, IN
www.beckman.com/centrifuges

Beckman Coulter Life Sciences empowers those seeking answers to life's important scientific and healthcare questions. Our precision instruments help researchers study complex biological problems and accelerate answers in the life sciences.

Benchling
San Francisco, CA
www.benchling.com

Benchling is the industry's leading life sciences R&D cloud. Benchling offers a suite of cloud applications that allows scientists to accelerate, measure, and forecast R&D – from discovery through bioprocessing – all in one place.

BioAnalytix
Cambridge, MA
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We generate best-in-class pre-clinical and CMC analytical data packages and provide scientific, technical and regulatory support to advance, de-risk and accelerate all stages of biopharmaceutical development from clone through commercialization.

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BioMarin Pharmaceutical
Novato, CA
www.bmrn.com

BioMarin focusses on developing first-in-class & best-in-class therapies that provide meaningful advances to patients with serious, life-threatening rare genetic diseases. BioMarin has a dedicated & professional approach to gene therapy research.

Bio-Rad Laboratories
Hercules, CA
www.bio-rad.com

Bio-Rad provides innovative products to minimize downtime and to provide the right answer the first time. We offer tools and services that empower the development, analysis, and manufacturing of cutting-edge cell and gene therapies.

Bio-Techne
Minneapolis, MN
www.bio-techne.com

Bio-Techne provides flexible and pioneering tools to simplify your workflow at every step of the manufacturing process. From CAR T cells to pluripotent stem cells, let us help you get your therapy to market.

Bristol Myers Squibb
Summit, NJ
www.bms.com

Bristol Myers Squibb is a leading global biopharma company focused on discovering, developing and delivering innovative medicines for patients with serious diseases.

Brooks Life Sciences GENEWIZ Inc.
South Plainfield, NJ
www.genewiz.com

Brooks Life Sciences and GENEWIZ offer comprehensive tools and solutions throughout the cell and gene therapy workflow, as well as novel AAV sequencing and synthetics solutions to support the development of safe and effective therapies.

Catalent Cell & Gene Therapy
Somerset, NJ
www.catalent.com

Catalent Cell & Gene Therapy is a full-service partner for adeno-associated virus (AAV) vectors and CAR-T immunotherapies, with deep experience in viral vector scale-up and production.

Cedars-Sinai Biomanufacturing Center
Los Angeles, CA
www.cedars-sinai.org/biomanufacturing

Cedars-Sinai Biomanufacturing Center (CBC) is a new 22,000 square feet, state-of-the-art biomanufacturing facility located in West Hollywood, California.

Cell Press
Cambridge, MA
www.cell.com

Cell Press publishes over 50 scientific journals including the Molecular Therapy family of journals. We bring our editorial excellence, commitment to innovation, reach and visibility, and passion for advocacy to all areas of scientific exploration.

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CellGenix

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Portsmouth, NH
www.cellgenix.com

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www.cevec.com

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Children's Hospital of Philadelphia
Philadelphia, PA
ccmt.research.chop.edu/cores.php

The CHOP Research and Clinical Vector Core provide state-of-the-art technology in lentivirus (LV) and AAV-based vectors for gene transfer in research, pre-clinical pharm-tox studies, and early-phase clinical trials.

CLEARPOINT NEURO

ClearPoint Neuro, Inc.
Irvine, CA
www.clearpointneuro.com

ClearPoint Neuro strives to support our partners through all stages of therapy development. Our services provide translational continuity as we navigate through the pre-clinical & clinical landscape w/ an industry-leading range & caliber of services.

COBRA BIOLOGICS

Cobra Biologics Limited
Keele, United Kingdom
www.cobrabio.com

Cobra provides a comprehensive service offering, with multi-functional and experienced project teams nurturing customers' DNA and Viral Vector products from pre-clinical through to clinical and commercial manufacture within GMP approved facilities.

CPC
Roseville, MN
www.cpcworldwide.com/cgt

CPC - Colder Products Company, is the leader in single-use connection technology offering a wide variety of cell and gene therapy connection solutions that ensure ease of use and a robust connection.

CURIOX

Curiox Biosystems
San Carlos, CA
www.curiox.com

Faster and easily automated, Laminar Wash™ systems deliver higher quality data through better cell retention, improved preservation of cellular physiology and viability, and exceptional study-to-study and operator-to-operator reproducibility.

Cygnus Technologies
Southport, NC
www.cygnustechnologies.com

Cygnus Technologies, part of Maravai LifeSciences, is the biopharmaceutical industry's partner in host cell protein (HCP) and other process-related impurity detection and analytics as well as in innovative viral clearance solutions.

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Cytiva

Marlborough, NJ
www.cytivalifesciences.com

Cytiva is a global provider of technologies and services that help advance and accelerate the development and manufacture of therapeutics.

Delphi Genetics

Gosselies, Belgium
www.delphigenetics.com

Delphi Genetics is a Belgian-based one-stop-shop CDMO specialized in plasmid DNA production for C&G Therapy application, including plasmid for viral vectors (AAV, LV) and linear plasmids for mRNA.
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Lancaster, PA
www.eurofinsus.com/bpt

The largest network of harmonized GMP product testing laboratories worldwide, Eurofins BPT offers expert cell and molecular biology, biochemistry, biosafety, and microbiology testing to support the development of cell and gene therapy products.

Eurofins Viracor BioPharma

Lee's Summit, MO
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FDA/Center for Biologics Evaluation and Research

Silver Spring, MD
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GeneWerk
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GeneWerk specializes in integration site analysis and safety assessment for gene- and cell therapy products. We also offer a wide variety of services, including gene-editing on/off-targets, TCR/BCR repertoire, VCN, RCL/RCR, and impurities detection.

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La Jolla, CA
www.helixmith.com/eng

Helixmith currently has a plasmid DNA-based drug, VM202, in clinical development for neurological and cardiovascular diseases. Helixmith also has early phase pipeline in AAV-based gene therapy & CAR-T cell therapy for solid tumors.

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Integrated DNA Technologies (IDT)
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Invicro is a leading contract research organization (CRO) that provides end-to-end imaging and pathology services for drug development firms. Invicro provides scientific and operational expertise to support the development of personalized therapies.

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www.isoplexis.com

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Legend Biotech
Piscataway, NJ
www.legendbiotech.com

Legend Biotech is a global clinical-stage biopharmaceutical company engaged in the discovery and development of novel cell therapies for oncology and other indications.

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www.lonza.com/viral

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www.malvernpanalytical.com

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www.liebertpub.com

Mary Ann Liebert Inc. publishes groundbreaking journals including Human Gene Therapy, The CRISPR Journal, and GEN (Genetic Engineering & Biotechnology News) which cover research, developments, and technologies that drive gene therapy advances.

Matica Biotechnology
College Station, TX
www.maticabio.com

Matica Biotechnology is a CDMO specializing in viral vector production for cell & gene therapies. We create manufacturing solutions, delivering advanced therapies utilizing innovative & highly flexible technologies.

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Gaithersburg, MD
www.maxcyte.com

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Miltenyi Biotec
San Diego, CA
www.miltenyibiotec.com

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www.mogrify.co.uk

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www.myriadelab.com/en

VIDEODROP by Myriade makes it possible to measure the Size & Concentration of lentivirus & adenvirus in the range of 70 nm & 10 microns, in real time (40s), in a single drop (5µL), without labelling, without purification if required on viscous samples

National Gene Vector Biorepository (NGVB) & NHLBI Primate Center for Gene Therapy
Indianapolis, IN
www.ngvbcc.org

NHLBI funds 2 gene therapy resources: The NGVB provides reagents, archiving and testing services (NGVBCC.org). The PCGT assists in evaluating the safety and efficiency of gene transfer strategies (pcgt.ucdavis.edu). Services are free when eligible!

National Organization for Rare Disorders (NORD)
Danbury, CT
www.rarediseases.org

NORD is a patient advocacy organization dedicated to individuals with rare diseases. NORD and its members are committed to the identification, treatment, and cure of rare disorders through education, advocacy, research, and patient services.

NHLBI Gene Therapy Resource Program
Silver Spring, MD
www.gtrp.org

The NHLBI Gene Therapy Resource Program (GTRP) provides translational resources at no cost to qualified US-based investigators studying gene therapy for heart, lung, blood, and sleep disorders.

Nordmark Biochemicals
Uetersen, Germany
www.nordmark-pharma.de/en/home/

Nordmark Biochemicals offers products for cell isolation, including Collagenase NB 6 GMP Grade for stem cell isolation and animal-free Collagenase AF-1 GMP Grade. We provide translational - research and GMP Grade-collagenases for research to clinic.

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Novartis & Novartis Medical
Deerfield, IL
www.novartis.com/our-company/novartis-pharmaceuticals/novartis-gene-therapies

AveXis is now Novartis Gene Therapies. Novartis Gene Therapies is dedicated to developing and commercializing gene therapies for patients and families devastated by rare and life-threatening neurological genetic diseases.

OBiO 和元生物 GENE THERAPY CDMO

OBiO Technology
Shanghai, China
www.obiosh.com

OBiO Technology is a biopharmaceutical company founded in 2013. Our CDMO platform offers plasmid, AAV, lentivirus, adenovirus, oncolytic viruses, and cell therapy cGMP manufacturing service, to pharmaceutical companies in the global GCT field.

Ology Bioservices
Alachua, FL
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Ology Bioservices is a biologics-focused CDMO serving clients from early-stage development through commercialization. Ology Bio provides services for cell & gene therapies, live viral vaccines & vectors, oncolytic viruses, and monoclonal antibodies.

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PHC Corporation of North America is a leader in laboratory equipment for biopharmaceutical, life sciences, academic, healthcare and government markets. The company is a subsidiary of PHC Holdings Corporation, Tokyo, Japan, which is a global healthcare company.

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ANNUAL MEETING EXHIBITORS

Virtual Exhibit Hall open 24hrs online. Exhibitors available:
Tues: 10:30a-12p, 2-3:30p, 5:15-6:45p | Wed. & Thr.: 10:45a-12:15p, 2-3:30p, 5:15-6:45p | Fri.: 10:45a-12:15p

Precision For Medicine
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Precision for Medicine is the first full-service, global clinical research organization purpose-built to accelerate precision medicine research and development. Our expertise in rare and orphan diseases includes 150+ projects in 80+ diseases.

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ANNUAL MEETING EXHIBITORS

Virtual Exhibit Hall open 24hrs online. Exhibitors available:
Tues: 10:30a-12p, 2-3:30p, 5:15-6:45p | Wed. & Thr.: 10:45a-12:15p, 2-3:30p, 5:15-6:45p | Fri.: 10:45a-12:15p

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ANNUAL MEETING EXHIBITORS

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Tues: 10:30a-12p, 2-3:30p, 5:15-6:45p | Wed. & Thr.: 10:45a-12:15p, 2-3:30p, 5:15-6:45p | Fri.: 10:45a-12:15p

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Schedule AT A GLANCE

ASGCT 2021 Virtual ANNUAL MEETING

ALL TIMES LISTED IN EDT

TUESDAY, MAY 11, 2021

ALL DAY ACCESS	**DIGITAL ABSTRACT PRESENTATIONS** **EXHIBIT HALL** Connect with Exhibitors: 10:30 AM - 12:00 PM 2:00 PM - 3:30 PM 5:15 PM - 6:45 PM
9 - 10 AM	**CHAT LOUNGE NETWORKING**
10 - 11:45 AM	**EDUCATION SESSIONS** • Gene Therapy for Hematologic Disorders *Co-Chairs: John Tisdale, M.D. and Matthew Porteus, M.D., Ph.D.* • Predictive Animal Models for Preclinical Testing of Gene Immunotherapies *Co-Chairs: Renata Stripecke, Ph.D. and Satiro De Oliveira, M.D.* • Stem Cell Expansion *Chair: Mitchell Horwitz,M.D.* Scientific Symposia • Cutting Edge Gene and Cell Therapy Research in Japan (Organized by JSGCT) *Co-Chairs: Noriyuki Kasahara, M.D., Ph.D. and Takafumi Nakamura, Ph.D.* • Gene Therapy Development Challenges and Opportunities in Low- and Middle-Income Countries (Organized by the Global Outreach Committee) *Co-Chairs: Kenneth Cornetta, M.D. and Jayandharan Rao, Ph.D.* • Genome Editing - Clinical and Preclinical Updates (Organized by the Genome Editing Committee *Co-Chairs: Benjamin Kleinstiver, Ph.D. and Angela Whatley, Ph.D.* • Payment Policies for Non-Policy Specialists: Joining the Conversation (Organized by the Commercialization Committee *Co-Chairs: Mark Skinner, J.D. and Jeremy Allen* • Recent Advances and Future Directions of Gene and Cellular Therapies in Immune Oncology (Organized by the Cancer Gene and Cell Therapy Committee) *Co-Chairs: Robert Sobol, M.D. and Katy Rezvani,M.D., Ph.D.* **SPECIAL SYMPOSIUM** • COVID-19: Vaccines to the Rescue

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TUESDAY, MAY 11, 2021

10:30 - 12 PM	**EXHIBITOR SHOWCASES** • Catalent Cell & Gene Therapy- 10:30-11:15 • STEMCELL Technologies- 10:30-11:15 • Aldevron- 11:15-12 • MilliporeSigma- 11:15-12
12 - 1 PM	**FIRESIDE CHAT** *Jennifer Doudna, Ph.D., UC Berkeley*
1:30 - 3:30 PM	**MENTOR MEET-UP EVENT** *Sponsored by Caribou Biosciences, Dyno Therapeutics, Rocket Pharmaceuticals, and Terumo Blood and Cell Technologies*
2:00 - 3:30 PM	**INDUSTRY SPONSORED SYMPOSIA** • Corning Incorporated • Maxcyte, Inc. • Sarepta Therapeutics, Inc. • Terumo Blood and Cell Technologies
3:30 - 5:15 PM	**EDUCATION SESSIONS** • AAV Vectors from Basic Biology to Clinical Application and Back *Co-Chairs: Hildegard Buning, Ph.D., and Alberto Auricchio, M.D.* 3:30 PM - 5:15 PM • Gene Therapy in Cancer *Co-Chairs: Rayne Rouce, M.D. and Renata Stripecke, Ph.D.* 3:30 PM - 5:15 PM • In Vivo Gene Editing *Co-Chairs: Juliana Alvarez Argote, M.D. and Blythe Sather, Ph.D.* 3:30 PM - 5:15 PM • Issues in gene therapy: Considerations for efficient development and access *Chair: John Tisdale, M.D.* 3:30 PM - 5:15 PM

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ALL TIMES LISTED IN EDT

TUESDAY, MAY 11, 2021

3:30 - 5:15 PM	**PLENARY SESSION** • Career Development Award Presentations
5:30 - 7:30 PM	**ABSTRACT SESSIONS** • Advances in Ex Vivo Modified Cell Therapies *Co-Chairs: Adrian Gee, Ph.D. and Joseph Gold, Ph.D.* 5:30 PM - 7:15 PM • Cancer - Oncolytic Viruses *Co-Chairs: Paola Grandi, Ph.D. and Melissa Kotterman, Ph.D.* 5:30 PM - 7:15 PM • Delivery Technologies and CRISPR for Therapeutics *Co-Chairs: Nicole Gaudelli, Ph.D. and Alejandro Chavez, M.D., Ph.D.* 5:30 PM - 7:00 PM • Development of AAV Capsid Variants *Co-Chairs: Deep Bhattacharya, Ph.D. and Christine Le Bec, Ph.D.* 5:30 PM - 7:00 PM • Gene Therapy for Inborn Errors of Metabolism *Co-Chairs: Giuseppe Ronzitti, Ph.D. and Gloria Gonzalez-Aseguinolaza, Ph.D.* 5:30 PM - 7:15 PM • Genetic Blood and Immune Disorders *Co-Chairs: Denise Sabatino, Ph.D. and Cyndi Dunbar, M.D.* 5:30 PM - 7:15 PM • Musculo-Skeletal Diseases *Co-Chairs: Olivier Danos, Ph.D. and Rita Perlingeiro Ph.D.* 5:30 PM - 7:15 PM • Novel AAV Capsids for Brain, Eye and Muscle Tissues *Co-Chairs: Nicole Paulk, Ph.D. and Daniel Lipinski, D.Phil.* 5:30 PM - 7:15 PM • Preclinical Gene Therapy for Neurologic Diseases I *Co-Chairs: Ana Rita Batista, Ph.D. and Martin Hicks, Ph.D.* 5:30 PM - 7:15 PM

Schedule AT A GLANCE

ASGCT 2021 Virtual ANNUAL MEETING

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TUESDAY, MAY 11, 2021

5:15 - 6:45 PM	INDUSTRY SPONSORED SYMPOSIA • Precision for Medicine • Thermo Fisher Scientific
5:15 - 6:15 PM	NETWORKING ROULETTE Sponsored by Teknova
5:15 - 7 PM	TOOLS AND TECHNOLOGY FORUM I

Schedule AT A GLANCE

ASGCT 2021 Virtual ANNUAL MEETING

ALL TIMES LISTED IN EDT

WEDNESDAY, MAY 12, 2021

	DIGITAL ABSTRACT PRESENTATIONS
ALL DAY ACCESS	**EXHIBIT HALL** Connect with Exhibitors: 10:45 AM - 12:15 PM 2:00 PM - 3:30 PM 5:15 PM - 6:45 PM
9 - 10 AM	**CHAT LOUNGE NETWORKING**
10 - 11:45 AM	**SCIENTIFIC SYMPOSIA** • Building Your Elevator Pitch (Organized by the Communications Committee) *Chair: Edith Pfister, Ph.D.* • Cutting Edge Gene and Cell Therapy Research in Europe (Organized by ESGCT) *Co-Chairs: Juan Bueren, Ph.D. and Alberto Auricchio, M.D.* • Entering and Thriving in Industry: Guidance for Academic, Clinical, and Industry Professionals (Organized by the Bio-Industry Committee) *Co-Chairs: Bartholomew Tortella, M.D. and Steven Howe, Ph.D.* • mmunological Barriers to GeneTherapy: Are They Surmountable? (Organized by the Immune Responses to Gene & Cell Therapy Committee) *Co-Chairs: Maria Castro, Ph.D. and Roberto Calcedo, Ph.D.* • Novel Viral Gene Transfer Vectors and Applications (Organized by the Viral Gene Transfer Vectors Committee) *Co-Chairs: Masato Yamamoto, M.D., Ph.D. and Phillip Tai, Ph.D.* • Regulatory Lessons Learned from COVID-19: Anomaly to Precedent (Organized by the Regulatory Affairs Committee) *Co-Chairs: Kit Shaw, Ph.D. and S. Kaye Spratt, Ph.D.* • Toxicities and Limitations of Gene Therapy (Organized by the Gene & Cell Therapy of Genetic and Metabolic Diseases Committee) *Co-Chairs: Paris Margaritis, D.Phil. and Moanaro Biswas, Ph.D.* • Translational Gene and Cell Therapy Studies in Cardiovascular Medicine (Organized by Cardiovascular Gene & Cell Therapy Committee) *Co-Chairs: Sangeetha Vadakke-Madathil, Ph.D. and Margaret Sleeper, V.M.D.*

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ASGCT 2021 Virtual ANNUAL MEETING

ALL TIMES LISTED IN EDT

WEDNESDAY, MAY 12, 2021

10 - 11:45 AM	**SPECIAL SYMPOSIUM** • Jerry Mendell Award for Translational Science Symposium *Supported by Dr. Suku and Ann Nagendran*
10:45 - 12:15 PM	**EXHIBITOR SHOWCASES** • Unchained Labs - 10:45 AM - 11:30 AM • Wyatt Technology - 10:45 AM - 11:30 AM • Bio-Rad Laboratories -11:30 AM - 12:15 PM • Halo Labs - 11:30 AM - 12:15 PM
12:15 - 2 PM	**GEORGE STAMATOYANNOPOULOS MEMORIAL LECTURE AND PRESENTATION OF THE EXCELLENCE IN RESEARCH AWARDS** Sponsored by REGENXBIO
2 - 3:30 PM	**INDUSTRY SPONSORED SYMPOSIA** • Cytiva • Miltenyi Biotec • Vertex Pharmaceuticals Inc. • Voyager Therapeutics
2 - 3 PM	**CHAT LOUNGE NETWORKING**
3:30 - 5:15 PM	**PLENARY SESSION** Outstanding New Investigator Symposium Sponsored by BURROUGHS WELLCOME FUND
5:15 - 7:15 PM	**ABSTRACT SESSIONS** • AAV Biology, Engineering, Immunology and Animal Modeling *Co-Chairs: Allison Bradbury, Ph.D. and Miguel Sena-Esteves, Ph.D.* 5:30 PM - 7:15 PM • CAR Modified Cellular Therapies *Co-Chairs: Maria-Grazia Roncarolo, M.D. and Pietro Genovese, Ph.D.* 5:30 PM - 7:00 PM • Gene Therapies for Hemoglobinopathies *Co-Chairs: John Chapin, M.D. and Pankaj Mandal, Ph.D.* 5:30 PM - 7:15 PM

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ASGCT 2021 Virtual ANNUAL MEETING

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WEDNESDAY, MAY 12, 2021

5:15 - 7:15 PM	**ABSTRACT SESSIONS** • Immune Responses to AAV Vectors *Co-Chairs: Ying Kai Chan, Ph.D. and Manish Muhuri, Ph.D.* 5:30 - 7:00 PM • Novel Factors in AAV Transduction and AAV Genomes *Co-Chairs: David Markusic, Ph.D. and Amanda Dudek, Ph.D.* 5:30 PM - 7:15 PM • Preclinical Gene Therapy for Neurologic Disorders II *Co-Chairs: Gwladys Gernoux, Ph.D. and Juliette Hordeaux, D.V.M., Ph.D.* 5:30 PM - 7:00 PM • Synthetic/Molecular Conjugates and Physical Methods for Delivery *Co-Chairs: Angela Pannier, Ph.D. and Kenya Kamimura, M.D., Ph.D.* 5:30 PM - 7:15 PM • Targeted Gene and Cell Therapy for Cancer *Co-Chairs: Michael Milone, M.D., Ph.D. and Hernando Lopez-Bertoni, Ph.D.* 5:30 PM - 7:15 PM • Upstream Process Development for AAV Vector Production *Co-Chairs: Sanford Boye and Laura Adamson-Small, Ph.D.* 5:30 PM - 7:15 PM
5:15 - 6:45 PM	**INDUSTRY SYMPOSIA** • 908 Devices • Pall Corporation
5:15 - 6:15 PM	**NETWORKING ROULETTE**
5:15 - 7:00 PM	**TOOLS AND TECHNOLOGY FORUM II**

Schedule AT A GLANCE

ASGCT 2021 Virtual ANNUAL MEETING

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THURSDAY, MAY 13, 2021

ALL DAY ACCESS	**DIGITAL ABSTRACT PRESENTATIONS** **EXHIBIT HALL** Connect with Exhibitors: 10:45 AM - 12:15 PM 2:00 PM - 3:30 PM 5:15 PM - 6:45 PM
9 - 10 AM	**CHAT LOUNGE NETWORKING**
10 - 11:45 AM	**EDUCATION SESSIONS** • Career and Workforce Development Issues in Gene and Cell Therapy *Co-Chairs: Juliana Alvarez Argote, M.D. and Rayne Rouce, M.D.* 10:00 AM - 11:45 AM • Gene Therapies for Liver Diseases *Chair: Nuria Morral, Ph.D.* 10:00 AM - 11:45 AM • Therapeutic Applications of EVs: From Diagnostics to Drug Delivery (Organized by the Nanoagents & Synthetic Formulations Committee) *Co-Chairs: Rajagopal Ramesh, Ph.D. and Assem Ziady, Ph.D.* 10:00 AM - 11:45 AM **SCIENTIFIC SYMPOSIA** • International Focus on Stem Cell Gene Therapy (Organized by the International Committee) *Co-Chairs: Toni Cathomen, Ph.D. and Alessandro Aiuti, M.D., Ph.D.* 10:00 AM - 11:45 AM • Racial Justice in the Gene Therapy Field (Organized by the Ethics & Diversity and Inclusion Committees) *Co-Chairs: Rayne Rouce, M.D. and David Segal, Ph.D.* 10:15 AM - 11:45 AM • Reporter Gene Imaging And The 3 Rs In Cell And Gene Therapy *Co-Chairs: Stephen Russell, M.D., Ph.D.* 10:00 AM - 11:45 AM • Vaccine Development: Successes and Emerging Challenges (Organized by the Infectious Diseases and Vaccines Committee) *Co-Chairs: David Weiner, Ph.D. and Sterghios Moschos, Ph.D.* 10:00 AM - 11:45 AM

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ASGCT 2021 Virtual ANNUAL MEETING

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THURSDAY, MAY 13, 2021

10 - 11:45 AM	**SCIENTIFIC SYMPOSIA** • Vector Manufacturing and Downstream Processing *Co-Chairs: Anne Galy, Ph.D. and Boro Dropulic, Ph.D.* 10:00 AM - 11:45 AM • Viral Vector Safety: A Renewed Focus on Vector Safety and Innate Immune Responses to Leading Viral Vectors (Organized by the Translational Science Committee) *Co-Chairs: H. Trent Spencer, Ph.D. and Nicole Paulk, Ph.D.* 10:00 AM - 11:45 AM
10:45 - 12:15 PM	**EXHIBITOR SHOWCASES** • BIA Separations now a Sartorius company - 10:45 AM - 11:30 AM • Bristol Myers Squibb - 10:45 AM - 11:30 AM • Brooks Life Sciences GENEWIZ Inc. - 11:30 AM - 12:15 PM • Solentim - 11:30 AM - 12:15 PM
12:15 - 2 PM	**OUTSTANDING ACHIEVEMENT AWARD LECTURE AND PRESENTATION OF THE SONIA SKARLATOS PUBLIC SERVICE AWARD** Sponsored by AskBio
2 - 3:30 PM	**INDUSTRY SPONSORED SYMPOSIA** • Charles River Laboratories • FUJIFILM Diosynth Biotechnologies • GenScript • Precision NanoSystems Inc.
2 - 3 PM	**CHAT LOUNGE NETWORKING**
3:30 - 5:15 PM	**PLENARY SESSION** Presidential Symposium and Presentation of Top Abstracts Sponsored by MODALIS

Schedule AT A GLANCE

ASGCT 2021 Virtual ANNUAL MEETING

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THURSDAY, MAY 13, 2021

	ABSTRACT SESSIONS
5:15 - 7:15 PM	• AAV Therapies for Neurological and Sensory Diseases *Co-Chairs: Phillip Tai, Ph.D. and Lluis Samaranch, Ph.D.* 5:30 PM - 7:15 PM
	• Advances in Cellular and Immunotherapies *Co-Chairs: Rayne Rouce, M.D., and Daniel Bauer, M.D., Ph.D.* 5:30 PM - 7:15 PM
	• CAR-Based Cancer Gene Therapy *Co-Chairs: Monica Casucci, Ph.D. and Daniel Abate-Daga, Ph.D.* 5:30 PM - 7:15 PM
	• Cardiovascular and Pulmonary Gene Therapy *Judith Greengard, Ph.D. and Mai ElMallah, M.D.* 5:30 PM - 7:00 PM
	• Clinical Trials and Advanced Preclinical Studies for Neurologic Diseases *Co-Chairs: Heather Gray-Edwards, D.V.M., Ph.D. and Patricia Dickson, M.D.* 5:30 PM - 7:15 PM
	• Downstream Process of Vector Manufacturing *Co-Chairs: Chris Morrison, Ph.D. and Eric Horowitz, Ph.D.* 5:30 PM - 7:15 PM
	• Immunotherapy and Vaccines *Co-Chairs: Matt Gardner, Ph.D. and Allison Keeler-Klunk, Ph.D.* 5:30 PM - 7:15 PM
	• New Gene Editing Technologies and Applications *Co-Chairs: Alexis Komor, Ph.D. and T.J. Cradick, Ph.D.* 5:30 PM - 7:15 PM
	• Novel AAV Biology and Platform Technologies *Co-Chairs: Lauren Woodard, Ph.D. and Anna Maurer, Ph.D.* 5:30 PM - 7:15 PM
	INDUSTRY SPONSORED SYMPOSIA
5:15 - 6:45 PM	• Dyno Therapeutics
	• L7 Informatics, Inc.
5:15 - 6:15 PM	**NETWORKING ROULETTE**
5:15 - 7:00 PM	**TOOLS AND TECHNOLOGY FORUM III**

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ASGCT 2021 Virtual ANNUAL MEETING

ALL TIMES LISTED IN EDT

FRIDAY, MAY 14, 2021

ALL DAY ACCESS	**DIGITAL ABSTRACT PRESENTATIONS** **EXHIBIT HALL** Connect with Exhibitors: 10:45 AM - 12:15 PM
9 - 10 AM	**CHAT LOUNGE NETWORKING**
10 - 11:45 AM	**SCIENTIFIC SYMPOSIA** • Hot Topics and Remaining Challenges in RNAi and Oligonucleotide Therapy for 2021 (Organized by the Oligonucleotide and RNAi Therapeutics Committee) *Chair: Paloma Giangrande, Ph.D.* 10:00 AM - 11:45 AM • New Advances in Physical Gene Delivery and Nucleic Acid Vectorology (Organized by the Physical Delivery, Therapeutics & Vector Development Committee) *Co-Chairs: Loree Heller, Ph.D. and Carol Miao, Ph.D.* 10:00 AM - 11:45 AM • Newborn Screening: Innovative Policies and Technologies to Eliminate the Diagnostic Odyssey (Organized by the Government Relations Committee) *Co-Chairs: Diane Berry, Ph.D. and Philip Reilly, M.D., J.D.* 10:00 AM - 11:45 AM • Race to Respiratory Therapies for COVID-19 (Organized by the Respiratory and GI Tract Gene and Cell Therapy Committee) *Chair: Amy Ryan, Ph.D.* 10:00 AM - 11:45 AM • RNA Therapies for Neurologic and Ophthalmic Disorders (Organized by the Neurologic & Opthalmic Gene and Cell Therapy Committee) *Co-Chairs: Kourous Rezaei, M.D. and Jason Shepherd, Ph.D.* 10:00 AM - 11:45 AM • Safety and Efficacy of Body-Wide Therapy for Musculo-Skeletal Diseases (Organized by the Musculo-Skeletal Gene & Cell Therapy Committee) *Co-Chairs: Jyoti Jaiswal, Ph.D. and Christina Pacak, Ph.D.* 10:00 AM - 11:45 AM • Vaccine Nanotechnology for Rapid Response Applications (Organized by the Nanoagents and Synthetic Formulations Committee) *Co-Chairs: Jordan Green, Ph.D. and Julie Champion, Ph.D.* 10:00 AM - 11:45 AM

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ASGCT 2021 Virtual ANNUAL MEETING

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FRIDAY, MAY 14, 2021

10:45 - 11:30 AM	**EXHIBITOR SHOWCASES** • 10x Genomics • Informa Pharma Intelligence
12:15 - 2 PM	**ABSTRACT SESSIONS** • Base Editing and Gene Editing Approaches *Co-Chairs: Giulia Pavani, Ph.D. and Shengdar Tsai, Ph.D.* 12:15 PM - 2:00 PM • Cancer Immunotherapy *Co-Chairs: Jan Joseph Melenhorst, Ph.D. and Sarwish Rafiq, Ph.D.* 12:15 PM - 2:00 PM • Gene Therapy for Lysosomal Storage Disorders *Lina Colella, Ph.D. and Pasquale Piccolo, Ph.D.* 12:15 PM - 2:00 PM • Lentiviral Vector Manufacturing *Co-Chairs: Magalie Penaud-Budloo, Ph.D. and Matthias Hebben, Ph.D.* 12:15 PM - 2:00 PM • Metabolic and Muscle Diseases, Tissue and Immunological Engineering *Co-Chairs: Douglas Martin, Ph.D. and Isabelle Richard, Ph.D.* 12:15 PM - 2:00 PM • New Technologies Advancing Gene Therapy for Neurologic Diseases *Co-Chairs: Dan Wang, Ph.D. and Paul Valdmanis, Ph.D.* 12:15 PM - 2:00 PM • Oligonucleotide Therapeutics *Co-Chairs: Mark Kay, M.D., Ph.D. and Paloma Giangrande, Ph.D.* 12:15 PM - 2:00 PM • Pharmacology/Toxicology Studies or Assay Development *Co-Chairs: Cristina Baricordi, Ph.D. and Carmen Unzu, Ph.D.* 12:15 PM - 2:00 PM • RNA Virus Vectors *Co-Chairs: Brian Bigger, Ph.D. and Andrew Wilber, Ph.D.* 12:15 PM - 1:15 PM • AAV Vectors - Clinical Studies *Co-Chairs: Steven Gray, Ph.D. and Diana Bharucha-Goebel, M.D.* 1:15 PM - 2:00 PM

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TUESDAY, MAY 11, 2021

ALL DAY

DIGITAL ABSTRACT PRESENTATIONS

EXHIBIT HALL

Be sure to connect with exhibit booth staff during listed times

9:00 AM - 10:00 AM

CHAT LOUNGE NETWORKING

10:00 AM - 11:45 AM

EDUCATION SESSIONS

Gene Therapy for Hematologic Disorders
Co-Chairs: John Tisdale, M.D. and Matthew Porteus, M.D., Ph.D.

10:00 AM - 10:35 AM	**Gene Addition Therapy For Hematologic Disorders:The Long Road Towards The Cure** *Giuliana Ferrari, Ph.D., SR-TIGET, Scientific Institute San Raffaele*
10:35 AM - 11:10 AM	**Gene Disruption Through Gene Editing For The Treatment Of Hematologic Disorders** *Daniel Bauer, M.D., Ph.D., Boston Children's Hospital*
11:10 AM - 11:45 AM	**Base Editing To Treat Blood Disease Without Double-Strand DNA Breaks** *David Liu, Ph.D., Broad Institute, Harvard University, and HHMI*

Predictive Animal Models for Preclinical Testing of Gene/Immunotherapies
Co-Chairs: Renata Stripecke, Ph.D. and Satiro De Oliveira, M.D.

10:00 AM - 10:35 AM	**Creating Non-Human Primate Models Of Neurodegenerative Disease** *Jodi McBride, Oregon National Primate Research Center*
10:35 AM - 11:10 AM	**Utilization Of An In Vivo Pbmc Humanized Mouse Model For Determining Bispecific Antibody Related Cytokine Release Syndrome** *James Keck, The Jackson Laboratory*
11:10 AM - 11:45 AM	**Testing Human CAR-T Cells And Oncolytic Virus In Mice Challenged With Human Tumors** *Amanda Rosewell Shaw, Baylor College of Medicine*

Schedule

ASGCT 2021 Virtual ANNUAL MEETING

ALL TIMES LISTED IN EDT

TUESDAY, MAY 11, 2021

10:00 AM - 11:45 AM

— EDUCATION SESSIONS —

Stem Cell Expansion
Co-Chair: Mitchell Horwitz, M.D.

10:00 AM - 10:35 AM	**De Novo Generation Of Hematopoietic Stem/ Progenitor Cells For Cellular Therapy** *Andre Larochelle, M.D., Ph.D., National Institutes of Health*
10:35 AM - 11:10 AM	**Cord Blood Expansion-Biology And Techniques** *Elizabeth Shpall, M.D., The University of Texas M.D. Anderson Cancer Center*
11:10 AM - 11:45 AM	**Cord Blood Hematopoietic Stem Cell Expansion - Clinical Results** *John Wagner, M.D., University of Minnesota*

ALL TIMES LISTED IN EDT

TUESDAY, MAY 11, 2021

10:00 AM - 11:45 AM

SCIENTIFIC SYMPOSIA

Cutting Edge Gene and Cell Therapy Research in Japan (Organized by JSGCT)
Co-Chairs: Noriyuki Kasahara, M.D., Ph.D. and Takafumi Nakamura, Ph.D.

10:00 AM - 10:26 AM — **Plasmid DNA-Based Gene Therapy: From Regenerative Medicine to Vaccine**
Ryuichi Morishita, Ph.D., Department of Clinical Gene Therapy, Osaka University

10:26 AM - 10:52 AM — **Contribution of Circulating Mesenchymal Stem Cells in Regenerating Injured Tissue Stem Cells: Implication for Stem Cell Gene Therapy**
Katsuto Tamai, Osaka University

10:52 AM - 11:18 AM — **PET Analysis in Gene Therapy for Aromatic L-Amino Acid Decarboxylase Deficiency**
Yoshiyuki Onuki, Ph.D., Jichi Medical University

11:18 AM - 11:45 AM — **Treatment Strategies for Refractory Gastroesophageal Cancer Using Oncolytic HeRPEs G47Δ**
Kotaro Sugawara, M.D., Ph.D., Institute of Medical Science, The University of Tokyo

Schedule

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TUESDAY, MAY 11, 2021

10:00 AM - 11:45 AM

SCIENTIFIC SYMPOSIA

Gene Therapy Development Challenges and Opportunities in Low - and Middle-Income Countries (Organized by the Global Outreach Committee)
Co-Chairs: Kenneth Cornetta, M.D. and Jayandharan Rao, Ph.D.

10:00 AM - 10:20 AM	**Overview Of Limitations And Progress In Gene Therapy Development In Low- And Middle-Income Countries** *Kenneth Cornetta, M.D., Indiana University*
10:20 AM - 10:40 AM	**Regulatory Pathways For Gene And Cell Therapies In Brazil** *João Batista Silva Junior, ANVISA*
10:40 AM - 11:00 AM	**Challenges And Opportunities For Gene Therapy Development For Hemophilia In South Africa** *Johnny Mahlangu, M.B.Ch.B., M.Med., University of Witwatersrand*
11:00 AM - 11:20 AM	**Opportunities For Clinical Trials Of Indigenously Developed Gene Therapies In India** *Rahul Purwar, Ph.D., Indian Institute of Technology Bombay*
11:20 AM -11:45 AM	**Panel Discussion**

Genome Editing - Clinical and Preclinical Updates
(Organized by the Genome Editing Committee)
Co-Chairs: Benjamin Kleinstiver, Ph.D. and Angela Whatley

10:00 AM - 10:26 AM	**In Vivo CRISPR Base Editing Of PCSK9 In Primates And Durable Cholesterol Reduction** *Andrew Bellinger, M.D., Ph.D., Verve Therapeutics*
10:26 AM - 10:52 AM	**Development Of Systemic CRISPR-Based Therapeutics** *Laura Sepp-Lorenzino, Ph.D., Intellia Therapeutics*
10:52 AM - 11:18 AM	**Ind Enabling Studies For Gene Correction For Sickle Cell Disease** *Annalisa Lattanzi, Ph.D., Stanford University*
10:52 AM - 11:18 AM	**Lca10 In Vivo Eye Clinical Trials** *Charles Albright, Ph.D., Editas Medicine*
11:18 AM - 11:45 AM	**Development Of CRISPR-Enhanced Bacteriophages For The Treatment Of Urinary Tract Infections** *Dave Ousterout, Ph.D., Locus Biosciences*

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TUESDAY, MAY 11, 2021

10:00 AM - 11:45 AM

SCIENTIFIC SYMPOSIA

Payment Policies for Non-Policy Specialists: Joining the Conversation
(Organized by the Commercialization Committee)
Co-Chair: Mark Skinner, J.D. and Jeremy Allen

10:00 AM - 10:25 AM	**Gene Therapy Payment Systems In The United States**	*Beth Halpern, Hogan Lovells*
10:25 AM - 10:50 AM	**Gene & Cell Therapy Payment Systems In Europe**	*Christian Hill, MAP BioPharma*
10:50 AM - 11:15 AM	**Current Drug Pricing And Payment Policy Debates: Applications To Gene Therapy**	*Remy Brim, Ph.D., BGR Group*
11:20 AM -11:45 AM	**Panel Discussion**	

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TUESDAY, MAY 11, 2021

10:00 AM - 11:45 AM

SCIENTIFIC SYMPOSIA

Recent Advances and Future Directions of Gene and Cellular Therapies in Immune Oncology (Organized by the Cancer Gene and Cell Therapy Committee)
Co-Chairs: Robert Sobol, M.D. and Katy Rezvani, M.D., Ph.D.

10:00 AM - 10:26 AM	**Cancer Immunotherapy: Where We Are And Where We Are Going**	*Antoni Ribas, University of California-Los Angeles*
10:26 AM - 10:52 AM	**Updates On Oncolytic Viral Therapy And Combinational Approaches For Gliomas And Other Solid Tumors**	*Juan Fueyo, M.D., M.D. Anderson Cancer Center*
10:52 AM - 11:18 AM	**Next Wave Of Innovation In NK Cell Therapies For Cancer**	*Jeffrey Miller, University of Minnesota*
11:18 AM - 11:44 AM	**Novel Vaccine Technologies To Prevent And Treat Cancer**	*David Weiner, The Wistar Institute*

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TUESDAY, MAY 11, 2021

SPECIAL SYMPOSIUM

COVID-19: Vaccines to the Rescue

10:00 AM - 11:45 AM **Roundtable Discussion**

Stephen Russell, M.D., Ph.D., Mayo Clinic
Sarah Gilbert, Ph.D., University of Oxford
Larry Corey, M.D., Fred Hutchinson Cancer Research Center
Gregory Poland, M.D., Mayo Clinic
Ligia Pinto, Ph.D., Frederick National Laboratory for Cancer Research
Philip Dormitzer, M.D., Ph.D., Pfizer

10:30 AM - 12:00 PM

EXHIBIT HALL OPEN
EXHIBITORS AVAILABLE TO CONNECT

Exhibit Hall is Accessible 24 Hours

10:30 AM - 12:00 PM

EXHIBITOR SHOWCASE

Catalent Cell & Gene Therapy – 10:30 AM - 11:15 AM

Strategies and Solutions for Gene Therapy Development and Manufacturing
Thomas VanCott, Ph.D., Catalent Cell & Gene Therapy

STEMCELL Technologies – 10:30 AM - 11:15 AM

Serum- and Feeder-Free Differentiation of Erythroid Progenitor Cells from hPSCs
Crystal Chau and Selena Hallahan, STEMCELL Technologies

Aldevron – 11:15 AM - 12:00 PM

Meeting Global Demand for Critical Biologics
Kevin Ballinger, Michelle Berg, Tom Foti, and Ken Bonnell, Aldevron

MilliporeSigma – 11:15 AM - 12:00 PM

A Platform Standard for Viral Vector Manufacturing and Commercialization
Eva Fong and Jessica Hilmoe, MilliporeSigma

Schedule

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TUESDAY, MAY 11, 2021

12:00 PM - 1:00 PM

FIRESIDE CHAT

Jennifer Doudna, Ph.D.,
UC Berkeley

1:30 PM - 3:30 PM

MENTOR MEET-UP EVENT

Sponsored by:

CARIBOU BIOSCIENCES · DYNO THERAPEUTICS · rocket pharma · TERUMO BLOOD AND CELL TECHNOLOGIES

MilliporeSigma

Don't Miss the Following Presentations

Tuesday, May 11th

EXHIBITOR SHOWCASE
11:15 am–12:00 pm

A Platform Standard for Viral Vector Manufacturing and Commercialization

Eva Fong, Sr. Scientist and Jessica Hilmoe, Technical Leads Manager

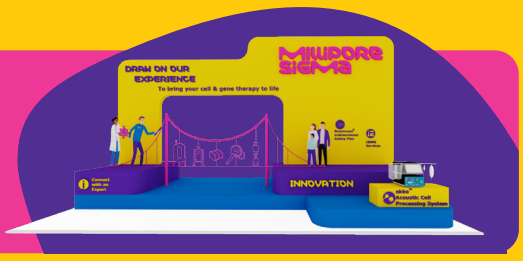
TOOLS & TECHNOLOGY FORUM
5:15–5:30 pm

Biosafety in Gene Therapy: Applying the Latest Regulatory Guidance for RCL Testing

Leyla Diaz, Ph.D., Principal Scientist

Explore the latest developments in cell & gene therapy

Explore Today



Schedule

ASGCT 2021 Virtual ANNUAL MEETING

ALL TIMES LISTED IN EDT

TUESDAY, MAY 11, 2021

2:00 PM - 3:30 PM

INDUSTRY SPONSORED SYMPOSIA

Corning Incorporated - Scaling AAV and Cell Production to Manufacturing Levels with Intensified Adherent Cell Culture Systems

CORNING

2:00 PM - 2:15 PM	**The Case for Scaling Up Adherent Cell Culture Systems** *Todd Upton, Ph.D., Corning Life Sciences*
2:15 PM - 2:45 PM	**The CDMO Perspective on Viral Vector Production - Challenges and Opportunities** *Plamena Kirova, Andelyn Biosciences*
2:45 PM - 3:15 PM	**Using a Scalable, Intensified Fixed Bed System for High-Yield Viral Vector Production** *Zara Melkoumian, Ph.D., Corning Life Sciences*

Maxcyte, Inc. - Building next generation engineered cell medicines - Leveraging the benefits of non-viral transfection in increasingly complex product development

MaxCyte®

Introduction
Sarah Haecker Meeks, Ph.D., MaxCyte, Inc.

2:00 PM - 2:10 PM	**NKARTA Therapeutics** *James Trager, Ph.D., NKARTA Therapeutics*
2:10 PM - 2:20 PM	**Genespire** *Julia Berretta, Ph.D., Genespire*
2:20 PM - 2:30 PM	**NHLBI** *John Tisdale, M.D., National Institutes of Health, NHLBI*
2:30 PM - 2:40 PM	**VOR Biopharma** *Sadik Kassim, Ph.D., VOR Biopharma*
2:40 PM - 2:50 PM	**Myeloid Therapeutics** *Daniel Getts, Ph.D., Myeloid Therapeutics*
2:50 PM - 3:30 PM	**Panel Discussion** *Moderated by Sarah Haecker Meeks, Ph.D., MaxCyte, Inc.*

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TUESDAY, MAY 11, 2021

2:00 PM - 3:30 PM

INDUSTRY SPONSORED SYMPOSIA

Sarepta Therapeutics, Inc. - AAVrh74 Gene Transfer Platform: Advancing Investigational Therapies for Patients with Duchenne & Limb Girdle Muscular Dystrophies

SAREPTA THERAPEUTICS

2:00 PM - 2:05 PM	**Welcome and Introduction** *Louise Rodino-Klapac, Ph.D., Sarepta Therapeutics, Inc.*
2:05 PM - 2:40 PM	**AAV Gene Transfer Therapy: Challenges and Future Directions** *Louise Rodino-Klapac, Ph.D., Sarepta Therapeutics, Inc.*
2:40 PM - 3:00 PM	**Duchenne Muscular Dystrophy Study 9001-102 Interim Findings** *Perry Shieh, M.D., Ph.D., David Geffen School of Medicine at UCLA, University of California*
3:00 PM - 3:20 PM	**Limb Girdle Muscular Dystrophy Study 9003-101 Interim Findings** *Erica Koenig, Ph.D., Sarepta Therapeutics, Inc.*
3:20 PM - 3:30 PM	**Closing Remarks & Q&A** *Moderated by Louise Rodino-Klapac, Ph.D.* *Panelists: Perry Shieh, M.D., Ph.D. and Erica Koenig, Ph.D.*

Terumo Blood and Cell Technologies - Combining flexibility and automation - key to resource maximization in a multi-product research facility

TERUMO BLOOD AND CELL TECHNOLOGIES

2:00 PM - 2:45 PM	**Optimizing Large-Scale Cell Expansion for iPS Cell-Based Applications** *Fernanda Mesquita, Ph.D., Texas Heart Institute*
2:45 PM - 3:30 PM	**The Role of Flexible Automation in Enabling THI's Research Initiatives** *Camila Hochman-Mendez, Ph.D., Texas Heart Institute*

Schedule

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TUESDAY, MAY 11, 2021

2:00 PM - 3:00 PM

CHAT LOUNGE NETWORKING

3:30 PM - 5:15 PM

EDUCATION SESSIONS

AAV Vectors From Basic Biology to Clinical Application and Back
Co-Chairs: Hildegard Buning, Ph.D., and Alberto Auricchio, M.D.

3:30 PM - 4:05 PM	**AAVs - What We Know 56 Years After Discovery** *Terence R. Flotte, Ph.D., University of Massachusetts Medical School*
4:05 PM - 4:40 PM	**Development Pipeline - AAV Vectors for Pompe/CNS** *Nathalie Cartier-Lacave, M.D., INSERM U1169*
4:40 PM - 5:15 PM	**Gene Therapy for Orphan Diseases** *Kathrin Meyer, Ph.D., Nationwide Children's Hospital*

Gene Therapy in Cancer
Co-Chairs: Rayne Rouce, M.D. and Renata Stripecke, Ph.D.

3:30 PM - 3:56 PM	**Strategy and Manufacturing of Cells Expressing CARs to Fight Cancer and the Tumor Microenvironment** *Cliona Rooney, Ph.D., Baylor College of Medicine*
3:56 PM - 4:22 PM	**Testing T Cells in Clinical Trials Against Liquid and Solid Cancer** *Marcela Maus, M.D., Ph.D., Massachusetts General Hospital*
4:22 PM - 4:48 PM	**Oncolytic Viral Therapies: Two Knives Against Cancer** *Paola Grandi, Ph.D., CG Oncology*
4:48 PM - 5:15 PM	**Systemic Immunogene Therapy for Cancer** *Jack Roth, M.D., University of Texas M.D. Anderson Cancer Center*

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3:30 PM - 5:15 PM

EDUCATION SESSIONS

In Vivo Gene Editing
Co-Chairs: Juliana Alvarez Argote, M.D. and Blythe Sather, Ph.D.

3:30 PM - 3:56 PM	**Introduction to In Vivo Gene Editing** *Paula Cannon, Ph.D., University of Southern California*
3:56 PM - 4:22 PM	**In Vivo Gene Editing With the Kamicas9 Self-Inactivating System** *Nicole Deglon, Ph.D., Lausanne University Hospital (CHUV)*
4:22 PM - 4:48 PM	**Gene and Epigenome Editing for Disease Therapies** *Juan Carlos Izpisua Belmonte, Ph.D., The Salk Institute for Biological Studies*
4:48 PM - 5:15 PM	**Safety Issues and Solutions for In Vivo Gene Editing** *William McKillop, Ph.D., Medical College of Wisconsin*

Issues in Gene Therapy: Considerations for Efficient Development and Access
Chair: John Tisdale, M.D.

3:30 PM - 4:05 PM	**Addressing Capacity Constraints to Viral Vector Manufacturing** *Sarah Yuan, Ph.D., bluebird bio*
4:05 PM - 4:40 PM	**Value and Access Considerations in Pricing Gene Therapies** *Sarah Pitluck, Spark Therapeutics*
4:40 PM - 5:15 PM	**Patient Perspective: The Value of Gene Therapy** *Charles Hough*

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TUESDAY, MAY 11, 2021

3:30 PM - 5:15 PM

SCIENTIFIC SYMPOSIA

Next Generation CAR T-Cell Therapies (Joint Session with ASTCT)
Co-Chairs: Hans-Peter Kiem M.D., Ph.D. and John DiPersio, M.D., Ph.D.

3:35 PM - 3:55 PM	**Engineering Human Pluripotent Stem Cells to Produce NK Cells With Improved Anti-Tumor Activity** *Dan Kaufman, M.D., Ph.D., University of California - San Diego*
3:55 PM - 4:15 PM	**INKt-CAR** *Rob Negrin, M.D., Stanford University*

Emerging Pluripotent Cell-Based Therapies (Organized by the Stem Cell Committee)
Co-Chairs: Punam Malik, M.D. and Masatoshi Suzuki, Ph.D., D.V.M.

3:30 PM - 3:56 PM	**hESC-Derived Dopaminergic Neurons for Parkinson's** *Lorenz Studer, M.D., Memorial Sloan Kettering Cancer Center*
3:56 PM - 4:22 PM	**Clinical Data on iPSC-Derived RPE for Macular Degeneration** *Masayo Takahashi, M.D., Ph.D., Vison Care Inc., Kobe Eye Center*
4:22 PM - 4:48 PM	**Hypoimmunogenic iPSCs** *Sonja Schrepfer, M.D., Ph.D., Sana Biotechnology*
4:48 PM - 5:15 PM	**CRISPR Genome Editing to Generate Immune-Compatible iPSCs** *Akitsu Hotta, Ph.D., CiRA, Kyoto University*

Immunological Complications and Solutions in HCT (Organized by the Hematologic & Immunologic Gene and Cell Therapy Committee)
Co-Chairs: Olivier Humbert, Ph.D. and Isabelle Riviere, Ph.D.

3:30 PM - 3:56 PM	**Overview of Immune Complications In HCT** *John Wagner, M.D., University of Minnesota*
3:56 PM - 4:22 PM	**Complement Responses in HCT** *Eleni Gavriilaki, M.D., Ph.D., George Papanicolaou Hospital*
4:22 PM - 4:48 PM	**Engineering Tregs** *Megan Levings, Ph.D., University of British Columbia*
4:48 PM - 5:15 PM	**Impact of Pre-Existing Transgene Product Immunity in Engraftment of Gene Modified HSC** *H. Trent Spencer, Ph.D., Emory University School of Medicine*

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3:30 PM - 5:15 PM

PLENARY SESSIONS

Career Development Award Presentations

3:50 PM - 4:07 PM	**Improved Strategies for Site-Specific Gene Insertion Using Non-Homologous End Joining** *Geoffrey Rogers, Ph.D., University of Southern California*
4:24 PM - 4:41 PM	**Development of Non-Viral Mechanisms of Targeting Lung Epithelial Cells for Prenatal Gene Editing in a Large Animal Model** *Kshitiz Singh, Ph.D., Children's Hospital of Philadelphia*
4:41 PM - 4:58 PM	**Characterizing and Overcoming the Host Response to Genome Editing Therapy** *Christopher Nelson, Ph.D., University of Arkansas*
4:58 PM - 5:15 PM	**Engineered CAR-T Cells to Overcome Alloimmunity in Transplant Rejection** *Kalpana Parvathaneni, Ph.D., University of Pennsylvania*

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TUESDAY, MAY 11, 2021

5:15 PM - 6:45 PM

EXHIBIT HALL OPEN - EXHIBITORS AVAILABLE TO CONNECT

Exhibit Hall is Accessible 24 Hours

5:30 PM - 7:30 PM

ABSTRACT SESSIONS

Advances in Ex Vivo Modified Cell Therapies
Co-Chairs: Adrian Gee, Ph.D. and Joseph Gold, Ph.D.

5:30 PM - 5:45 PM	**1: Development of an Optimized Lentiviral Transduction Process for Ex Vivo CD34+ Hematopoietic Stem Cell Gene Therapy Drug Product Manufacture** *Pervinder Sagoo, Ph.D., Orchard Therapeutics Europe Ltd.*
5:45 PM - 6:00 PM	**2: Non-Viral Integration of Large Cargo in Primary Human T Cells by CRISPR/Cas9 Guided Homology Mediated End Joining** *Matthew Johnson, Ph.D., University of Minnesota*
6:00 PM - 6:15 PM	**3: Epigenetic Modulation of Aging to Increase CAR-T Cell Fitness** *Benedetta Nicolis di Robilant, Ph.D., Dorian Therapeutics*
6:15 PM - 6:30 PM	**4: Effective and Efficient Intracellular Delivery Achieved with the Cell Squeeze® Technology Enables Rapid, Scaled, and Reproducible Production of Cell Therapies** *David Chirgwin, SQZ Biotechnologies*
6:30 PM - 6:45 PM	**5: Sequential CRISPR-mediated Engineering and Clonal Banking for the Generation of Multiplexed Engineered Master Pluripotent Cell Lines for the Mass Manufacture of Off-the-Shelf Immune Cells Targeting Solid Cancers** *Ramzey Abujarour, Ph.D., Fate Therapeutics*
6:45 PM - 7:00 PM	**6: Generation of Engineered Tregs (EngTregs) from Umbilical Cord Blood Derived CD4+ T Cells via HDR-Dependent FOXP3 Gene Editing** *Shivani Patel, Seattle Children's Research Institute*
7:00 PM - 7:15 PM	**7: VOR33: A Clinic-Ready CRISPR/Cas9 Engineered Hematopoietic Stem Cell Transplant for the Treatment of Acute Myeloid Leukemia** *John Lydeard, Ph.D., Vor Biopharma*

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5:30 PM - 7:30 PM

ABSTRACT SESSIONS

Cancer - Oncolytic Viruses
Co-Chairs: Paola Grandi, Ph.D. and Melissa Kotterman, Ph.D.

5:30 PM - 5:45 PM	**8: Preclinical Toxicology Assessment of an Oncolytic Measles Virus Armed With H. pylori Immunostimulatory Bacterial Antigen in Preparation for a Phase I Trial in Breast Cancer Patients** *Kimberly Viker, Mayo Clinic*
5:45 PM - 6:00 PM	**9: Validating Secreted IFNβ as an In Vivo Biomarker of Intratumoral Replication of VSV-IFNβ-NIS** *Lianwen Zhang, Mayo Clinic*
6:00 PM - 6:15 PM	**10: Development of Novel Oncolytic Vector Based on Alternative Adenovirus Serotype 6 for Glioblastoma and Breast Cancer Therapy** *Margarita Romanenko, Ph.D., University of Minnesota*
6:15 PM - 6:30 PM	**11: Human Cytomegalovirus Engineered for Glioma Therapy** *Haifei Jiang, M.D., Ph.D., Mayo Clinic*
6:30 PM - 6:45 PM	**12: Virulent Velogenic Newcastle Disease Virus is More Oncolytic Than Attenuated and Lentogenic Newcastle Viruses** *Ahmed Majeed Al-Shammari, Ph.D., Mustansiriyah University, Iraqi Center for Cancer and Medical Genetic Research*
6:45 PM - 7:00 PM	**13: Generation and Characterization of Replication-Competent Oncolytic Foamy Virus Vectors** *Karol Budzik, Mayo Clinic*
7:00 PM - 7:15 PM	**14: An Oncolytic Adenoviral Vector Expressing an Anti-PD-L1 scFv Reduces Tumor Growth in a Melanoma Mouse Model** *Maria Vitale, University of Naples*

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5:30 PM - 7:30 PM

ABSTRACT SESSIONS

Delivery Technologies and CRISPR for Therapeutics
Co-Chairs: Nicole Gaudelli, Ph.D. and Alejandro Chavez, M.D., Ph.D.

5:30 PM - 5:45 PM	**15: CRISPR/Cas9-Mediated Targeted Gene Insertion Platform Achieves Durable, Normal Human Alpha-1 Antitrypsin Protein Levels in Non-Human Primates** *Sean Burns, M.D., Intellia Therapeutics*
5:45 PM - 6:00 PM	**16: Direct rAAV-Mediated In Vivo Gene Editing of Hematopoietic Stem Cells** *Ishani Dasgupta, Ph.D., University of Massachusetts Medical School*
6:00 PM - 6:15 PM	**17: CRISPR-Cas9 Genome Editing of Human CD34+ Cells at Gamma-Globin Promoter to Induce Fetal Hemoglobin as Sickle Cell Disease Therapy** *Varun Katta, St. Jude Children's Research Hospital*
6:15 PM - 6:30 PM	**18: In Utero Lipid Nanoparticle Delivery of CRISPR Technology to Correct Hereditary Tyrosinemia Type 1** *Kshitiz Singh, Ph.D., Children's Hospital of Philadelphia*
6:30 PM - 6:45 PM	**19: Correction of DMD Mutations in Human iPS-Derived Muscle Cells by Single-Cut CRISPR/Cas9-Based Gene Editing** *Ziad Al Tanoury, Ph.D., Vertex Cell and Genetic Therapies*
6:45 PM - 7:00 PM	**20: Cell-Based Delivery Strategies for Artificial Transcription Factors in Preclinical Animal Models** *Peter Deng, Ph.D., UC Davis*

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5:30 PM - 7:30 PM

ABSTRACT SESSIONS

Development of AAV Capsid Variants
Co-Chairs: Deep Bhattacharya, Ph.D. and Christine Le Bec, Ph.D.

5:30 PM - 5:45 PM	**22: A Novel Liver-Tropic AAV Capsid sL65 Shows Superior Transduction and Efficacy in Humanized Mice and Non-Human Primates** *Jing Liao, Ph.D., LogicBio Therapeutics*
5:45 PM - 6:00 PM	**23: Efficient Design of Optimized AAV Capsids Using Multi-Property Machine Learning Models Trained Across Cells, Organs and Species** *Eric Kelsic, Ph.D., Dyno Therapeutics*
6:00 PM - 6:15 PM	**24: Risk-Adjusted Selection for Validation of Sequences in AAV Design Using Composite Sampling** *Lauren Wheelock, Ph.D., Dyno Therapeutics*
6:15 PM - 6:30 PM	**25: A Comparison of Methods Used for the Determination of Full and Empty rAAV Particles** *Bryan Troxell, Ph.D., StrideBio, Inc.*
6:30 PM - 6:45 PM	**26: A Tetracycline Enabled Self-Silencing Adenovirus (TESSA) Platform Delivers High-Quality, High-Titre, Multi-Serotype Recombinant Adeno-Associated Virus (AAV) Stocks** *Maria Patricio, Ph.D., Oxgene*
6:45 PM - 7:00 PM	**27: Next Generation AAV Drug Products: Enhanced Stability & Clinical Ease for High Titer Preparations** *Lori Karpes, Ph.D., Homology Medicines*

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5:30 PM - 7:30 PM

ABSTRACT SESSIONS

Gene Therapy for Inborn Errors of Metabolism
Co-Chairs: Giuseppe Ronzitti, Ph.D. and Gloria Gonzalez-Aseguinolaza, Ph.D.

5:30 PM - 5:45 PM	**29: Coadministration of AAV Expressing M.D.R3 (VTX-803) and ImmTOR Allows for Vector Re-Administration to Treat Progressive Familial Intrahepatic Cholestasis Type 3 (PFIC3) in Juvenile Mice** *Nicholas Weber, Ph.D., Vivet Therapeutics*
5:45 PM - 6:00 PM	**30: Preclinical Evaluation of Combined Adeno-Associated Virus and Nanoparticle Delivery of piggyBac® Transposon System for Durable Transgene Expression in the Growing Neonatal Murine Liver** *Jingjing Jiang, Ph.D., Poseida Therapeutics*
6:00 PM - 6:15 PM	**31: Targeting Aberrant Acylation as a Novel Approach for Treating Methylmalonic Acidemia (MMA) and Related Other Organic Acidemias** *Sangho Myung, National Institutes of Health, NHGRI*
6:15 PM - 6:30 PM	**32: AAV Liver Gene Therapy-mediated Inhibition Of FGF23 Signaling as a Therapeutic Strategy for X-linked Hypophosphatemia** *Giuseppe Ronzitti, Ph.D., Genethon*
6:30 PM - 6:45 PM	**33: Comparison of Gene Addition Therapy in Genetically Distinct Mouse Models of Classical Phenylketonuria** *Daelyn Richards, Ph.D., Oregon Health and Science University*
6:45 PM - 7:00 PM	**34: AAV8 Gene Therapy as a Potential Treatment in Adults with Late-Onset Ornithine Transcarbamylase (OTC) Deficiency: Updated Results from a Phase 1/2 Clinical Trial** *Cary Harding, M.D., Oregon Health and Science University*
7:00 PM - 7:15 PM	**35: AAV-Mediated Delivery of MiRNA-34B/C Improves Liver Fibrosis** *Pasquale Piccolo, Ph.D., Telethon Institute of Genetics and Medicine*

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TUESDAY, MAY 11, 2021

5:30 PM - 7:30 PM

ABSTRACT SESSIONS

Genetic Blood and Immune Disorders
Co-Chairs: Denise Sabatino, Ph.D. and Cyndi Dunbar, M.D.

5:30 PM - 5:45 PM	**36: Follow-Up of a Phase I/II Gene Therapy Trial in Patients with Fanconi Anemia, Subtype A** *Juan Bueren, Ph.D., CIEMAT/CIBERER/IIS-FJD, UAM*
5:45 PM - 6:00 PM	**37: Liver Gene Therapy with Lentiviral Vectors Corrects Hemophilia A in Mice and Achieves Normal-Range Factor VIII Activity in Non-Human Primates** *Michela Milani, Ph.D., San Raffaele Telethon Institute for Gene Therapy*
6:00 PM - 6:15 PM	**38: Towards Clinical Translation of Hematopoietic Cell Gene Editing for Treating Hyper-IgM Type 1** *Valentina Vavassori, Ph.D., San Raffaele Telethon Institute for Gene Therapy; Vita-Salute San Raffaele University*
6:15 PM - 6:30 PM	**39: A Phase 1/2 Study of Lentiviral-Mediated Ex-Vivo Gene Therapy for Pediatric Patients with Severe Leukocyte Adhesion Deficiency-I (LAD-I): Interim Results** *Donald Kohn, M.D., UCLA*
6:30 PM - 6:45 PM	**40: Autologous Ex Vivo Lentiviral Gene Therapy for the Treatment of ADA-SCID** *Claire Booth, Ph.D., UCL GOSH Institute of Child Health*
6:45 PM - 7:00 PM	**41: Efficient Ex-Vivo Selection of Gene Edited Human Hematopoietic Stem/Progenitor Cells** *Martina Fiumara, San Raffaele Telethon Institute for Gene Therapy; Vita-Salute San Raffaele University*
7:00 PM - 7:15 PM	**42: Targeted Genome Editing of Hematopoietic Stem Cells for Treating Recombination Activating Gene 1 (RAG1) Immunodeficiency** *Maria Carmina Castiello, Ph.D., San Raffaele Telethon Institute for Gene Therapy*

Schedule

ASGCT 2021 Virtual ANNUAL MEETING

ALL TIMES LISTED IN EDT

TUESDAY, MAY 11, 2021

5:30 PM - 7:30 PM

ABSTRACT SESSIONS

Musculo-Skeletal Diseases
Co-Chairs: Olivier Danos, Ph.D. and Rita Perlingeiro Ph.D.

5:30 PM - 5:45 PM
43: The Long-Term Efficiency of the scAAV. U7.ACCA Vector in Inducing Dystrophin Expression in Adult Dup2 Mice
Liubov Gushchina, Ph.D., Nationwide Children's Hospital

5:45 PM - 6:00 PM
44: Towards an Off-the-Shelf Cell Therapy for Bone Healing: Use of an Immortalized, Genetically Modified Cell Line as a Proof of Concept
Rodolfo De la Vega, M.D., Mayo Clinic

6:00 PM - 6:15 PM
45: Correction of Clcn1 Mis-Splicing Reverses Muscle Fiber Type Transition in Mice with Myotonic Dystrophy
Ningyan Hu, Massachusetts General Hospital

6:15 PM - 6:30 PM
46: Characterization of Acute Toxicity After High-Dose Systemic Adeno-Associated Virus in Nonhuman Primates, Including Impact of Vector Characteristics
Juliette Hordeaux, Ph.D., University of Pennsylvania

6:30 PM - 6:45 PM
47: Long-Term Hematopoietic Stem Cell Lentiviral Gene Therapy Corrects Neuromuscular Manifestations in Preclinical Study of Pompe Mice
Niek van Til, Ph.D., AVROBIO, Inc.; Vrije Universiteit and Amsterdam Neuroscience

6:45 PM - 7:00 PM
48: Downregulation of the Genetic Modifier PITPNA as Means of Therapy in Duchenne Muscular Dystrophy
Matthias Lambert, Ph.D., Boston Children's Hospital

7:00 PM - 7:15 PM
49: Non-Genotoxic Conditioning to Increase Gene Therapy Safety in a Rare Bone Disease
Valentina Capo, Ph.D., IRCCS San Raffaele Scientific Institute; CNR-IRGB

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5:30 PM - 7:30 PM

ABSTRACT SESSIONS

Novel AAV Capsids for Brain, Eye and Muscle Tissues
Co-Chairs: Nicole Paulk, Ph.D. and Daniel Lipinski, D.Phil.

5:30 PM - 5:45 PM	**50: Endothelial-Tropic AAVs for Genetic Access to Whole-Brain Vasculature in Wild-Type Mouse Strains following Non-Invasive Systemic Delivery** *Xinhong Chen, Caltech*
5:45 PM - 6:00 PM	**51: RNA-Driven Evolution of AAV Capsid Libraries Identifies Variants with High Transduction Efficiency in Non-Human Primate Central Nervous System** *Mathieu Nonnenmacher, Ph.D., Voyager Therapeutics*
6:00 PM - 6:15 PM	**52: Expanding the Utility of Intravitreal AAV via a Capsid Variant That Overcomes Neutralization by Anti-AAV2 NAbs in Human Vitreous** *Siddhant Gupte, University of Florida*
6:15 PM - 6:30 PM	**53: Breaking Thru the Human Blood Brain Barrier: Discovering AAV Vectors Targeting the Central Nervous System Using a Transwell Model** *Ren Song, Ph.D., Stanford University School of Medicine*
6:30 PM - 6:45 PM	**54: Expanding the AAV Toolbox for Cerebellar Transduction: Identifying and Characterizing Novel Variants in Non-Human Primates and Mice** *Megan Keiser, Ph.D., Children's Hospital of Philadelphia*
6:45 PM - 7:00 PM	**55: Capsid Display of Cell-Penetrating Peptides Yields AAVs with Enhanced Brain Penetration in Both Rodents and Primates** *Fengfeng Bei, Ph.D., Harvard Medical School*
7:00 PM - 7:15 PM	**56: Engineering AAV6-Based Vectors for Improved Ocular Transduction Following Intravitreal and Intracameral Injection** *Sean Crosson, Ph.D., University of Florida*

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5:30 PM - 7:30 PM

ABSTRACT SESSIONS

Preclinical Gene Therapy for Neurologic Diseases I
Co-Chairs: Ana Rita Batista, Ph.D. and Martin Hicks, Ph.D.

5:30 PM - 5:45 PM	**57: ST3GAL5 Gene Replacement in CNS Restores Gangliosides Production and Improves Survival in a Mouse Model of GM3 Synthase Deficiency** *Huiya Yang, University of Massachusetts Medical School*
5:45 PM - 6:00 PM	**58: CRISPR/Cas9 Strategies to Treat Spinocerebellar Ataxia Type 1** *Kelly Fagan, University of Pennsylvania*
6:00 PM - 6:15 PM	**59: Rescue of Molecular and Motor Phenotypes in CGG Knock-In Mice With CRISPR Mediated Deletion of the Trinucleotide Repeat** *Carolyn Yrigollen, Ph.D., Children's Hospital of Philadelphia*
6:15 PM - 6:30 PM	**60: Transthyretin Gene Therapy as a Modulator of Alzheimer's Disease Progression** *Ana Rita Batista, Ph.D., University of Massachusetts Medical School*
6:30 PM - 6:45 PM	**61: CRISPR/Cas9-Mediated Excision of ALS/FTD-causing Hexanucleotide Repeat Expansion in C9ORF72 Rescues Major Disease Mechanisms n Vivo and In Vitro** *Katharina Meijboom, D.Phil., University of Massachusetts Medical School*
6:45 PM - 7:00 PM	**62: C9ORF72 Variant-Specific RNA Interference Rescues C9-ALS/FTD Molecular Hallmarks In Vivo and In Vitro** *Katharina Meijboom, D.Phil., University of Massachusetts Medical School*
7:00 PM - 7:15 PM	**63: Restoration of Scn1a Expression after Symptom Onset in a Novel Model of Dravet Syndrome Rescues Seizures and Behavioral Alterations** *Gaia Colasante, Ph.D., Ospedale San Raffaele*

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TUESDAY, MAY 11, 2021

5:15 PM - 6:45 PM

INDUSTRY SYMPOSIA

Precision for Medicine - Considerations for the Clinical Development of Cell & Gene Therapies
Moderator: David Parker, Ph.D., Precision for Medicine

PRECISION for medicine

5:15 PM - 6:00 PM	**Cell Therapy** *Megan Liles, Precision for Medicine; John Khoury, Project Farma; Alex Grosvenor, Precision Value & Health; Joachim Fruebis, BlueRock Therapeutics; Osvaldo Flores, Century Therapeutics; and Sadik Kassim, VOR Biopharma*
6:00 PM - 6:45 PM	**Gene Therapy** *Deborah Phippard, Precision for Medicine; Tony Khoury, Project Farma; Phil Cyr, Precision Value & Health; Tim Kelly, Asklepios Biopharmaceutical Inc. (AskBio); Ottavio Vitolo, Alcyone Therapeutics; and Steven Zelenkofske, SwanBio Therapeutics*

Thermo Fisher Scientific - Scalable AAV manufacturing – addressing challenges across the workflow
Moderator: Natasha Lucki, Ph.D., Thermo Fisher Scientific

ThermoFisher SCIENTIFIC

5:15 PM - 5:45 PM	**Scalable, High-Titer, Simplified AAV Production in the AAV-MAX Helper Free AAV Production System** *Chao Yan Liu, Ph.D., Thermo Fisher Scientific*
5:45 PM - 6:15 PM	**Benefits of Using a Media Panel to Address the Diversity of HEK293 Cell Lines** *Gino Stolfa, Ph.D., Thermo Fisher Scientific*
6:15 PM - 6:45 PM	**CGT Regulatory Landscape and Virtual Inspections** *Monica Commerford, Ph.D., Thermo Fisher Scientific*

5:15 PM - 6:15 PM

Networking Roulette

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Schedule

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TUESDAY, MAY 11, 2021

5:15 PM - 7:00 PM

TOOLS AND TECHNOLOGIES FORUM

Tools and Technology Forum I

5:15 PM - 5:30 PM	**Biosafety in Gene Therapy: Applying the Latest Regulatory Guidance for RCL Testing** *Leyla Diaz, Ph.D., MilliporeSigma*	MilliporeSigma
5:30 PM - 5:45 PM	**Design, Manufacturing and Analytics of New AAV Reference Materials - A Case Study** *Jeffrey Hung, Ph.D., Vigene Biosciences*	Vigene Biosciences Excellence in Gene Delivery
5:45 PM - 6:00 PM	**Host Cell Protein Analytics in Viral Vector Manufacturing** *Alla Zilberman, Cygnus Technologies*	CYGNUS TECHNOLOGIES part of Maravai LifeSciences
6:00 PM - 6:15 PM	**Mass Photometry - A New Tool to Study Biomolecules** *Gabriella Kiss, Refeyn*	REFEYN WEIGHING MOLECULES WITH LIGHT
6:15 PM - 6:30 PM	**Transient Transfection at Large-Scale for Clinical AAV9 Vector Manufacturing** *Denis Kole, Pall Corporation*	PALL CORPORATION
6:30 PM - 6:45 PM	**Videodrop: Rapid Characterization of Lentiviral Vectors in a Droplet for a Better Bioproduction Follow-Up** *Marie Berger, PharM.D., MYRIADE*	myriade
6:45 PM - 7:00 PM	**Trends in Cell and Gene Therapy: De-risking Platform and Product Development with Mass Spectrometry** *Chen Li, BioAnalytix*	BioAnalytix A Protagen Protein Services Company

Schedule

ASGCT 2021 Virtual ANNUAL MEETING

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WEDNESDAY, MAY 12, 2021

ALL DAY

DIGITAL ABSTRACT PRESENTATIONS

EXHIBIT HALL

Be sure to connect with exhibit booth staff during listed times

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9:00 AM - 10:00 AM

CHAT LOUNGE NETWORKING

10:00 AM - 11:45 AM

SCIENTIFIC SYMPOSIA

Building Your Elevator Pitch (Organized by the Communications Committee)
Chair: Edith Pfister, Ph.D.

10:00 AM - 10:26 AM	**5 Steps for a Polished Grant Submission**	*Kelly Turner, Ph.D., Baylor College of Medicine*
10:26 AM - 10:52 AM	**Advice for Early-Stage Cell Therapy Professionals**	*Rayne Rouce, M.D., Baylor College of Medicine*
10:52 AM - 11:18 AM	**Communicating Your Research to Investors**	*Manisha Pai, Vertex Pharmaceuticals*
11:18 AM - 11:45 AM	**Communicating Complex Science With a Public Audience**	*Roxanne Khamsi, Freelance Journalist*

Schedule

ASGCT 2021 Virtual ANNUAL MEETING

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WEDNESDAY, MAY 12, 2021

10:00 AM - 11:45 AM

SCIENTIFIC SYMPOSIA

Cutting Edge Gene and Cell Therapy Research in Europe (Organized by ESGCT)
Co-Chairs: Juan Bueren, Ph.D. and Alberto Auricchio, M.D.

10:00 AM - 10:26 AM	**AAV Capsid Engineering for In Vivo Gene Therapy** *Hildegard Büning, Ph.D., Hannover Medical School*
10:26 AM - 10:52 AM	**Dressing Viruses in Tumors' Clothing: Cloning-Free Platforms to Trigger Tumor-Specific Immune Response** *Vincenzo Cerullo, Ph.D., University of Helsinki*
10:52 AM - 11:18 AM	**LV-Mediated Gene Therapy of Pyruvate Kinase Deficiency** *Jose-Carlos Segovia, Ph.D., Centro de Investigaciones Energéticas Medioambientales y Tecnológicas (CIEMAT) and Centro de Investigación Biomédica en Red de Enfermedades Raras (CIBERER)*
11:18 AM - 11:45 AM	**Liver-Directed Gene Therapy Clinical Trial for Mucopolysaccharidosis VI** *Nicola Brunetti-Pierri, M.D., Telethon Institute of Genetics and Medicine*

Entering and Thriving in Industry: Guidance for Academic, Clinical, and Industry Professionals (Organized by the Bio-Industry Committee)
Co-Chairs: Bartholomew Tortella, M.D. and Steven Howe, Ph.D.

10:00 AM - 10:15 AM	**Preparing For a Career In Industry: What New Entrants Need to Know** *Nathaniel Berendson, GlaxoSmithKline (GSK)*
10:15 AM - 10:30 AM	**When to Spin Out: One Academic Perspective** *Matthew Porteus, M.D., Ph.D., Stanford University School of Medicine*
10:30 AM - 10:45 AM	**Attracting, Developing, and Retaining the Best Young Academic Science Talent: A Guide for Those in Industry** *Kate Barclay, Ph.D., UK BioIndustry Association*
10:45 AM - 11:00 AM	**Life in The Industry Matrix: Journeying Through the Industry Professional Environment** *Freda Lewis-Hall, M.D., Pfizer (retired)*
11:00 AM - 11:45 AM	**Panel Discussion**

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WEDNESDAY, MAY 12, 2021

10:00 AM - 11:45 AM

SCIENTIFIC SYMPOSIA

Immunological Barriers to Gene Therapy: Are They Surmountable? (Organized by the Immune Responses to Gene & Cell Therapy Committee)
Co-Chairs: Maria Castro, Ph.D. and Roberto Calcedo, Ph.D.

10:00 AM - 10:35 AM	**Complement Activation Following AAV Gene Delivery** *Pascal Deschatelets, Ph.D., Apellis Pharmaceuticals*
10:35 AM - 11:10 AM	**Cytokine Storm in Response to Gene and Cell Transfer** *Stephan Grupp, M.D., Ph.D., University of Pennsylvania Perelman School of Medicine*
11:10 AM - 11:45 AM	**CARs and Armoured CARs: Improving CAR T Cell Therapy for Cancer** *Renier Brentjens, M.D., Ph.D., Memorial Sloan-Kettering Cancer Center*

Novel Viral Gene Transfer Vectors and Applications
(Organized by the Viral Gene Transfer Vectors Committee)
Co-Chairs: Masato Yamamoto, M.D., Ph.D. and Phillip Tai, Ph.D.

10:00 AM - 10:26 AM	**Novel Poxvirus Vectors** *Takafumi Nakamura, Ph.D., Tottori University*
10:26 AM - 10:52 AM	**Induced Pluripotent Stem Cells Using Single-Cycle Measles Virus Vector** *Patricia Devaux, Ph.D., Mayo Clinic*
10:52 AM - 11:18 AM	**HSV Delivery of Genetic Circuits** *Ron Weiss, Ph.D., Massachusetts Institute of Technology*
11:18 AM - 11:45 AM	**In Vivo Hematopoietic Stem Cell Gene Therapy With Hdad5/35++ Adenovirus Vectors** *Andre Lieber, M.D. Ph.D., University of Washington*

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10:00 AM - 11:45 AM

SCIENTIFIC SYMPOSIA

Regulatory Lessons Learned From COVID-19: Anomaly to Precedent
(Organized by the Regulatory Affairs Committee)
Co-Chairs: Kit Shaw, Ph.D. and S. Kaye Spratt, Ph.D.

10:00 AM - 10:15 AM	**EMA Experience on How Emergency Measures Will Inform Flexibility for Gene Therapies** *Emer Cooke, European Medicines Agency*
10:15 AM - 10:30 AM	**Clinical Trial Efficacy Assessments During the Covid-19 Pandemic** *Wilson Bryan, M.D., Food and Drug Administration*
10:30 AM - 10:45 AM	**Creating a Remote Data-Collection Paradigm in a Rare Pediatric Disease: A Case Study** *Genevieve Laforet, M.D., Ph.D., Aspa Therapeutics*
10:45 AM - 11:00 AM	**Incorporating Regulatory Guidance to Provide Continuity for Ongoing Clinical Trials** *Jonathan Cotliar, M.D., Science37*
11:00 AM -11:45 AM	**Panel Discussion**

Toxicities and Limitations of Gene Therapy
(Organized by the Gene & Cell Therapy of Genetic and Metabolic Diseases Committee)
Co-Chairs: Paris Margaritis, D.Phil. and Moanaro Biswas, Ph.D.

10:00 AM - 10:26 AM	**Mtm Patient Deaths** *Carsten Bonnemann, M.D., National Institutes of Health, NINDS*
10:26 AM - 10:52 AM	**The Challenges of DMD Gene Therapy Clinical Trials** *Michael Binks, M.D., Pfizer Worldwide Research, Development and Medical*
10:52 AM - 11:18 AM	**Adeno-Associated Virus-Related Toxicities in Nonhuman Primates** *Juliette Hordeaux, D.V.M., Ph.D., Gene Therapy Program, University of Pennsylvania*
11:18 AM - 11:45 AM	**Hemophilia - Addressing Durability and Variability of Gene Therapy** *Glenn Pierce, M.D., Ph.D., Third Rock Ventures*

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10:00 AM - 11:45 AM

SCIENTIFIC SYMPOSIA

Translational Gene and Cell Therapy Studies in Cardiovascular Medicine
(Organized by Cardiovascular Gene & Cell Therapy Committee)
Co-Chairs: Sangeetha Vadakke-Madathil, Ph.D. and Margaret Sleeper, V.M.D.

10:00 AM - 10:26 AM	**Gene Therapy With Cyclin-A2 in Pigs** *Hina Chaudhry, M.D., Mount Sinai Hospital*
10:26 AM - 10:52 AM	**Gene Therapy of Myosin Binding Protein C in Hypertrophic Cardiomyopathy** *Julian Stelzer, Ph.D., Case Western Reserve University*
10:52 AM - 11:18 AM	**Cardiac Regenerative Strategies** *Eldad Tzahor, Ph.D., Weizmann Institute of Science*
11:18 AM - 11:45 AM	**Cardiac Phosphoinositide 3-Kinase (P110A) as a Therapeutic Target for Diabetic Cardiomyopathy - An AAV-Mediated Gene Therapy Approach** *Rebecca Ritchie, Ph.D., Monash University*

10:00 AM - 11:45 AM

SPECIAL SYMPOSIUM

JERRY MENDELL AWARD FOR TRANSLATIONAL SCIENCE SYMPOSIUM

Translational Science Overcomes Obstacles for Treatment of Children With Neuromuscular Disease

Supported by Dr. Suku and Ann Nagendran

Jerry Mendell, M.D., Nationwide Children's Hospital

Schedule

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WEDNESDAY, MAY 12, 2021

10:45 AM - 12:15 PM

EXHIBIT HALL OPEN - EXHIBITORS AVAILABLE TO CONNECT

Exhibit Hall is Accessible 24 Hours

10:45 AM - 12:15 PM

EXHIBITOR SHOWCASES

Unchained Labs - 10:45 AM - 11:30 AM

Get Hassle-Free AAV & LNP Sample Prep and Characterization From Unchained Labs' Gene Therapy Squad
Kevin Lance, Ph.D., Unchained Labs

Wyatt Technology - 10:45 AM - 11:30 AM

Use DLS, SEC-MALS and FFF-MALS towards Well Characterized Gene Vectors
Michelle Chen, Ph.D.; Bob Collins; and Eric Seymour, Wyatt Technology

Bio-Rad Laboratories - 11:30 AM - 12:15 PM

Implementing Droplet Digital PCR in Clinical Trial Testing
Mark Wissel, Ph.D., Eurofins Viracor BioPharma Services and Tara Ellison, Ph.D., Bio-Rad Laboratories

Halo Labs - 11:30 AM - 12:15 PM

Introducing the Aura CL for Cell and Gene Therapy Aggregation, Particle Analysis and ID
Bernardo Cordovez, Ph.D., Halo Labs

12:15 PM - 2:00 PM

GEORGE STAMATOYANNOPOULOS MEMORIAL LECTURE AND PRESENTATION OF THE EXCELLENCE IN RESEARCH AWARDS

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Schedule

ASGCT 2021 Virtual ANNUAL MEETING

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WEDNESDAY, MAY 12, 2021

2:00 PM - 3:30 PM

INDUSTRY SPONSORED SYMPOSIA

EXHIBIT HALL OPEN - EXHIBITORS AVAILABLE TO CONNECT

Exhibit Hall is Accessible 24 Hours

Cytiva - Platform processing for AAV production

2:00 PM - 3:30 PM — *Mats Lundgren, Ph.D., Cytiva*

Miltenyi Biotec - Latest Advances in Cell and Gene Therapy

2:00 PM - 2:05 PM — **Introduction**
Matthew Porteus, M.D., Ph.D., Stanford University School of Medicine

2:05 PM - 2:30 PM — **Development of WU-NK-101, an Off-the-Shelf Memory NK Cell Therapy for the Treatment of AML**
Kenneth Chrobak, Ph.D., Wugen

2:30 PM - 2:55 PM — **In Vivo and In-Vitro Characterization of MART-1 Specific T Cells Generated Using the AIM Technology and Prodigy System**
Ruipeng Wang, NexImmune

2:55 PM - 3:20 PM — **Manufacturing Genome Edited Hematopoietic Stem Cells: From Now to the Future**
Matthew Porteus, M.D., Ph.D., Stanford University School of Medicine

Schedule

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2:00 PM - 3:30 PM

INDUSTRY SPONSORED SYMPOSIA

Vertex Pharmaceuticals Inc. - Restoring and Replacing: Strategies for Potential New Treatment Approaches

2:00 PM - 2:05 PM	**Welcome and Introduction** *Bastiano Sanna, Ph.D., Vertex Pharmaceuticals Inc.*
2:05 PM - 2:25 PM	**Reviewing the Latest Advances in Genetic and Cell Therapy Technologies** *Bastiano Sanna, Ph.D., Vertex Pharmaceuticals Inc.*
2:25 PM - 2:45 PM	**Restoring at the Molecular Level: CRISPR/Cas9-Mediated Gene Editing Technology** *Eric N. Olson, Ph.D., UT Southwestern Medical Center*
2:45 PM - 3:05 PM	**Replacement at the Cellular Level: Transplantable Stem Cell-Derived Technology** *Douglas A. Melton, Ph.D., Harvard University*
3:05 PM - 3:30 PM	**Panel Discussion**

Voyager Therapeutics - Advancing AAV Gene Therapy for CNS Disease

2:00 PM - 2:05 PM	**Welcome and Introduction** *Omar Khwaja, M.D., Ph.D., Voyager Therapeutics*
2:05 PM - 2:15 PM	**Voyager Therapeutics' Commitment to Advancing AAV Gene Therapy for CNS Disease** *Andre Turenne, Voyager Therapeutics*
2:15 PM - 2:35 PM	**Building a Better AAV Capsid for CNS Gene Therapies** *David Schaffer, Ph.D., University of California, Berkeley*
2:35 PM - 2:55 PM	**Optimizing the Transgene for AAV CNS Gene Therapies** *Guangping Gao, Ph.D., University of Massachusetts Medical School*
2:55 PM - 3:15 PM	**Evolving Approaches to Direct Delivery of AAV CNS Gene Therapies** *Mark Richardson, M.D., Ph.D., Massachusetts General Hospital*
3:15 PM - 3:30 PM	**Q&A –** *Moderated by Omar Khwaja, M.D., Ph.D.* *Panelists: David Schaffer, Ph.D.; Guangping Gao, Ph.D.; Mark Richardson, M.D., Ph.D.*

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2:00 PM - 3:00 PM

CHAT LOUNGE NETWORKING

3:30 - 5:15 PM

OUTSTANDING NEW INVESTIGATOR SYMPOSIUM

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PLENARY SESSIONS

3:30 PM - 3:56 PM	**Mechanisms Underlying CAR T Cell Function** *Marcela Maus, M.D., Ph.D., Massachusetts General Hospital*
3:56 PM - 4:22 PM	**Enhancing Genome Editing Technologies Using Protein Engineering** *Benjamin Kleinstiver, Ph.D., Massachusetts General Hospital*
4:22 PM - 4:48 PM	**Using the Blood to Treat the Brain: Engineering the Hematopoietic System to Treat Non-Hematological Diseases** *Natalia Gomez-Ospina, M.D., Ph.D., Stanford*
4:48 PM - 5:15 PM	**Gene Therapy Approaches to β-hemoglobinopathies** *Annarita Miccio, Ph.D., Institut Imagine*

5:15 PM - 6:45 PM

EXHIBIT HALL OPEN - EXHIBITORS AVAILABLE TO CONNECT

Exhibit Hall is Accessible 24 Hours

Schedule

ASGCT 2021 Virtual ANNUAL MEETING

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WEDNESDAY, MAY 12, 2021

5:15 PM - 7:15 PM

ABSTRACT SESSIONS

AAV Biology, Engineering, Immunology and Animal Modeling
Co-Chairs: Allison Bradbury, Ph.D. and Miguel Sena-Esteves, Ph.D.

5:30 PM - 5:45 PM	**64: A Multi-Mechanistic Anti-Angiogenic AAV Gene Therapy Product Candidate, 4D-150, for the Treatment of Wet Age-Related Macular Degeneration (wAM.D.) and Diabetic Macular Edema (DME): Intravitreal Biodistribution, Transgene Expression, Safety and Efficacy in Non-Human Primates** *Peter Francis, M.D., Ph.D., 4D Molecular Therapeutics*
5:45 PM - 6:00 PM	**65: Evolving Synthetic AAV Variants for Genome Editing in Immune Cell Populations** *Jonathan Ark, Molecular Genetics and Microbiology, Duke University*
6:00 PM - 6:15 PM	**66: Real Time Blood Brain Barrier Disruption In A Multi-Species Model** *Ana Rita Batista, Ph.D., University of Massachusetts Medical School*
6:15 PM - 6:30 PM	**67: AAV2:2.Retro-Mediated Delivery of Mutant Huntingtin Throughout Cortico-Basal Ganglia Circuitry Leads to the Progressive Development of Motor and Cognitive Decline, Along With Microstructural Changes in White and Gray Matter, in a Novel Rhesus Macaque Model of Huntington's Disease** *Alison Weiss, Ph.D., Oregon National Primate Research Center/OHSU*
6:30 PM - 6:45 PM	**68: Investigating Mechanisms of Variability of AAV5-hFVIII-SQ Expression in Mice** *Bridget Yates, BioMarin Pharmaceutical Inc.*
6:45 PM - 7:00 PM	**69: Thermoresponsive Polymer-AAV Nanoparticle Vectors Improved Transgene Expression on Immunized Murine Model** *Kai Wang, Ph.D., The University of North Carolina at Chapel Hill*
7:00 PM - 7:15 PM	**70: AAV Vector Dose Dependent Redundant and Non-Redundant Roles of TLR9 and IL1R Signaling in CD8 T Cell Activation Upon Muscle Gene Transfer** *Ning Li, Ph.D., IU School of Medicine*

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5:15 PM - 7:15 PM

ABSTRACT SESSIONS

CAR Modified Cellular Therapies
Co-Chairs: Maria-Grazia Roncarolo, M.D. and Pietro Genovese, Ph.D.

5:30 PM - 5:45 PM	**71: Pre-Selected CAR_T_N/SCM Outperform CARTBULK In Driving Tumor Eradication In The Absence Of Severe CRS And ICANS** *Silvia Arcangeli, Ph.D., San Raffaele Hospital*
5:45 PM - 6:00 PM	**72: CD5 CAR T-Cells Avoid Self-Elimination by Continuously Degrading CD5 Protein** *Royce Ma, Baylor College of Medicine*
6:00 PM - 6:15 PM	**77: Precise Targeting of AML With First-in-Class OR / NOT Logic-Gated Gene Circuits in CAR-NK Cells** *Brian Garrison, Ph.D., Senti Biosciences*
6:15 PM - 6:30 PM	**74: Investigating the Therapeutic Efficacy of Disruption of Cell Intrinsic Checkpoint Regulator CTLA-4 in Chimeric Antigen Receptor T cells** *Sangya Agarwal, University of Pennsylvania Perelman School of Medicine*
6:30 PM - 6:45 PM	**75: Non-Human Primate Derived CD20 CAR T Cells Elicit a Bystander Effect on CD8 but Not CD4 CAR-T Cells** *Ulrike Gerdemann, M.D., Dana Farber Cancer Institute; Boston Children's Hospital*
6:45 PM - 7:00 PM	**76: Enhanced Generation of T-Cell Derived Naïve Pluripotent Cells as a Renewable Cell Source for the Mass Manufacture of Off-the-Shelf CAR T Cell Therapies** *Yi-Shin Lai, Ph.D., Fate Therapeutics*

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WEDNESDAY, MAY 12, 2021

5:15 PM - 7:15 PM

ABSTRACT SESSIONS

Gene Therapies for Hemoglobinopathies
Co-Chairs: John Chapin, M.D. and Pankaj Mandal, Ph.D.

5:30 PM - 5:45 PM
78: Early Results From a Phase 1/2 Study of ARU-1801 Gene Therapy for Sickle Cell Disease (SCD): Safety and Efficacy of a Modified Gamma Globin Lentivirus Vector and Reduced Intensity Conditioning Transplant
Punam Malik, M.D., Cincinnati Children's Hospital Medical Center

5:45 PM - 6:00 PM
79: Immune Reconstitution in Transfusion Dependent Beta-Thalassemia Patients Treated With Hematopoietic Stem Cell Gene Therapy
Samantha Scaramuzza, Ph.D., San Raffaele Telethon Institute for Gene Therapy

6:00 PM - 6:15 PM
80: Multiplex Base Editing of Hematopoietic Stem and Progenitor Cells to Enrich Therapeutic Cells Post Engraftment
Olivier Humbert, Ph.D., Fred Hutchinson Cancer Research Center

6:15 PM - 6:30 PM
81: In Vivo HSC Gene Therapy for Hemoglobinopathies: A Proof of Concept Evaluation in Rhesus Macaques
Chang Li, Ph.D., University of Washington

6:30 PM - 6:45 PM
82: Hematopoietic Reconstitution and Lineage Commitment in HSC Gene Therapy Patients Are Influenced by the Disease Background
Andrea Calabria, Ph.D., San Raffaele Telethon Institute for Gene Therapy

6:45 PM - 7:00 PM
83: Lentiviral Mediated Gene Therapy for Pyruvate Kinase Deficiency: Updated Results of a Global Phase 1 Study for Adult and Pediatric Patients
José Luis López Lorenzo, M.D., Hospital Universitario Fundación Jiménez Díaz; Instituto de Investigación Sanitaria Fundación Jiménez Díaz

7:00 PM - 7:15 PM
83: Base Editing of the -200 Region of the γ-Globin Promoters Leads to Fetal Hb Reactivation and Rescues the Sickle Cell Disease Phenotype in Primary Patient Cells
Panagiotis Antoniou, Institut Imagine

ALL TIMES LISTED IN EDT

WEDNESDAY, MAY 12, 2021

5:15 PM - 7:15 PM

ABSTRACT SESSIONS

Immune Responses to AAV Vectors
Co-Chairs: Ying Kai Chan, Ph.D. and Manish Muhuri, Ph.D.

5:30 PM - 5:45 PM
85: Declining FVIII Activity Following Hepatic AAV Gene Transfer Because of Translational Shutdown Linked to an Immune Response
John Butterfield, University of Florida

5:45 PM - 6:00 PM
86: Requirements for Cross-Presenting Dendritic Cells and CpG Motifs in CD8+ T Cell Response to AAV Gene Transfer
Thais Bertolini, Ph.D., Indiana University School of Medicine

6:00 PM - 6:15 PM
87: Defining and Overcoming Preexisting T-Cell Adaptive Immunity to SaCas9 CRISPR-Cas Genome Editors
Andrea Lee, St. Jude Children's Research Hospital

6:15 PM - 6:30 PM
88: Clinical Outcomes in Patients With and Without Pre-Existing Neutralizing Antibodies to the Vector: 6 Month Data From the Phase 3 HOPE-B Gene Therapy Trial of Etranacogene Dezaparvovec
Michael Recht, M.D., Ph.D., The Hemophilia Center at Oregon Health and Science University

6:30 PM - 6:45 PM
91: Novel miRNA-Binding Sites That Recruit miR-652 and miR-223 in AAV Vector Designs Boost Transgene Levels and Synergistically Suppress Cell-Mediated Immunity
Manish Muhuri, Ph.D., University of Massachusetts Medical School

6:45 PM - 7:00 PM
90: IL-1a and IL-1b Are Essential for Inflammasome Independent CD8+ T Cell Responses to Hepatic AAV Gene Transfer
Sandeep Kumar, Ph.D., Indiana University

ALL TIMES LISTED IN EDT

WEDNESDAY, MAY 12, 2021

5:15 PM - 7:15 PM

ABSTRACT SESSIONS

Novel Factors in AAV Transduction and AAV Genomes
Co-Chairs: David Markusic, Ph.D. and Amanda Dudek, Ph.D.

5:30 PM - 5:45 PM	**92: Chemical Mediated Recruitment of Epigenetic Modifiers Regulate Adeno-Associated Virus Episomal Transgene Expression** *Jessica Umana, University of North Carolina - Chapel Hill*
5:45 PM - 6:00 PM	**93: The Human Silencing Hub (HUSH Complex) is a Potent Regulator of AAV Transgene Silencing** *Anshuman Das, Ph.D., Duke University*
6:00 PM - 6:15 PM	**94: Effects of Sexual Dimorphism and Genetic Background on AAV Tissue Transduction in Mice Following Intravenous Administration of a Diverse Capsid Pool** *Elad Firnberg, Ph.D., REGENXBIO, Inc.*
6:15 PM - 6:30 PM	**95: High Throughput Screening of Diverse Mini-Promoter Libraries Within AAV via Expression Linked Promoter Selection (ELiPS)** *Kazuomori Lewis, University of California, Berkeley*
6:30 PM - 6:45 PM	**96: GMEB2 is a Conserved Cellular AAV Restriction Factor That Inhibits Transduction of Human Stem Cells** *Amanda Dudek, Ph.D., Stanford University*
6:45 PM - 7:00 PM	**97: Rationally Designed Inverted Terminal Repeats Improve AAV Vector Production** *Liujiang Song, Ph.D., University of North Carolina, Chapel Hill*
7:00 PM - 7:15 PM	**98: Characterization of AAV Inverted Terminal Repeats by Atomic Force Microscopy** *Marianne Laugel, University of Nantes*

Schedule

ASGCT 2021 Virtual ANNUAL MEETING

ALL TIMES LISTED IN EDT

WEDNESDAY, MAY 12, 2021

5:15 PM - 7:15 PM

ABSTRACT SESSIONS

Preclinical Gene Therapy for Neurologic Disorders II
Co-Chairs: Gwladys Gernoux, Ph.D. and Juliette Hordeaux, D.V.M., Ph.D.

5:30 PM - 5:45 PM	**99: A Novel Exon Specific U1 snRNA Therapeutic Strategy to Prevent Retinal Degeneration in Familial Dysautonomia** *Anil Chekuri, Ph.D., Massachusetts General Hospital Research Institute; Harvard Medical School*
5:45 PM - 6:00 PM	**105: Efficacy of a Vectorized Anti-Tau Antibody Using Systemic Dosing of a Blood Brain Barrier Penetrant AAV Capsid in Mouse Models of Tauopathies** *Wencheng Liu, Ph.D., Voyager Therapeutics*
6:00 PM - 6:15 PM	**101: Evolution of Modified AAV Vectors in Rhesus Macaque Cochlea** *Paul Ranum, Ph.D., The Children's Hospital of Philadelphia*
6:15 PM - 6:30 PM	**102: A Novel Retinal Gene Therapy Strategy for Batten Disease and Beyond** *Maura Schwartz, The Research Institute at Nationwide Children's Hospital*
6:30 PM - 6:45 PM	**103: Reprogramming to Recover Youthful Epigenetic Information and Restore Vision** *Yuancheng Lu, Ph.D., Harvard Medical School*
6:45 PM - 7:00 PM	**104: Efficacious, Safe, and Stable Inhibition of Corneal Neovascularization With rAAV-KH902 in a Mouse Model of Corneal Alkali Injury** *Wenqi Su, M.D., University of Massachusetts Medical School*

Schedule

ASGCT 2021 Virtual ANNUAL MEETING

ALL TIMES LISTED IN EDT

WEDNESDAY, MAY 12, 2021

5:15 PM - 7:15 PM

ABSTRACT SESSIONS

Synthetic/Molecular Conjugates and Physical Methods for Delivery
Co-Chairs: Angela Pannier, Ph.D. and Kenya Kamimura, M.D., Ph.D.

5:30 PM - 5:45 PM	**106: Combinatorial Modified mRNA Induces Cardiovascular Regeneration Post Muscle Ischemic Injury** *Keerat Kaur, Ph.D., Icahn School of Medicine, Mount Sinai*
5:45 PM - 6:00 PM	**107: Sustained Episomal Transgene Expression In Vivo Driven by Non-Viral DNA Delivery to Rodent Liver** *Stoil Dimitrov, M.D., Ph.D., Moderna*
6:00 PM - 6:15 PM	**108: Optimization of Transcutaneous Ultrasound Mediated Gene Delivery Into Large Animals** *Megan Manson, Seattle Children's Research Institute*
6:15 PM - 6:30 PM	**109: Assembling Several mRNA Strands for Facilitating mRNA Delivery With and Without Using Carriers** *Satoshi Uchida, M.D., Ph.D., Kyoto Prefectural University of Medicine; Kawasaki Institute of Industrial Promotion*
6:30 PM - 6:45 PM	**110: Development of Hydrodynamics-Based Gene Therapy for Liver Cancer** *Kenya Kamimura, M.D., Ph.D., Niigata University*
6:45 PM - 7:00 PM	**111: Hematopoietic Stem and Progenitor Cells-Targeted Polymeric Nanoparticles for In Vivo Gene Therapy** *Rkia El kharrag, Ph.D., Fred Hutchinson Cancer Research Center*
7:00 PM - 7:15 PM	**112: Delivery of CRISPR/Cas9 for Recovering the Expression of the Endogenous FVIII in Hemophilia A Mice** *Chun-Yu Chen, Ph.D., Seattle Children's Hospital Research Institute*

Schedule

ASGCT 2021 Virtual ANNUAL MEETING

ALL TIMES LISTED IN EDT

WEDNESDAY, MAY 12, 2021

5:15 PM - 7:15 PM

ABSTRACT SESSIONS

Targeted Gene and Cell Therapy for Cancer
Co-Chairs: Michael Milone, M.D., Ph.D. and Hernando Lopez-Bertoni, Ph.D.

5:30 PM - 5:45 PM	**113: Gene-based Immune Reprogramming Overcomes the Immunosuppressive Microenvironment of Liver Metastases and Enables Protective T Cell Responses** *Thomas Kerzel, San Raffaele Telethon Institute for Gene Therapy*
5:45 PM - 6:00 PM	**114: Inducible Tumor-Targeted Interferon-α Gene Therapy Inhibits Glioblastoma Multiforme in Mouse Model Without Adverse Systemic Effects** *Filippo Birocchi, San Raffaele Telethon Institute for Gene Therapy*
6:00 PM - 6:15 PM	**115: Design and Demonstration of Potent In Vitro and In Vivo Activity for CART-ddBCMA, a BCMA-Targeted CAR-T Cell Therapy Incorporating a Non-scFv Binding Domain** *Janine Buonato, Ph.D., Arcellx*
6:15 PM - 6:30 PM	**116: A SOX2 Engineered Epigenetic Silencer Factor Represses the Cancer Genetic Program and Eradicate Glioblastoma Development** *Vania Broccoli, Ph.D., San Raffaele Scientific Institute*
6:30 PM - 6:45 PM	**117: Oncolytic Adeno-Immunotherapy Expressing IL-12p70 and Immune Checkpoint Blockade PD-L1 Minibody Modulates the Host Immune System to Enable HER2.CAR T-Cells to Cure Pancreatic Tumors** *Amanda Rosewell Shaw, Ph.D., Baylor College of Medicine*
6:45 PM - 7:00 PM	**118: UCARTCS1A, an Allogeneic CAR T-Cell Therapy Targeting CS1 in Patients With Relapsed/Refractory Multiple Myeloma (RRMM): Preliminary Translational Results From a First-in-Human Phase I Trial (MELANI-01)** *Krina Patel, M.D., The University of Texas M.D. Anderson Cancer Center*
7:00 PM - 7:15 PM	**119: A Drug-Regulated Anti-CD33 Chimeric Antigen Receptor With Potent Anti-AML Activity and a Reversible On-Off Switch** *Jacob Applebaum, M.D., Ph.D., Seattle Children's Therapeutics; University of Washington*

Schedule

ASGCT 2021 Virtual ANNUAL MEETING

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WEDNESDAY, MAY 12, 2021

5:15 PM - 7:15 PM

ABSTRACT SESSIONS

Upstream Process Development for AAV Vector Production
Co-Chairs: Sanford Boye and Laura Adamson-Small, Ph.D.

5:30 PM - 5:45 PM	**120: Co-Identification and Characterization of Host and Viral Protein Interactomes During AAV Production by Two Different Proximity Labeling Methods** *Ji Sun Lee, Ph.D., University of Massachusetts Medical School*
5:45 PM - 6:00 PM	**121: The Effects of ITR Structure and Plasmid Backbone on Plasmid Stability and Yield** *Ruofan Wang, Ph.D., Vigene Biosciences*
6:00 PM - 6:15 PM	**122: Increasing Gene Therapy Vector Production Using Viral Sensitizer Molecules** *Jean-Simon Diallo, Ph.D., Virica Biotech, Inc.*
6:15 PM - 6:30 PM	**123: Transcriptional Response of HEK293 Cells to Clinical-Scale Recombinant Adeno Associated Virus Production by Transient Transfection** *Cheng-Han Chung, Ph.D., Pfizer, Inc.*
6:30 PM - 6:45 PM	**124: Vector Engineering of pRep-Cap and pHelper Enhanced AAV Productivity by Triple Transfection in Suspension HEK293 Cells** *Bingnan Gu, Ph.D., Lonza Houston Inc.*
6:45 PM - 7:00 PM	**125: High Titer rAAV Production Upon Upstream Process Development of Stable Helper-Virus Free ELEVECTA® Producer Cells** *Juliana Coronel, Ph.D., Cevec Pharmaceuticals GmbH*
7:00 PM - 7:15 PM	**126: Genome-Wide CRISPR Activation Screen Reveals That SKA2 and ITPRIP Increase AAV Manufacturing via Cell Cycle Modulation** *Hyuncheol Lee, D.V.M., Ph.D., University of California, Berkeley*

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ASGCT 2021 Virtual Annual Meeting

ALL TIMES LISTED IN EDT

WEDNESDAY, MAY 12, 2021

5:15 PM - 6:45 PM

INDUSTRY SYMPOSIUM

Industry Symposium: 908 Devices - Mass Spectrometry-Based Process Analytical Technologies for Cell Therapies

Industry Symposium: Pall Corporation

5:15 PM - 6:15 PM

Networking Roulette

Schedule

ASGCT 2021 Virtual ANNUAL MEETING

ALL TIMES LISTED IN EDT

WEDNESDAY, MAY 12, 2021

5:15 PM - 7:00 PM

TOOLS AND TECHNOLOGY FORUM

Tools and Technology Forum II

5:15 PM - 5:30 PM
Emerging Technologies: Optimizing Mammalian Cell Culture Cultivation and Analysis
Holly Hattaway, PHC Corporation of North America

5:30 PM - 5:45 PM
Streamlined Detection and Characterization of CRISPR Editing Using the rhAmpSeq™ CRISPR Analysis System
Gavin Kurgan, Ph.D., Integrated DNA Technologies (IDT)

5:45 PM - 6:00 PM
Fibro Chromatography in Downstream AAV Processing
Peter Guterstam, Ph.D., Cytiva

6:00 PM - 6:15 PM
Seeing is Believing - 3D Visualization of Vector-Mediated Expression in Whole Animals
Hemi Dimant, Ph.D., Invicro, A Konica Minolta Company

6:15 PM - 6:30 PM
Quality Matters - Advanced AAV Vector Manufacturing for Reliable Preclinical Results
Christian Thirion, SIRION Biotech

6:30 PM - 6:45 PM
The Impact of Closed Systems on Cell and Gene Therapy Scalability
Jayanthi Grebin, CPC

6:45 PM - 7:00 PM
Automated Parallel Chromatography to Accelerate Downstream Process Development in Gene Therapy
Jana Langhoff, Tecan; Tim Schroeder, Repligen

Schedule

ASGCT 2021 Virtual ANNUAL MEETING

ALL TIMES LISTED IN EDT

THURSDAY, MAY 13, 2021

ALL DAY

DIGITAL ABSTRACT PRESENTATIONS

EXHIBIT HALL

Be sure to connect with exhibit booth staff during listed times

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9:00 AM - 10:00 AM

CHAT LOUNGE NETWORKING

10:00 AM - 11:45 AM

EDUCATION SESSIONS

Career and Workforce Development Issues in Gene and Cell Therapy
Co-Chairs: Juliana Alvarez Argote, M.D. and Rayne Rouce, M.D.

10:00 AM - 10:26 AM — **Alternate Careers in Vector Production and Consulting**
Jeffrey Medin, Ph.D., Medical College of Wisconsin

10:26 AM - 10:52 AM — **Building and Developing a Team to Make Translation Possible**
Parameswaran Hari, M.D., Medical College of Wisconsin

10:52 AM - 11:18 AM — **The Value of Diversity and Inclusion in the Scientific Workforce**
Lynn Gordon, M.D., Ph.D., University of California Los Angeles

11:18 AM - 11:45 AM — **Dealing With Research Interruptions: From Parenting to the Pandemic**
Catherine Bollard, M.D., M.B.Ch.B., Children's National Hospital/The George Washington University

Schedule

ASGCT 2021 Virtual ANNUAL MEETING

ALL TIMES LISTED IN EDT

THURSDAY, MAY 13, 2021

10:00 AM - 11:45 AM

EDUCATION SESSIONS

Gene Therapies for Liver Diseases
Chair: Nuria Morral, Ph.D.

10:00 AM - 10:35 AM	**Overview of Liver Gene Therapies for Inborn Errors of Metabolism** *Gloria Gonzalez-Aseguinolaza, Ph.D., Cima-Universidad de Navarra and Vivet Therapeutics*
10:35 AM - 11:10 AM	**Creation and Treatment of Murine Models of Liver-Based Inherited Enzyme Deficiencies Using CRISPR/Cas9 Gene Editing Technology** *Cary Harding, M.D., Oregon Health & Sciences University*
11:10 AM - 11:45 AM	**Lipid Nanoparticles for Therapeutic Gene Targeting to the Liver** *Pieter Cullis, Ph.D., University of British Columbia, Vancouver*

Therapeutic Applications of EVs: From Diagnostics to Drug Delivery
(Organized by the Nanoagents & Synthetic Formulations Committee)
Co-Chairs: Rajagopal Ramesh, Ph.D. and Assem Ziady, Ph.D.

10:00 AM - 10:35 AM	**Purification of Tissue Specific EVs** *Erez Eitan, Ph.D., Neurodex*
10:35 AM - 11:10 AM	**Bacterial-Derived Outer Membrane Vesicles for Gene Delivery** *Angela Pannier, Ph.D., University of Nebraska-Lincoln*
11:10 AM - 11:45 AM	**Exosome Delivery of Sars CoV-2 Vaccines** *Linda Marban, Ph.D., Capricor*

Schedule

ASGCT 2021 Virtual ANNUAL MEETING

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THURSDAY, MAY 13, 2021

10:00 AM - 11:45 AM

SCIENTIFIC SYMPOSIA

International Focus on Stem Cell Gene Therapy
(Organized by the International Committee)
Co-Chairs: Toni Cathomen, Ph.D. and Alessandro Aiuti, M.D., Ph.D.

10:00 AM - 10:26 AM	**iPS Cell-Based Therapy for Parkinson's Disease** *Jun Takahashi, M.D., Ph.D., Kyoto University*
10:26 AM - 10:52 AM	**Combined Cell and Gene Therapy of Epidermolysis Bullosa** *Michele De Luca, M.D. University of Modena and Reggio Emilia*
10:52 AM - 11:18 AM	**Stem Cell Gene Therapy for Primary Immunodeficiencies** *Claire Booth, D.Phil., UCL Great Ormond Street Institute of Child Health*
11:18 AM - 11:45 AM	**Gene Therapy in Fanconi Anemia: Current Strategies to Enable the Correction of HSCs** *Paula Rio, Ph.D., Centro de Investigaciones Energéticas Medioambientales y Tecnológicas and Centro de Investigación Biomédica en Red de Enfermedades Raras (CIEMAT/CIBERER)/Instituto de Investigación Sanitaria Fundación Jiménez (IIS-FJD, UAM)*

Racial Justice in the Gene Therapy Field
(Organized by the Ethics & Diversity and Inclusion Committees)
Co-Chairs: Rayne Rouce, M.D. and David Segal, Ph.D.

10:00 AM - 10:20 AM	**Addressing Racial Disparities Due to Poverty: Partnering With Biotechnology Companies** *Rob Perez, Life Science Cares*
10:20 AM - 10:40 AM	**NHLBI Cure Sickle Cell Initiative: An Update** *Traci Mondoro, Ph.D., National Institutes of Health, NHLBI*
10:40 AM - 11:00 AM	**The Significance of Training and Mentorship for Underrepresented Groups in the Science Workforce** *Melody Smith, M.D., Memorial Sloan Kettering Cancer Center*
11:00 AM - 11:20 AM	**Supporting and Enacting Change** *Rayne Rouce, M.D., Baylor College of Medicine*
11:20 AM - 11:45 AM	**Panel Discussion**

Schedule

ASGCT 2021 Virtual ANNUAL MEETING

ALL TIMES LISTED IN EDT

THURSDAY, MAY 13, 2021

10:00 AM - 11:45 AM

SCIENTIFIC SYMPOSIA

Reporter Gene Imaging And The 3 Rs In Cell And Gene Therapy
Co-Chairs: Stephen Russell, M.D., Ph.D.

10:00 AM - 10:26 AM	**Experimental Design Strategies in Primate Trials That Strengthen the Translational Bridge to Clinical Success in Cell And Gene Therapy**	*Melanie Graham, Ph.D., University of Minnesota*
10:26 AM - 10:52 AM	**Longitudinal Non-Invasive Tracking of iPSC-Derived Tissues in Non-Human Primates**	*Cynthia Dunbar, M.D., National Institutes of Health, NHLBI Translational Stem Cell Biology Branch*
10:52 AM - 11:18 AM	**Imaging the In Vivo Fate of Genetically Labeled CAR T Cells**	*Saad Kenderian, M.B., Ch.B., Mayo Clinic*
11:18 AM - 11:45 AM	**Imaging the In Vivo Fate of Genetically Labeled Hepatocyte Progenitor Cells**	*Joseph Lillegard, M.D., Ph.D., Mayo Clinic*

Vaccine Development: Successes and Emerging Challenges
(Organized by the Infectious Diseases and Vaccines Committee)
Co-Chairs: David Weiner, Ph.D. and Sterghios Moschos, Ph.D.

10:10 AM - 10:35 AM	**Successes in Vaccine Development**	*Ugur Sahin, M.D., Ph.D., Biontech*
10:35 AM - 11:00 AM	**Successes in Vaccine Development**	*Tonya Villafana, Ph.D., AstraZeneca*
11:00 AM - 11:25 AM	**Blowback, Re-Emergence, and Antigenic Drift of Infectious Disease**	*Scott Hensley, Ph.D., University of Pennsylvania*
11:25 AM - 11:45 AM	**Panel Discussion**	

Schedule

ASGCT 2021 Virtual ANNUAL MEETING

ALL TIMES LISTED IN EDT

THURSDAY, MAY 13, 2021

10:00 AM - 11:45 AM

SCIENTIFIC SYMPOSIA

Vector Manufacturing and Downstream Processing
Co-Chairs: Anne Galy, Ph.D. and Boro Dropulic, Ph.D.

10:00 AM - 10:26 AM	**Retroviral Vector Production** *Isabelle Riviere, Ph.D., Memorial Sloan-Kettering Cancer Center*
10:26 AM - 10:52 AM	**Large Scale Production of Lentiviral Vectors** *Anne Galy, Ph.D., Genethon*
10:26 AM - 10:52 AM	**Large Scale Production of AAV Vectors** *Patrick Santambien, Ph.D., Genethon*
10:52 AM - 11:18 AM	**Academic Vector Production for Early Phase Clinical Studies** *Johannes van der Loo, Ph.D., Children's Hospital of Philadelphia*
11:18 AM - 11:45AM	**Lentiviral Vector Manufacture** *Boro Dropulic, Ph.D., CARing Cross*

Viral Vector Safety: A Renewed Focus on Vector Safety and Innate Immune Responses to Leading Viral Vectors
(Organized by the Translational Science Committee)
Co-Chairs: H. Trent Spencer, Ph.D. and Nicole Paulk, Ph.D.

10:00 AM - 10:21 AM	**A Late Gene Transcription Factor of Rhesus Cytomegalovirus Vectors is Required to Elicit MHC-II and MHC-E-Restricted CD8 T Cells and Protect Against SIV Challenge** *Klaus Früh, Ph.D., Oregon Health and Science University*
10:21 AM - 10:42 AM	**Human Immune Response To AAV Vectors** *Katherine High, M.D., Asklepios BioPharmaceuticals*
10:42 AM - 11:03 AM	**AAV Vectors: Are They Safe?** *Arun Srivastava, Ph.D, University of Florida*
11:03 AM - 11:24 AM	**Safety and Use of Retroviral Vectors** *Christopher Doering, Ph.D., Emory University*
11:24 AM - 11:45 AM	**Safety of Adenoviral Vectors: A Platform for Therapy of Genetic Diseases, Cancer, and Novel Vaccines** *Dmitry Shayakhmetov, Ph.D., Emory University School of Medicine*

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THURSDAY, MAY 13, 2021

10:45 AM - 12:15 PM

EXHIBIT HALL OPEN - EXHIBITORS AVAILABLE TO CONNECT

Exhibit Hall is Accessible 24 Hours

EXHIBITOR SHOWCASES

10:45 AM - 11:30 AM

SARTORIUS

BIA Separations now a Sartorius company - Chromatin (DNA) Removal From Harvest Before an AAV Capture Step Greatly Improves Robustness, Purity, and Yield of the Overall Downstream Process
Ales Strancar, Ph.D., BIA Separations now a Sartorius company

10:45 AM - 11:30 AM

Bristol Myers Squibb

Bristol Myers Squibb - A New Treatment Option for Adults with Relapsed or Refractory (R/R) Large B-Cell Lymphoma (LBLC) After Two or More Lines of Systemic Therapy
Krish Patel, M.D., Swedish Cancer Institute; William Mir, Bristol Myers Squibb; and Erin McCaffrey, Pharm.D., Bristol Myers Squibb

11:30 AM - 12:15 PM

GENEWIZ A Brooks Life Sciences Company

Brooks Life Sciences GENEWIZ Inc. - InnovativeGenomics & Cold Chain Solutions for Development of Cell and Gene Therapies
Elizabeth Louie, GENEWIZ, A Brooks Life Sciences Company; and Kathi Shea, Brooks Life Sciences

11:30 AM - 12:15 PM

Solentim

Solentim - Robust Workflow for Single Cell Cloning of iPSCs for Making cGMP Master Cell Banks
Ian Taylor, Ph.D., Solentim

12:15 PM - 2:00 PM

OUTSTANDING ACHIEVEMENT AWARD LECTURE AND PRESENTATION OF THE SONIA SKARLATOS PUBLIC SERVICE AWARD

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Schedule

ASGCT 2021 Virtual ANNUAL MEETING

ALL TIMES LISTED IN EDT

THURSDAY, MAY 13, 2021

2:00 PM - 3:30 PM

INDUSTRY SPONSORED SYMPOSIA

EXHIBIT HALL OPEN - EXHIBITORS AVAILABLE TO CONNECT

Exhibit Hall is Accessible 24 Hours

Charles River Laboratories - Accelerating and Achieving Your Cell Therapy Program Goals: A Focus on CAR T Development

charles river

2:00 PM - 2:25 PM	**The Importance of Starting Material for Cell Therapies - Your CAR Depends on It** *Dominic Clarke, Ph.D., HemaCare at Charles River Laboratories*
2:25 PM - 2:45 PM	**CAR-T Binder Discovery Simplified: Any Target, 50 Antibodies. Engineered Specifically for CAR Formats.** *Sarah Ives, PSM, Distributed Bio at Charles River Laboratories*
2:45 PM - 3:05 PM	**In Vitro Efficacy and Safety Testing of Your Cellular Therapy - A Good CAR Inspection** *Sabrina de Munnik, Ph.D., Charles River Laboratories*
3:05 PM - 3:30 PM	**Take Your CAR and Drive It - Charting Your Path to Market** *Steven Miklasz, MSc, CBA, Charles River Laboratories*

FUJIFILM Diosynth Biotechnologies - Beyond the Horizon: What's Next for Advanced Therapies?

FUJIFILM Diosynth biotechnologies

2:00 PM - 3:30 PM	*Moderated by Ian Goodwin, FUJIFILM Diosynth Biotechnologies* *Panelists: Lois Chandler, Ph.D., Gene Biotherapeutics Inc.; Shon Green, Ph.D., Umoja Biopharma Inc.; and Leslie Wolfe, Ph.D., Generation Bio*

Schedule

ASGCT 2021 Virtual ANNUAL MEETING

ALL TIMES LISTED IN EDT

THURSDAY, MAY 13, 2021

2:00 PM - 3:30 PM

INDUSTRY SPONSORED SYMPOSIA

GenScript - Innovative Solutions and Approaches in Immunotherapy

GenScript Make Research Easy

2:00 PM - 2:30 PM	**De Novo Design of a Self-Assembling Super Antigen: A Potential Cancer Immunotherapy via Controlled T Cell Activation** *Possu Huang, Ph.D., Stanford University*
2:30 PM - 3:00 PM	**Library-Selected AAV Variants Can Effectively Translate to Non-Human Primates in the Spinal Cord and Cochlea** *Killian Hanlon, Ph.D., Harvard Medical School; Massachusetts General Hospital*
3:00 PM - 3:30 PM	**Precise and Efficient Non-Viral CRISPR Gene Editing Solutions** *Lumeng Ye, Ph.D., GenScript*

Precision NanoSystems Inc. - Accelerating Genetic Medicine Development With Lipid Nanoparticles (LNPs)

PRECISION NANOSYSTEMS Create Transformative Medicines™

2:00 PM - 2:10 PM	**Genetic Medicines From Discovery to Commercial Production** *Andrew Geall, Ph.D., Precision NanoSystems Inc.*
2:10 PM - 2:30 PM	**Effects of Polymeric and Lipid Nanoparticle Self-Amplifying RNA Formulation on Protein Expression and Vaccine Immunogenicity** *Anna Blakney, Ph.D., University of British Columbia*
2:30 PM - 2:50 PM	**Engineering of RNA CAR T Cells WNovel Lipid Nanoparticles for Gene Delivery** *Samuel Clarke, Ph.D., Precision NanoSystems Inc.*
2:50 PM - 3:10 PM	**Manufacturing Considerations for the Development of Lipid Nanoparticles Using Microfluidics** *Yvonne Perrie, Ph.D., University of Strathclyde*
3:10 PM - 3:30 PM	**Round Table Discussion: Genetic Medicine Development With Lipid Nanoparticles** *Chair: Vicki Stronge, Ph.D., Precision NanoSystems Inc.* *Panelists: Anna Blakney, Ph.D.; Samuel Clarke, Ph.D.; Andrew Geall, Ph.D.; and Yvonne Perrie, Ph.D.*

Schedule

ASGCT 2021 Virtual ANNUAL MEETING

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THURSDAY, MAY 13, 2021

2:00 PM - 3:00 PM

CHAT LOUNGE NETWORKING

3:30 PM - 5:15 PM

PRESIDENTIAL SYMPOSIUM AND PRESENTATION OF TOP ABSTRACTS

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PLENARY SESSIONS

3:30 PM - 4:15 PM	**Oncolytic Immunovirotherapy: Uniting Genetic Engineering, Virology, Immunology, and Cancer Research** *Robert Martuza, M.D., Harvard Medical School and Massachusetts General Hospital*
4:15 PM - 4:30 PM	**127: Base Editing Rescues Sickle Cell Disease in Human Hematopoietic Stem Cells and in Mice** *Jonathan Yen, Ph.D., St. Jude Children's Research Hospital*
4:30 PM - 4:45 PM	**128: Safety and Efficacy Results with a Single Dose of Autologous CRISPR-Cas9-Modified CD34+ Hematopoietic Stem and Progenitor Cells (HSPCs) in Transfusion-Dependent β-Thalassemia (TDT) and Sickle Cell Disease (SCD)** *Tony W. Ho, M.D., CRISPR Therapeutics*
4:45 PM - 5:00 PM	**129: Immunostimulatory Bacterial Antigen-armed Oncolytic Measles Virotherapy S ignificantly Increases the Potency of Anti-PD1 Checkpoint Therapy** *Eleni Panagioti, Ph.D., Mayo Clinic*
5:00 PM - 5:15 PM	**130: In-Vivo Engineered B Cells Retain Memory and Secrete High Titers of Anti-HIV Antibodies in Mice** *Alessio David Nahmad, M.Sc., Tel Aviv University*

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5:15 PM - 6:45 PM

EXHIBIT HALL OPEN - EXHIBITORS AVAILABLE TO CONNECT

Exhibit Hall is Accessible 24 Hours

5:15 PM - 7:15 PM

ABSTRACT SESSIONS

AAV Therapies for Neurological and Sensory Diseases
Co-Chairs: Phillip Tai, Ph.D. and Lluis Samaranch, Ph.D.

5:30 PM - 5:45 PM	**131: AAV-Mediated GJB2 Gene Therapy Rescues Hearing Loss and Cochlear Damage in a Mouse Model of Congenital Hearing Loss Caused by Conditional Connexin26 Knockout** *Pranav Mathur, Ph.D., Otonomy Inc.*
5:45 PM - 6:00 PM	**132: AAV9 Mediated Delivery of PUF RNA Targeting System Corrects Molecular and Functional Defects in a Myotonic Dystrophy Type 1MOUSE Model** *Ranjan Batra, Ph.D., Locanabio, Inc.*
6:00 PM - 6:15 PM	**133: Intracorneal and Sequential Contralateral Dosing of AAV-opt-ARSB Reverses MPS VI Corneal Clouding** *Matthew Hirsch, Ph.D., University of North Carolina, Chapel Hill*
6:15 PM - 6:30 PM	**134: Efficacy and Biodistribution of Anc80-RK-hRPGRIP1 Gene Therapy in a Mouse Model of Rpgrip1 Deficiency and in Non-Human Primate** *Binit Kumar, Ph.D., PTC Therapeutics Inc.*
6:30 PM - 6:45 PM	**135: Bicistronic AAV Gene Therapy for Tay-Sachs and Sandhoff Diseases** *Toloo Taghian, Ph.D., University of Massachusetts Medical School*
6:45 PM - 7:00 PM	**136: Gene Therapy Rescues Cone and Rod Function in a Pre-Clinical Model of CDHR1-Associated Retinal Degeneration Through Restoration of Photoreceptor Outer Segments** *Imran Yusuf, M.D., University of Oxford*
7:00 PM - 7:15 PM	**137: scAAV9 Gene Replacement Therapy for Epileptic SLC13A5 Deficiency** *Rachel Bailey, Ph.D., University of Texas Southwestern Medical Center*

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ABSTRACT SESSIONS

Advances in Cellular and Immunotherapies
Co-Chairs: Rayne Rouce, M.D. and Daniel Bauer, M.D., Ph.D.

5:30 PM - 5:45 PM	**138: Dissecting the Transcriptional and Epigenetic Landscape of hiPSC-Derived Neural Stem and Progenitor Cells: Implications for Cell Therapy Approaches** *Vasco Meneghini, Ph.D., San Raffaele Telethon Institute for Gene Therapy*
5:45 PM - 6:00 PM	**139: Receptor Engineered TRuC Tregs Maintain a Regulatory Phenotype and Are Suppressive in a Murine Model of Hemophilia A** *Jyoti Rana, Ph.D., IUPUI*
6:00 PM - 6:15 PM	**140: Functional Benefit of Mitochondrially Augmented HSPCs: Improved Engraftment and Alterations in Immune Cell Differentiation** *Noa Sher, Ph.D., Minovia Therapeutics Ltd*
6:15 PM - 6:30 PM	**141: Memory Enriched Epstein-Barr Virus {EBV} Specific T-cells With Broader Target Antigen Repertoire For The Treatment Of EBV+ Malignancies** *Sandhya Sharma, Baylor College of Medicine*
6:30 PM - 6:45 PM	**142: Non-Viral Engineering of CAR-NK Cells Using the TC Buster Transposon System™** *Emily Pomeroy, University of Minnesota*
6:45 PM - 7:00 PM	**143: Adoptively Transferred, In Vitro-Generated Alloantigen-Specific Type 1 Regulatory T (Tr1) Cells Persist Long-Term In Vivo** *Alma-Martina Cepika, M.D., Ph.D., Stanford University School of Medicine*
7:00 PM - 7:15 PM	**144: Endothelial Progenitor Cells Engineered to Overexpress Endothelial NO-Synthase May Improve Infarct Healing: Results from the Enhanced Angiogenic Cell Therapy - Acute Myocardial Infarction (ENACT-AMI) Trial** *Duncan Stewart, M.D., Ottawa Hospital Research Institute*

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ABSTRACT SESSIONS

CAR-Based Cancer Gene Therapy
Co-Chairs: Monica Casucci, Ph.D. and Daniel Abate-Daga, Ph.D.

5:30 PM - 5:45 PM	**145: Chimeric Antigen Receptor Macrophages (CAR-M) Induce Anti-Tumor Immunity and Synergize With Immune Checkpoint Inhibitors in Pre-Clinical Solid Tumor Models** *Stefano Pierini, Ph.D., Carisma Therapeutics*
5:45 PM - 6:00 PM	**146: Bivalent CD19/CD20-Specific CAR T Cells With 4-1BB and Mutated CD28 Co-Stimulatory Domains Show Enhanced Function** *Emiliano Roselli, Ph.D., Moffitt Cancer Center*
6:00 PM - 6:15 PM	**147: Combining IAP Inhibitors With CAR T Cell Therapy to Treat Glioblastoma** *Edward Song, University of Pennsylvania*
6:15 PM - 6:30 PM	**148: B-CLL-Mediated Insufficient Activation Is CAR-Independent** *McKensie Collins, University of Pennsylvania*
6:30 PM - 6:45 PM	**149: CAR Design and Expression Determine Hyper-Proliferative States in TET2 Deficient T Cells** *Nayan Jain, Memorial Sloan Kettering Cancer Center*
6:45 PM - 7:00 PM	**150: Tumor-Responsive, Multifunctional CAR-NK Cells Cooperate With Impaired Autophagy to Infiltrate and Target Glioblastoma** *Jiao Wang, Ph.D., Purdue University*
7:00 PM - 7:15 PM	**151: ADCLEC.syn1 Is a Novel Combinatorial CAR Platform for Enhanced Therapeutic Index in AML** *Sascha Haubner, M.D., Memorial Sloan Kettering Cancer Center*

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ABSTRACT SESSIONS

Cardiovascular and Pulmonary Gene Therapy
Judith Greengard, Ph.D. and Mai ElMallah, M.D.

5:30 PM - 5:45 PM	**152: Systemic Hps1 Gene Augmentation Prevents Pulmonary Manifestations in a Mouse Model of Hermansky-Pudlak Syndrome** *May Malicdan, M.D., Ph.D., National Institutes of Health*
5:45 PM - 6:00 PM	**153: Generation of a Human 3D Lung Model for Therapeutic Gene Editing in Surfactant Protein B Deficiency** *Helena Meyer-Berg, University of Oxford*
6:00 PM - 6:15 PM	**154: Vectored Immunoprophylaxis for COVID-19 (COVIP)** *Yue Du, Ph.D., University of Oxford*
6:15 PM - 6:30 PM	**155: Impact of Transplant Immunosuppression on In Vivo Lung-Selective CRISPR/Cas9 Therapeutics for Lung Transplantation** *Kumi Mesaki, University Health Network*
6:30 PM - 6:45 PM	**156: First Proof-of-Concept of miQURE™ Based Gene Targeting in the Liver: Lipid Lowering and Atherosclerosis Suppression by AAV-miQURE™-Mediated ANGPTL3 Targeting** *Vanessa Zancanella, Ph.D., uniQure Biopharma*
6:45 PM - 7:00 PM	**158: Electroporation Mediated Gene Transfer of MRCKα to the Lungs of Mice Effectively Treats Pre-Existing Acute Lung Injury** *Jing Liu, University of Rochester School of Medicine and Dentistry*

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ABSTRACT SESSIONS

Clinical Trials and Advanced Preclinical Studies for Neurologic Diseases
Co-Chairs: Heather Gray-Edwards, D.V.M., Ph.D. and Patricia Dickson, M.D.

5:30 PM - 5:45 PM	**159: Gene Therapy Candidate for Metachromatic Leukodystrophy (MLD): Summary of Preclinical In Vivo Data Following an Intravenous Delivery of HMI-202** *Jacinthe Gingras, Ph.D., Homology Medicines Inc.*
5:45 PM - 6:00 PM	**160: Gene Replacement Therapy for Angelman Syndrome** *Justin Percival, Ph.D., University of Pennsylvania*
6:00 PM - 6:15 PM	**161: An AAV-miRNA for Androgen Receptor Knockdown in Spinal and Bulbar Muscular Atrophy** *Eileen Workman, Ph.D., University of Pennsylvania*
6:15 PM - 6:30 PM	**162: AXO-AAV-GM1 for the Treatment of GM1 Gangliosidosis: Preliminary Results From a Phase I-II Trial** *Cynthia Tifft, M.D., Ph.D., National Institutes of Health, NHGRI*
6:30 PM - 6:45 PM	**163: AXO-Lenti-PD Gene Therapy for Parkinson's Disease: Efficacy, Safety, and Tolerability Data from the Second Cohort in Open-Label Dose Evaluation Study SUNRISE-PD at 6 Months Post Administration** *Gavin Corcoran, M.D., Sio Gene Therapies Inc.*
6:45 PM - 7:00 PM	**164: Safety Evaluation of IV-Administered BBP-812, an AAV9-Based Gene Therapy for the Treatment of Canavan Disease, in Mice and Juvenile Cynomolgus Macaques** *David Scott, Ph.D., Aspa Therapeutics*
7:00 PM - 7:15 PM	**165: Gene Replacement Therapy for SURF1-Related Leigh Syndrome Using AAV9** *Qinglan Ling, Ph.D., UT Southwestern*

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ABSTRACT SESSIONS

Downstream Process of Vector Manufacturing
Co-Chairs: Chris Morrison, Ph.D. and Eric Horowitz, Ph.D.

5:30 PM - 5:45 PM	**166: Novel Platform for Transport and Delivery of Recombinant Adeno-Associated Virus without Need for Cold Storage during Transit** *Maria Croyle, Ph.D., University of Texas at Austin*
5:45 PM - 6:00 PM	**167: Exposing the Content of Different AAV Fractions after Ultracentrifugation** *David Dobnik, Ph.D., National Institute of Biology*
6:00 PM - 6:15 PM	**168: Optimization of Affinity Purification for Adeno-associated Viral Vectors** *Huiren Zhao, Amgen Inc.*
6:15 PM - 6:30 PM	**169: Characterization of rAAV Key Quality Attributes Generated From A Highly Optimized, Hela 3.0 Producer Cell Line (PCL) Production Platform** *Nicholas Richards, Ultragenyx Pharmaceuticals, Inc.*
6:30 PM - 6:45 PM	**170: AAVX Resin Binding Site Identification via Library Screening Analysis on Novel AAV Vectors** *Zachary Thorpe, Affinia Therapeutics*
6:45 PM - 7:00 PM	**171: Analysis of Gene Therapy Products by Charge Detection Mass Spectrometry** *Benjamin Draper, Ph.D., Megadalton Solutions*
7:00 PM - 7:15 PM	**172: Use of SPTFF in Continuous Downstream Manufacturing of Adeno-Associate Viruses** *Rajeshwar Chinnawar, Ph.D., Pall Biotech*

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ABSTRACT SESSIONS

Immunotherapy and Vaccines
Co-Chairs: Matt Gardner, Ph.D. and Allison Keeler-Klunk, Ph.D.

5:30 PM - 5:45 PM	**173: A Humanized EBV Mouse Model to Evaluate the Safety/Impact of Human Treg Cell Therapy on Antiviral Immune Responses** *Swati Singh, Seattle Children's Research Institute*
5:45 PM - 6:00 PM	**174: Candidate Selection in BALB/c Mice towards a Single Dose AAV-based COVID19 Prophylactic Vaccine** *Wenlong Dai, Ph.D., Schepens Eye Research Institute and Massachusetts Eye and Ear Infirmary*
6:00 PM - 6:15 PM	**175: AAV Specific CAR Regulatory T Cells Mitigate Immune Responses Against AAV Gene Therapy** *Motahareh Arjomandnejad, Ph.D., University of Massachusetts Medical School*
6:15 PM - 6:30 PM	**176: Engineered Protein M Analogs Enhance the Ability to Suppress Vector Neutralizing Antibodies and Generate a Window for Successful Gene Delivery** *Charles Askew, Ph.D., University of North Carolina, Chapel Hill*
6:30 PM - 6:45 PM	**177: In Vivo HSC Gene Therapy with High-Level, Erythroid-Specific Expression of a Secreted SARS-CoV-2 Decoy Receptor** *Andre Lieber, M.D., Ph.D., University of Washington*
6:45 PM - 7:00 PM	**178: A Platform for Genome Editing of Human B Cells to Produce Single-Chain Antibody-Like Molecules That Recapitulate Antibody Functionality** *Geoffrey Rogers, Ph.D., University of Southern California*
7:00 PM - 7:15 PM	**179: Immune Modulation Preceding AAV9-GLB1 Gene Therapy Preserves the Possibility for Re-Dosing in Children with GM1 Gangliosidosis** *Precilla D'Souza, National Institutes of Health, NHGRI*

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ABSTRACT SESSIONS

New Gene Editing Technologies and Applications
Co-Chairs: Alexis Komor, Ph.D. and T.J. Cradick, Ph.D.

5:30 PM - 5:45 PM
180: Modulation of DNA Repair Pathways by HDR-CRISPR Promotes Seamless Genome Editing in Primary Human Hematopoietic Cells
Antonio Carusillo, University of Freiburg

5:45 PM - 6:00 PM
181: Capturing and Characterizing Single Cell Allelic Heterogeneity of CRISPR-Cas9 Gene Editing In Vivo
Jesse Weber, Children's Hospital of Philadelphia and University of Pennsylvania

6:00 PM - 6:15 PM
182: Dual-HDR Editing Strategies for the Development of Islet-Specific Regulatory T Cells (EngTregs) for Restoration of Immune Tolerance in Type 1 Diabetes
Ahmad Boukhris, Seattle Children's Research Institute

6:15 PM - 6:30 PM
183: A Dimeric, Luminescent Biosensor for Imaging Unique DNA Sequences in Individual Cells
Nicholas Heath, University of California - Davis

6:30 PM - 6:45 PM
184: Efficient CRISPR-Cas9-Mediated Gene Knockout and Interallelic Gene Conversion in Human Induced Pluripotent Stem Cells Using Non-Integrative Bacteriophage-Chimeric Retrovirus-Like Particles
John De Vos, M.D., Ph.D., INSERM

6:45 PM - 7:00 PM
185: Triggering P53 Activation and Trapping of Transcriptionally Active Recombinant AAV Sequences Are Inadvertent Consequences of HSC Genome Editing
Samuele Ferrari, Ph.D., San Raffaele Telethon Institute for Gene Therapy

7:00 PM - 7:15 PM
186: Accurate Quantification of CRISPR/Cas9 Induced Large Deletions, Insertions and Chromosomal Rearrangements Using SMRT Sequencing with Unique Molecular Identifiers
So Hyun Park, Ph.D., Rice University

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ABSTRACT SESSIONS

Novel AAV Biology and Platform Technologies
Co-Chairs: Lauren Woodard, Ph.D. and Anna Maurer, Ph.D.

5:30 PM - 5:45 PM	**187: Effect of pH and Temperature on AAV2 Capsid Structure and Stability** *Joshua Hull, University of Florida*
5:45 PM - 6:00 PM	**188: Development of a Split Rep/Cap System to Improve AAV Capsid Production** *Derek Carbaugh, Ph.D., AskBio*
6:00 PM - 6:15 PM	**189: Intravenous Administration of a Barcoded and Pooled AAV Library for the Comprehensive Characterization and Comparison of Capsid Tropisms** *April Giles, Ph.D., REGENXBIO*
6:15 PM - 6:30 PM	**190: AAV Capsid Property Estimation is Improved by Combining Single-Molecule ID Tags and Hierarchical Bayesian Modeling of Experimental Processes** *Kathy Lin, Ph.D., Dyno Therapeutics*
6:30 PM - 6:45 PM	**191: Hydroxylation of N-acetylneuraminic Acid Influences the In Vivo Tropism of N-linked Sialic Acid-Binding Adeno-Associated Viruses AAV1, AAV5 and AAV6** *Estrella Lopez-Gordo, Ph.D., Icahn School of Medicine at Mount Sinai*
6:45 PM - 7:00 PM	**192: High-Throughput Screening of AAV Productivity to Enable Rapid Capsid Characterization** *Jenny Egley, REGENXBIO*
7:00 PM - 7:15 PM	**193: AAV-GPseq Analysis of Vectors from HEK293 and BEV/Sf9 Production Platforms Reveals Differential Genome Heterogeneity and Enrichment of Potential Innate Immune DNA Epitopes in Empty Capsids** *Ngoc Tam Tran, Ph.D., University of Massachusetts Medical School*

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5:15 PM - 6:45 PM

INDUSTRY SPONSORED SYMPOSIA

Dyno Therapeutics - Building Dyno Therapeutics

DYNO THERAPEUTICS

5:15 PM - 5:35 PM	**Dyno's Origins: Starting a Gene Therapy Company** *Q&A with co-founders: Eric Kelsic, Ph.D. and Sam Sinai, Ph.D., Dyno Therapeutics*
5:35 PM - 5:59 PM	**Dyno's Science Today: Engineering Best-in-Class Capsids** *Q&A with Dyno Scientists: Cem Sengel, Ph.D.; Kathy Lin, Ph.D.; Christopher Reardon; and Elina Locane, Ph.D., Dyno Therapeutics*
5:59 PM - 6:21 PM	**Dyno's Culture Today: How We Work Together as a Team** *Q&A with Dyno Executives: Andrea Szekely-Hill, Paige Swanson, and Tyson Bertmaring, Dyno Therapeutics*
6:21 PM - 6:45 PM	**Dyno's Future: Maximizing Our Positive Impact on Patients** *Q&A with Dyno R&D: Eric Kelsic, Ph.D.; Jamie Kwasnieski, Ph.D.; Sylvain Lapan, Ph.D.; and Jeffrey Gerold, Ph.D., Dyno Therapeutics*

L7 Informatics, Inc. - A Case Study in Digital Transformation in Advanced Therapeutics

L7INFORMATICS

5:15 PM - 6:45 PM	*Moderated by John Conway, 20/15 Visioneers* *Speakers: Ernie Bognar, Ph.D., Gradalis; Trent Carrier, Ph.D., MBA, L7 Informatics, Inc.; Vasu Rangadass, Ph.D., L7 Informatics, Inc.; and Jeff McDaniel, L7 Informatics, Inc.*

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WEDNESDAY, MAY 12, 2021

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Networking Roulette

5:15 PM - 7:00 PM

TOOLS AND TECHNOLOGY FORUM

Tools and Technology Forum III

5:15 PM - 5:30 PM
Combining Highest rAAV Manufacturing Performance with Highest Quality Standards to Support CGT Industry
Mathieu Porte, Polyplus-transfection

5:30 PM - 5:45 PM
ELEVECTA® and CAP® - Leading Technologies for Large Scale Adenoviral and AAV Vector Manufacturing
Petra Nitschke, CEVEC Pharmaceuticals

5:45 PM - 6:00 PM
Insights for Plasmid DNA Manufacturing in 2021
Stephen Rodriguez, VGXI Inc.

6:00 PM - 6:15 PM
Viral Vector Safety Assessment in Cell and Gene Therapy
Wei Wang, Ph.D., MBA, GeneWerk

6:15 PM - 6:30 PM
AAV Xpress ELISAs - High Performance, Fast Results
Dana Holzinger, Ph.D., PROGEN

6:30 PM - 6:45 PM
Characterization of Adeno Associated Viruses
Sahana Mollah, SCIEX

6:45 PM - 7:00 PM
High Throughput Upstream Processing and Quality Analysis via Microfluidic Capillary Electrophoresis
James Geiger, Ph.D., PerkinElmer

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ALL DAY

DIGITAL ABSTRACT PRESENTATIONS

EXHIBIT HALL

Be sure to connect with exhibit booth staff during listed times

9:00 AM - 10:00 AM

CHAT LOUNGE NETWORKING

10:00 AM - 11:45 AM

SCIENTIFIC SYMPOSIA

Hot Topics and Remaining Challenges in RNAi and Oligonucleotide Therapy for 2021 (Organized by the Oligonucleotide and RNAi Therapeutics Committee)
Chair: Paloma Giangrande, Ph.D.

10:00 AM - 10:26 AM	**CNS Toxicity in Higher Mammals Related to AAV-Mediated RNAi Gene Therapy** *Beverly Davidson, Ph.D., Children's Hospital of Philadelphia*
10:26 AM - 10:52 AM	**Splice Correction and Reduction of Toxic DMPK RNA In Vitro and In Vivo Utilizing Novel Antibody Targeted Antisense Oligonucleotides** *Romesh Subramanian, Ph.D., Dyne Therapeutics*
10:52 AM - 11:18 AM	**Discussion of N-Lorem Foundation's Mission and Progress to Create Individual Antisense Therapies for Patients in The United States With Ultra-Rare Diseases Caused by Genetic Mutations That Affect Approximately N=1-10 Patients in the World** *Stanley Crooke, M.D. Ph.D., n-Lorem Foundation*
11:18 AM - 11:45 AM	**Opportunities and Challenges in Development of ASOs for Treatment of Neuromuscular Disease** *Toby Ferguson, M.D., Ph.D., Biogen*

New Advances in Physical Gene Delivery and Nucleic Acid Vectorology
(Organized by the Physical Delivery, Therapeutics & Vector Development Committee)
Co-Chairs: Loree Heller, Ph.D. and Carol Miao, Ph.D.

10:00 AM - 10:26 AM	**Electroporation to Deliver DNA Coronavirus Vaccine** *Kate Broderick, Ph.D., Inovio Pharmaceuticals, Inc.*
10:26 AM - 10:52 AM	**RNA-Guide DNA Insertion With CRISPR-Associated Transposases** *Feng Zhang, Ph.D., Massachusetts Institute of Technology*
10:52 AM - 11:18 AM	**mRNA as Therapeutics to Treat Rare Genetic Disorders** *Paolo Martini, Ph.D., Moderna*
11:18 AM - 11:45 AM	**Ultrasound-Targeted Microbubble Destruction to Facilitate Gene Delivery** *Mark Borden, Ph.D., University of Colorado*

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SCIENTIFIC SYMPOSIA

Newborn Screening: Innovative Policies and Technologies to Eliminate the Diagnostic Odyssey (Organized by the Government Relations Committee)
Co-Chairs: Diane Berry, Ph.D. and Philip Reilly, M.D., J.D.

10:00 AM - 10:15 AM	**Newborn Screening: An Inside Look at the Landscape, Challenges, and Opportunities for Strengthening Newborn Health** *Max Bronstein, MGB Consulting*
10:15 AM - 10:30 AM	**The Impact of Newborn Screening Policies for Genetic Diseases on Children and Families** *Elisa Seeger, Aiden Jack Seeger Foundation*
10:30 AM - 10:45 AM	**How Will Gene and Cell Therapies Transform Newborn Screening?** *Don Bailey, Ph.D., RTI International*
10:45 AM - 11:00 AM	**Screenplus: A Multi-Disorder, Multi-Tiered, Consented Pilot Newborn Screening Program** *Melissa Wasserstein, M.D., Montefiore Medical Center*
11:00 AM - 11:15 AM	**Advances in Diagnostic Testing Technology: Implications for NBS** *Ryan Taft, Ph.D., Illumina*
11:15 AM - 11:45 AM	**Panel Discussion**

Race to Respiratory Therapies for COVID-19
(Organized by the Respiratory and GI Tract Gene and Cell Therapy Committee)
Chair: Amy Ryan, Ph.D.

10:00 AM - 10:35 AM	**Animal Models for SARS-CoV-2 Research** *Tracy Webb, D.V.M., Ph.D., Colorado State University and Danielle Adney, Ph.D., National Institutes of Health, NIAID*
10:35 AM - 11:10 AM	**AAVCOVID: An AAV-Based, Single Dose, Rt Stable Covid-19 Vaccine Candidate** *Luk Vandenberghe, Ph.D., Grousbeck Gene Therapy Center, Mass Eye and Ear, Harvard & The Broad Institute of Harvard and MIT*
11:10 AM - 11:45 AM	**Airway Vaccine Approach SARS-Cov-2 Using RAAV** *James Wilson, M.D., Ph.D. University of Pennsylvania*

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SCIENTIFIC SYMPOSIA

RNA Therapies for Neurologic and Ophthalmic Disorders (Organized by the Neurologic & Opthalmic Gene and Cell Therapy Committee)
Co-Chairs: Kourous Rezaei, M.D. and Jason Shepherd, Ph.D.

10:00 AM - 10:26 AM	**Splicing Modulation Therapy for Inherited Retinal Diseases** *Rob Collin, Ph.D., Radboud University Medical Centre*
10:26 AM - 10:52 AM	**The Use of ODNs for Cellular Therapy of Neurodegenerative Disorders** *Holly Kordasiewicz, Ph.D., Ionis Pharmaceuticals*
10:52 AM - 11:18 AM	**From Impossible to Possible: A Personal Journey From Diagnosis to Drug Development for Angelman Syndrome** *Allyson Berent, D.V.M., Foundation for Angelman Syndrome Therapeutics*
11:18 AM - 11:45 AM	**Shifting the Paradigm of Gene Therapy for Neuromuscular Diseases** *Pavlina Konstantinova, Ph.D., Vectory*

Safety and Efficacy of Body-Wide Therapy for Musculo-Skeletal Diseases (Organized by the Musculo-Skeletal Gene & Cell Therapy Committee)
Co-Chairs: Jyoti Jaiswal, Ph.D. and Christina Pacak, Ph.D.

10:00 AM - 10:26 AM	**Overview of Data From Clinical Trials of AAV Gene Therapy for DMD** *Perry Shieh, M.D., Ph.D., University of California, Los Angeles*
10:26 AM - 10:52 AM	**Safety/Efficacy of Systemic Genome Editing for DMD** *Christopher Nelson, Ph.D., University of Arkansas*
10:52 AM - 11:18 AM	**Optimization Studies to Enable a Phase 1 Clinical Trial of iPSC-Derived Myogenic Progenitors for DMD** *Rita Perlingeiro, Ph.D., University of Minnesota*
11:18 AM - 11:45 AM	**rAAV Controlled Gene Delivery to Heal Cartilage Defects Combined With Biomaterials in Large Animals** *Magali Cucchiarini, Ph.D., Saarland University Medical Center*

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SCIENTIFIC SYMPOSIA

Vaccine Nanotechnology for Rapid Response Applications (Organized by the Nanoagents and Synthetic Formulations Committee)
Co-Chairs: Jordan Green, Ph.D. and Julie Champion, Ph.D.

10:00 AM - 10:26 AM	**Polyplex Based DNA Nanoparticles as Vaccines**	*Darrell Irvine, Ph.D., Massachusetts Institute of Technology*
10:26 AM - 10:52 AM	**Identifying Vital Immunogenic Epitopes in Pathogens as Therapeutic Targets**	*Guarav Gaiha, M.D., Ragon Institute of MGH*
10:52 AM - 11:18 AM	**Replicating Viral RNA as Gene Expression Platform**	*Jesse Erasmus, Ph.D., University of Washington*
11:18 AM - 11:45 AM	**Development of RNA Vaccines**	*Drew Weissman, Ph.D., University of Pennsylvania*

10:00 AM - 11:45 AM

ORAL ABSTRACT SESSIONS

Clinical Trials Spotlight Symposium
Chair: Terence R. Flotte, M.D.

10:00 AM - 10:15 AM	**194: Systemic AAV Delivery Activates the Classical Complement Pathway Leading to Thrombotic Microangiopathy**	*Stephanie Salabarria, BH.Sc., University of Florida*
10:15 AM - 10:30 AM	**195: Activity of Banked (Off-the-Shelf) CD30. CAR-Modified Epstein-Barr Virus-Specific T Cells in Patients with CD30-Positive Lymphoma**	*David Quach, Ph.D.*
10:30 AM - 10:45 AM	**196: Updated Results From HGB-206 LentiGlobin for Sickle Cell Disease Gene Therapy Study: Group C Data and Group A AML Case Investigation**	*John Tisdale, M.D., National Institutes of Health*
10:45 AM - 11:00 AM	**197: ADVM-022 Intravitreal Gene Therapy for Neovascular AM.D. - Results From the Phase 1 OPTIC Study**	*Szilárd Kiss, M.D., Weill Cornell Medicine*

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ORAL ABSTRACT SESSIONS

11:00 AM - 11:15 AM
198: Natural Killer T Cells Expressing a GD2-CAR and IL-15 Are Safe and Can Induce Complete Remission in Children with Relapsed Neuroblastoma - A First-in-Human, Phase 1 Trial
Andras Heczey, M.D., Baylor College of Medicine

11:15 AM - 11:30 AM
199: Long Term Follow Up for the Development of Subsequent Malignancies in Patients Treated With Genetically Modified Immune Effectors
David Steffin, M.D., Baylor College of Medicine

11:30 AM - 11:45 AM
200: Updated Results of Transpher A, a Multicenter, Single-Dose, Phase 1/2 Clinical Trial of ABO-102 Gene Therapy for Sanfilippo Syndrome Type A (Mucopolysaccharidosis IIIA)
Kevin Flanigan, M.D., Nationwide Children's Hospital

10:45 AM - 12:15 PM

EXHIBIT HALL OPEN - EXHIBITORS AVAILABLE TO CONNECT

Exhibit Hall is Accessible 24 Hours

10:45 AM - 11:30 PM

EXHIBITOR SHOWCASES

10x Genomics - 10:45 AM - 11:30 AM

10x GENOMICS

Single Cell Multiomics to Accelerate Cancer Immune Cell Therapy Research

10:45 AM - 11:15 AM **Multi-modal and Multi-tissue Profiling of Glioblastoma Patients Treated with CAR T Cell Therapy**
Nicholas Banovich, Ph.D., Translational Genomics Research Institute (TGen)

11:15 AM - 11:30 AM **Single Cell and Spatial Multiomics to Accelerate Cancer Immune Cell Therapy Research**
Abbey Cutchin, 10x Genomics

Informa Pharma Intelligence - 10:45 AM - 11:30 AM

Pharma Intelligence Informa

Trends and Challenges in Cell & Gene Therapy
Ly Nguyen-Jatkoe, Ph.D., Informa Pharma Intelligence

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12:15 PM - 2:00 PM

ABSTRACT SESSIONS

Base Editing and Gene Editing Approaches
Co-Chairs: Giulia Pavani, Ph.D. and Shengdar Tsai, Ph.D.

12:15 PM - 12:30 PM	**201: Intracellular Rnase Activity Limits the Efficiency of mRNA-Based Gene Editing in Nonhuman Primate Hematopoietic Stem and Progenitor Cells** *Christopher Peterson, Ph.D., Fred Hutchinson Cancer Research Center*
12:30 PM - 12:45 PM	**205: Cleavage-Free dCas9 Knock-In Gene-Editing Tool Leveraging RNA-Guided Targeting of Recombineering Proteins** *Le Cong, Ph.D., Stanford University*
12:45 PM - 1:00 PM	**203: Novel CRISPR-Associated Transposase Systems for Targeted DNA Integration** *Daniela Goltsman, Ph.D., Metagenomi*
1:00 PM - 1:15 PM	**204: CRISPR-Cas9 Cytidine and Adenosine Base Editing of Splice-Sites Mediates Highly-Efficient Disruption of Proteins in Primary and Immortalized Cells** *Mitchell Kluesner, University of Minnesota*
1:15 PM - 1:30 PM	**202: Enhancing the RNA Base-Editing Activity, Functionality and Specificity of the ADAR2 Deaminase Domain** *Dhruva Katrekar, Ph.D., University of California - San Diego*
1:30 PM - 1:45 PM	**206: Novel CRISPR Associated Gene Editing Systems Discovered in Metagenomic Samples Enable Efficient and Specific Genome Engineering for Cell Therapy Development** *Gregory Cost, Ph.D., Metagenomi*
1:45 PM - 2:00 PM	**207: Base Editing Approaches for the Treatment of β-hemoglobinopathies through Disruption of the Erythroid-Specific BCL11A Enhancer to Reactivate Fetal Hb** *Panagiotis Antoniou, Institut Imagine*

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ABSTRACT SESSIONS

Cancer Immunotherapy
Co-Chairs: Jan Joseph Melenhorst, Ph.D. and Sarwish Rafiq, Ph.D.

12:15 PM - 12:30 PM
208: Clinical Application of CRISPR Edited Tumor Infiltrating Lymphocytes in Gastrointestinal Cancer
Beau Webber, Ph.D., University of Minnesota

12:30 PM - 12:45 PM
209: Single-Cell Sequencing Approach for the Discovery of Mutant IDH1 Reactive T Cell Receptors From a Glioma Vaccine Trial and an MHC-Humanized Mouse Model
Khwab Sanghvi, German Cancer Research Center

12:45 PM - 1:00 PM
210: Epigenome Editing Enables PD1 Silencing in Primary T Cells
Maria Silvia Roman Azcona, University of Freiburg

1:00 PM - 1:15 PM
211: mRNA Delivery of an Evolved Bispecific Single Domain Antibody to Synergize the Immune Checkpoint Blockade Therapy for Liver Malignancies
Rihe Liu, Ph.D., University of North Carolina - Chapel Hill

1:15 PM - 1:30 PM
212: Exhausted, Tumor-Specific T Cells Can Be Exploited to Generate a Library of T-Cell Receptors for Adoptive T-Cell Therapy in Blood Malignancies
Francesco Manfredi, M.D., San Raffaele University

1:30 PM - 1:45 PM
213: Changes in the Tumor Microenvironment in Patients with Glioblastoma Multiforme Treated with IFN-a Immune Cell & Gene Therapy (TEM-GBM_001 Study)
Bernhard Gentner, M.D., San Raffaele Telethon Institute for Gene Therapy

1:45 PM - 2:00 PM
214: Small Molecule-Regulated Gene Circuit for Controlling Cytokine Expression in Cell Therapies
Michelle Hung, Ph.D., Senti Bio

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ABSTRACT SESSIONS

Gene Therapy for Lysosomal Storage Disorders
Co-Chairs: Lina Colella, Ph.D. and Pasquale Piccolo, Ph.D.

12:15 PM - 12:30 PM
215: RGX-121 Gene Therapy for the Treatment of Severe Mucopolysaccharidosis Type II: Interim Analysis of the First in Human Study
Roberto Giugliani, M.D., Medical Genetics Service, HCPA

12:30 PM - 12:45 PM
216: AVR-RD-01, an Investigational Lentiviral Gene Therapy for Fabry Disease: Clinical Data Trends from Phase 1 and Phase 2 Studies up to 3.5 Years
Mark Thomas, M.D., Royal Perth Hospital

12:45 PM - 1:00 PM
217: Updated Results of Transpher B, a Multicenter, Single-Dose, Phase 1/2 Clinical Trial of ABO-101 Gene Therapy for Sanfilippo Syndrome Type B (Mucopolysaccharidosis IIIB)
Maria de Castro, M.D., Hospital Clínico Universitario de Santiago de Compostela

1:00 PM - 1:15 PM
218: Safety and Efficacy of Liver-Directed Gene Therapy in Patients with Mucopolysaccharidosis Type VI
Nicola Brunetti-Pierri, M.D., Telethon Institute of Genetics and Medicine

1:15 PM - 1:30 PM
219: Ex Vivo Hematopoietic Stem Cell Gene Therapy for Mucopolysaccharidosis Type IH (Hurler Syndrome): An Interim Analysis with a Median Follow Up of 19 Months
Bernhard Gentner, M.D., San Raffaele Telethon Institute for Gene Therapy

1:30 PM - 1:45 PM
220: A Targeted AAV Gene Therapy Product Candidate, 4D-310, for the Treatment of Fabry Disease: Intravenous Biodistribution, Transgene Expression and Safety in Non-Human Primates
Kevin Whittlesey, Ph.D., 4D Molecular Therapeutics

1:45 PM - 2:00 PM
221: Efficacy of Intracerebroventricular Adeno-Associated Virus Encoding Iduronidase with Dorsal Root Ganglia-Detargeting Sequences in a Mouse Model of Mucopolysaccharidosis Type I
Juliette Hordeaux, D.V.M., Ph.D., University of Pennsylvania

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ABSTRACT SESSIONS

Lentiviral Vector Manufacturing
Co-Chairs: Magalie Penaud-Budloo, Ph.D. and Matthias Hebben, Ph.D.

12:15 PM - 12:30 PM	**222: Stabilization of Lentiviral Vector Genomic RNA during Production Using a Novel RNA Chaperone Leads to Increased Yields** *Jordan Wright, Ph.D., Oxford BioMedica*
12:30 PM - 12:45 PM	**223: Knockout of Entry Receptors in Virus Producer Cells for Improved Titer and Quality BaEVRless-Pseudotyped Retroviral Particles** *Denise Klatt, Ph.D., Dana Farber Cancer Institute*
12:45 PM - 1:00 PM	**224: Development of pEMBR™- An Improved Adenovirus Helper Plasmid for AAV Production** *David Dismuke, Ph.D., Forge Biologics*
1:00 PM - 1:15 PM	**225: Genetically Engineering Packaging Cells to Enhance Titer and Infectivity of Lentiviral Vectors** *Jiaying Han, University of California - Los Angeles*
1:15 PM - 1:30 PM	**226: Long-Term Expression Comparison of Adeno-Associated Virus (AAV) Vector Produced in HEK293 vs Sf Cell Lines** *Britta Handyside, Ph.D., BioMarin Pharmaceutical Inc.*
1:30 PM - 1:45 PM	**227: Processing of Lentiviral Vectors Pseudotypes Using Anion Exchange and Affinity Chromatography** *Yuefei Huang, Ph.D., U.S. Food and Drug Administration*
1:45 PM - 2:00 PM	**228: Separation of Empty Capsids from Full Capsids for AAV Gene Therapy Using a Flow through and Step Elution Approach** *Danielle Ladwig, Voyager Therapeutics*

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ABSTRACT SESSIONS

Metabolic and Muscle Diseases, Tissue and Immunological Engineering
Co-Chairs: Douglas Martin, Ph.D. and Isabelle Richard, Ph.D.

12:15 PM - 12:30 PM
229: Immunogenicity of An AAV-based, Room-Temperature Stable, Single Dose COVID-19 Vaccine in Mouse and NHP
Nerea Zabaleta, Ph.D., Grousbeck Gene Therapy Center

12:30 PM - 12:45 PM
230: Systemic AAV9 Gene Therapy Rescues Propionic Acidemia (PA) Mice From Neonatal Lethality and Provides Sustained Therapeutic Benefits
Lina Li, M.D., Ph.D., National Human Genome Research Institute

12:45 PM - 1:00 PM
231: A Randomized, Double-Blind, Placebo-Controlled, Gene-Delivery Clinical Trial of rAAVrh74.MHCK7.micro-dystrophin for Duchenne Muscular Dystrophy
Jerry Mendell, M.D., Nationwide Children's Hospital

1:00 PM - 1:15 PM
232: Unprecedented Low Dose of AAV-Mediated Gene Transfer Corrects the Pathology in a Model for Fukutin-Related-Protein Deficiencies
Isabelle Richard, Ph.D., Genethon

1:15 PM - 1:30 PM
233: AAV Mediated Apelin Transduction Facilitates Cell Cycle Entry of cTnT-Positive Cells in the Heart
Andrew Park, AstraZeneca

1:30 PM - 1:45 PM
234: Vectorized SARS-CoV-2 Human IgG Expression in Mice and Ovine Animal Models Is Feasible and Well Tolerated
Amira Rghei, University of Guelph

1:45 PM - 2:00 PM
235: Intravenous AAV5 Gene Therapy with Human CYP21A1 Corrects Phenotypic Deficiencies of the 21-Hydroxylase Knockout Mouse Model and Demonstrates Durability and Safety in Non-Human Primates and Mice
Rachel Eclov, Ph.D., BridgeBio

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ABSTRACT SESSIONS

New Technologies Advancing Gene Therapy for Neurologic Diseases
Co-Chairs: Dan Wang, Ph.D. and Paul Valdmanis, Ph.D.

12:15 PM - 12:30 PM	**236: Combined Transgene and Intron-Derived miRNA Therapy Reverses Motor Phenotypes in SCA1 Mice** *Ellie Carrell, Ph.D., Raymond G. Perelman Center for Cellular and Molecular Therapeutics*
12:30 PM - 12:45 PM	**237: Combining MALDI-Based Metabolic Imaging and Molecular Analysis for Effective and Informative Assessment of Therapeutic Efficacy in Sub-Anatomical Regions of the CNS After rAAV Gene Therapy** *Dominic Gessler, M.D., Ph.D., University of Massachusetts*
12:45 PM - 1:00 PM	**238: Evolution of Modified AAV in Rhesus Macaque Brain** *Paul Ranum, Ph.D., The Children's Hospital of Philadelphia*
1:00 PM - 1:15 PM	**239: Real-Time MR Tracking of AAV Gene Therapy with Enzyme-Activated MR Probes** *Toloo Taghian, Ph.D., University of Massachusetts Medical School*
1:15 PM - 1:30 PM	**240: Inclusion of a Degron Reduces Levels of Undesired Inteins after AAV-Mediated Protein Trans-Splicing in the Retina** *Patrizia Tornabene, Telethon Institute of Genetics and Medicine*
1:30 PM - 1:45 PM	**241: Drug-Regulated Splicing Switch for Gene Expression Control** *Alex Monteys, Ph.D., University of Pennsylvania*
1:45 PM - 2:00 PM	**242: Targeted Gene Therapy with Engineered Systemic AAVs for the Central and Peripheral Nervous Systems Prevents Motor Coordination Phenotypes in a Mouse Model of Friedreich's Ataxia** *Acacia Hori, California Institute of Technology*

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12:15 PM - 2:00 PM

ABSTRACT SESSIONS

Oligonucleotide Therapeutics
Co-Chairs: Mark Kay, M.D., Ph.D. and Paloma Giangrande, Ph.D.

12:15 PM - 12:30 PM
243: Robust RNA Editing via Recruitment of Endogenous ADARs Using Circular Guide RNAs
Dhruva Katrekar, Ph.D., University of California - San Diego

12:30 PM - 12:45 PM
244: In Vivo Delivery of AAV.U7 Induce Efficient Exon Skipping for a Mutational Hotspot of the DM.D. Gene Results in Protein Restoration & Force Improvement in Skeletal Muscles, Heart & Diaphragm
Dhanarajan Rajakumar, Ph.D., Nationwide Children's Hospital

12:45 PM - 1:00 PM
245: A Versatile Platform for ADAR-Mediated RNA Editing In Vivo in Preclinical Models
Prashant Monian, Ph.D., Wave Life Sciences

1:00 PM - 1:15 PM
246: Human miRNA mir-675 Inhibits DUX4 Expression And May Be Exploited As A Potential Treatment For Facioscapulohumeral Muscular Dystrophy
Nizar Saad, Ph.D., Nationwide Children's Hospital

1:15 PM - 1:30 PM
247: The FORCE™ Platform Achieves Robust Knock Down of Toxic Human Nuclear DMPK RNA and Foci Reduction in DM1 Cells and in Newly Developed hTfR1/DMSXL Mouse Model
Stefano Zanotti, Ph.D., Dyne Therapeutics

1:30 PM - 1:45 PM
248: In Vivo Delivery of Suppressor tRNA Overcomes a Pathogenic Nonsense Mutation in Mice
Jiaming Wang, Ph.D., University of Massachusetts Medical School

1:45 PM - 2:00 PM
249: The 3´-tsRNAS Are Aminoacylated Further Implicating Their Role in Ribosome Biogenesis During Tissue Homeostasis and Cancer
Mark Kay, M.D., Ph.D., Stanford University School of Medicine

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ABSTRACT SESSIONS

Pharmacology/Toxicology Studies or Assay Development
Co-Chairs: Cristina Baricordi, Ph.D. and Carmen Unzu, Ph.D.

12:15 PM - 12:30 PM
250: Liquid-Biopsy-Integration-Site-Sequencing Allows Safety Studies and Longitudinal Monitoring of Vector Integration Sites in LV and AAV-Based In-Vivo GT Applications
Daniela Cesana, Ph.D., San Raffaele Telethon Institute for Gene Therapy

12:30 PM - 12:45 PM
251: Physiologically Based Pharmacokinetic Modeling for the Biodistribution of Adeno-Associated Virus Serotype 8 after Intravenous Administration in Mice and Non-Human Primates
Kefeng Sun, Ph.D., Shire Human Genetic Therapies

12:45 PM - 1:00 PM
252: LC-MS Confirmation of Single Amino Acid Correction by Base Editing
Bo Yan, Ph.D., Beam Therapeutics

1:00 PM - 1:15 PM
253: Lack of Germline Transmission in Male Mice Following Administration of AAV5-hFVIII-SQ, an Investigational Gene Therapy for Hemophilia A
Cheng Su, Ph.D., BioMarin Pharmaceutical Inc.

1:15 PM - 1:30 PM
254: DNA Sequence Analysis of Recombinant Adeno-Associated Viral Integrations Events Recovered from Hepatocellular Carcinomas in Mice Reveals Enhancer Insertion as the Mechanism of Vector Genotoxicity
Randy Chandler, Ph.D., National Institutes of Health

1:30 PM - 1:45 PM
255: Towards a Clinical Trial of Gene Therapy for Retinitis Pigmentosa Associated with Usher Syndrome Type IB
Rita Ferla, Ph.D., Telethon Institute of Genetics and Medicine

1:45 PM - 2:00 PM
256: A GLP Safety and Biodistribution Study of AXO-Lenti-PD Manufactured via Two Processes Delivered at a Higher Volume and Flow Rate
Thomas Pack, Ph.D., Sio Gene Therapies

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ABSTRACT SESSIONS

RNA Virus Vectors
Co-Chairs: Brian Bigger, Ph.D. and Andrew Wilber, Ph.D.

12:15 PM - 12:30 PM — **257: Combinatorial Relief of Multiple Innate Immune Blocks Allows Efficient Gene Engineering of Quiescent Human Hematopoietic Stem Cells**
Erika Valeri, San Raffaele Telethon Institute for Gene Therapy

12:30 PM - 12:45 PM — **258: Administration During Liver Growth Improves the Efficiency of Lentiviral Vector Based In Vivo Gene Therapy in Mice**
Francesco Starinieri, San Raffaele Telethon Institute for Gene Therapy

12:45 PM - 1:00 PM — **259: MicroRNA Detargeting Proves Superior to Genetic Attenuation for Balancing Safety and Efficacy of Oncolytic Mengovirus in Immunodeficient Glioblastoma Mouse Model**
Yogesh Suryawanshi, M.D., Ph.D., Mayo Clinic

1:00 PM - 1:15 PM — **260: CTCF-Based Chromatin Insulators and Enhancers in Lentiviral Vectors Impact Genome Topology and Vector Safety**
Monica Volpin, Ph.D., San Raffaele Telethon Institute for Gene Therapy

AAV Vectors - Clinical Studies
Co-Chairs: Steven Gray, Ph.D. and Diana Bharucha-Goebel, M.D.

1:15 PM - 1:30 PM — **261: AAV8-Mediated Liver-Directed Gene Therapy as a Potential Therapeutic Option in Adults with Glycogen Storage Disease Type Ia (GSDIa): Updated Phase 1/2 Clinical Trial Results**
David Rodriguez-Buritica, M.D., University of Texas McGovern Medical School

1:30 PM - 1:45 PM — **262: Safety, β-Sarcoglycan Expression, and Functional Outcomes from Systemic Gene Transfer of rAAVrh74.MHCK7.SGCB in Limb Girdle Muscular Dystrophy Type 2E/R4**
Louise Rodino-Klapac, Ph.D., Sarepta Therapeutics

1:45 PM - 2:00 PM — **263: IGNITE-DMD Phase I/II Study of SGT-001 Microdystrophin Gene Therapy for Duchenne Muscular Dystrophy**
Carl Morris, Ph.D., Solid Biosciences

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