American Society of Gene + Cell Therapy

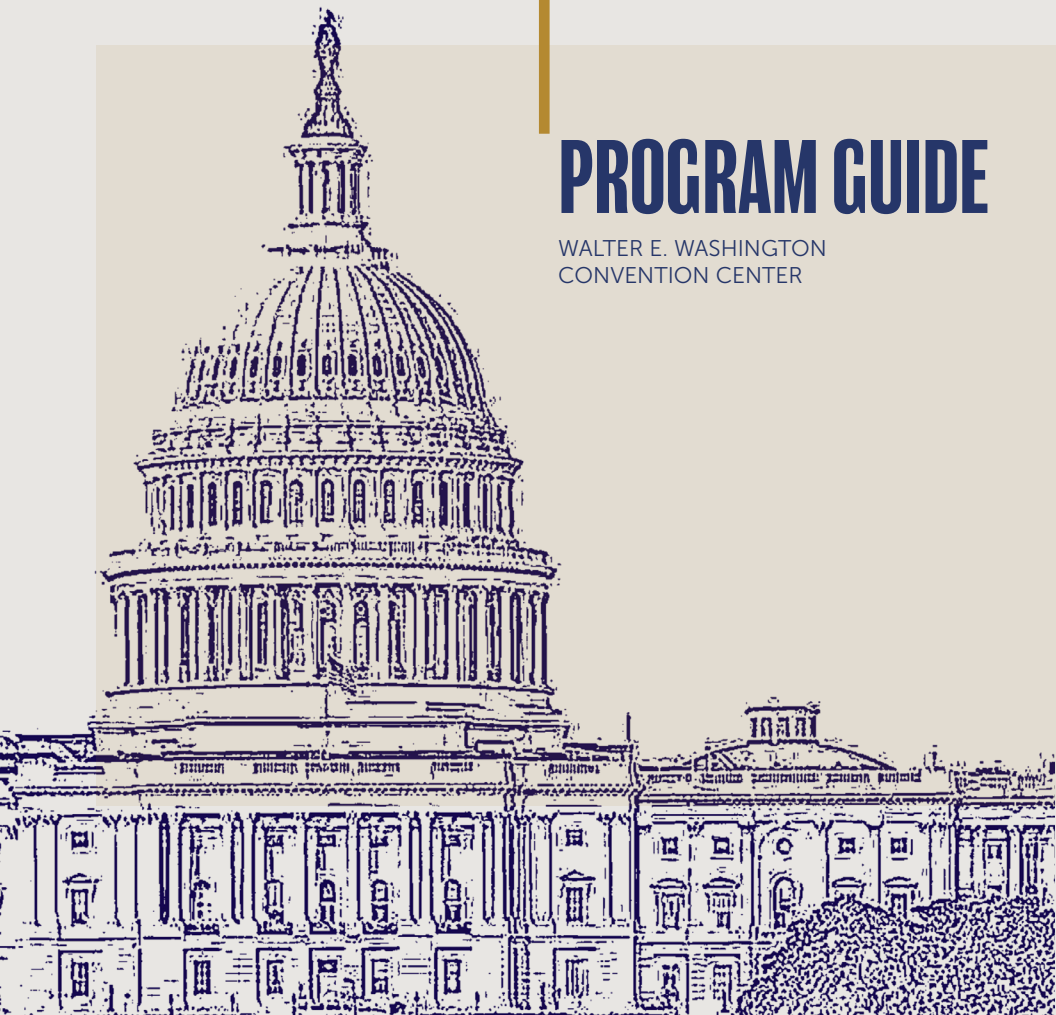
ASGCT 25TH ANNUAL MEETING

MAY 16–19 · 2022

WASHINGTON DC

PROGRAM GUIDE

WALTER E. WASHINGTON
CONVENTION CENTER



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A WELCOME LETTER FROM BEVERLY L. DAVIDSON, PHD

Dear Colleagues,

On behalf of the American Society of Gene & Cell Therapy (ASGCT), I am thrilled to welcome you to our first Annual Meeting back in person since 2019. After almost three years apart, I feel especially honored to join you and thousands of our colleagues from around the world who believe in the 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.

Whether you're gathering with us in person in Washington, D.C. or virtually, I hope you're looking forward to a week that will be packed with the latest research developments in gene and cell therapy. Please remember that all attendees will have on-demand access to the plenary lectures, oral abstract presentations, scientific symposia, education sessions, virtual poster hall, and more, for 30 days following the meeting.

I would like to extend a warm welcome to Drew Weissman, MD, PhD, and ASGCT Past President Kathy High, MD, who will present the George Stamatoyannopoulos Memorial Lecture on Wednesday morning and the Presidential Symposium on Wednesday afternoon, respectively. We'll also hear from former NIH Director Francis Collins, MD, PhD, who will receive the first ASGCT Founders Award recognizing his contributions to the field.

I'd like to congratulate our Annual Meeting award winners: Don Kohn, MD, recipient of the Outstanding Achievement Award; Lindsey George, MD, Matthew Hirsch, PhD, Morgan Maeder, PhD, and Christopher Peterson, PhD, recipients of the Outstanding New Investigator Award; Kathy High, MD, recipient of the Jerry Mendell Award for Translational Science; and P.J. Brooks, PhD, recipient of the Sonia Skarlatos Public Service Award. Thank you for your important work to move the field forward.

If you're here in person, make sure to visit the Exhibit Hall to learn about the latest products and services from dozens of companies in the space. For those who are on the job hunt or looking to build connections, our Career Fair and Mentor Meet-Up are open to all meeting registrants.

Finally, I would like to thank all of you involved in the planning and execution of the Annual Meeting, especially the Program Committee, Scientific and Education Committees, abstract reviewers, staff, and all of our volunteers. I can't think of a better way to celebrate 25 years of our Society's progress than to hold our first-ever hybrid Annual Meeting for what we hope will be our largest audience to date.

Thank you for supporting ASGCT and enjoy the meeting!

Sincerely,
Beverly L. Davidson, PhD
President, ASGCT

GENERAL MEETING INFORMATION

Mission + Vision

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

Abstract Publications

All abstracts accepted for presentation at the ASGCT 25th Annual Meeting have been published in a May supplement of *Molecular Therapy* and are searchable for free on annualmeeting.asgct.org.

Continuing Medical Education

The ASGCT Board of Directors decided that the Society will not offer CME credit for the 25th 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.

Disclosures are published on the Annual Meeting website agenda and at the beginning of every presentation.

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 National Institutes of Health (NIH) and Food and Drug Administration (FDA) faculty during the educational program.

Evaluation Method

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

Needs

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

Target Audience

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

Dates + Location

The ASGCT 25th Annual Meeting will be a hybrid event. The in-person portion will be held at the Walter E. Washington Convention Center in Washington, D.C. A virtual registration will also be offered. Both virtual and in-person attendees will have access to the virtual platform and on-demand content.

The meeting will begin on the morning of Monday, May 16, 2022 and continue through 12 PM (ET) on Thursday, May 19, 2022. The Exhibit Hall will be open daily 9:45 AM to 6:30 PM (ET) Monday, May 16 through Wednesday, May 18 in Hall D. All ASGCT content and select sponsor-generated content will be available to registrants on-demand for 30 days following the conclusion of the meeting.

Photography + Video Recording

ASGCT contracts with photographers and videographers throughout the Annual Meeting to assist in the creation of future marketing materials. By attending the 25th Annual Meeting you understand and agree that your image may be recorded and used in ASGCT promotional materials.

Printing Instructions

To print this program:

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

HEALTH AND SAFETY

Vaccine Requirement

The safety of meeting attendees is ASGCT's top priority and we believe the most effective way to help ensure that safety is for everyone to be vaccinated against COVID-19. **Please note that proof of vaccination is required for all in-person meeting attendees prior to traveling to Washington, D.C.**

We define "fully vaccinated" to be:

- At least 14 days past the final administration of the second dose in a two-dose series (i.e. Pfizer or Moderna vaccines),

OR

- At least 14 days past a single-dose administration (i.e. Johnson & Johnson vaccine)

ASGCT will accept a full course of immunization with any vaccine that has been authorized by the World Health Organization (WHO) or the U.S. Food and Drug Administration (FDA). The product may be either fully authorized or authorized under an Emergency Use Authorization (EUA) or equivalent at the time of travel. Vaccination with an agent that is not included on the WHO or FDA website as authorized will not be accepted.

Booster shots are not required but are recommended for any eligible communities. For those unable to travel or meet the vaccination requirement, ASGCT is offering a virtual registration option.

Vaccine Verification

Prior to arriving on site, attendees will be asked to provide proof that they are fully vaccinated with a **vaccine approved by the WHO or FDA.** ASGCT is partnering with Safe Expo for vaccination verification services to make your entry to the meeting faster, safer, and more secure. In-person meeting attendees are required to upload your vaccination card to the **secure vaccination verification portal** before the event. Please visit the Safe Expo desk near registration for assistance with vaccine verification.

COVID-19 Testing Available On-site

A negative COVID-19 test is not required to attend the meeting, but testing is available for your convenience if you choose to utilize it. Onsite testing will be available at the Annual Meeting during the hours below. ASGCT is working with Safe Expo to manage the onsite health and safety plan. If you want to test onsite, Safe Expo is offering three options. You will be responsible for the cost of your test.

Rapid Antigen Test – $50 each
Rapid Molecular NAAT LAMP Test* – $150 each
Rapid RT-PCR – $200 each

*This test is different from a PCR, which requires a 24–48-hour turnaround. This test is acceptable in Canada, Japan, Korea, and most of Europe, but please double check your local health guidelines to be sure.

Testing Hours

COVID-19 testing is available in Salon D on the street level of the convention center during the following hours. To save time, please **schedule your test in advance.**

Sunday, May 15: 8:00 AM to 5:00 PM
Monday, May 16: 8:00 AM to 5:00 PM
Tuesday, May 17: 8:00 AM to 5:00 PM
Wednesday, May 18: 8:00 AM to 5:00 PM

If you test positive for COVID-19 at any point immediately before or during the 25th Annual Meeting, you must surrender your badge and **follow CDC guidance on quarantine and isolation.** You will not be allowed to attend sessions in person until a negative test can be produced, although you will still have access to the virtual platform. Information on individuals who test positive, including their names, will be provided to staff overseeing the COVID-19 testing and response efforts. Safe Expo will report positive test results to the Washington D.C. Public Health Service as required by law. The identity of individuals who test positive will be kept confidential to the general public and meeting participants.

International Attendees

International attendees should always check current travel restrictions to the U.S. on the **CDC website** before planning travel arrangements. In addition, please monitor regulations in your home country to ensure that you will be able to return from the U.S.

Other Guidelines

Anyone experiencing flu-like symptoms or symptoms associated with COVID-19 must refrain from attending the meeting in-person. The CDC states the symptoms of COVID-19 include: fever or chills, cough, shortness of breath or difficulty breathing, fatigue, muscle or body aches, headaches, new loss of taste or smell, sore throat, congestion or running nose, nausea or vomiting, diarrhea, or a fever (100.4º F or higher). As a reminder, all registered attendees will have access to view the event virtually.

If you are attending in person and you experience any COVID-19 symptoms, please refrain from attending and notify healthandsafety@asgct.org as soon as possible.

Please visit the **Health & Safety section** of the Annual Meeting website for the most up-to-date information.

ON-SITE SAFETY MEASURES

Masks

ASGCT is requiring its staff and all on-site attendees to wear masks regardless of whether it is required by law at the time of the meeting. Masks must be always covering both mouth and nose except when actively eating or drinking, or when giving a presentation as an invited program speaker. The mask requirement applies to the convention center, hotels, shuttle buses, and anywhere an in-person ASGCT event is being held.

Disposable surgical masks will be available for free if you do not have a proper facial covering.

Physical Distancing

Meeting rooms will be set in theater style at their maximum capacity. Attendees can choose to sit next to someone or distance themselves if they prefer more personal space. All in-person attendees will have access to the virtual platform to view sessions in real time if they feel uncomfortable with the space in meeting rooms.

Sanitization

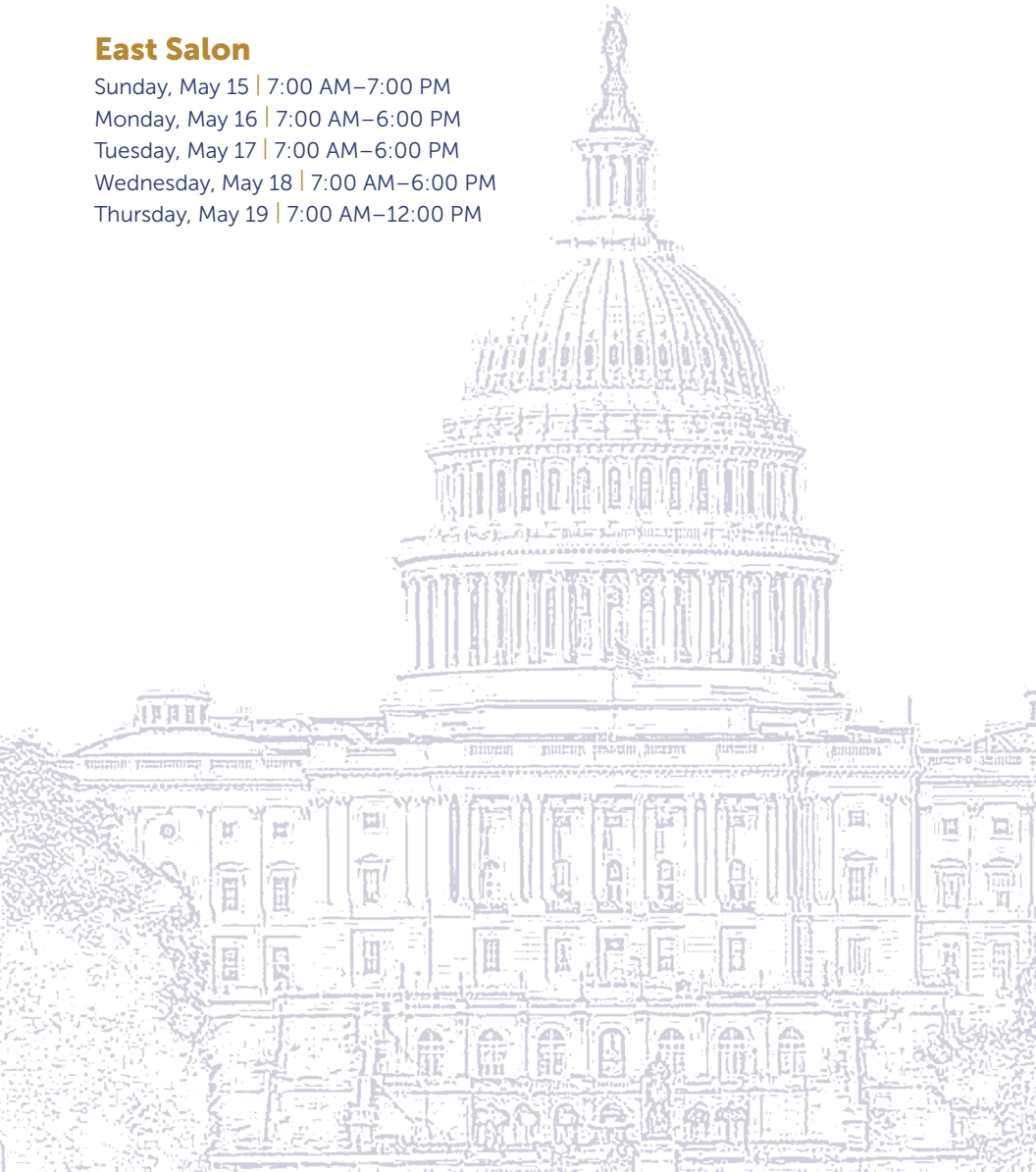
Even though current research shows that COVID-19 transmission is unlikely to occur from touching surfaces, convention center staff will regularly clean and disinfect surfaces to promote healthy sanitation.

The convention center also has hand sanitizer locations which are stocked and deployed strategically. They are monitored and replenished frequently.

REGISTRATION

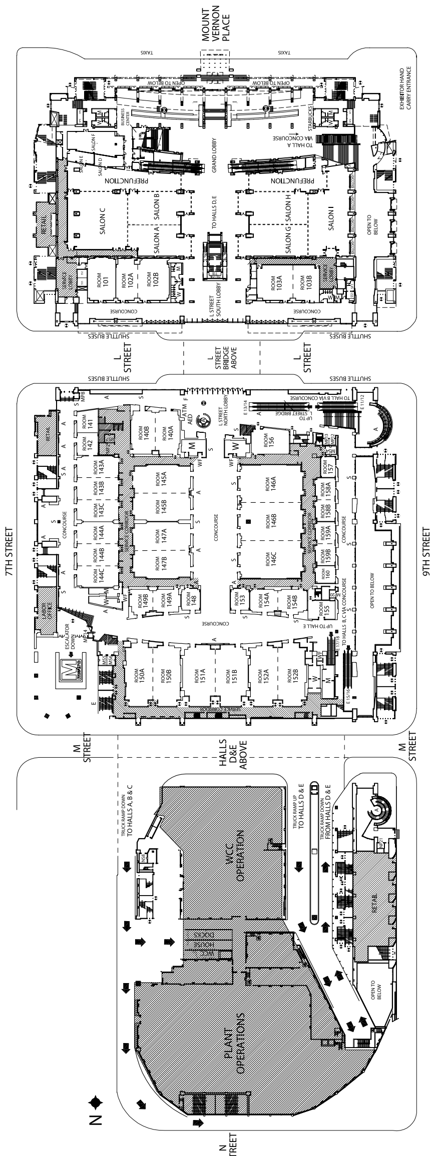
East Salon

Sunday, May 15 | 7:00 AM–7:00 PM
Monday, May 16 | 7:00 AM–6:00 PM
Tuesday, May 17 | 7:00 AM–6:00 PM
Wednesday, May 18 | 7:00 AM–6:00 PM
Thursday, May 19 | 7:00 AM–12:00 PM

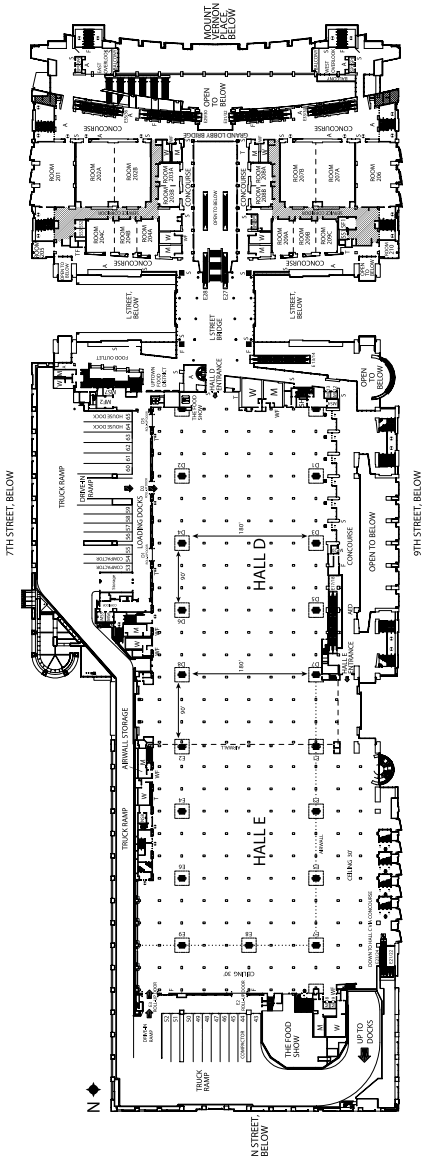


WALTER E. WASHINGTON CONVENTION CENTER

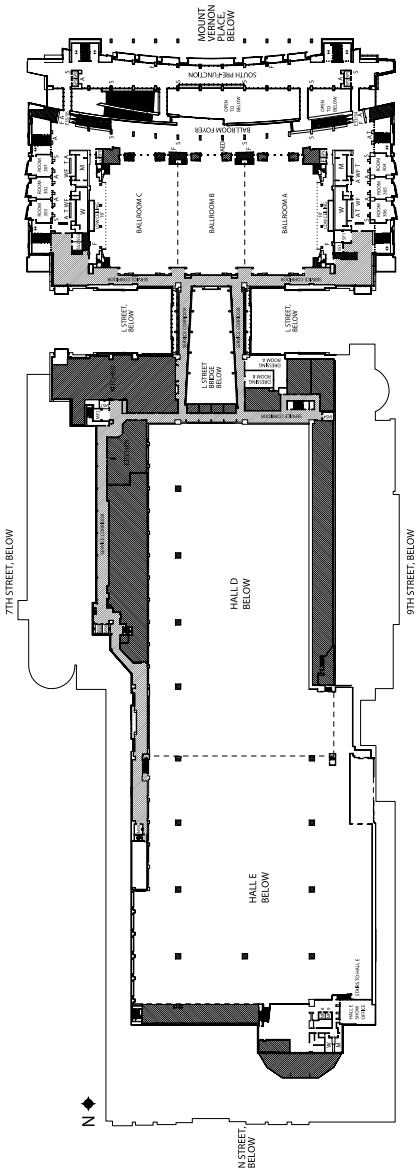
Street Level



Level Two


MOUNT VERNON ROOM BELOW
OPEN TO BELOW
CONCOURSE
GRAND LOBBY BRIDGE
ROOM 201
ROOM 200A
ROOM 200B
ROOM 200C
ROOM 200D
ROOM 200E
ROOM 200F
ROOM 200G
ROOM 201A
ROOM 201B
ROOM 201C
ROOM 201D
ROOM 201E
ROOM 201F
ROOM 201G
L STREET, BELOW
L STREET BRIDGE
L STREET ENTRANCE
OPEN TO BELOW
7TH STREET, BELOW
9TH STREET, BELOW
TRUCK RAMP
DRIVE-UP RAMP
LOADING DOCKS
HALL D
HALL D CONCOURSE
OPEN TO BELOW
HALL E ENTRANCE
AIRWALL STORAGE
HALLE
CEILING 30'
CEILING 39'
THE FOOD SHOW
UP TO DOCKS
N
N STREET, BELOW

Level Three


MOUNT VERNON PLACE BELOW
SOUTH PRE-FUNCTION
BALLROOM FOYER
BALLROOM C
BALLROOM B
BALLROOM A
L STREET BELOW
L STREET BRIDGE BELOW
7TH STREET, BELOW
9TH STREET, BELOW
HALL D BELOW
HALL E BELOW
N
N STREET BELOW

PROGRAM COMMITTEE + ABSTRACT PLANNING COMMITTEE

2022 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 25th Annual Meeting.

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

MEMBERS
Jeffrey Chamberlain, PhD, *University of Washington*
Helen Heslop, PhD, *Baylor College of Medicine*
Hans-Peter Kiem, MD, PhD, *Fred Hutchinson Cancer Research Center*
Maritza McIntyre, PhD, *Advanced Therapies Partners*
Carol Miao, PhD, *Seattle Children's Research Institute*
Stephen J. Russell, MD, PhD, *Mayo Clinic*

2022 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
Beverly L. Davidson, PhD, *Children's Hospital of Philadelphia*

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

ASGCT SECRETARY + ABSTRACT CHAIR
Terence R. Flotte, MD, *University of Massachusetts Medical School*

MEMBERS
Jeffrey Chamberlain, PhD, *University of Washington*
Helen Heslop, PhD, *Baylor College of Medicine*

THANK YOU

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CAPSIDA BIOTHERAPEUTICS
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cytiva
DYNO THERAPEUTICS
FORGE BIOLOGICS
L7 INFORMATICS
labcorp
MaxCyte
PackGene
TAYSHA GENE THERAPIES
TERUMO BLOOD AND CELL TECHNOLOGIES
Miltenyi Biotec
UMass Chan MEDICAL SCHOOL
uniQure
voyager THERAPEUTICS

AWARD RECIPIENTS

Outstanding Achievement Award

Donald B. Kohn, MD
Distinguished Professor, Department of Microbiology, Immunology & Molecular Genetics, UCLA

Outstanding New Investigator Award

Lindsey George, MD
Director, Clinical In Vivo Gene Therapy, Children's Hospital of Philadelphia

Matthew Hirsch, PhD
Associate Professor, Department of Ophthalmology, University of North Carolina

Morgan Maeder, PhD
Director, Payload Sciences, Chroma Medicine

Christopher Peterson, PhD
Staff Scientist, Clinical Research Division, Fred Hutchinson Cancer Research Center

The Outstanding New Investigator Award is supported by:

Spark THERAPEUTICS

Jerry Mendell Award for Translational Science

Katherine High, MD
President of Therapeutics, AskBio

Sonia Skarlatos Public Service Award

Philip J. Brooks, PhD
Acting Director Office of Rare Diseases Research, National Center for Advancing Translational Sciences, National Institutes of Health

Founders Award

Francis Collins, MD, PhD
Former Director, NIH

Excellence in Research Awards

Excellence in Research Award winners will be announced during the George Stamatoyannopolous lecture on Tuesday, May 17th.

The Excellence in Research Awards are supported by:

EXHIBIT HALL DIRECTORY (as of 4/1/22)

The Exhibit Hall is located in Hall D, and will be open daily 9:45 AM to 6:30 PM (ET) Monday, May 16 through Wednesday, May 18. Please view interactive floor plan **here.**

EXHIBITOR	BOOTH NUMBER
10x Genomics	219
908 Devices	541
ABL Inc.	341
Agilent Technologies	518
Akron Biotech	635
Aldevron	309
Alfa Wassermann Separation Technologies	337
American Society for Transplantation and Cellular Therapy	128
AmplifyBio	246
Andelyn Biosciences	148
Anemocyte Srl	412
ArcticZymes Technologies	241
Avid Bioservices, Inc.	542
Avirmax, Inc.	619
Axion BioSystems	613
Azzur Group	520
B Medical Systems	661
Baker	317
Batavia Biosciences	643
Beckman Coulter Life Sciences	170
BioCentriq	144
Biocut Systems	261
Biognosys	233
BioIVT	158
Biomere	571
Bio-Rad Laboratories	245
BioSharingNetwork	215
Bio-Techne	452
Bristol Myers Squibb	543
Catalent Cell & Gene Therapy	234
Cellipont Bioservices	526
CellVec Pte Ltd	562
Celonic AG	235
Center for Breakthrough Medicines	204
CEVEC Pharmaceuticals	363
Charles River	369
Children's Hospital of Philadelphia	565
Clean Cells	164
ClearPoint Neuro, Inc.	373

EXHIBITOR	BOOTH NUMBER
Comecer	244
Cook MyoSite	277
Corning, Inc.	536
CPC	210
Curiox Biosystems	564
Cystic Fibrosis Foundation	665
Cytiva	609
Cytotheryx	266
Cytovance Biologics	426
Dyne Therapeutics	549
Eurofins BioPharma Product Testing	462
Eurofins Viracor BioPharma	466
Evozyne	243
Exemplar Genetics	430
Flash Therapeutics	252
Forecyte Bio USA Limited	365
Forge Biologics	214
FuGENE Transfection (Fugent LLC)	559
FUJIFILM Diosynth Biotechnologies	420
FUJIFILM WAKO CHEMICALS U.S.A. CORPORATION	544
Gator Bio	238
G-CON Manufacturing	276
Gene Tools, LLC	510
Genezen Laboratories, Inc.	464
GenIbet	514
GenoSafe	136
GENSCRIPT USA INC.	280
GTS Scientific	126
Gyros Protein Technologies	557
Halo Labs	432
Informa Pharma Intelligence	114
Innoforce US	118
Integrated DNA Technologies (IDT)	166
InVitria	442
Iota Sciences	450
KromaTiD	645
L7 Informatics	272
Labcorp Drug Development (formerly Covance)	480

EXHIBITOR	BOOTH NUMBER
Leroy Biotech	359
Lonza	570
Lovelace Biomedical	558
LUMICKS	217
Malvern Panalytical	533
Matica Biotechnology	142
MaxCyte	257
MaxQ Research	561
MilliporeSigma	351
Miltenyi Biotec	263
Mirus Bio	527
MISSION BIO	548
Molecular Devices, LLC	275
MYRIADE	339
NanoView Biosciences Ltd	529
National Organization for Rare Disorders (NORD)	116
Ncardia	226
NECI	274
NHLBI Gene Therapy Resource Program (GTRP)	560
NOF CORPORATION	221
Northern Biomedical Research	476
Novartis AG	122
NxGEN Vector Solutions, LLC	260
OBiO Technology	258
Olympus	270
Open Therapeutics	205
OrganaBio	106
OriGen Biomedical	637
Oxford Biomedica (UK) Limited	240
PackGene Biotech INC	627
Pall Corporation	504
Penn Vector Core	551
PeproTech, Inc., part of Thermo Fisher Scientific	375
PerkinElmer	220
Pfizer	269
Pharmaron	631
PhenoVista Biosciences	617
Polyplus-transfection	569
Powered Research, LLC	134
PPD, part of Thermo Fisher Scientific	349
Precision For Medicine	566
Precision NanoSystems Inc.	641
PROGEN	458
Promega Corporation	547

EXHIBITOR	BOOTH NUMBER
ProtaGene	140
Proteintech Group, Inc.	532
PTC Therapeutics, Inc.	512
Puresyn, Inc.	563
QIAGEN	516
Refeyn	357
Renovate Biosciences	207
Repligen	203
Resilience	160
Rocket Pharmaceuticals	138
Sabai Global, and subsidiaries of Clinical Biosafety Services, Shield Consulting and Castle IRB	227
Sarepta Therapeutics	428
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SIRION Biotech	218
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Taysha Gene Therapies	472
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Versiti	108
Vertex Pharmaceuticals	313
VGXI, Inc.	319
Vinta Bio, Inc.	528
Viralgen Vector Core	648
Virica Biotech	112
Virovek Incorporation	546
VIVEbiotech	264
Wacker Biotech	254
Waisman Biomanufacturing	104
WuXi Advanced Therapies	436
Wyatt Technology	553
Yecuris	176
Yposkesi	448
Zymo Research Corp.	250

SCHEDULE AT A GLANCE (ALL TIMES LISTED IN ET)

MONDAY, MAY 16, 2022

7:00 AM–6:00 PM

REGISTRATION

EAST SALON

9:45 AM–6:30 PM

EXHIBIT HALL

HALL D

Connect with Exhibitors

Coffee Social | 9:45–10:15 AM

Coffee Social | 3:15–3:45 PM

Networking Reception & Poster Session I | 5:30–6:30 PM

8:00–9:45 AM

EDUCATION SESSIONS

BALLROOM B

- **Off-the-Shelf Cell Therapies—Beyond T-Cells**
  Co-chair: Blythe Sather, PhD Ballroom B

BALLROOM C

- **AAV Vectors—From Basic Biology to Regulatory Hurdles**
  Chair: Erik Barton, PhD

8:00–9:45 AM

SCIENTIFIC SYMPOSIA

ROOM 206

- **Accelerated Approval for Gene Therapies**
  Co-chairs: Jeremy Allen and Jennifer Wellman

ROOM 207

- **Function and Therapeutics Applications of Circular RNAs (circRNAs)**
  Co-chairs: Mark Kay, PhD and Paloma Giangrande, PhD

ROOM 204

- **Intersection Between Genetic Therapy and Society—Nothing About Us Without Us**
  Chair: Maritza McIntyre, PhD

SALON G

- **Translating Science Into Medicine: Moving From Bench to Startup (Session 1 of 2)**
  Co-chairs: H. Trent Spencer, PhD and Nicole Paulk, PhD

SCHEDULE AT A GLANCE (ALL TIMES LISTED IN ET)

MONDAY, MAY 16, 2022

8:00–9:45 AM

SCIENTIFIC SYMPOSIA

ROOM 102

- **Preparing for Research Partnerships With Patient Advocates**
  Co-chairs: Rachel Bailey, PhD and Jennifer Helfer, PhD

ROOM 201

- **Stem Cells in Tissue Repair and Regeneration: Insights From Model Systems**
  Co-chairs: TK and Sangeetha Vadakke-Madathil, PhD

BALLROOM A

- **The Ultimate Personalized Gene and Cell Therapy for Treatment of Cancer**
  Co-chairs: Daniela Bischof, PhD and Sunil Raikar, MD

SCHEDULE AT A GLANCE (ALL TIMES LISTED IN ET)

MONDAY, MAY 16, 2022

9:00–11:30 AM

EXHIBITOR SHOWCASES

ROOM 209 | INDUSTRY INTERACTIONS

GenScript USA Inc. | 9:00–9:30 AM

10x Genomics | 10:00–10:30 AM

InVitria | 11:00–11:30 AM

10:15 AM–12:00 PM

ORAL ABSTRACT SESSIONS

ROOM 204

- **AAV Gene Therapy in Large Animal Models**
  Co-chairs: Heather Gray-Edwards, DVM, PhD and Juliette Hordeaux, DVM, PhD

ROOM 201

- **Biology of Gene Edited Cells**
  Co-chairs: Matthew Porteus, MD, PhD and Paula Rio, PhD

SALON H

- **Oligonucleotide Therapeutics**
  Co-chairs: Shen Shen, PhD and Michelle Hastings, PhD

ROOM 206

- **Cardiovascular and Pulmonary Diseases**
  Co-chairs: Margaret Sleeper, VMD and Uta Griesenbach, PhD

SALON G

- **Musculo-skeletal Diseases**
  Co-chairs: Perry Shieh, MD, PhD and Lindsay Wallace, PhD

ROOM 102

- **Immune Responses to AAV Vectors**
  Co-chairs: Julie Crudele, PhD and Ying Kai Chan, PhD

BALLROOM A

- **Vector Manufacturing and Engineering I: Deciphering AAV Vector Genomes**
  Co-chairs: H. Trent Spencer, PhD and John Gray, PhD

12:00–1:30 PM

INDUSTRY SPONSORED SYMPOSIA

Labcorp Drug Development (formerly Covance) | Room 202

MaxCyte | Ballroom B

Sarepta Therapeutics | Ballroom C

Terumo Blood and Cell Technologies | Room 207

SCHEDULE AT A GLANCE (ALL TIMES LISTED IN ET)

MONDAY, MAY 16, 2022

12:00–1:00 PM

STARTUP SHOWCASES

ROOM 209 | INDUSTRY INTERACTIONS

Chair: Li Ou, PhD

1:30–3:15 PM

SCIENTIFIC SYMPOSIA

SALON H

- **Competing With Best Available Care: Perspectives on Lowering Burden of Treatment With Cell and Gene Therapies**
  Co-chairs: Nimi Chhina, PhD and Kristin Van Goor, PhD

ROOM 102

- **Cutting-edge Gene and Cell Therapy Research in Japan**
  Co-chairs: Noriyuki Kasahara, MD, PhD and Takafumi Nakamura, PhD

BALLROOM A

- **Immune Responses to Gene Therapy**
  Co-chairs: Allison Keeler, PhD and Melissa Rhodes, PhD

SALON G

- **Translating Science Into Medicine: Moving From Bench to Startup (Session 1 of 2)**
  Co-chairs: H. Trent Spencer, PhD and Madhusudan Peshwa, PhD

ROOM 206

- **Career Development Award Presentations**

1:30–3:00 PM

EXHIBITOR SHOWCASES

ROOM 209 | INDUSTRY INTERACTIONS

PerkinElmer | 1:30–2:00 PM

PTC Therapeutics, Inc. | 2:30–3:00 PM

SCHEDULE AT A GLANCE (ALL TIMES LISTED IN ET)

MONDAY, MAY 16, 2022

1:30–3:15 PM

ORAL ABSTRACT SESSIONS

ROOM 201

- **Gene and Cell Therapy Trials in Progress**
  Co-Chairs: Kevin Flanigan, MD and Barry Byrne, MD, PhD

3:30–6:00 PM

TOOLS AND TECHNOLOGY FORUM I

ROOM 209 | INDUSTRY INTERACTIONS

Co-chairs: Afrooz Rashnonejad, PhD and Li Ou, PhD

SCHEDULE AT A GLANCE (ALL TIMES LISTED IN ET)

MONDAY, MAY 16, 2022

3:45–5:30 PM — ORAL ABSTRACT SESSIONS

ROOM 204
- **AAV Preclinical CNS Gene Therapy**
  Co-chairs: Allison Bradbury, PhD and Brian Bigger, PhD

BALLROOM A
- **Gene Editing in Blood and Immune Disorders**
  Co-chairs: Maria-Grazia Roncarolo, MD and Jose Segovia, PhD

SALON H
- **Synthetic Nanoparticle-Based Gene Transfer**
  Co-chairs: Hai-Quan Mao, PhD and Carol Miao, PhD

SALON G
- **Tools and Approaches for Inborn Errors of Metabolism**
  Co-chairs: Gloria Gonzalez-Aseguinolaza, PhD and Charles Venditti, MD, PhD

ROOM 102
- **CAR T-cells and Beyond**
  Co-chairs: Chiara Bonini, MD and Avery Posey, PhD

ROOM 206
- **Cancer - Oncolytic Viruses**
  Co-chairs: Paola Grandi, PhD, MBA and Marta Alonso, PhD

ROOM 207
- **Cell-Based Cancer Immunotherapies I**
  Co-chairs: Barbra Sasu, PhD and Daniel Abate-Daga, PhD

ROOM 202
- **Hematopoietic Stem Cell Gene Therapy**
  Co-chairs: Bernhard Gentner, MD and Anne Galy, PhD

ROOM 201
- **Vector Manufacturing and Engineering II: Next Generation Methods**
  Co-chairs: Kerry Dooriss, PhD and Johannes Van Der Loo, PhD

5:30–7:00 PM — INDUSTRY SPONSORED SYMPOSIA

Pall Corporation | Ballroom B

Thermo Fisher Scientific - Patheon | Ballroom C

5:30–6:30 PM — NETWORKING RECEPTION & POSTER SESSION I

EXHIBIT HALL | HALL D

SCHEDULE AT A GLANCE (ALL TIMES LISTED IN ET)

TUESDAY, MAY 17, 2022

7:00 AM–6:00 PM

REGISTRATION

EAST SALON

9:45 AM–6:30 PM

EXHIBIT HALL

HALL D

Connect with Exhibitors

Coffee Social | 9:45–10:15 AM

Coffee Social | 3:15–3:45 PM

Networking Reception & Poster Session II | 5:30–6:30 PM

8:00–9:45 AM

EDUCATION SESSIONS

ROOM 202

- **Cancer Gene Therapy**
  Chair: Christopher LaRocca MD

BALLROOM C

- **CRISPR/Cas9 Gene Editing—Concepts to *In Vivo* Editing**
  Chair: Nuria Morral, PhD

ROOM 207

- **Integrating Retroviral Vectors**
  Chair: John Tisdale, MD

8:00–9:45 AM

SCIENTIFIC SYMPOSIA

ROOM 102

- **Gene- and Cell-based Therapies for Lung and GI**
  Chair: Alisha Gruntman, DVM, PhD

BALLROOM B

- **Intended and Unintended Roles of Viral Vector Heterogeneity in Gene Therapy**
  Co-chairs: Chen Ling, PhD and Brian Bigger, PhD

SALON G

- **Musculoskeletal Gene Therapy: Progresses and Challenges**
  Co-chairs: Scott Q. Harper, PhD and Chunping Qiao, PhD

BALLROOM A

- **Fireside Chat—State of the Field: Emerging Regulatory Trends**
  Chair: Keith Wonnacott, PhD

SCHEDULE AT A GLANCE (ALL TIMES LISTED IN ET)

TUESDAY, MAY 17, 2022

8:00–9:45 AM

SCIENTIFIC SYMPOSIA

ROOM 204

- **Symposium on Translating Tissue Engineering and Regenerative Medicine Approaches Into Therapies**
  Co-chairs: Steven Becker, PhD and Lloyd Rose, PhD

ROOM 206

- **The Ethical Gray Zone? Perspectives on the Development and Governance of Gene and Cell Therapies for Human Enhancement**
  Co-chairs: Eric Juengst, PhD and Benjamin Hurlbut, PhD

ROOM 201

- **Therapeutic Applications of RNA Therapy Strategies**
  Co-chairs: Lauren E. Woodard, PhD and Loree Heller, PhD

8:00–9:45 AM

LATE BREAKING ORAL ABSTRACT SESSIONS

SALON H

8:15–9:45 AM

EXHIBITOR SHOWCASES

ROOM 209 | INDUSTRY INTERACTIONS

Bio-Rad Laboratories | 8:15–8:45 AM

Catalent Cell & Gene Therapy | 9:15–9:45 AM

10:15 AM–12:00 PM

SPONSORED BY: REGENXBIO

GEORGE STAMATOYANNOPOULOS MEMORIAL LECTURE AND AWARDS PRESENTATION

HALL E

12:00–1:30 PM

INDUSTRY SPONSORED SYMPOSIA

Corning Life Sciences | Ballroom C

Cytiva | Ballroom B

Taysha Gene Therapies | Room 207

Vertex Pharmaceuticals Inc. | Room 202

SCHEDULE AT A GLANCE (ALL TIMES LISTED IN ET)

TUESDAY, MAY 17, 2022

12:00–1:30 PM

EXHIBITOR SHOWCASES

ROOM 209 | INDUSTRY INTERACTIONS

Bio-Techne | 12:00–12:30 PM

Precision for Medicine | 1:00-1:30 PM

1:30–3:00 PM

SPONSORED BY:

OUTSTANDING NEW INVESTIGATOR SYMPOSIUM

HALL E

3:00–5:00 PM

NETWORKING EVENT—CAREER FAIR

SOUTH PRE-FUNCTION

Connect with employers and get a complimentary professional headshot! Make sure you are **registered for the Annual Meeting** to attend the Career Fair.

3:30–6:00 PM

TOOLS AND TECHNOLOGY FORUM II

ROOM 209 | INDUSTRY INTERACTIONS

Co-chairs: Blythe Sather, PhD and Le Cong, PhD

SCHEDULE AT A GLANCE (ALL TIMES LISTED IN ET)

TUESDAY, MAY 17, 2022

3:45–5:30 PM

ORAL ABSTRACT SESSIONS

BALLROOM A

- **Discoveries in Fundamental AAV Biology**
  Co-chairs: Nicole Paulk, PhD and Anna Maurer, PhD

SALON G

- **Enhanced AAV Targeting**
  Chair: Ana Rita Batista, PhD

HALL E

- **Gene Editing in Cancer and Complex Diseases**
  Co-chairs: Angelo Lombardo, PhD and Mara Pavel-Dinu, PhD

SALON H

- **Physical Methods and Extracellular Vesicle-Based Gene Transfer**
  Chair: Richard Heller, PhD

ROOM 201

- **Inborn Errors of Metabolism Gene and Cell Therapies: Proof-of-Concepts and Beyond**
  Co-chairs: Gerald Lipshutz, MD and Stephanie Cherqui, PhD

ROOM 204

- **Applications of Improved Gene Therapy Methods in Neurologic Disorders**
  Co-chairs: Kathrin Meyer, PhD and Steven Gray, PhD

ROOM 102

- **Enhancing CAR-T Cell Efficacy**
  Co-chairs: Craig Sauter, MD and Barbara Savoldo, MD, PhD

ROOM 202

- **Gene Therapy for Immunologic Diseases**
  Co-chairs: Donald Kohn, MD and Claire Booth, MBBS, PhD

ROOM 206

- **Immune Responses to Gene Delivery and Vaccine Approaches**
  Co-chairs: Matthew Gardner, PhD and Manish Muhuri, PhD

5:30–7:00 PM

INDUSTRY SPONSORED SYMPOSIA

Anemocyte | Ballroom C

Charles River Laboratories | Room 207

QIAGEN LLC | Ballroom B

SCHEDULE AT A GLANCE (ALL TIMES LISTED IN ET)

TUESDAY, MAY 17, 2022

5:30–6:30 PM

NETWORKING RECEPTION & POSTER SESSION II

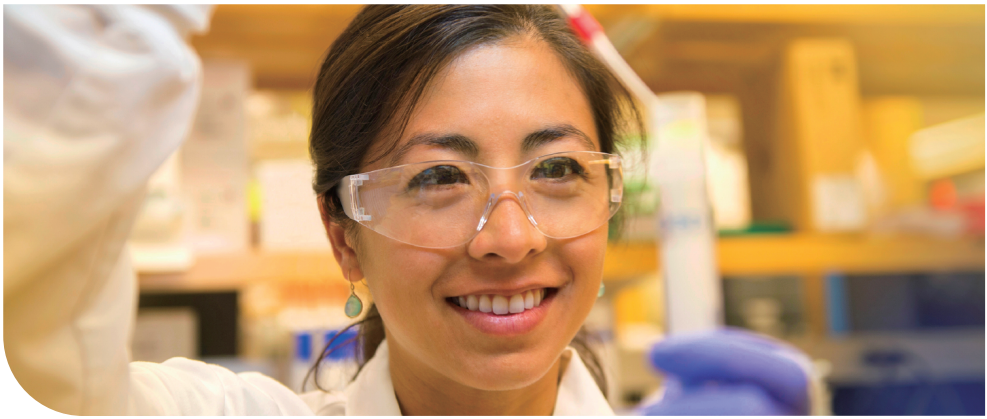
EXHIBIT HALL | HALL D

6:00–8:00 PM

NETWORKING EVENT—NEW MEMBER MEETUP

ROOM 103

The New Member Mixer welcomes attendees who have joined ASGCT over the past three years. Usually reserved for members joining in the past 12 months, we've expanded the mixer to include those who have joined since we were last in-person in 2019. This informal setting will introduce new members to the Society and highlight the many ways they can use our resources as their professional hub. Attendees will also hear from a longtime member who has adeptly used their Society membership throughout their career.



SCHEDULE AT A GLANCE (ALL TIMES LISTED IN ET)

WEDNESDAY, MAY 18, 2022

7:00 AM–6:00 PM

REGISTRATION

EAST SALON

9:45 AM–6:30 PM

EXHIBIT HALL

HALL D

Connect with Exhibitors

Coffee Social | 9:45–10:15 AM

Coffee Social | 3:15–3:45 PM

Networking Reception & Poster Session III | 5:30–6:30 PM

8:00–9:45 AM

EDUCATION SESSIONS

BALLROOM B

- **Gene Therapy Beyond Cancer**
  Chair: Satiro De Oliveira, MD

ROOM 204

- **Pre-clinical Models**
  Co-chairs: Erik Barton, PhD and Hildegard Buning, PhD

8:00–9:45 AM

SCIENTIFIC SYMPOSIA

ROOM 206

- **Combating Misinformation in Science**
  Co-chairs: Karen Bulaklak, PhD, Sarepta Therapeutics and Edith Pfister, PhD, UMASS Chan Medical School

ROOM 102

- **Challenges and Status in Development of Gene Therapies in Low- and Middle-income Countries**
  Co-chairs: Richard Koya, MD, PhD and Savita Rangarajan, MD

BALLROOM A

- **Gene Editing: New Technology Advances**
  Co-chairs: Thomas J. Cradick, PhD and Paula Rio, PhD

SALON G

- **Inborn Metabolic Issues**
  Co-chairs: Anne Galy, PhD and Alessandro Aiuti, MD, PhD

ROOM 207

- **Non-viral Delivery: A Diverse Toolbox Comes of Age**
  Co-chairs: Stefan Radtke, PhD and Chantal Pichon, PhD

SCHEDULE AT A GLANCE (ALL TIMES LISTED IN ET)

WEDNESDAY, MAY 18, 2022

8:00–9:45 AM

SCIENTIFIC SYMPOSIA

ROOM 201

- **Overview of Cardiac Gene Therapy**
  Co-chairs: Jeffrey L. Ellsworth, PhD and Ryan L. Boudreau, PhD

ROOM 202

- **Vector-associated Neural and Ocular Inflammation**
  Co-chairs: Christine N. Kay, MD and Paul A. Sieving, MD

8:00–9:45 AM

LATE BREAKING ORAL ABSTRACT SESSIONS

BALLROOM C

8:15–9:45 AM

EXHIBITOR SHOWCASES

ROOM 209 | INDUSTRY INTERACTIONS

Informa Pharma Intelligence | 8:15–8:45 AM

Curiox Biosystems | 9:15–9:45 AM

10:15–11:15 AM

PLENARY SESSION

HALL E

The Outstanding Achievement Award Symposium

Chair: Beverly L. Davidson, PhD

11:15 AM–12:00 PM

PLENARY SESSION

HALL E

Founder's Award Presentation

Chair: Beverly L. Davidson, PhD

12:00–1:30 PM

INDUSTRY SPONSORED SYMPOSIA

Dyno Therapeutics | Room 202

Miltenyi Biotec | Room 207

Thermo Fisher Scientific | Ballroom B

12:00–12:30 PM

EXHIBITOR SHOWCASES

ROOM 209 | INDUSTRY INTERACTIONS

L7 Informatics | 12:00–12:30 PM

SCHEDULE AT A GLANCE (ALL TIMES LISTED IN ET)

WEDNESDAY, MAY 18, 2022

1:30–3:15 PM

PLENARY SESSION

SPONSORED BY:

HALL E

Presidential Symposium and Presentation of Top Abstracts

Chair: Beverly L. Davidson, PhD

3:30–6:00 PM

TOOLS AND TECHNOLOGY FORUM III

ROOM 209 | INDUSTRY INTERACTIONS

Co-chairs: Kay Whye Peng, PhD and Rachel Bailey, PhD

SCHEDULE AT A GLANCE (ALL TIMES LISTED IN ET)

WEDNESDAY, MAY 18, 2022

3:45–5:30 PM — ORAL ABSTRACT SESSIONS

SALON H
- **RNA Virus Vectors**
  Co-chairs: John Tisdale, MD and Mario Amendola, PhD

BALLROOM A
- **AAV Developments in Liver, T-cells, and Toxicity**
  Co-chairs: Nicole Paulk, PhD and Amanda Dudek, PhD

HALL E
- **New Gene Editing Technologies and Applications**
  Co-chairs: Luca Biasco, PhD and Paula Cannon, PhD

ROOM 202
- **Novel Therapeutic Targets to treat CNS Disorders**
  Co-chairs: Miguel Sena-Esteves, PhD and Rebecca Ahrens-Nicklas, MD, PhD

ROOM 102
- **Harnessing Innate Immunity for Cancer Immunotherapy**
  Co-chairs: Gianpietro Dotti, MD and Sarwish Rafiq, PhD

SALON G
- **Cell-Based Cancer Immunotherapies II**
  Co-chairs: Masato Yamamoto, MD, PhD and Irina Balyasnikova, PhD

ROOM 206
- **Engineered Cell Therapies**
  Co-chairs: Bakhos Tannous, PhD and M Graca Almeida-Porada, MD, PhD

ROOM 201
- **Vector Manufacturing and Engineering 3: Improving Vector Design and System Performance**
  Chair: Christopher Doering, PhD

ROOM 204
- **Pharmacology/Toxicology Studies or Assay Development I**
  Co-chairs: Heikki Turunen, PhD and Eva Andres-Mateos, MD, PhD

5:30–6:30 PM — NETWORKING RECEPTION & POSTER SESSION III

EXHIBIT HALL | HALL D

SCHEDULE AT A GLANCE (ALL TIMES LISTED IN ET)

WEDNESDAY, MAY 18, 2022

8:00–11:00 PM

NETWORKING EVENT—CLOSING NIGHT RECEPTION

INTERNATIONAL SPY MUSEUM

Join us for a night of fun during the Closing Night Reception at Washington, D.C.'s **International Spy Museum!** Enjoy the museum exhibits, light appetizers, and drinks.

You can purchase a ticket ($50 per person) for the reception before or after you **register for the meeting.** Space is limited!

SCHEDULE AT A GLANCE (ALL TIMES LISTED IN ET)

THURSDAY, MAY 19, 2022

7:00 AM–12:00 PM

REGISTRATION

EAST SALON

8:00–9:45 AM

SCIENTIFIC SYMPOSIA

ROOM 201

- **Effective Regulatory Interactions**
  Co-chairs: Megan Zoschg-Canniere, PhD and Kit Shaw, PhD

BALLROOM B

- **Emerging Clinical and Translational Safety Topics in Cell and Gene Therapy**
  Co-chairs: Megan Hoban, PhD and TK

BALLROOM A

- **Finding a "Cure": The Promise and Perils of Gene Therapy, *In Utero* and Beyond**
  Co-chairs: Randy J. Chandler, PhD and Stephanie Cherqui, PhD

ROOM 207

- **Global Experience With COVID-19 Vaccines**
  Co-chairs: Shan Lu, MD, PhD and David B. Weiner, PhD

SALON G

- **Panel Discussion: What are the Most Pressing Issues of the Future?**
  Co-chairs: TK and Rayne Rouce, MD

ROOM 204

- **Cutting-edge Gene and Cell Therapy Research, in Memory of Dr. Manfred Schmidt**
  Co-chairs: Juan A. Bueren, PhD and Hildegard Büning, PhD

8:00–9:45 AM

ORAL ABSTRACT SESSIONS

BALLROOM C

- **Clinical Trials Spotlight Symposium**
  Chair: Maria Grazia Roncarolo, MD

SCHEDULE AT A GLANCE (ALL TIMES LISTED IN ET)

THURSDAY, MAY 19, 2022

10:15 AM–12:00 PM ORAL ABSTRACT SESSIONS

BALLROOM A

- **Novel AAV Capsids for the Brain, Eye and Kidney**
  Co-chairs: Nicole Paulk, PhD and Andrew Steinsapir

BALLROOM C

- **New Technologies for AAV Gene Therapy**
  Co-chairs: Phillip Tai, PhD and Aravind Asokan, PhD

BALLROOM B

- **AAV Vectors - Clinical Studies**
  Co-chairs: Kathryn Wagner, MD, PhD and Christian Mueller, PhD

ROOM 202

- **Delivery Technologies and *In Vivo* Gene Editing**
  Co-chairs: TJ Cradick, PhD and Daniela Cesana, PhD

ROOM 201

- **Breakthroughs in Neuromuscular and Hearing Disorders**
  Co-chairs: Scott Q. Harper, PhD and Rachel M. Bailey, PhD

SALON G

- **Ophthalmic and Auditory Diseases**
  Co-chairs: Hemant Khanna, PhD and Mariacarmela Allocca

ROOM 207

- **Cell-Based Cancer Immunotherapies III**
  Co-chairs: Saad Kenderian and Michael Milone, MD, PhD

ROOM 204

- **Cell Therapies for Hematological Disorders**
  Co-chairs: Andre Larochelle, MD, PhD and Punam Malik, MD, PhD

ROOM 206

- **Cell Therapy Product Engineering, Development or Manufacturing**
  Co-chairs: Isabelle Riviere, PhD and Maksim Mamonkin, PhD

ROOM 102

- **Pharmacology/Toxicology Studies or Assay Development II**
  Co-chairs: Angela Lynch, PhD and Eva Andres-Mateos, PhD

SCHEDULE

ALL TIMES LISTED IN EDT

SCHEDULE (ALL TIMES LISTED IN ET)

MONDAY, MAY 16, 2022

7:00 AM–6:00 PM	REGISTRATION HOURS—EAST SALON

9:45 AM–6:30 PM	EXHIBIT HALL OPEN—HALL D
9:45–10:15 AM	Exhibit Hall Coffee Social
3:15–3:45 PM	Exhibit Hall Coffee Social
5:30–6:30 PM	Exhibit Hall Networking Reception & Poster Session I

EDUCATION SESSIONS

BALLROOM B
Off the Shelf Cell Therapies—Beyond T-cells
Chair: Blythe Sather, PhD, Tune Therapeutics

8:00–8:24 AM	**Programming T-cell Therapies with CRISPR** *Alexander Marson, MD, PhD, UCSF*
8:24–8:48 AM	**The Next Generation of γδT Cell-based Therapies** *Lawrence S. Lamb, Jr, PhD, IN8Bio*
8:48–9:12 AM	**Epigenetic Programming to Enhance Cell Therapy** *Charles A. Gersbach, PhD, Duke University*
9:12–9:36 AM	**Progress in Pluripotent Cell Therapy Manufacturing: A Personal 22-year Journey** *Steve Oh, PhD, Bioprocessing Technology Institute, A*STAR*

SCHEDULE (ALL TIMES LISTED IN ET)

MONDAY, MAY 16, 2022

EDUCATION SESSION

BALLROOM C
AAV Vectors—From Basic Biology to Regulatory Hurdles
Chair: Erik S. Barton, PhD, Pfizer, Inc.

8:00–8:20 AM	**How Comparative Studies of Mammalian- and Insect-cell-derived AAV Can Enable Design of Vector Manufacturing Platforms** *Nicole Paulk, PhD, UCSF*
8:20–8:40 AM	**Understanding and Controlling Mechanisms of AAV Immune-response-associated Toxicities** *J. Fraser Wright, PhD, Stanford University School of Medicine*
8:40–9:05 AM	**Harnessing AAV Membrane-associated Accessory Protein to Improve Vector Supply and Quality** *Kari J. Airenne, PhD, Kuopio Center for Gene and Cell Therapy (KCT)*
9:05–9:25 AM	**Understanding and Mitigating the Potential for AAV-associated Oncogenesis** *Denise Sabatino, PhD, Children's Hospital of Philadelphia*
9:25–9:45 AM	**Bridging AAV Biology to Platform and Process Innovation** *Aravind Asokan, PhD, Duke University*

SCHEDULE (ALL TIMES LISTED IN ET)

MONDAY, MAY 16, 2022

SCIENTIFIC SYMPOSIA

ROOM 206
Accelerated Approval for Gene Therapies
Co-chairs: Jeremy Allen, Spark Therapeutics and Jennifer Wellman, Akouos

8:00–8:15 AM	**Overview of the Accelerated Approval Pathway: Definitions, Legislative History, and Purpose** *Anna K. Abram, Akin Gump Strauss Hauer & Feld LLP*
8:15–8:30 AM	**Patient Perspective: How Patients Weigh Benefit/Risk of Treatment, and How to Weigh Unmet Need** *Teonna Woolford, Sickle Cell Reproductive Health Education Directive*
8:30–8:45 AM	**Current Developments in Policy: Pathway Development Consortium** *Nina Hunter, PhD, REGENXBIO; Pathway Development Consortium*
8:45–9:00 AM	**Reimbursing Products After Accelerated Approval: A Payer Perspective**
9:00–9:15 AM	**The Significance of Accelerated Approval to Rare Disease Drug Development** *Emil Kakkis, MD, PhD, Ultragenyx*
9:15–9:45 AM	**Panel Discussion**

SCHEDULE (ALL TIMES LISTED IN ET)

MONDAY, MAY 16, 2022

SCIENTIFIC SYMPOSIA

ROOM 207

Function and Therapeutics Applications of Circular RNAs (circRNAs)

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

8:00–8:35 AM	**Circular RNAs in Innate Immunity** *Ling-Ling Chen, PhD, Shanghai Institute of Biochemistry and Cell Biology*
8:35–9:10 AM	**Circular RNA Immunology** *Grace Chen, PhD, Yale University*
9:10–9:45 AM	**In-situ CAR Therapy Using oRNA Lipid Nanoparticles (LNPs) Regresses Tumors in Mice** *Tom Barnes, PhD, Orna Therapeutics*

SCHEDULE (ALL TIMES LISTED IN ET)

MONDAY, MAY 16, 2022

SCIENTIFIC SYMPOSIA

ROOM 204
Intersection Between Genetic Therapy and Society—Nothing About Us Without Us
Chair: Maritza C. McIntyre, PhD, Advanced Therapies Partners

8:00–8:24 AM	**Does Where You Live Matter?** *Michael Louella, University of Washington*
8:24–8:48 AM	**Intersection Between Genetic Therapy and Society** *Carla Elena Echeveste, All of Us*
8:48–9:12 AM	**International Efforts to Expand Diversity of Genomic Data to Improve Health Outcomes** *Julie Makani, H3A Africa*
9:12–9:36 AM	**Attracting and Retaining Diversity in Research** *Danielle Quarles, Sana Biotechnology*

SCHEDULE (ALL TIMES LISTED IN ET)

MONDAY, MAY 16, 2022

SCIENTIFIC SYMPOSIA

SALON G

Translating Science Into Medicine: Moving From Bench to Startup (Session 1 of 2)

Co-chairs: H. Trent Spencer, PhD, Emory University School of Medicine and Nicole Paulk, PhD, UCSF

8:00–8:48 AM	**Translating Science into Medicine: Moving from Bench to Startup** *Deborah Palestrant, PhD, 5AM Ventures*
8:48–9:36 AM	**How to Raise Seed/Series A Funding From Institutional Biotech VCs for a CGT Startup** *Timothy Miller, PhD, Forge Biologics*

ROOM 102

Preparing for Research Partnerships With Patient Advocates

Co-chairs: Rachel M. Bailey, PhD, UT Southwestern Medical Center and Jennifer Helfer, PhD, Encoded Therapeutics

8:00–8:24 AM	**Understanding the Patient Advocate's Role** *Florian Eichler, MD, Massachusetts General Hospital*
8:24–8:48 AM	**Moving From Preclinical to Clinical Trials—The Translational Path** *Yael Weiss, MD, PhD, Mahzi Therapeutics*
8:48–9:12 AM	**A Parent's Journey Through Drug Development: From Diagnosis to Preclinical Work to Clinical Trial** *Allyson Berent, DVM, DACVIM, Foundation for Angelman Syndrome Therapeutics; GeneTx Biotherapeutics; Angelman Syndrome Biomarker and Outcome Measure Consortium*
9:12–9:36 AM	**Lessons Learned From a Patient Advocate** *Sharon King, Taylor's Tale*

SCHEDULE (ALL TIMES LISTED IN ET)

MONDAY, MAY 16, 2022

SCIENTIFIC SYMPOSIA

ROOM 201
Stem Cells in Tissue Repair and Regeneration: Insights From Model Systems
Chair: Sangeetha Vadakke-Madathil, PhD, Icahn School of Medicine at Mount Sinai

8:00–8:35 AM	**Interspecies Chimerism and Organogenesis** *Jun Wu, PhD, UT Southwestern Medical Center*
8:35–9:10 AM	**Sheep Model of Diseases Generated by CRISPR/Cas9** *Irina Polejaeva, PhD, Utah State University*
9:10–9:45 AM	**Engraftment of CD34 + Cells Gene-edited at the Sickle Cell Disease Locus in Non-human Primate Models** *John Tisdale, MD, National Institutes of Health; National Heart, Lung, and Blood Institute*

BALLROOM A
The Ultimate Personalized Gene and Cell Therapy for Treatment of Cancer
Co-chairs: Daniela Bischof, PhD, Indiana University School of Medicine and Sunil S. Raikar, MD, Emory University

8:00–8:25 AM	**Strength/Limitation of Gene and Cell Therapy—Route of Delivery** *Richard Vile, PhD, Mayo Clinic*
8:25–8:50 AM	**Cell Therapy Approaches with Genome-edited T-cells** *Chiara Bonini, MD, Ospedale San Raffaele*
8:50–9:15 AM	**Safety of Cell Therapy - CAR T-cells New CAR Engineering** *Gianpietro Dotti, MD, UNC School of Medicine*
9:15–9:40 AM	**Personalizing Oncolytic Virotherapy and Immunovirotherapy Approaches** *Evanthia Galanis, MD, DSc, Mayo Clinic*

TAYSHA GENE THERAPIES

VISIT US AT BOOTH 472 TO LEARN MORE.

WHO WE ARE

Taysha Gene Therapies is on a mission to eradicate monogenic CNS disease. We have combined our team's proven experience in gene therapy drug development with the world-class UT Southwestern Gene Therapy Program to build an extensive, fully integrated AAV9 gene therapy pipeline with a goal of dramatically improving patients' lives.

Please join us for a lunch symposium

Accepting the Challenge:

Innovative Approaches and Translational Strategies in Gene Therapy Development

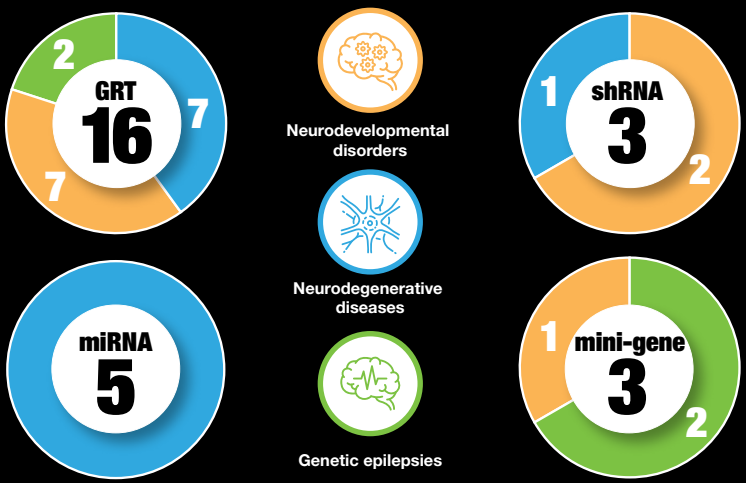
Tuesday, May 17 from noon to 1:30 PM ET in Room 207

Complimentary lunch will be provided.

Presented by Dr Suyash Prasad, Dr Steven Gray, Dr Kimberly Goodspeed, and Dr Jagdeep Walia

Taysha Gene Therapies has a diverse pipeline focused exclusively on monogenic disorders of the central nervous system

Therapies in Development



AAV, adeno-associated virus: CNS, central nervous system: GRT, Gene Replacment Therapy: miRNA, microRNA: shRNA, short hairpin RNA.

Contact us at medinfo@tayshagtx.com.
https://tayshagtx.com/

TSHA-0168-2022

SCHEDULE (ALL TIMES LISTED IN ET)

MONDAY, MAY 16, 2022

EXHIBITOR SHOWCASES

ROOM 209
Industry Interactions
GenScript USA Inc. | Fully Non-viral T cell Engineering With Hybrid ssDNA Repair Templates

9:00–9:30 AM — **Brian Shy, MD, PhD**
Associate Medical Director, UCSF

ROOM 209
Industry Interactions
10x Genomics | Case Study: Sequencing Strategies to Determine Cellular States During Reprogramming and Regeneration

10:00–10:30 AM — **Peter Andersen, MS, PhD**
Assistant Professor, The Johns Hopkins School of Medicine Department of Medicine

ROOM 209
Industry Interactions
InVitria | Exhibitor Showcase

11:00–11:30 AM — **Presenter(s) to be announced**

SCHEDULE (ALL TIMES LISTED IN ET)

MONDAY, MAY 16, 2022

ORAL ABSTRACT SESSIONS

ROOM 204
AAV Gene Therapy in Large Animal Models
Co-chairs: Heather Gray-Edwards, DVM, PhD, UMass Chan Medical School and Juliette Hordeaux, DVM, PhD, University of Pennsylvania

10:15–10:30 AM	**1: *In vivo* Selection of Randomly Integrated rAAV Vectors** *Amita Tiyaboonchai, PhD, Oregon Health and Science University*
10:30–10:45 AM	**2: Bicistronic AAV Gene Therapy for Tay-Sachs and Sandhoff Diseases in the Sheep Model of Tay-Sachs** *Toloo Taghian, PhD, UMass Chan Medical School*
10:45–11:00 AM	**3: Vagus Nerve Delivery of AAV9 to Treat Autonomic Nervous System Dysfunction in Giant Axonal Neuropathy** *Rachel Bailey, PhD, UT Southwestern Medical Center*
11:00–11:15 AM	**4: Assessment of Gene Therapy Treatment in the Pompe Disease Canine Model** *Megan Pope, University of Florida, Powell Gene Therapy Center*
11:15–11:30 AM	**6: The Porcine Model for In Utero Gene Therapy** *Apeksha Dave, MD, CHOP*
11:30 AM–11:45 PM	**7: Temporary Mechanical Support Improves Cardiac AAV Gene Transfer Efficacy in a Pig HF Model** *Renata Mazurek, MD, Mount Sinai*

SCHEDULE (ALL TIMES LISTED IN ET)

MONDAY, MAY 16, 2022

ORAL ABSTRACT SESSIONS

ROOM 201
Biology of Gene Edited Cells
Co-chairs: Matthew Porteus, MD, PhD, Stanford University and Paula Rio, PhD, CIEMAT

10:15–10:30 AM	**8: Double Strand Break Free Genome Editing to Target Hematopoietic Stem and Progenitor Cells: Therapeutic Applicability in Fanconi Anemia** *Laura Ugalde, CIEMAT/CIBERER/IIS-FJD*
10:30–10:45 AM	**9: Cellular Senescence and Inflammatory Programs are Unintended Consequences of CRISPR-Cas9 Gene Editing in Hematopoietic Stem and Progenitors Cells** *Anastasia Conti, SR-TIGET*
10:45–11:00 AM	**10: The Choice of Template Delivery Mitigates the Genotoxic Risk and Adverse Impact of Editing in Human Hematopoietic Stem Cells** *Aurelien Jacob, PhD, San Raffaele Telethon Institute for Gene Therapy, IRCCS San Raffaele Scientific Institute*
11:00–11:15 AM	**11: Assessing Stealth and Sensed Base Editing in Human Hematopoietic Stem/Progenitor Cells** *Martina Fiumara, IRCCS San Raffele Hospital*
11:15–11:30 AM	**12: DNA Barcode as a Useful Tool to Study Hematopoietic Stem Cell Fate in Gene Editing Strategies** *Isabel Ojeda-Perez, PhD, Cell Technology Division, Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas (CIEMAT) and Centro de Investigación Biomédica en Red de Enfermedades Raras (CIBERER)*
11:30–11:45 AM	**13: High Frequency of AAV Integration at Double Strand Breaks Induced in Preclinical Model of Gene Therapy and in Edited Long-Term Engrafted HSPCs** *Daniela Cesana, PhD, SR-TIGET*
11:45 AM–12:00 PM	**14: Base Editing of a γ-globin *cis*-regulatory Element in Human Hematopoietic Stem Cells for Reactivation of Therapeutic Fetal Hemoglobin** *Panagiotis Antoniou, PhD, IMAGINE Institute, INSERM UMR1163*

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MONDAY, MAY 16, 2022

ORAL ABSTRACT SESSIONS

SALON H
Oligonucleotide Therapeutics
Co-chairs: Shen Shen, PhD, Vertex Pharmaceuticals and Michelle Hastings, PhD, Rosalind Franklin University

10:15–10:30 AM	**15: Development of an AIMer for the Treatment of Alpha-1 Antitrypsin Deficiency** *Prashant Monian, PhD, Wave Life Sciences*
10:30–10:45 AM	**16: STK-002, an Antisense Oligonucleotide (ASO) for the Treatment of Autosomal Dominant Optic Atrophy (ADOA), Localizes to Retinal Ganglion Cells (RGC) and Upregulates OPA-1 Protein Expression After Intravitreal Administration to Non-human Primates (NHP)** *Aditya Venkatesh, PhD, Stoke Therapeutics*
10:45–11:00 AM	**17: Repeat Dosing with DYNE-101 is Well Tolerated and Leads to a Sustained Reduction of *DMPK* RNA Expression in Key Muscles for DM1 Pathology in hTfR1/DMSXL Mice and NHPs** *Stefano Zanotti, PhD, Dyne Therapeutics*
11:00–11:15 AM	**18: SNCA Reduction for the Treatment of Synucleinopathie** *Bradford Elmer, PhD, Sanofi*
11:15–11:30 AM	**19: Alpha-synuclein Lowering and Rescue of Motor Phenotype by miRNA-based AAV Gene Therapy in *In Vivo* Parkinson's Disease Models** *Seyda Açar Broekmans, PhD, uniQure Biopharma BV*
11:30–11:45 AM	**20: Discovery of Translation Initiation Elements Enabled by a Parallel Arrayed Screen of Full-length Viral UTRs in Synthetic Circular RNA** *Alexander Wesselhoeft, Orna Therapeutics*
11:45 AM–12:00 PM	**21: Tumor-targeted miRNA Agent for Pediatric Glioblastoma** *Sunam Mander, PhD, University of Illinois at Chicago*

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ORAL ABSTRACT SESSIONS

ROOM 206
Cardiovascular and Pulmonary Diseases
Co-chairs: Margaret Sleeper, VMD, University of Florida School of Veterinary Medicine and Uta Griesenbach, PhD, Imperial College Faculty of Medicine

10:15–10:30 AM	**22: Development of Passive Immunoprophylaxis Against SARS-CoV-2 using Elderly and Immunodeficient Mice Models** *Yue Du, PhD, University of Oxford*
10:30–10:45 AM	**23: F/HN Pseudotyped Lentiviral Vector Uses Alpha 2,3 Sialylated N-Acetyllactosamine to Efficiently Transduce Human Airway Cells** *Rosie Munday, PhD, University of Oxford*
10:45–11:00 AM	**24: Extended Results from First-In-Human Clinical Trial Of RP-A501 (AAV9:LAMP2B) Gene Therapy Treatment for Danon Disease** *Barry Greenberg, PhD, UC San Diego Medical Center*
11:00–11:15 AM	**25: Amphiphilic Peptides Deliver Adenine Base Editor RNPs to Rhesus Monkey Airway Epithelial Cells *In Vivo*** *Katarina Kulhankova, MD, PhD, University of Iowa*
11:15–11:30 AM	**26: Gene Therapy Induced Cholesterol Catabolism to Treat Atherosclerosis and NASH** *Mourad Toporsian, PhD, Repair Biotechnologies, Inc*
11:30–11:45 AM	**27: AAV Gene Therapy Using a Genetic Suppressor Treats *LMNA* Dilated Cardiomyopathy in a *Lmna* Mouse Model Following a Decline in Ejection Fraction** *Yin Loon Lee, PhD, Nuevocor Pte Ltd*
11:45 AM–12:00 PM	**28: A First-in-Human Phase 1 Clinical Gene Therapy Trial for the Treatment of Heart Failure Using a Novel Re-Engineered Adeno-Associated Vector** *Roger Hajjar, MD, Asklepios BioPharmaceutical, Inc.*

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ORAL ABSTRACT SESSIONS

SALON G
Musculo-skeletal Diseases
Co-chairs: Perry Shieh, MD, PhD, UCLA
and Lindsay Wallace, PhD, Nationwide Children's Research Institute

10:15–10:30 AM	**29: Muscle-specific Tyrosine Kinase Chimeric Autoantibody Receptor T-Cells (MuSK-CAART): A Precision Cellular Immunotherapy for Antigen-specific B Cell Depletion in MuSK Myasthenia Gravis** *Sangwook Oh, PhD, University of Pennsylvania*
10:30–10:45 AM	**30: Novel Single AAV Vector Treatment for Congenital Muscular Dystrophy Type 1A (MDC1A) Using CRISPR-GNDM® Technology** *Yuanbo Qin, PhD, Modalis Therapeutics Inc.*
10:45–11:00 AM	**31: Regeneration of Articular Cartilage, Suppression of Synovial Inflammation, and Alleviation of Joint Pain After Intra-articular Injection of ICM-203 in Canine OA Model** *Minsun Park, Yonsei University*
11:00–11:15 AM	**32: Lentiviral Vector-Based Gene Therapy for Type II Collagen Disorders** *David Favre, PhD, Innoskel, SAS*
11:15–11:30 AM	**33: Multicenter AAV Gene Therapy Studies for SMARD1/CMT2S Establish Safety and Efficacy in Multiple Animal Models and Pave the Way for Initiation of a Phase I/II Clinical Trial** *Kathrin Meyer, PhD, Nationwide Childrens Hospital*
11:30–11:45 AM	**34: AAV-CRISPR-Cas13 Gene Therapy for FSHD: DUX4 Gene Silencing Efficacy and Immune Responses to Cas13b Protein** *Afrooz Rashnonejad, PhD, The Abigale Wexner Research Institute of Nationwide Children's Hospital*
11:45 AM–12:00 PM	**35: Development of Dual AAV-mediated RNAi and Protein Expression Therapy for Myotonic Dystrophy** *Matthew Karolak, PhD, University of Washington*

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ORAL ABSTRACT SESSIONS

ROOM 102
Immune Responses to AAV Vectors
Co-chairs: Julie Crudele, PhD, University of Washington and Ying Kai Chan, PhD, Harvard University

10:15–10:30 AM	**36: Characterizing AAV-mediated Immune Responses in a Mouse Model of Duchenne Muscular Dystrophy** *Melissa Spencer, UCLA*
10:30–10:45 AM	**37: Functional Assessment of T-cell Responses to AAV8 Empty Capsids in Healthy Volunteers** *Holly Schroeder, PhD, AskBio*
10:45–11:00 AM	**38: Characterization of the Innate Immune Response to AAV in Human Blood and the Central Role of Complement** *Corinne Smith, PhD, Spark Therapeutics*
11:00–11:15 AM	**39: Deimmunized Micro-dystrophin Vectors Blunt Patient Immunity *in vitro* & Restore Cardiac Functional Deficits *In Vivo* in *mdx*^{4cv} DMD Mice** *Guy Odom, PhD, University of Washington*
11:15–11:30 AM	**40: Pretreatment With IVIG Reduces Peripheral Transduction of AAV9 Delivered to the CNS** *Cara West, Affinia Therapeutics, Inc.*
11:30–11:45 AM	**41: Differential T-cell Immune Responses to Deamidated Adeno-associated Virus Vector** *So Jin Bing, Food and Drug Administration*
11:45 AM–12:00 PM	**42: Interplay Between Plasmacytoid Dendritic Cells and Kupffer Cells in IL-1R1-MyD88 Driven Cellular Immune Responses to Hepatic AAV Gene Transfer** *Sandeep Kumar, PhD, Indiana University*

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ORAL ABSTRACT SESSIONS

BALLROOM A

Vector Manufacturing and Engineering I: Deciphering AAV Vector Genomes

Co-chairs: H. Trent Spencer, PhD, Emory University School of Medicine and John Gray, PhD, Vertex Pharmaceuticals

10:15–10:30 AM	**43: Direct ITR-To-ITR Nanopore Sequencing of Plasmid and Vector Preparations and Their Implications for *In Vivo* Gene-editing Vector Performance** *Suk Namkung, ScM, UMass Chan Medical School*
10:30–10:45 AM	**44: Physico- and Biochemical Characterization of Linear Covalently Closed dbDNA™ for rAAV Manufacturing** *Felix Bastida. DVM, PhD. TAAV Biomanufacturing Solutions, S.L.*
10:45–11:00 AM	**45: Assessment and Comparison of Digital PCR Platforms for AAV Viral Genome Titration** *Stuart Nelson, MS, Prevail Therapeutics*
11:00–11:15 AM	**46: rAAV Vector Breakpoints Determined Using Single-molecule, Modified Base Sequencing** *Donald Selby, PhD, Homology Medicines*
11:15–11:30 AM	**47: Positioning Short-hairpin Stopper Sequences Outside of the ITRs Reduces Encapsidation of Non-vector DNA in rAAV Preparations** *Mitchell Yip, UMass Chan Medical School*
11:30–11:45 AM	**48: Characterization of Residual DNA in rAAV Products Made in the Baculovirus/Sf9 Platform** *Daniel Barajas, PhD, BioMarin Pharmaceutical*
11:45 AM–12:00 PM	**49: NGS Based Evaluation of AAV Genome Integrity for Improved Production and Function** *Keith Connolly, Modalis Therapeutics*

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MONDAY, MAY 16, 2022

INDUSTRY SPONSORED SYMPOSIA

ROOM 202

Labcorp Drug Development (formerly Covance) | Strategies and Approaches to Optimize Your Non-clinical and Clinical Development for Cell and Gene Therapies

12:00–1:30 PM

Maryland Franklin, PhD
VP and Head, Enterprise Cell & Gene Therapy, Labcorp Drug Development

Brian McIntosh, PhD
Lead, Cell & Gene Therapy/ Safety Assessment/Toxicology, Labcorp Drug Development

Mark Cameron, MS
Director, Scientific Development, Preclinical Oncology, Labcorp Drug Development

Darby Thomas, PhD
Director, Enterprise Cell & Gene Therapy, Labcorp Drug Development

Alicia M. Baker McDowell, DRSc, MS
Head of Regulatory Strategy, Product Development & Market Access Consulting, Labcorp Drug Development

Xcell Biosciences Representative
Xcell Biosciences

Akanksha Gupta, PhD
Executive Director, Enterprise Cell & Gene Therapy, Labcorp Drug Development

Paul Byrne, MS
Associate Director, Enterprise Cell & Gene Therapy, Labcorp Drug Development

Fred Derosier, DO
VP, Rare Diseases & Pediatrics and Clinical Lead, Enterprise, Labcorp Drug Development

Ningchun Liu, PhD
Director, Enterprise Cell & Gene Therapy, Labcorp Drug Development

TrakCel Representative
TrakCel

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MONDAY, MAY 16, 2022

INDUSTRY SPONSORED SYMPOSIA

BALLROOM B

MaxCyte | Industry Sponsored Symposium

12:00–1:30 PM — **Presenter(s) to be Announced**

BALLROOM C

Sarepta Therapeutics | Clinical Horizons for Investigational AAV-Based Gene Transfer Therapy: The Rationale Behind rAAVrh74 as a Platform for Neuromuscular Diseases

12:00–1:30 PM — **Presenter(s) to be Announced**

ROOM 207

Terumo Blood and Cell Technologies | Balancing Scale, Risk and Cost: How to Automate Your T cell Manufacturing Effectively

12:00–1:30 PM — **Stuart Gibb, PhD**
Scientific Strategy Lead; Terumo Blood and Cell Technologies

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MONDAY, MAY 16, 2022

STARTUP SHOWCASES

ROOM 209
Industry Interactions
Chair: Li Ou, PhD, Capsida Biotherapeutics

12:00–12:10 PM	**AviadoBio | A Revolution in Gene Therapy for Neurodegenerative Disorders** *Lisa Deschamps, MBA, CEO, AviadoBio*
12:10–12:20 PM	**CellFE | Building the Future of Advanced Therapies Through Microfluidics** *Alla Zamarayeva, PhD, CEO and Cofounder, CellFE*
12:20–12:30 PM	**Nuevocor | Leveraging Mechanobiology to Treat Genetic Cardiomyopathies** *Yann Chong Tan, PhD, CEO, Nuevocor*
12:30–12:40 PM	**Rejuvenation Technologies | Extending Telomeres to Extend the Cellular and Human Lifespan** *Colin Maraganore, Business Development Associate, Rejuvenation Technologies*
12:40–12:50 PM	**AAVnerGene Inc. | New Methods in AAV Production, Characterization and Capsid Selection** *Daozhan Yu, PhD, CEO, AAVnerGene Inc.*
12:50–1:00 PM	**To be Announced**

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MONDAY, MAY 16, 2022

SCIENTIFIC SYMPOSIA

SALON H
Competing With Best Available Care: Perspectives on Lowering Burden of Treatment With Cell and Gene Therapies
Co-chairs: Nimi Chhina, PhD, BioMarin and Kristin Van Goor, PhD, Vertex Pharmaceuticals

1:30–1:45 PM	**Assessing Best Available Care and CGT: A Disease-Agnostic Viewpoint** *Craig Martin, Global Genes*
1:45–2:00 PM	**FDA Considerations on Risk/Benefit for Best Available Care Versus New Innovative Therapies** *Peter Bross, MD, Food and Drug Administration*
2:00–2:15 PM	**Assessing Best Available Care and Cell and Gene Therapy in the Diabetes Context** *Marjana Marinac, PharmD, Juvenile Diabetes Research Foundation*
2:15–2:30 PM	**Case Study of Zolgensma: Determining Labeling Requirements for Gene Therapies to Guide Patient and Physician Choice** *Sitra Tauscher-Wisniewski, MD, Novartis Gene Therapies*
2:30–2:45 PM	**Considerations for Gene Therapy Development in Diseases with Available Therapies With Unmet Medical Need** *Jill Jarecki, PhD, BioMarin*
2:45–3:15 PM	**Panel Discussion**

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SCIENTIFIC SYMPOSIA

ROOM 102
Cutting Edge Gene and Cell Therapy Research in Japan
Co-chairs: Noriyuki Kasahara, MD, PhD, UCSF and Takafumi Nakamura, PhD, Tottori University School of Medicine

1:30–1:56 PM	**An RNA Gene Drug DVC1-0101 Based on Recombinant Sendai Virus Vector to Treat Severe Intermittent Claudication: Topline Results From Multicenter, Double-blinded, Placebo-controlled Phase IIb Clinical Trial** *Yoshikazu Yonemitsu, MD, PhD, Kyushu University*
1:56–2:22 PM	**Transposon-mediated CAR T-cells** *Shigeki Yagyu, MD, PhD, Kyoto Prefectural University of Medicine*
2:22–2:48 PM	**Gene Therapy in a Mouse Model of OTC Deficiency With Engineered AAV3 Vector** *Kazuhiro Muramatsu, MD, PhD, Jinchi Medical University*
2:48–3:14 PM	**Development of BBB-penetrating Heteroduplex Oligonucleotides Regulating CNS Genes** *Takanori Yokota, MD, PhD, Tokyo Medical and Dental University*

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SCIENTIFIC SYMPOSIA

BALLROOM A
Immune Responses to Gene Therapy
Co-chairs: Allison M. Keeler, PhD, University of Massachusetts Chan Medical School and Melissa Rhodes, PhD, Kriya Therapeutics

1:30–1:55 PM	**Innate Immune Responses in Gene Engineering** *Anna Kajaste-Rudnitski, PhD, San Raffaele Telethon Institute for Gene Therapy*
1:55–2:20 PM	**Systemic and Local Immune Responses to Intraocular AAV Vector Administration** *Deniz Dalkara, PhD, Institut de la Vision*
2:20–2:45 PM	**Mechanisms, Monitoring, and Mitigation of Host Immune Responses to AAV Gene Therapy Vectors** *Kei Kishimoto, PhD, Selecta Biosciences*
2:45–3:10 PM	**Drug-mediated Modulation of Immune Responses to Gene Therapy** *Klaudia Kuranda, PhD, Spark Therapeutics*

SALON G
Translating Science Into Medicine: Moving From Bench to Startup (Session 2 of 2)
Co-chairs: H. Trent Spencer, PhD, Emory University School of Medicine and Madhusudan Peshwa, PhD, Tessera Therapeutics

1:30–2:18 PM	**Launching Innovation Into Gene Therapy Companies** *Sheila Mikhail, JD, AskBio*
2:18–3:06 PM	***Ex Vivo* Gene-edited Cell Therapy Products** *Jane Grogan, PhD, Graphite Bio*

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SCIENTIFIC SYMPOSIA

ROOM 206
Career Development Award Presentations
Chair, Le Cong, PhD, Stanford University

1:35–1:55 PM	**Engineering Chimeric Gene Therapy Vectors with Enhanced Packaging Capacity** *Victoria Madigan, MIT*
1:55–2:15 PM	**Therapeutic Isoform Specific Knockkdown Strategies for Limb Girdle Muscular Dystrophy D1** *Andrew Findlay, Washington University*
2:15–2:35 PM	**Spatially-controlled Brain Gene Editing Guided by Non-invasive Focused Ultrasound** *Yeh-Hsing Lao, Columbia University*
2:35–2:55 PM	**Polymeric Gene Delivery Nanoparticles to Treat Multiple Sclerosis** *Stephany Tzeng, John Hopkins University*
2:55–3:15 PM	**Allo-iNKT Cells are Safe and Persist in MHC-mismatched Dogs** *Antonia Rotolo, University of Pennsylvania*

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EXHIBITOR SHOWCASES

ROOM 209 | Industry Interactions
PerkinElmer | Exhibitor Showcase

1:30–2:00 PM — Presenter(s) to be announced

ROOM 209 | Industry Interactions
PTC Therapeutics, Inc. | Pioneering In Gene Therapies: An Update From PTC Therapeutics

2:30-3:00 PM

Matthew B. Klein, MD, MS, FACS
Chief Operating Officer, PTC Therapeutics, Inc.

Philippe Moyen
Chief of Staff & Head of CMC Program Management – Technical Operations, PTC Therapeutics, Inc.

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ORAL ABSTRACT SESSIONS

ROOM 201
Gene and Cell Therapy Trials in Progress
Co-chairs: Kevin Flanigan, MD, Nationwide Children's Hospital and Barry Byrne, MD, PhD, University of Florida

1:30–3:15 PM

50: High Anti-Sickling Potency of a Gamma Globin in the Phase 1/2 MOMENTUM Study of ARU-1801 Gene Therapy and Reduced Intensity Conditioning for Sickle Cell Disease
Punam Malik, MD, Cincinnati Children's Hospital Medical Center

51: Safety and Outcomes of Intravenous scAAV9.U7-ACCA for the Treatment of Duchenne Muscular Dystrophy Caused by Exon 2 Duplications
Megan Waldrop, MD, Nationwide Children's Hospital

52: RGX-121 Gene Therapy for the Treatment of Severe Mucopolysaccharidosis Type II (MPS II): Interim Analysis of Data from the First in Human Study
Roberto G Hiugliani, MD, Department of Genetics, UFRGS, Medical Genetic Service, HCPA

53: Up to 10.5 Years of Follow-up in 17 Subjects Treated With Hematopoietic Stem and Progenitor Cell Lentiviral Gene Therapy for Wiskott-Aldrich Syndrome
Francesca Ferrua, IRCCS San Raffaele Scientific Institute

54: Anti-GD2 CAR NKT Cells are Safe and Produce Antitumor Responses in Patients with Relapsed/Refractory Neuroblastoma
Andras Heczey, MD, Baylor College of Medicine

55: Exploratory Immuno-Safety Profile of EDIT-101, a First-in-Human *in vivo* CRISPR Gene Editing Therapy for *CEP290*-related Retinal Degeneration
Brian Duke, Editas Medicine, Inc.

56: Hematopoietic Stem Cell Gene Therapy for Cystinosis: Updated Results from a Phase 1/2 Clinical Trial
Stephanie Cherqui, PhD, UC San Diego

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TOOLS AND TECHNOLOGY FORUM I

ROOM 209
Industry Interactions
Co-chairs: Afrooz Rashononejad, PhD, The Abigail Wexner Research Institute of Nationwide Children's Hospital and Li Ou, PhD, Capsida Biotherapeutics

3:30–3:45 PM	**Forecyte Bio USA Limited | Accelerating Discovery to Clinical with Trusted CDMO Services of Plasmids, Viral Vectors and Cells** *Shuyuan Zhang, CTO, Technology*
3:45–4:00 PM	**Polyplus-transfection | Next-Generation Transfection Reagent for Large Scale AAV Manufacturing** *Mathieu Porte, R&D Bioproduction Manager, Polyplus-transfection*
4:00–4:15 PM	**STEMCELL Technologies | Generation of Large Numbers of Megakaryocytes from Human Pluripotent Stem Cells** *Leon Lin, PhD, Senior Scientist, RND, STEMCELL Technologies*
4:15–4:30 PM	**WuXi Advanced Therapies | TESSA Technology: Scalable, Plasmid Free rAAV Manufacture** *Ryan Cawood, Chief Scientific Officer, WuXi Advanced Therapies*
4:30–4:45 PM	**Pall Corporation | Quality by Design for Adeno-associated Virus AAV Products** *Peiqing Zhang, Strategic Technology Partnership Leader, Pall Corporation*
4:45–5:00 PM	**Molecular Devices | Next Generation Clone Screening Workflows From Molecular Devices** *Paula L. Feinberg-Zadek, PhD,BioPharma Field Applications Scientist II, Molecular Devices*
5:00–5:15 PM	**Cytiva | Purify Small Drug Volumes Better — GMP Manufacturing of Viral Vectors** *Fredrik Lundström, Senior Product Manager Downstream Hardware Single-use Bioprocess, Cytiva*

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TOOLS AND TECHNOLOGY FORUM I

5:15–5:30 PM	**MilliporeSigma | Bioprocess Development Using the VirusExpress® 293 AAV Platform** *Eva Fong Principal Scientist, Virus and Gene Therapy Bioprocessing, MilliporeSigma*
5:30–5:45 PM	**Dyne Therapeutics | FORCE™ Platform for Targeted Delivery of Oligonucleotide Therapeutics in Muscle Diseases** *Ashish Dugar, Senior VP, Global Head of Medical Affairs, Dyne Therapeutics*
5:45–6:00 PM	**Andelyn Biosciences | Reliable Scale-Up to 2000L in Gene Therapy Manufacturing** *Wade Macedone, Chief Operations Officer, Andelyn Biosciences*

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ORAL ABSTRACT SESSIONS

ROOM 204
AAV Preclinical CNS Gene Therapy
Co-chairs: Allison Bradbury, PhD, Nationwide Children's Hospital and Brian Bigger, PhD, University of Manchester

3:45–4:00 PM	**57: Selection of Clinical Doses for SBT101, an AAV9-h*ABCD1* Vector for the Treatment of Adrenomyeloneuropathy** *D.W. Anderson, SwanBio Therapeutics Ltd*
4:00–4:15 PM	**58: AAV-ARSA Mediated Gene Replacement for the Treatment of Metachromatic Leukodystrophy** *Shyam Ramachandran, PhD, Sanofi*
4:15–4:30 PM	**59: Three Examples of Long-term AIDS-virus Suppression Using AAV-Delivered Monoclonal Antibodies** *Jose Martinez-Navio, Miller School of Medicine, University of Miami*
4:30–4:45 PM	**60: AAV-mediated Delivery of Anti-HIV Antibodies to the CNS** *Jose Martinez-Navio, Miller School of Medicine, University of Miami*
4:45–5:00 PM	**61: Antibody Gene Therapy for Rabies Encephalitis** *Amanda Gross, PhD, Auburn University*
5:00–5:15 PM	**62: Development of an Intrathecal AAV9/AP4M1 Gene Therapy for Hereditary Spastic Paraplegia 50 (SPG50)** *Xin Chen, UT Southwestern Medical Center*
5:15–5:30 PM	**63: CNS Penetrant AAV Vectors Encoding HER2 Antibodies Reduce Tumor Burden in Models of Breast Cancer Brain Metastasis** *Dan Laks, PhD, Voyager Therapeutics*

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ORAL ABSTRACT SESSIONS

BALLROOM A
Gene Editing in Blood and Immune Disorders
Co-chairs: Maria-Grazia Roncarolo, MD, Stanford University and Jose Segovia, PhD, CIEMAT

3:45–4:00 PM	**64: Two is Better Than One: CRISPR/Cas9 Based Gene Editing with FOXP3 Isoforms for IPEX Therapy** *Esmond Lee, PhD, Stanford*
4:00–4:15 PM	**65: Preclinical Safety and Feasibility Study of a CRISPR/Cas9 Gene Editing Platform to Treat Wiskott Aldrich Syndrome** *Alessia Cavazza, PhD, UCL Institute of Child Health*
4:15–4:30 PM	**66: Development of a Beta-globin Gene Replacement Strategy as a Therapeutic Approach for β-Thalassemia** *Beeke Wienert, PhD, Graphite Bio, Inc.*
4:30–4:45 PM	**67: Adenine Base Editor-mediated Correction of Three Prevalent and Severe β-thalassemia Mutations** *Giulia Hardouin, Imagine Institute, INSERM UMR1163*
4:45–5:00 PM	**68: Improvement of *PKLR*-gene Editing in Human Hematopoietic Stem and Progenitor Cells Towards its Clinical Application for Pyruvate Kinase Deficiency** *Isabel Ojeda-Pérez, PhD, Cell Technology Division, Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas (CIEMAT) and Centro de Investigación Biomédica en Red de Enfermedades Raras (CIBERER)*
5:00–5:15 PM	**69: Site-specific Editing Methods to Reverse Severe Combined Immunodeficiency (SCID) in Athabascan-speaking Native Populations** *Patricia Claudio Vázquez, University of Minnesota*
5:15–5:30 PM	**70: Development of a Double shmiR Lentivirus Effectively Targeting Both BCL11A and ZNF410 for Enhanced Induction of Fetal Hemoglobin to Treat β-hemoglobinopathies** *Boya Liu, Boston Children's Hospital*

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ORAL ABSTRACT SESSIONS

SALON H
Synthetic Nanoparticle-Based Gene Transfer
Co-chairs: Hai-Quan Mao, PhD, Johns Hopkins University and Carol Miao, PhD, Seattle Children's Research Institute

3:45–4:00 PM	**71: Building a Genetic Medicine Platform for DNA-encoded Antibody Therapeutics** *Kevin Hollevoet, PhD, PharmAbs - KU Leuven University*
4:00–4:15 PM	**72: Mechanisms of siRNA Delivery by Cyclic Amphipathic Peptides** *Uday Baliga, BS, MS, University of Rochester*
4:15–4:30 PM	**73: *In Vivo* Spatially Targeted Nonviral Optical Delivery of Genes in Mice to NHPs** *Sanghoon Kim, PhD*
4:30–4:45 PM	**74: Particle Size Engineering to Enhance mRNA Delivery Efficiency via Biodegradable Carriers *In Vivo*** *Yizong Hu, Johns Hopkins University*
4:45–5:00 PM	**75: Delivery of CRISPR/Cas9 mRNA LNPs to Repair a Small Deletion in FVIII Gene in Hemophilia A Mice** *Chun-Yu Chen, PhD, Seattle Children's Research Institute*
5:00–5:15 PM	**76: Ministring DNA: A Durable and Safe Non-viral Delivery Platform** *Ting-Yen Chao, Seattle Children's Research Institute*
5:15–5:30 PM	**77: Nano-structures for Efficient and Transgene-free Immune Cell Transfection** *Andy Tay, PhD, National University of Singapore (NUS)*

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ORAL ABSTRACT SESSIONS

Salon G
Tools and Approaches for Inborn Errors of Metabolism
Co-chairs: Gloria Gonzalez-Aseguinolaza, PhD, Vivet Therapeutics and Charles Venditti, MD, PhD, NIH

3:45–4:00 PM	**78: Nuclease-Free Promoterless Genome Editing for Wilson Disease** *Agnese Padula, Telethon Institute of Genetics and Medicine (TIGEM)*
4:00–4:15 PM	**79: Systemic Gene Therapy Using the Novel Adeno-Associated Viral Vector 44.9** *Randy Chandler, PhD, NHGRI, NIH*
4:15–4:30 PM	**80: Single, Systemic Administration of BEAM-301 Mitigates Fasting Hypoglycemia and Restores Metabolic Function in a Transgenic Mouse Model of Glycogen Storage Disease Type Ia** *Yvonne Aratyn-Schaus, PhD, Beam Therapeutics*
4:30–4:45 PM	**81: CRISPR-mediated Insertion of a Targeted GAA Transgene into Hepatocytes Provides Effective, Long-lasting Gene Therapy in Neonate and Adult Pompe Disease Mice** *Andrew Baik, MD, Regeneron Pharmaceuticals*
4:45–5:00 PM	**82: A Novel Human Liver Chimeric Mouse Model Lacking the Murine *AAVR* Gene for Validation of AAV Gene Therapy Vectors** *Tong Chen, MD, PhD, Duke University*
5:00–5:15 PM	**83: Lentiviral-Based Genetic Correction of IL10RB-Defect to Treat Very Early Onset-Inflammatory Bowel Disease** *Adele Mucci, Dana Farber/Boston Children's Cancer and Blood Disorders Center, Harvard Medical School*
5:15–5:30 PM	**84: *Ex vivo* Editing of Hematopoietic Stem Cells for Erythroid Expression of Therapeutic Proteins *In Vivo* for LAL-D Therapy** *Marine Laurent, Genethon*

SCHEDULE (ALL TIMES LISTED IN ET)

MONDAY, MAY 16, 2022

ORAL ABSTRACT SESSIONS

ROOM 102
CAR T-cells and Beyond
Co-chairs: Chiara Bonini, MD, Università Vita Salute San Raffaele and Avery Posey, PhD, University of Pennsylvania

3:45–4:00 PM	**85: A Genome-Scale Screen for Synthetic Drivers of T-cell Proliferation** *Mateusz Legut, PhD, NY Genome Center*
4:00–4:15 PM	**86: CD45RA Expressing PBMCs Effect the Outgrowth of Epstein-Barr Virus Antigen Specific T-cells** *Sandhya Sharma, Baylor College of Medicine*
4:15–4:30 PM	**87: IFNg Impedes Antigen-Specific Proliferation of CAR-T with a CD28, but not 4-1BB, Costimulatory Domain** *Stefanie Bailey, PhD, Massachusetts General Hospital*
4:30–4:45 PM	**88: Naturally Occurring CD7- T-cells Mark a Functional Effector and Persistent CAR T-cell Population** *Jaquelyn Zoine, PhD, St. Jude Children's Research Hospital*
4:45–5:00 PM	**89: Tim-4-Chimeric Engulfment Receptor (CER) T-cell Therapy Elicits Phosphatidylserine-Dependent Cytotoxic and Antigen-Presenting Cell-Like Function and Synergizes with Approved BTK Inhibitors for the Treatment of Hematologic Malignancies** *Daniel Corey, MD, CERo Therapeutics*
5:00–5:15 PM	**90: Peptide-scFv Bispecific CAR T-cells targeting Acute Myeloid Leukemia** *Jaquelyn Zoine, PhD, St Jude Children's Research Hospital*
5:15–5:30 PM	**91: CD28-based B7-H3 CAR T-cells Have Superior Anti-glioma Efficacy in an Immune Competent Glioma Model While Suppressive Macrophages at Tumor Edges are Associated with Therapeutic Resistance** *Dalia Haydar, PharmD, PhD, St Jude Children's Research Hospital*

SCHEDULE (ALL TIMES LISTED IN ET)

MONDAY, MAY 16, 2022

ORAL ABSTRACT SESSIONS

ROOM 206
Cancer — Oncolytic Viruses
Co-chairs: Paola Grandi, PhD, MBA , CG Oncology and Marta Alonso, PhD, University Hospital of Navarra

3:45–4:00 PM	**92: The Potential of Oncolytic Virotherapy in Synovial Sarcoma** *Steven Robinson, Mayo Clinic*
4:00–4:15 PM	**93: Oncolytic Adenovirus Expressing IFN-alpha with Chemoradiation (GEM+nab-PTX) Synergistically Inhibits Pancreatic Cancer Cell Growth *In Vitro* and *In Vivo*** *Shuhei Shinoda, MD, PhD, University of Minnesota*
4:15–4:30 PM	**94: Oncolytic HSV-1 rQNestin34.5v2 Sensitizes IDH1-mutated Glioma to Immunotherapy** *Eleni Panagioti, PhD, Brigham and Womens Hospital, Harvard Medical School*
4:30–4:45 PM	**95: Characterization of the Cancer-Targeted Oncolytic Adenoviruses with Fiber-Knob Modification** *Mizuho Sato-Dahlman, PhD, University of Minnesota*
4:45–5:00 PM	**96: Optimizing NIS-expression for Oncolytic Adenovirus-based Radiotherapy and Imaging of Breast Cancer** *Robert Sacha, PhD, University of Minnesota*
5:00–5:15 PM	**97: Tumor Regression of Oncolytic Adenovirus-treated Melanoma Rely on the Gut Microbiome** *Lorella Tripodi, PhD, CEINGE Biotecnologie Avanzate*
5:15–5:30 PM	**98: Oncolytic Adenovirus with Hyaluronidase Activity that Evades Neutralizing Antibodies and Allows Re-administration: VCN-11** *Ramon Alemany, PhD, VCN Biosciences*

SCHEDULE (ALL TIMES LISTED IN ET)

MONDAY, MAY 16, 2022

ORAL ABSTRACT SESSIONS

ROOM 207
Cell-based Cancer Immunotherapies I
Co-chairs: Barbra Sasu, PhD, Allogene
and Daniel Abate-Daga, PhD, H. Lee Moffitt Cancer Center and Research Institute

3:45–4:00 PM	**99: Endowing Universal CAR T-cell With Immune-Evasive Properties Using TALEN-Gene Editing** *Julien Valton, PhD, Cellectis INC*
4:00–4:15 PM	**100: Engineering Stealth CAR T-cells to Evade the Host Immune Responses** *Korneel Grauwet, PhD/Ir, Massachusetts General Hospital / Harvard Medical School*
4:15–4:30 PM	**101: Tumor-directed, Myeloid Cell-based Cytokine Gene Delivery Unleashes CAR T Cells in the Immunosuppressive Glioblastoma Microenvironment to Control Tumor Growth** *Federico Rossari, MD*
4:30–4:45 PM	**102: Activation Regulated Gene Circuit for Controlling Payload Expression in Cell Therapies** *Michelle Hung, PhD, Senti Biosciences*
4:45–5:00 PM	**103: Preclinical Development of Safe and Effective T-Cell Receptors Specific for Mutant KRAS G12V and G12D Peptides** *Tijana Martinov, PhD, Fred Hutchinson Cancer Research Center*
5:00–5:15 PM	**104: Disruption of H3K9me3-mediated Gene Silencing Augments CAR T-cell Functional Persistence** *Nayan Jain, Memorial Sloan Kettering Cancer Center*
5:15–5:30 PM	**105: Mechanisms Regulating the Resistance of Normal T-Cells to CD5 CAR-mediated Cytotoxicity** *Royce Ma, Baylor College of Medicine*

SCHEDULE (ALL TIMES LISTED IN ET)

MONDAY, MAY 16, 2022

ORAL ABSTRACT SESSIONS

ROOM 202
Hematopoietic Stem Cell Gene Therapy
Co-chairs: Bernhard Gentner, MD, SR-TIGET and Anne Galy, PhD, GENETHON

3:45–4:00 PM	**106: Inhibition of P38-MAPK Counteracts Culture Stress Induced by e *x vivo* Expansion of Hematopoietic Stem and Progenitor Cells (HSPCs) for Efficient Genetic Engineering** *Lucrezia Della Volpe, PhD, San Raffaele Telethon Institute for Gene Therapy*
4:00–4:15 PM	**107: Targeting *CX3CR1* Gene to Improve Microglia Reconstitution and Transgene Delivery into the CNS Upon Hematopoietic Stem and Progenitor Cell Transplant** *Annita Montepeloso PhD, Dana-Farber/ Boston Children's Cancer and Blood Disorders*
4:15–4:30 PM	**108: *Ex vivo* Lentiviral-mediated Gene Therapy for Patients with Fanconi Anemia [Group A]: Updated Results From Global RP-L102 Clinical Trials** *A Czechowicz, MD, PhD, Stanford University School of Medicine and Lucile Packard Children's Hospital*
4:30–4:45 PM	**109: Preclinical Evidences Towards Lentiviral Gene Therapy for *RPS19*-Diamond Blackfan Anemia Patients** *Susana Navarro, PhD, CIEMAT/CIBERER/IIS-FJD*
4:45–5:00 PM	**110: A Mouse Model of Severe Alpha-Thalassemia With Abnormal Iron Metabolism, Erythropoiesis and Coagulation can be Rescued by a Novel Gene Therapy Approach** *Maxwell Chappell, University of Pennsylvania*
5:00–5:15 PM	**111: Treating Sickle Cell Disease With Lentiviral Vectors Combining an Anti-Sickling β^{AS3}-globin Gene with *BCL11A* and *ZNF410* MicroRNA Adapted Short Hairpin RNAs** *Kevyn Hart, University of California, Los Angeles*
5:15–5:30 PM	**112: Transplantation Without Myeloablation: Novel Conditioning Enables Robust Repopulation of Macrophage/ microglia Niches by Bone Marrow-derived Cells** *Natalia Gomez-Ospina, MD, PhD, Stanford*

SCHEDULE (ALL TIMES LISTED IN ET)

MONDAY, MAY 16, 2022

ORAL ABSTRACT SESSIONS

ROOM 201
Vector Manufacturing and Engineering II: Next Generation Methods
Co-chairs: Kerry Dooriss, PhD, City of Hope
and Johannes Van Der Loo, PhD, Children's Hospital of Philadelphia

3:45–4:00 PM	**113: Stable BaEVRless Producer Cell Line for the Production and In Vivo Application of Alpha-Retroviral Particles** *Denise Klatt, PhD, Dana Farber Cancer Institute, Harvard Medical School*
4:00–4:15 PM	**114: Generation and Characterization of Rep Expressing AAV Packaging Cell Lines** *Lovro Jalšić, Université Laval*
4:15–4:30 PM	**115: Synthetic Biology Approach to Nucleic Acid Clearance in Lentiviral Vector Production** *Sadfer Ali, EngD, University College London*
4:30–4:45 PM	**116: Modification and Optimization of an AAV Purification Process to Accommodate Increased Upstream Yield and Reduce Manufacturing Bottlenecks** *Nick DiGioia, LogicBio Therapeutics Inc*
4:45–5:00 PM	**117: Adenovirus Purification Method Using Scalable System With Single Use Anion Exchange Fiber Chromatography Capsule** *Masato Yamamoto, MD, PhD, University of Minnesota*
5:00–5:15 PM	**118: Membrane-Associated Accessory Protein Variants Improve Adeno-Associated Virus Production in HEK293 Cells** *Adam Schieferecke, MS, University of California, Berkeley*
5:15–5:30 PM	**119: AAV Manufacturing with Stable Helper-virus Free ELEVECTA® Producer Cells for Industrial Scale Vector Production** *Ines do Carmo Gil Goncalves, PhD, CEVEC Pharmaceuticals GmbH*

SCHEDULE (ALL TIMES LISTED IN ET)

MONDAY, MAY 16, 2022

INDUSTRY SPONSORED SYMPOSIA

BALLROOM B

Pall Corporation | Successful Gene Therapy Scale-up, Start-up and Facility Expansion

5:30–7:00 PM

Clive Glover, PhD
General Manager, Gene Therapy, Pall Corporation

REGENXBIO Representative
REGENXBIO

Emily Moran
VP of Vector Manufacturing, Center for Breakthrough Medicine

Avi Nandi
VP Process Development, Center for Breakthrough Medicine

BALLROOM C

Thermo Fisher Scientific-Patheon | Integrating Development and Manufacturing Services to Bring Viral Vector Products to Market

5:30–7:00 PM

Kim Watanabe, PhD
General Manager/Site Head, Translational Services, Thermo Fisher Scientific

Akanksha Nagpal, PhD
Sr Director, Bioprocess Development, Thermo Fisher Scientific

Christopher Murphy
Vice President/General Manager, Viral Vector Services, Thermo Fisher Scientific

SCHEDULE (ALL TIMES LISTED IN ET)

MONDAY, MAY 16, 2022

HALL D
Networking Reception & Poster Session I
5:30–6:30 PM

Pharma Intelligence Consulting

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Wednesday, May 18 | 8:15am - 8:45am (EST)

Presented by:

LY NGUYEN-JATKOE, Ph.D.
Executive Director, Pharma Custom Intelligence

DAN CHANCELLOR
Director, Thought Leadership and Consulting

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SCHEDULE (ALL TIMES LISTED IN ET)

TUESDAY, MAY 17, 2022

7:00 AM–6:00 PM	REGISTRATION HOURS—EAST SALON

9:45 AM–6:30 PM	EXHIBIT HALL OPEN—HALL D
9:45–10:15 AM	Exhibit Hall Coffee Social
3:15–3:45 PM	Exhibit Hall Coffee Social
5:30–6:30 PM	Exhibit Hall Networking Reception & Poster Session II

EDUCATION SESSIONS

ROOM 202
Cancer Gene Therapy
Chair: Christopher LaRocca MD

8:00–8:24 AM	**CRISPR/Cas-based Editing Approaches to Produce T-cells Resistant to Immune Suppression** *Theresa Kaeuferle, PhD, LMU Klinikum*
8:24–8:48 AM	**Oncolytic Virotherapy: Progress and Challenges of Clinical Translation** *Julia Davydova, MD, PhD, University of Minnesota*
8:48–9:12 AM	**Updates on NK Cells for Cancer Therapy** *Nathan Schloemer, MD, Children's Wisconsin/Medical College of Wisconsin*
9:12–9:36 AM	**Optimizing CAR-T Therapy for Brain Tumors** *Ryuma Tanaka, MD, Children's Wisconsin/Medical College of Wisconsin*

SCHEDULE (ALL TIMES LISTED IN ET)

TUESDAY, MAY 17, 2022

EDUCATION SESSIONS

BALLROOM C
CRISPR/Cas9 Gene Editing—Concepts to *In Vivo* Editing
Chair: Nuria Morral, PhD, Indiana University School of Medicine

8:00–8:24 AM	**TK** *TK*
8:24–8:48 AM	**CRISPR/Cas9-guided Orthotopic T-cell Receptor Replacement to Engineer Close to Physiological Antigen-specific T-cells** *Dirk Busch, MD,Institute for Medical Microbiology, Immunology, and Hygiene, Technical University of Munich*
8:48–9:12 AM	***In Vivo* CRISPR Base Editing of PCSK9 Durably Lowers Cholesterol in Primates** *Sekar Kathiresan, MD, Verve Therapeutics*
9:12–9:36 AM	**Base Editing and Prime Editing in Mouse Models** *Wen Xue, PhD, University of Massachusetts Medical School*

ROOM 207
Integrating Retroviral Vectors
Chair: John Tisdale, MD, National Institutes of Health; National Heart, Lung, and Blood Institute

8:00–8:32 AM	**Gene Modified HSCs Bring Despair and Hope** *Marina Cavazzana, MD, PhD, Hôpital Necker-Enfants Malades*
8:32–9:04 AM	**Are Globin Vectors Lacking an Insulator Safe?** *Michel Sadelain, PhD, Memorial Sloan Kettering Cancer Center*
9:04–9:36 AM	**Potential and Identified Safety Issues With Integrating Vectors** *Melissa Bonner, PhD, bluebird bio*

SCHEDULE (ALL TIMES LISTED IN ET)

TUESDAY, MAY 17, 2022

SCIENTIFIC SYMPOSIA

ROOM 102
Gene- and Cell-based Therapies for Lung and GI
Co-chairs: Maria Limberis, PhD, Spirovant Sciences Inc and Alisha Gruntman, DVM, PhD, University of Massachusetts Chan Medical School

8:00–8:25 AM	**Advances in Macrophage-based Cell Therapies: Alveolar Proteinosis as a Paradigm Disease** *Bruce Trapnell, MD, Cincinnati Children's Hospital Medical Center*
8:25–8:50 AM	**Cell- and EV-based Approaches for COVID-19 Respiratory Failure: A Success?** *Maroun Khoury, PhD, Center of Interventional Medicine for Precision and Advanced Cellular Therapy*
8:50–9:15 AM	**Development of Selective Organ Targeting (SORT) Lipid Nanoparticles (LNPs) for Lung-specific Delivery** *Daniel J. Siegwart, PhD, UT Southwestern Medical Center*
9:15–9:40 AM	**Human Mini Lungs Grown in Lab Dishes, Also in GI** *Soumita Das, PhD, UC San Diego*

SCHEDULE (ALL TIMES LISTED IN ET)

TUESDAY, MAY 17, 2022

SCIENTIFIC SYMPOSIA

BALLROOM B
Intended and Unintended Roles of Viral Vector Heterogeneity in Gene Therapy
Co-chairs: Chen Ling, PhD, Fudan University and Brian Bigger, PhD, University of Manchester

8:00–8:25 AM	**RNA Virus Genome Diversity: Mechanisms and Consequences for Therapeutic Vectors** *Roberto Cattaneo, PhD, Mayo Clinic*
8:25–8:50 AM	**DNA Virus Heterogeneity—AAV Vectors With Expansion to Other DNA Viral Vectors** *Phillip W.L. Tai, PhD, University of Massachusetts Chan Medical School*
8:50–9:15 AM	**Lentiviral Vector Heterogeneity in Clinical Applications** *Megan D. Hoban, PhD, bluebird bio*
9:15–9:40 AM	**Sizing and Counting Empty and Filled AAV Particles by High Resolution Native Mass Spectrometry and Single Molecule Charge Detection Mass Spectrometry** *Albert J.R. Heck, PhD, Utrecht University*

SCHEDULE (ALL TIMES LISTED IN ET)

TUESDAY, MAY 17, 2022

SCIENTIFIC SYMPOSIA

SALON G
Musculoskeletal Gene Therapy: Progresses and Challenges
Co-chairs: Scott Q. Harper, PhD, Nationwide Childen's Hospital and Chunping Qiao, PhD, REGENXBIO

8:00–8:25 AM — **Genome Editing and Immuno-engineering for Arthritis Therapy**
Farshid Guilak, PhD, Washington University

8:25–8:50 AM — **Immunomodulation as an Adjunctive Therapy to AAV Systemic Dosing to Improve Safety, Increase Expression and Allow for Repeated AAV Dosing**
Manuela Corti, PhD, University of Florida

8:50–9:15 AM — **Genotype-phenotype Correlations in DMD Patients and AAV-U7-SnRNA-mediated Exon Skipping for DMD**
Kevin M. Flanigan, MD, Nationwide Children's Hospital

9:15–9:40 AM — **Directed Evolution of a Family of AAV Capsid Variants Enabling Potent Muscle-directed Gene Delivery Across Species**
Sharif Tabebordbar, PhD, Kate Therapeutics

BALLROOM A
Fireside Chat | State of the Field: Emerging Regulatory Trends
Chair: Keith Wonnacott, PhD, Lexeo Therapeutics

8:00–9:45 AM — *Peter Marks, MD, PhD, Food and Drug Administration*
Ana Hidalgo-Simon, MD, PhD, European Medicines Agency

SCHEDULE (ALL TIMES LISTED IN ET)

TUESDAY, MAY 17, 2022

SCIENTIFIC SYMPOSIA

ROOM 204

Symposium on Translating Tissue Engineering and Regenerative Medicine Approaches into Therapies

Co-chairs: Steven Becker, PhD, National Institutes of Health and Lloyd F. Rose, PhD, U.S. Army Medical Materiel Development Activity/BioFabUSA

8:00–8:20 AM	**Decellularized Heart Scaffold** *Doris A. Taylor, PhD, RegenMedix Consulting*
8:20–8:40 AM	**Projects Done With the BioFabUSA Tissue Foundry** *Tom Bollenbach, PhD, Advanced Regenerative Manufacturing Institute/BioFabUSA*
8:40–9:00 AM	**Preclinical Spinal Cord Repair Research With Neural Stem Cells** *Mark Tuszynski, MD, PhD, UC San Diego*
9:00–9:45 AM	**Round Table Discussion** *Moderator: Laura Ricles, PhD, Food and Drug Administration*

SCHEDULE (ALL TIMES LISTED IN ET)

TUESDAY, MAY 17, 2022

SCIENTIFIC SYMPOSIA

ROOM 206

The Ethical Gray Zone? Perspectives on the Development and Governance of Gene and Cell Therapies for Human Enhancement

Co-chairs: Eric Juengst, PhD, UNC Chapel Hill and Benjamin Hurlbut, PhD, Arizona State University

8:00–8:20 AM	**Introduction: Defining and Differentiating Disease Versus Enhancement** *Sheila Jasanoff, PhD, JD, Harvard University*
8:20–8:40 AM	**The Case for Rethinking Health-related Enhancements in Specific Social Contexts: NASA's Space Radiation Program** *Lisa Scott-Carnell, PhD, National Aeronautics and Space Administration*
8:40–9:00 AM	**Special Ethical Considerations Relevant to Governance of Enhancement Interventions** *Peter Mills, PhD, Nuffield Council on Bioethics*
9:00–9:20 AM	**Reflections on the Treatment Versus Enhancement Debate Through the Years: Key Points and Lessons Learned for the Present** *John Evans, PhD, UC San Diego*
9:20–9:45 AM	**Panel Discussion**

SCHEDULE (ALL TIMES LISTED IN ET)

TUESDAY, MAY 17, 2022

SCIENTIFIC SYMPOSIA

ROOM 201
Therapeutic Applications of RNA Therapy Strategies
Co-chairs: Lauren E. Woodard, PhD, Vanderbilt University Medical Center and Loree Heller, PhD, University of South Florida

8:00–8:25 AM	***In Vivo* Cellular Reprogramming by Targeted mRNA-LNP** *Hamideh Parhiz, PharmD, PhD, University of Pennsylvania*
8:25–8:50 AM	**Strategies for Developing mRNA-based Therapeutics for Rare Diseases** *Lisa Rice, PhD, Moderna Therapeutics*
8:50–9:15 AM	**Exosomes in Nucleic Acid Delivery** *Susmita Sahoo, PhD, Icahn School of Medicine*
9:15–9:40 AM	**Ultrasound-mediated Gene Delivery (UMGD)** *Costas Arvanitis, PhD, Georgia Institute of Technology*

SALON H
Late Breaking Oral Abstract Sessions
8:15 AM–9:45 PM

Due to the late-breaking nature of this session, individual presentations were not yet selected at time of publish.

See annualmeeting.asgct.org/program for more information

SCHEDULE (ALL TIMES LISTED IN ET)

TUESDAY, MAY 17, 2022

EXHIBITOR SHOWCASES

ROOM 209

Industry Interactions

Bio-Rad Laboratories | Droplet Digital PCR for Advancing Quality Manufacturing of Gene and Cell Therapies

8:15–8:45 AM — **Presenter(s) to be Announced**

ROOM 209

Industry Interactions

Catalent Cell & Gene Therapy | AAV Platform Process Accelerating Production from Gene to Clinic

9:15–9:45 AM — **George Buchman, PhD**
VP, Pre-clinical and Process Development, Catalent Cell & Gene Therapy

SCHEDULE (ALL TIMES LISTED IN ET)

TUESDAY, MAY 17, 2022

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KEYNOTE SESSIONS

HALL E
George Stamatoyannopoulos Memorial Lecture and Award Presentation

10:15–10:35 AM	ASGCT Update from President Beverly Davidson, PhD and CEO David Barrett, JD
10:35–10:55 AM	Presentation of the Career Development Awards and Diversity & Inclusion Awards
10:55–11:05 AM	Presentation of the Sonia Skarlatos Public Service Award to P.J. Brooks, PhD
11:05–11:15 AM	Presentation of the Jerry Mendell Award for Translational Science to Katherine High, MD
11:15 AM–12:00 PM	Keynote Address from Drew Weissman, PhD

SCHEDULE (ALL TIMES LISTED IN ET)

TUESDAY, MAY 17, 2022

INDUSTRY SPONSORED SYMPOSIA

BALLROOM C

Corning Life Sciences | Leveraging a Novel Adherent Cell Culture Technology for Highly Efficient and Intensified Cell and Gene Therapy Manufacturing

12:00–1:30 PM

Zara Melkoumian, PhD
Business Technology Director, Corning Life Sciences

Hannah Rasby
Sr. Process Development Engineer, Viacyte

Todd Upton, PhD
Innovation Portfolio Sr. Manager, Corning Life Sciences

BALLROOM B

Cytiva | Cells to Purified Capsids: How to Develop a Scalable rAAV Process

12:00–1:30 PM

Åsa Hagner McWhirter
Principal Scientist, R&D, Cytiva

ROOM 207

Taysha Gene Therapies | Accepting the Challenge: Innovative Approaches and Translational Strategies in Gene Therapy Development

12:00–1:30 PM

Suyash Prasad, MBBS, MRCP, MRCPCH, FFPM
Chief Medical Officer, Head of Research and Development, Taysha Gene Therapies

Steven Gray, PhD
Chief Scientific Advisor and Associate Professor, UTSW Gene Therapy Program, University of Texas Southwestern

Kimberly Goodspeed, MD
Assistant Professor, Department of Pediatrics, University of Texas Southwestern

Jagdeep S. Walia, MBBS, FRCPC, FCCMG
Full Professor, Director of Research (Pediatrics), Queen's University, Canada

SCHEDULE (ALL TIMES LISTED IN ET)

TUESDAY, MAY 17, 2022

INDUSTRY SPONSORED SYMPOSIA

ROOM 202

Vertex Pharmaceuticals, Inc. | Breaking Barriers: Managing the Immune Response to Cell and Gene Therapies

12:00–1:30 PM

John F. DiPersio, MD, PhD
Director, Center for Gene and Cellular Immunotherapy, Washington University School of Medicine

Leslie S. Kean, MD, PhD
Director, Pediatric Stem Cell Transplant Program, Dana-Farber Cancer Institute

Barry J. Byrne, MD, PhD
Director, Powell Gene Therapy Center, University of Florida

Mike Cooke
Senior Vice President, Cell and Genetic Therapy Research Team, Vertex Pharmaceuticals Inc.

EXHIBITOR SHOWCASES

ROOM 209

Industry Interactions

Bio-Techne | Characterization of Gene Therapy Biodistribution and Function - From AAV to ASO

12:00–12:30 PM

Michaeline Bunting, PhD
Director, Spatial Biology Division, Bio-Techne

ROOM 209

Industry Interactions

Precision for Medicine | Key Considerations for Design and Implementation of NAb Bioassays in Gene Therapy Development

1:00–1:30 PM

Travis Harrison, PhD
Vice President, Bioassay Solutions, Precision for Medicine

SCHEDULE (ALL TIMES LISTED IN ET)

TUESDAY, MAY 17, 2022

SPONSORED BY:

Spark THERAPEUTICS

HALL E | 1:30–3:00 PM
Outstanding New Investigator Symposium

SOUTH PRE-FUNCTION
Networking Event—Career Fair
3:00–5:00 PM

Connect with employers and get a complimentary professional headshot!
Make sure you are **registered for the Annual Meeting** to attend the Career Fair.

SCHEDULE (ALL TIMES LISTED IN ET)

TUESDAY, MAY 17, 2022

TOOLS AND TECHNOLOGY FORUM II

ROOM 209
Industry Interactions
Co-chairs: Blythe Sather, PhD and Le Cong, PhD, Stanford University

3:30–3:45 PM	**PROGEN | AAV Lateral Flow Test for Rapid Titer Determination** *Dana Holzinger, Head of Product Management, PROGEN*
3:45–4:00 PM	**Refeyn | SamuxMP - A Mass Photometer for Rapid AAV Analytics** *Gareth Rogers, Product Manager, Refeyn*
4:00–4:15 PM	**MYRIADE | VIDEODROP: Ideal Tool for Lentiviral Vector Bioproduction Follow-up** *Marie Berger, PharmD, Application Engineer, MYRIADE*
4:15–4:30 PM	**SIRION Biotech | Taking Gene Delivery to the Single Cell Level** *Christian Thirion, PhD, CEO and Founder, SIRION Biotech*
4:30–4:45 PM	**Curiox Biosystems**
4:45–5:00 PM	**Wyatt Technology | Biophysical Characterization and Quality Control of Vaccines and Gene Vectors With Light Scattering** *William Penny, PhD,Regional Account Manager & Application Scientist, Wyatt Technology*
5:00–5:15 PM	**Mission Bio | Harnessing Single-cell Multi-omics to Advance Cell and Gene Therapy Research** *Yue Wang, PhD, Business Development Manager, Cell and Gene Therapy, Mission Bio*
5:15–5:30 PM	**Synthego | Industrialized CRISPR: Accelerating Disease Research** *Peter Deng, PhD, Associate Product Manager, On-Market Strategy, Synthego*

SCHEDULE (ALL TIMES LISTED IN ET)

TUESDAY, MAY 17, 2022

TOOLS AND TECHNOLOGY FORUM II

5:30–5:45 PM	**PackGene Biotech INC | π-AlphaTM 293 AAV High-yield Production Technology to Improve Scalability and Biosafety** *Baker Lu, PhD, Business Development Director, North America, PackGene Biotech INC*
5:45–6:00 PM	**Unchained Labs | Hit the Gas Pedal on AAV Analysis and Prep with Uncle, Stunner and Big Tuna** *Kevin Lance, PhD, Director of Analytics Marketing, Unchained Labs*

SCHEDULE (ALL TIMES LISTED IN ET)

TUESDAY, MAY 17, 2022

ORAL ABSTRACT SESSIONS

BALLROOM A
Discoveries in Fundamental AAV Biology
Co-chairs: Nicole Paulk, PhD, UCSF and Anna Maurer, PhD, UC Berkeley

3:45–4:00 PM	**433: Structural Characterization of Patient-derived Anti-AAV9 Monoclonal Antibodies Generated Post-Zolgensma Treatment** *Mario Mietzsch, PhD, University of Florida*
4:00–4:15 PM	**434: Characterization of Alternative Reading Frame Proteins Generated from AAV Cassettes** *Ferzin Sethna, Ph.D, Asklepios Biopharmaceuticals*
4:15–4:30 PM	**435: Transcription, Translation, and Immunogenic Potential of P5-Associated rAAV Contaminants Post-infection** *Mark Brimble, PhD, St Jude Children's Research Hospital*
4:30–4:45 PM	**436: Unlocking Avian AAV Transduction in Mammalian Cells and Tissues for Immune Evasion and Redosing** *Ezra Loeb, Duke University*
4:45–5:00 PM	**437: Genome Packaging Efficiencies of Anc80 and AAV9 Vectors using Non-canonical Rep-ITR Combinations** *Anusha Sairavi, Oregon Health and Science University*
5:00–5:15 PM	**438: AAV Capsid Dynamics at the 5-fold Pore Controls Genome Release** *Joshua Hull, BS, University of Florida*
5:15–5:30 PM	**439: Furin is a Host Factor Restricting Adeno-associated Virus 4 Transduction** *Timothy Smith, Duke University*

SCHEDULE (ALL TIMES LISTED IN ET)

TUESDAY, MAY 17, 2022

ORAL ABSTRACT SESSIONS

SALON G
Enhanced AAV Targeting
Chair: Ana Rita Batista, PhD, UMass Chan Medical School

3:45–4:00 PM	**440: Effects of an Early Gene Therapy Targeting Oligodendrocytes in a Mouse Model of Adrenomyeloneuropathy** *Yasemin Gunes, UMR 1195 Inserm and University Paris Saclay*
4:00–4:15 PM	**441: Engineered AAV Capsids Exhibit Improved Transduction of the Central Nervous System after CSF Administration in Adult Cynomolgus Macaques** *David Ojala, Sangamo Therapeutics, Inc.*
4:15–4:30 PM	**442: A Direct Comparison of Five AAV Capsids for Intramuscular Inoculation in Non-human Primates** *Matthew Gardner, PhD, Emory University*
4:30–4:45 PM	**443: Systemic Administration of Novel Engineered AAV Capsids Facilitates Enhanced Transgene Expression in the Macaque Central Nervous System** *Alexandra Stanton, Broad Institute*
4:45–5:00 PM	**444: Astrocyte-restricted Gene Silencing Improves the Safety of AAV9-Mediated Gene Therapy for Alexander Disease** *Wassamon Boonying, PhD, University of Massachusetts Chan Medical School*
5:00–5:15 PM	**445: Positron Emission Tomography I-124-labeled AAV Assessment of CSF to Blood Diffusion and Consequent Systemic Distribution of AAV Capsids Following CSF Administration of AAV Vectors** *Jonathan Rosenberg, MD, Weill Cornell Medicine*
5:15–5:30 PM	**446: Identification of New AAV9 Engineered Capsids Targeting Mouse and Monkey Brains Through a Directed Evolution Approach in BALB/c Mice** *Giannelli Serena, PhD, Fondazione Centro San Raffaele*

SCHEDULE (ALL TIMES LISTED IN ET)

TUESDAY, MAY 17, 2022

ORAL ABSTRACT SESSIONS

HALL E
Gene Editing in Cancer and Complex Diseases
Co-chairs: Angelo Lombardo, PhD, SR-TIGET and Mara Pavel-Dinu, PhD, Stanford University

3:45–4:00 PM	**447: Targeting the Hepatitis BcccDNA with a Sequence-Specific ARCUS Nuclease to Eliminate Hepatitis B Virus *In Vivo*** *Cassandra Gorsuch, PhD, Precision BioSciences*
4:00–4:15 PM	**448: A Novel DNA Oligo-based Repair Strategy for the Functional Correction of Shwachman-Diamond Syndrome** *CY Zhang, PhD, Dana-Farber Cancer Institute*
4:15–4:30 PM	**449: Liver Gene-editing Based on Nickase Cas9 for the Treatment of Primary Hyperoxaluria Type I (PH1) is More Efficient when Using an All-in-one Delivery System** *Laura Torella, Center for Applied Medical Research (CIMA), University of Navarra*
4:30–4:45 PM	**450: Enhanced CRISPR/Cas9 Genome Editing in Heart and Skeletal Muscle with a Potent New AAV Variant** *Trevor Gonzalez, Duke University*
4:45–5:00 PM	**451: A MiniCEP290 Gene Replacement Therapy to Treat CEP 290-Leber Congenital Amourosis (LCA10)** *Bhubanananda Sahu, PhD, Iveric Bio*
5:00–5:15 PM	**452: A Novel Polyfunctional Editing Strategy for Adoptive T-cell Immunotherapy of Cancer** *Tania Baccega, IRCCS San Raffaele Scientific Institute*
5:15–5:30 PM	**453: Generation of Efficient Lipid Nanoparticles for Liver-Directed Gene Therapy and Genome Editing** *Claude Warzecha, PhD, University of Pennsylvania*

SCHEDULE (ALL TIMES LISTED IN ET)

TUESDAY, MAY 17, 2022

ORAL ABSTRACT SESSIONS

SALON H
Physical Methods and Extracellular Vesicle-Based Gene Transfer
Chair: Richard Heller, PhD, University of South Florida

3:45–4:00 PM	**454: DNA Sensor Palmitoylation in Mouse Skeletal Muscle Following DNA Electroporation** *Amanda Sales Conniff, PhD, University of South Florida*
4:00–4:15 PM	**455: Novel Non-thermal Plasma Based Delivery of Plasmid DNA** *Pavan Cherukuri, PhD, University of South Florida*
4:15–4:30 PM	**456: Electrotransfer Combined with Moderate Heat and Impedance Monitoring to Enhance Delivery of Agents to Multiple Tissues** *Richard Heller, PhD, University of South Florida*
4:30–4:45 PM	**457: Ultrasound Mediated Gene Delivery Specifically Targets Liver Sinusoidal Endothelial Cells for Sustained FVIII Expression in Hemophilia A Mice** *Savannah Lawton, Seattle Children's Research Institute*
4:45–5:00 PM	**458: Assessment of Commensal E. coli Outer Membrane Vesicles for Application in a Novel Oral Delivery System** *Kari Heck, University of Nebraska-Lincoln*
5:00–5:15 PM	**459: Extracellular Vesicle-mediated Therapeutic Delivery of RIG-I Agonists for Immunotherapy Against Breast Cancer** *Minh T.N. Le, PhD, Department of Pharmacology, Yong Loo Lin School of Medicine, National University of Singapore*
5:15–5:30 PM	**460: Engineering Cells to Produce miRNA-loaded Exosomes for Potential Biotherapeutics** *Andrew Hamann, PhD, University of Nebraska-Lincoln*

SCHEDULE (ALL TIMES LISTED IN ET)

TUESDAY, MAY 17, 2022

ORAL ABSTRACT SESSIONS

ROOM 201
Inborn Errors of Metabolism Gene and Cell Therapies: Proof-of-Concepts and Beyond
Co-chairs: Gerald Lipshutz, MD, David Geffen School of Medicine at UCLA and Stephanie Cherqui, PhD, UC San Diego

3:45–4:00 PM	**461: Safety and Efficacy of a Dual-function AAV9 *BCKDHA-BCKDHB* Gene Replacement Vector in Murine and Bovine Models of Classic Maple Syrup Urine Disease** *Kevin Strauss, MD, Clinic for Special Children*
4:00–4:15 PM	**462: Homology Independent Targeted Integration Leads to Highly Efficient Protein Expression and Secretion From Liver** *Federica Esposito, TIGEM*
4:15–4:30 PM	**463: Safety and Efficacy of DTX301 in Adults With Late-Onset Ornithine Transcarbamylase (OTC) Deficiency: A Phase 1/2 Trial** *Cary Harding, MD, Oregon Health & Science University*
4:30–4:45 PM	**464: Genome Editing in a Canine Model for Glycogen Storage Disease Type Ia** *Benjamin Arnson, Duke University School of Medicine*
4:45–5:00 PM	**465: Liver Directed Lentiviral Gene Therapy Ameliorates the Phenotype of Progressive Familiar Intrahepatic Cholestasis Type 2 in a Mouse Model** *Elena Barbon, PhD, San Raffaele Telethon Institute for Gene Therapy, IRCCS San Raffaele Scientific Institute*
5:00–5:15 PM	**466: Nuclease Enhancement of AAV Mediated Editing into Albumin in Neonatal Mice with Methylmalonic Acidemia (MMA)** *Leah Venturoni, PhD, NHGRI*
5:15–5:30 PM	**467: Updated Interim Results of Transpher A, a Multicenter, Single-Dose, Pivotal Clinical Trial of ABO-102 Gene Therapy for Sanfilippo Syndrome Type A (Mucopolysaccharidosis IIIA)** *Kevin M. Flanigan, MD, Nationwide Children's Hospital*

SCHEDULE (ALL TIMES LISTED IN ET)

TUESDAY, MAY 17, 2022

ORAL ABSTRACT SESSIONS

ROOM 204

Applications of Improved Gene Therapy Methods in Neurologic Disorders

Co-chairs: Kathrin Meyer, PhD, Nationwide Children's Hospital and Steven Gray, PhD, UT Southwestern

3:45–4:00 PM	**468: Intrathalamic Delivery of AVB.PGRN Rescues Pathology in *GRN* Null Mice and Achieves Widespread Cortical Expression in a Large Animal Model Without Expression in the Liver** *Christopher Shaw, MD, Kings College London*
4:00–4:15 PM	**469: AAV-based GDNF Expression in VTA Prevents Relapse to Alcohol-Drinking Behavior and Modifies Mesolimbic Dopamine Function in Rhesus Macaques: A Gene Therapy Approach to Treating Alcohol Use Disorder** *Victor Van Laar, PhD, The Ohio State University*
4:15–4:30 PM	**470: Vectorized Delivery of Tau Reduction Therapy as a Treatment Approach for Tauopathies** *Rachel Bailey, PhD, UT Southwestern Medical Center*
4:30–4:45 PM	**471: CAP-001: Systemic AAV Gene Therapy With Next Generation Capsids for MPS II Disease** *Nicholas Flytzanis, PhD, Capsida Biotherapeutics, Inc.*
4:45–5:00 PM	**472: Durable and Specific Rescue of UBE3A Expression in the Brain of an Angelman Syndrome Mouse Model Using an Artificial Transcription Factor** *Henriette O'Geen, PhD, UC Davis*
5:00–5:15 PM	**473: Gene Therapy Mediated Cross Correction for CDKL5 Deficiency Disorder** *Heather Born, PhD, University of Pennsylvania*
5:15–5:30 PM	**474: Safe And Efficacious Rescue Of GM3 Synthase Deficiency Mice By Spatially Regulated Gene Delivery** *Huiya Yang, UMass Chan Medical School*

SCHEDULE (ALL TIMES LISTED IN ET)

TUESDAY, MAY 17, 2022

ORAL ABSTRACT SESSIONS

ROOM 102
Enhancing CAR T-cell Efficacy
Co-chairs: Craig Sauter, MD, MSKCC
and Barbara Savoldo, MD, PhD, UNC Lineberger Comprehensive Cancer Center

3:45–4:00 PM	**475: Leucine Zipper-based Cytokine Receptors Augments CAR T-cell Immunotherapy for Solid Tumors** *Matthew Bell, St. Jude Children's Research Hospital*
4:00–4:15 PM	**476: Epigenome Editing Enables PD1 Silencing in CAR T-cells** *Maria Silvia Roman Azcona, PhD student, Institute for Transfusion Medicine and Gene Therapy*
4:15–4:30 PM	**477: TNFR2 as a Target to Improve CD19-Directed CAR T-cell Fitness and Antitumor Activity in Large B-cell Lymphoma** *Claudia Manriquez Roman, M.Sc., Mayo Clinic*
4:30–4:45 PM	**478: A High Expression of IL15 Receptor Alpha (IL15Rα) in Glioblastoma Microenvironment Enables IL15-armored CAR T-cells to Modulate Tumor Immunosuppression and Improve Survival in Syngeneic Models** *Irina V. Balyasnikova, PhD, Northwestern University Feinberg School of Medicine*
4:45–5:00 PM	**480: Allogeneic Donor-derived CD19-Chimeric Antigen Receptor (CAR) T-cells for Relapsed B-cell Malignancies After Hematopoietic Stem Cell Transplantation** *Ibrahim Muhsen, MD, Houston Methodist Hospital*
5:00–5:15 PM	**481: High-Affinity PD1-CD28 Chimeric Switch Receptors Enhance Costimulatory Signaling and Improve TCR and CAR T-cell Antitumor Activity** *Brooke Prinzing, PhD, St. Jude Children's Research Hospital*

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TUESDAY, MAY 17, 2022

ORAL ABSTRACT SESSIONS

ROOM 202
Gene Therapy for Immunologic Diseases
Co-chairs: Donald Kohn, MD, UCLA
and Claire Booth, MBBS, PhD, UCL Great Ormond Street Institute of Child Health

3:45–4:00 PM	**482: A Novel Engineered CRISPR-Associated Nuclease Accurately Removes ELANE Mutated Allele and Shifts HSC Differentiation Towards Neutrophils in Severe Congenital Neutropenia** *Rafi Emmanuel, PhD, Emendo Biotherapeutics*
4:00–4:15 PM	**483: Editing T-cell Repertoire by Thymic Epithelial Cell-Directed Gene Transfer Abrogates Risk of Type 1 Diabetes Development** *Andrea Annoni, PhD, San Raffaele Telethon Institute for Gene Therapy*
4:15–4:30 PM	**484: Therapeutic Gene Editing of T-cells Corrects CTLA4 Insufficiency** *Thomas Fox, MD, UCL*
4:30–4:45 PM	**485: A Simultaneous Knock-out Knock-in Gene Editing Strategy in HSPCs Potently Inhibits R5- and X4-tropic HIV Replication** *Amanda Dudek, PhD, Stanford University School of Medicine*
4:45–5:00 PM	**486: Transcriptional Mapping of Human Hematopoietic Stem and Progenitor Cells discriminates Chronic Granulomatous Disease Patients Able to Benefit From Gene Therapy Treatment** *Steicy Sobrino, Imagine Institute*
5:00–5:15 PM	**487: Base Editing of Hematopoietic Stem Cells Rescues T-cell Development for CD3d Severe Combined Immunodeficiency** *Grace McAuley, UCLA*
5:15–5:30 PM	**488: Chemotherapy-free Engraftment of Gene Edited Human Hematopoietic Stem Cells Leveraged on Mobilization and mRNA-based Engineering** *Attya Omer Javed, IRCCS San Raffaele Scientific Institute*

SCHEDULE (ALL TIMES LISTED IN ET)

TUESDAY, MAY 17, 2022

ORAL ABSTRACT SESSIONS

ROOM 206
Immune Responses to Gene Delivery and Vaccine Approaches
Co-chairs: Matthew Gardner, PhD, Emory University and Manish Muhuri, PhD, Biogen

3:45–4:00 PM	**489: Loss of AAV-FVIII Gene Therapy in Hemophilia A Mice Due to Shutdown of Hepatic Protein Production Rather Than Loss of Vector or Transduced Cells** *Kentaro Yamada, PhD, Indiana University School of Medicine*
4:00–4:15 PM	**490: Novel Early Checkpoint Modifier Demonstrates Broadened and Enhanced CD8+ T-cell Responses Across Multiple Preclinical Studies** *Hildegund Ertl, The Wistar Institute*
4:15–4:30 PM	**491: Mucosal Chemokine CCL27 Adjuvant Uniquely Improves Mucosal Responses to SARS-CoV-2 synDNA Antigens Providing Heterologous Protection Against Delta Variant Challenge** *Ebony Gary, PhD, The Wistar Institute*
4:30–4:45 PM	**492: An Intranasal saRNA/NLC Vaccine Induces Robust Mucosal and Systemic Immunity to SARS-CoV-2 in Mice** *Emily Voigt, PhD, Infectious Disease Research Institute*
4:45–5:00 PM	**493: AAV-mediated Expression of Monoclonal Antibodies Provides Protection in a Mouse Model of Marburg Virus Infection and Long-term Expression in an Ovine Model** *Amira Rghei, BS, University of Guelph*
5:00–5:15 PM	**494: Adenovirus Capsid Proteins-based Anti-fentanyl Vaccine Attenuates Fentanyl-induced Behaviors in Mice** *Bishnu De, Weill Cornell Medicine*
5:15–5:30 PM	**495: Pre-existing Maternal Humoral Immunity to Adeno-Associated Virus Impairs Fetal Gene Editing in a Serotype-specific Fashion** *John Riley, Children's Hospital of Philadelphia*

SCHEDULE (ALL TIMES LISTED IN ET)

TUESDAY, MAY 17, 2022

INDUSTRY SPONSORED SYMPOSIA

BALLROOM C

Anemocyte | Expert Talk—My Letter to Santa: Wishes and Reality Impacting Cell and Gene Therapies

5:30–7:00 PM

Federico Mingozzi, PhD, MBA
Chief Scientific Officer; Spark Therapeutics

Julianne Smith, PhD
Chief Development Officer, GeneSpire

Nathalie Belmonte, PhD
SVP Research & Translation, Quell Therapeutics

Jim Faulkner, PhD
Chief Technical Officer, Ascidian Therapeutics

Miguel Forte, MD, PhD
CEO, Bone Therapeutics, President-Elect ISCT, International Society for Cell & Gene Therapy

Luigi Naldini, MD, PhD
Co-founder, Genenta Science; Director of SR-Tiget, San Raffaele Telethon Institute for Gene Therapy

ROOM 207

Charles River Laboratories | Thinking With the End in Mind: How to Accelerate C&GT Research and Development

5:30–7:00 PM

Sam Chuang, PhD
Director, Scientific Advisory Services, Charles River Laboratories

Matt Hewitt
Executive Director, Scientific Services Cell & Gene Therapy, Charles River Laboratories

Steven Miklasz
Strategic Key Account & Technical Development Director, Charles River Laboratories

Dimpi Patel
Senior Director Global Cell & Gene Therapy Compliance, Charles River Laboratories

SCHEDULE (ALL TIMES LISTED IN ET)

TUESDAY, MAY 17, 2022

INDUSTRY SPONSORED SYMPOSIA

BALLROOM B
QIAGEN LLC | Industry Sponsored Symposium

5:30–7:00 PM

Miriam Menezes
Senior Scientist - Molecular Biology/Automation, Spark Therapeutics

John Kerwin
Technical Head, Gene Therapy Franchise, National Resilience

Dana Cipriano
VP of Testing and Analytical Services, The Center for Breakthrough Medicines

Mandy Conver
Associate Director, dPCR Commercial Operartions, QIAGEN LLC

A'Drian Pineda
Sr. Business Development Manager, Biopharma, QIAGEN LLC

Victoria Best
Director, Analytical Sciences, AmplifyBio

SCHEDULE (ALL TIMES LISTED IN ET)

TUESDAY, MAY 17, 2022

HALL D
Networking Reception & Poster Session II
5:30–6:30 PM

ROOM 103
Networking Event—New Member Meetup
6:00–8:00 PM

The New Member Mixer welcomes attendees who have joined ASGCT over the past three years. Usually reserved for members joining in the past 12 months, we've expanded the mixer to include those who have joined since we were last in-person in 2019. This informal setting will introduce new members to the Society and highlight the many ways they can use our resources as their professional hub. Attendees will also hear from a longtime member who has adeptly used their Society membership throughout their career.

SCHEDULE (ALL TIMES LISTED IN ET)

WEDNESDAY, MAY 18, 2022

7:00 AM–6:00 PM	REGISTRATION HOURS—EAST SALON

9:45 AM–6:30 PM	EXHIBIT HALL OPEN—HALL D
9:45–10:15 AM	Exhibit Hall Coffee Social
3:15–3:45 PM	Exhibit Hall Coffee Social
5:30–6:30 PM	Exhibit Hall Networking Reception & Poster Session III

EDUCATION SESSIONS

BALLROOM B
Gene Therapy Beyond Cancer
Chair: Satiro Nakamura de Oliveira, MD, UCLA

8:00–8:24 AM	**Gene Therapies for Inherited Bleeding Disorders** *David A. Wilcox, PhD, Medical College of Wisconsin; Children's Research Institute-Children's Wisconsin; Versiti Blood Research Institute*
8:24–8:48 AM	**Gene Therapies in Hemoglobinopathies** *Daniel Bauer, MD, PhD, Boston Children's Hospital*
8:48–9:12 AM	**Gene Therapies for Liver Disease** *Gloria Gonzalez-Aseguinolaza, PhD, CIMA-University of Navarra, Vivet Therapeutics*
9:12–9:36 AM	**Genetically Engineered CAAR T-cell Therapies for B Cell-mediated Autoimmune Diseases** *Aimee S. Payne, MD, PhD, University of Pennsylvania*

SCHEDULE (ALL TIMES LISTED IN ET)

WEDNESDAY, MAY 18, 2022

EDUCATION SESSIONS

ROOM 204
Pre-clinical Models
Co-Chair: Erik Barton, PhD, Pfizer, and Hildegard Buning, PhD, Hannover Medical School

8:00–8:24 AM	**Small Animal Models for Gene Therapy Research** *Karl-Dimiter Bissig, MD, PhD, Duke University*
8:24–8:48 AM	**Immuno-oncology Humanized Mice and PDX** *Saar Gill, MD, PhD, University of Pennsylvania*
8:48–9:12 AM	**Immune Competent Pet Dogs with Spontaneous Disease as Pre-clinical Models for Cellular Therapy** *Nicola Mason, BVetMed, PhD, University of Pennsylvania*
9:12–9:36 AM	**FDA/CBER Perspective for *In Vivo* Preclinical Testing of Cell and Gene Therapy Products** *Abigail L. Shearin, VMD, PhD, OTAT, Food and Drug Administration*

SCHEDULE (ALL TIMES LISTED IN ET)

WEDNESDAY, MAY 18, 2022

SCIENTIFIC SYMPOSIUM

ROOM 206
Combating Misinformation in Science
Co-chairs: Karen Bulaklak, PhD, Sarepta Therapeutics and Edith Pfister, PhD, UMASS Chan Medical School

8:00–8:24 AM	**Misinformation in and About Science** *Jevin West, PhD, University of Washington Center f or an Informed Public*
8:24–8:48 AM	**California's Initiatives to Advance Precision Medicine** *Julianne McCall, PhD, California Governor's Office of Planning and Research*
8:48–9:12 AM	**Breaking Down Gene Therapy and CRISPR Topics for Non-scientific Audiences** *Jennifer E. Adair, PhD, Fred Hutchinson Cancer Research Center*

SCHEDULE (ALL TIMES LISTED IN ET)

WEDNESDAY, MAY 18, 2022

SCIENTIFIC SYMPOSIUM

ROOM 102

Challenges and Status in Development of Gene Therapies in Low- and Middle-income Countries (LMICs)

Co-chairs: Richard Koya, MD, PhD, University of Chicago School of Medicine and Savita Rangarajan, MD, University Hospital Southampton

8:00–8:20 AM	**Investigator Perspective on Improving Transportability** *Jennifer E. Adair, PhD, Fred Hutchinson Cancer Research Center*
8:20–8:40 AM	**Bringing Safe, Effective, and Accessible Gene Therapies for HIV and Sickle Cell Disease to LMICs** *Mike McCune, MD, PhD, UCSF*
8:40–9:00 AM	**Challenges of Initiating Trials in LMICs** *Cissy Kityo, MD, Joint Clinical Research Center, Uganda*
9:00–9:20 AM	**Manufacturing: Barriers and Solutions to LMICs** *Kollengode V. Subramanian, Reliance Life Sciences, India*
9:20–9:45 AM	**Panel Discussion**

SCHEDULE (ALL TIMES LISTED IN ET)

WEDNESDAY, MAY 18, 2022

SCIENTIFIC SYMPOSIUM

BALLROOM A
Gene Editing: New Technology Advances
Co-chairs: Thomas J. Cradick, PhD, Excision BioTherapeutics and Paula Rio, PhD, Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas (CIEMAT)

8:00–8:25 AM	**Prime Editing: Precision Gene Editing Without Double-strand DNA Breaks** *Peter Chen, MD, PhD, Harvard University*
8:25–8:50 AM	**Heritable Control of Gene Expression by CRISPR Epigenome Editing** *James K. Nuñez, PhD, UC Berkeley*
8:50–9:15 AM	**Compact CRISPR Proteins for Genome Editing** *Pei-Qi Liu, Mammoth Biosciences*
9:15–9:40 AM	**Programmable Gene Insertion (PASTE)** *Omar Abudayyeh, PhD, MIT* *Jonathan Gootenberg, PhD, MIT*

SCHEDULE (ALL TIMES LISTED IN ET)

WEDNESDAY, MAY 18, 2022

SCIENTIFIC SYMPOSIUM

SALON G
Inborn Metabolic Issues
Co-chairs: Anne Galy, PhD, Genethon and Alessandro Aiuti, MD, PhD, San Raffaele Telethon Institute

8:00–8:26 AM	**Clinical Trial Results of Hematopoietic Stem Cell Gene Therapy for Mucopolysaccharidosis Type I Hurler** *Maria Ester-Bernardo, MD, PhD, San Raffaele Telethon Institute*
8:26–8:52 AM	**Pre-clinical Studies of Gene Therapy for Inherited Liver Disorders** *Gloria Gonzalez-Aseguinolaza, MD, PhD, CIMA, University of Navarra*
8:52–9:18 AM	**Clinical Trials of AAV for Aromatic L-amino Acid Decarboxylase Deficiency (AADC)** *Paul Wuh-Liang Hwu, MD, PhD, National Taiwan University Hospital*
9:18–9:44 AM	**Pre-clinical Development of Lentiviral Liver Gene Therapy for Pediatric Metabolic Diseases** *John Counsell, PhD, University College London*

SCHEDULE (ALL TIMES LISTED IN ET)

WEDNESDAY, MAY 18, 2022

SCIENTIFIC SYMPOSIA

ROOM 207
Non-viral Delivery: A Diverse Toolbox Comes of Age
Co-chairs: Stefan Radtke, PhD, Fred Hutchinson Cancer Research Center and Chantal Pichon, PhD, Center for Molecular Biophysics, CNRS

8:00–8:25 AM	**Engineered Exosomes as a Delivery System From Bench to Bedside** *Sriram Sathy, PhD, Codiak Biosciences Inc.*
8:25–8:50 AM	**LNP Formulation Engineering for Lung and Endothelial** *Ross Wilson, PhD, UC Berkeley*
8:50–9:15 AM	**SEND Harnesses Natural Proteins** *Feng Zhang, PhD, Massachusetts Institute of Technology*
9:15–9:40 AM	**Gene-activated Scaffolds for Bone Fracture** *Fergal J. O'Brien, PhD, Royal College of Surgeons in Ireland*

ROOM 201
Overview of Cardiac Gene Therapy
Co-chairs: Jeff L. Ellsworth, PhD, Stellar Research and Development, LLC and Ryan L. Boudreau, PhD, University of Iowa

8:00–8:25 AM	**Pre-clinical Development of Gene Therapy for Post-operative Atrial Fibrillation** *J. Kevin Donahue, MD, University of Massachusetts Chan Medical School*
8:25–8:50 AM	**Gene-edited Human Stem Cell-derived Cardiomyocytes for Cardiac Regeneration** *Silvia Marchiano, PhD, University of Washington*
8:50–9:15 AM	**Non-viral Delivery of Genes Into the Heart** *Valeria Chiono, PhD, Politecnico di Torino*
9:15–9:40 AM	**MicroRNA Manipulation to Control Myocardial Compliance** *Ryan L. Boudreau, PhD, University of Iowa*

SCHEDULE (ALL TIMES LISTED IN ET)

WEDNESDAY, MAY 18, 2022

SCIENTIFIC SYMPOSIA

ROOM 202
Vector-associated Neural and Ocular Inflammation
Co-chairs: Christine N. Kay, MD, Vitreoretinal Associates and Paul A. Sieving, MD, PhD, UC Davis

8:00–8:25 AM	**Strategies To Evade Gene Therapy Inflammation** *Ying Kai Chan, PhD, Harvard University*
8:25–8:50 AM	**Identifying and Preventing Unexpected Impacts of AAV-mediated Gene Delivery in the Nervous System** *Lisa M. Boulanger, PhD, Princeton University*
8:50–9:15 AM	**AAV-RS1 Trials for X-linked Retinoschisis: Disease Condition, Trial Parameters, and Immune Status** *Paul Sieving, MD, PhD, UC Davis*
9:15–9:40 AM	**Ocular Inflammation in AAV-mediated Clinical Trials** *Christine N. Kay, MD, Vitreoretinal Associates*

BALLROOM C
Late Breaking Oral Abstract Sessions
8:00 AM–9:45 PM

Due to the late-breaking nature of this session, individual presentations were not yet selected at time of publish.

See annualmeeting.asgct.org/program for more information.

SCHEDULE (ALL TIMES LISTED IN ET)

WEDNESDAY, MAY 18, 2022

EXHIBITOR SHOWCASES

ROOM 209
Industry Interactions
Informa Pharma Intelligence | The State of Advanced Genetic Therapies in 2022

8:15–8:45 AM

Ly Nguyen-Jatkoe, PhD
Executive Director, Americas, Informa Pharma Intelligence

Daniel Chancellor
Director, Thought Leadership and Consulting; Informa Pharma Intelligence

ROOM 209
Industry Interactions
Curiox Biosystems | Exhibitor Showcase

9:15–9:45 AM

Presenter(s) to be Announced

SCHEDULE (ALL TIMES LISTED IN ET)

WEDNESDAY, MAY 18, 2022

PLENARY SESSIONS

HALL E | 10:15–11:15 AM

Outstanding Achievement Award Symposium

Co-chairs: Beverly L. Davidson, PhD, Children's Hospital of Philadelphia and Donald B. Kohn, MD, UCLA

HALL E

Founder's Award Presentaion

Chair: Beverly L. Davidson, PhD, Children's Hospital of Philadelphia

11:15 AM–12:00 PM **Towards Scalable In Vivo Gene Editing**
Francis S. Collins, MD, PhD

Better Health, Brighter Future

Takeda is a global, R&D-driven biopharmaceutical company committed to discovering and delivering life-changing treatments and vaccines that have a lasting impact on society.

We aim to bring functional cures to patients no matter where they live in the world by developing the next generation of gene therapies through internal innovations and with our growing network of partners.

Since our founding in 1781 in a market stall in Osaka, Japan, our values endure by putting patient needs first, building trust with society, strengthening our reputation, and developing the business – in that order.

SCHEDULE (ALL TIMES LISTED IN ET)

WEDNESDAY, MAY 18, 2022

INDUSTRY SPONSORED SYMPOSIA

ROOM 202

Dyno Therapeutics | Transforming the Gene Therapy Landscape With AI-powered AAV Vectors

12:00–1:30 PM

Nicole Paulk, PhD
Assistant Professor of AAV Gene Therapy, UCSF

Eric Kelsic, PhD
CEO, Dyno Therapeutics

Jamie Kwasnieski, PhD
Head of Applied Biology, Dyno Therapeutics

Jeff Gerold, PhD
Head of Data Science, Dyno Therapeutics

Sylvain Lapan, PhD
Head of Gene Therapy, Dyno Therapeutics

Yvette Leung, MBA
Head of Corporate Development, Dyno Therapeutics

SCHEDULE (ALL TIMES LISTED IN ET)

WEDNESDAY, MAY 18, 2022

INDUSTRY SPONSORED SYMPOSIA

ROOM 207

Miltenyi Biotec | State-of-the-art Closed System Manufacturing of Cell and Gene Therapy Products With the CliniMACS Prodigy®

12:00–1:30 PM

Ian Johnston, PhD
Industrial and Academic Cooperations Manager, Senior Project Manager, Miltenyi Biotec

Kunal Patel, MS
Manager, Process Engineering, Miltenyi Biotec

BALLROOM B

Thermo Fisher Scientific | One Workflow, Two Approaches

12:00–1:30 PM

Julia Braun
Staff Scientist, Thermo Fisher Scientific

Celine Martin
Senior Product Manager, Thermo Fisher Scientific

SCHEDULE (ALL TIMES LISTED IN ET)

WEDNESDAY, MAY 18, 2022

EXHIBITOR SHOWCASES

ROOM 209

Industry Interactions

L7 Informatics | Digitalization – Implementing a Unified Platform to Enable Transformative Progress for Cell and Gene Therapy Timelines

12:00–12:30 PM

Matthew Hewitt, BA, PhD
Executive Director, Scientific Services Cell and Gene Therapy, Charles River

Vasu Rangadass, PhD
President & CEO, L7 Informatics

SCHEDULE (ALL TIMES LISTED IN ET)

WEDNESDAY, MAY 18, 2022

SPONSORED BY: Biogen

PLENARY SESSIONS

HALL E

Presidential Symposium and Presentation of Top Abstracts

Chair: Beverly L. Davidson, PhD, Children's Hospital of Philadelphia

1:30–2:15 PM	**Turning Genes into Medicines: The Long and Winding Road from Gene Discovery to Gene Therapeutics** *Katherine A. High, MD, Ask Bio*
2:15–2:30 PM	**808: Short- and Long-Term Hematopoietic Reconstitution After Transplantation is Stem Cell-Driven and Stochastic: Implications for Gene Therapy** *Stefan Radtke, PhD, Fred Hutchinson Cancer Research Center*
2:30–2:45 PM	**809: Correction of the Sickle Cell Mutation by *In Vivo* HSC Prime Editing in a Mouse Model** *Chang Li, PhD, University of Washington*
2:45–3:00 PM	**810: Generation of Islet-specific Engineered Regulatory T-cells (EngTregs) for Immune Tolerance Induction in Type 1 Diabetes using a Novel Dual-editing Strategy** *Martina Hunt, Seattle Childrens Research Institute*
3:00–3:15 PM	**811: AAV-Meganuclease-Mediated Gene Targeting Achieves Efficient and Sustained Transduction in Newborn and Infant Macaque Liver** *Lili Wang, University of Pennsylvania*

SCHEDULE (ALL TIMES LISTED IN ET)

WEDNESDAY, MAY 18, 2022

TOOLS AND TECHNOLOGY FORUM III

ROOM 209
Industry Interactions
Co-chairs: Kah Whye Peng, PhD and Rachel M. Bailey, PhD, UT Southwestern Medical Center

3:30–3:45 PM	**908 Devices | Actionable Information of Your Cell Culture Media to Boost Viral Vector Production** *Milla Neffling, PhD, Bioprocessing market segment manager, 908 Devices*
3:45–4:00 PM	**Univercells Technologies | An Innovative Platform for Integrated Continuous Viral Vector Production** *Tania Pereira Chilima, Chief Technology Officer, Univercells Technologies*
4:00–4:15 PM	**VIVEbiotech | VIVEbiotech's Approach to Lentiviral Vector Manufacturing** *Natalia Elizalde, PhD, Business Development Director, VIVEbiotech*
4:15–4:30 PM	**Forge Biologics | Platform Process Development for AAV Manufacturing** *David Dismuke, PhD, Chief Technical Officer, Forge Biologics*
4:30–4:45 PM	**SCIEX | Characterization of Lentiviral Vectors Using Capillary Electrophoresis Platform Technology** *Yan Lu, Mass Spec Analytic Scientist I, St. Jude Children's Research Hospital*
4:45–5:00 PM	**Virica Biotech | Overcoming Barriers in Viral Vector Manufacturing: Small Molecule Targeting of Antiviral Defenses** *Jean-Simon Diallo, PhD, Scientific Founder/CEO, Virica Biotech*
5:00–5:15 PM	**10x Genomics | Bridging the Worlds of Histology and Genomics with Visium CytAssist** *Spontaneous Russell, Senior Product Manager, Visium Spatial Solutions, 10x Genomics*
5:15–5:30 PM	**Precision NanoSystems | Rethink Genome Editing of CAR T-cells: Insights into Lipid Nanoparticle Delivery and Scalable Microfluidic Manufacturing** *Angela Zhang, PhD, Senior Product Manager, Precision NanoSystems*
5:30–5:45 PM	**NanoView Biosciences | Purification-Free Detection and Analysis of Viruses with ExoView®** *Clayton Deighan, PhD, NanoView Biosciences*

SCHEDULE (ALL TIMES LISTED IN ET)

WEDNESDAY, MAY 18, 2022

ORAL ABSTRACT SESSIONS

SALON H
RNA Virus Vectors
Co-chairs: John Tisdale, MD, NIH, NHLBI and Mario Amendola, PhD, Genethon

3:45–4:00 PM	**812: Development of a Lentiviral Vector Mediated B-cell Gene Therapy Platform for the Delivery of the Anti-HIV eCD4-Ig Immunoadhesin** *Eirini Vamva, Scripps Research Institute*
4:00–4:15 PM	**813: Taking a Good Look Under the Hood of Engineered Lentiviruses: Nanoview's Novel LentiView Technology Allows the Quantitative Profiling of Pseudotyped Lentiviral Particles** *George Daaboul, PhD, NanoView Biosciences Ltd*
4:15–4:30 PM	**814: Investigating Liver Tissue Dynamics to Improve *In Vivo* Gene Therapy with Lentiviral Vectors** *Francesco Starinieri , PhD, SR-TIGET*
4:30–4:45 PM	**815: CD90-Targeted Viral Vectors of Hematopoietic Stem Cell Gene Therapy** *Kurt Berckmueller, PhD, Fred Hutchinson Cancer Research Center*
4:45–5:00 PM	**816: SARS-CoV-2 Spike Protein-Pseudotyped Lentiviral Vectors (S-LV) for *In Vitro* and *In Vivo* Modelling of Emergent SARS-CoV-2 Variants** *Kamran Miah, PhD, MS, BS, University of Oxford*
5:00–5:15 PM	**817: A Single Injection of CD117 Antibody-drug Conjugate Allows for Efficient Engraftment of Gene-modified CD34+ Cells in a Rhesus Gene Therapy Model** *Naoya Uchida, MD, PhD, National Institutes of Health*
5:15–5:30 PM	**818: Bioinformatic-Guided Design of a Lentiviral Vector for X-Linked Chronic Granulomatous Diseases Recapitulates Endogenous *CYBB* Gene Regulation and Expression** *Ryan Wong, PhD, ImmunoVec*

SCHEDULE (ALL TIMES LISTED IN ET)

WEDNESDAY, MAY 18, 2022

ORAL ABSTRACT SESSIONS

BALLROOM A
AAV Developments in Liver, T-cells, and Toxicity
Co-chairs: Nicole Paulk, PhD, UC San Francisco and Amanda Dudek, PhD, Stanford University

3:45–4:00 PM	**819: Alteration of ITR Sequences for Attenuating the AAV Toxicity in Human Embryonic Stem Cells** *Liujiang Song, PhD, University of North Carolina*
4:00–4:15 PM	**820: Preclinical Evaluation of AAV Vectors in an *Ex Vivo* Human Whole Liver Explant Confirms the Potential of Bioengineered AAVs as Clinically Relevant Hepatotropic Vectors** *Marti Cabanes-Creus, PhD, Children's Medical Research Institute*
4:15–4:30 PM	**821: Optimized Novel AAV Capsids Selected For Improved Homology-Dependent Repair In Human T-cells** *Adrian Westhaus, University College London*
4:30–4:45 PM	**822: Selection of Engineered AAV Capsids With Enhanced Incorporation Into Extracellular Vesicles and Stable Liver Transduction *in vivo*** *Casey Maguire, PhD, The Massachusetts General Hospital*
4:45–5:00 PM	**823: Receptor and Antibody Interactions of AAV by *Cryo*-EM and Tomography** *Michael Chapman, PhD, University of Missouri*
5:00–5:15 PM	**824: Gene Expression From AAV Vectors in the Liver: A Comparative Study Across Species, Promoters and AAV Serotypes** *Subha Karumuthil-Melethil, PhD, REGENXBIO Inc.*
5:15–5:30 PM	**825: Evolution of a New AAV Variant with Murine T Lymphocyte Tropism using the MHC-Ib Molecule H2-Q7 as a Receptor** *Jonathan Ark, Duke University*

SCHEDULE (ALL TIMES LISTED IN ET)

WEDNESDAY, MAY 18, 2022

ORAL ABSTRACT SESSIONS

HALL E
New Gene Editing Technologies and Applications
Co-chairs: Luca Biasco, PhD, AVROBIO and Paula Cannon, PhD, USC

3:45–4:00 PM	**826: MitoTALENs as Genetic Tools to Reduce Mutant mtDNA Levels in the CNS of a Mouse Model Carrying a Heteroplasmic mtDNA Mutation** *Sandra Bacman, PhD, University of Miami*
4:00–4:15 PM	**827: Lowering Huntington Gene Expression by Targeted Base Editing of Cis-Regulatory Elements in its Promoter Sequence** *Tristan McCallister, University of Illinois Urbana Champaign*
4:15–4:30 PM	**828: Long-read Nanopore Sequencing Reveals Outcomes of AAV-CRISPR Editing in the Brain of Transgenic Mouse Models** *Bryan Simpson, The Children's Hospital of Philadelphia*
4:30–4:45 PM	**829: Optimization of Pre-mRNA Exon Editing for Efficient Rescue of ABCA4 Expression** *Kirk Burkhart, PhD, Ascidian Therapeutics*
4:45–5:00 PM	**830: Promoterless AAV Vectors with Homology Arms can Integrate and Express from Transcriptionally Active Sites in Non-targeted Loci** *Calvin Stephens, PhD, Stanford University*
5:00–5:15 PM	**831: SLEEK: A Method for Highly Efficient Knock-in and Expression of Transgene Cargos for Next-generation Cell-based Medicines** *John Zuris, PhD, Editas Medicine*
5:15–5:30 PM	**832: Programmable Deletion, Replacement, Integration and Inversion of Large DNA Sequences with Twin Prime Editing and Site-specific Recombinases** *Xin Gao, PhD, Broad Institute of Harvard and MIT*

SCHEDULE (ALL TIMES LISTED IN ET)

WEDNESDAY, MAY 18, 2022

ORAL ABSTRACT SESSIONS

ROOM 202
Novel Therapeutic Targets to treat CNS Disorders
Co-chairs: Miguel Sena-Esteves, PhD, UMass Chan Medical School and Rebecca Ahrens-Nicklas, MD, PhD, Children's Hospital of Philadelphia

3:45–4:00 PM	**833: Hematopoietic Stem and Progenitor Cell Gene Therapy Uniquely Benefits Multiple Sclerosis in the Animal Model** *Silvia Spadini, Division of Pediatric Hematology, Oncology and Stem Cell Transplantation, Woman's and Child Health Department, University of Padova*
4:00–4:15 PM	**834: A Self-regulating Gene Therapy for Rett Syndrome** *Stuart Cobb, PhD, Neurogene Inc.*
4:15–4:30 PM	**835: A New Gene Therapy Approach to Treat Niemann-Pick Type C2 Disease** *Sara Marcó, PhD, Universitat Autònoma de Barcelona*
4:30–4:45 PM	**836: Comparison of Therapeutic Efficacy and Durability of Gene Therapy for Tuberous Sclerosis Type 2 With Standard of Care Everolimus** *Edwina Abou Haidar* *Massachusetts General Hospital*
4:45–5:00 PM	**837: A Novel Gene Therapy for Rett Syndrome Through Reactivation of the Silent X Chromosome** *Samantha Powers, PhD, Nationwide Children's Hospital*
5:00–5:15 PM	**838: Second Generation AAV-mediated Gene Therapy to Mitigate Risk for Alzheimer's Disease in APOE4/4Homozygotes** *Rachel Montel, PhD, Weill Cornell Medicine*
5:15–5:30 PM	**839: Modulation of miR-181 Influences Dopaminergic Neuronal Degeneration in a Mouse Model of Parkinson's Disease** *Ryan Boudreau, PhD, University of Iowa*

SCHEDULE (ALL TIMES LISTED IN ET)

WEDNESDAY, MAY 18, 2022

ORAL ABSTRACT SESSIONS

ROOM 102
Harnessing Innate Immunity for Cancer Immunotherapy
Co-chairs: Gianpietro Dotti, MD, UNC Lineberger Cancer Center and Sarwish Rafiq, PhD, Emory University

3:45–4:00 PM	**840: Multiplex Base Editing of NK Cell to Enhance Cancer Immunotherapy** *Minjing Wang, University of Minnesota*
4:00–4:15 PM	**841: Engineered Induced Pluripotent Stem Cell-derived Natural Killer Cells Reactively Co-target TIGIT and CD73 in the Glioblastoma Tumor Microenvironment** *Kyle Lupo, Purdue University*
4:15–4:30 PM	**842: Consistent Expansion and Activation of Autologous Non-genetically Modified Natural Killer Cells With Enhanced Cytotoxicity (SNK01) from Heavily Pre-treated Patients With Advanced Solid Tumors** *Paul Song, MD, NKGen*
4:30–4:45 PM	**843: Multifunctional Natural Killer Cell Engager Releasing CXCL10 Augments Natural Killer Cell Recruitment and Anti-tumor Efficacy Against Glioblastoma** *Xue Yao, Purdue University*
4:45–5:00 PM	**844: Logic Gated FLT3 or CD33 Not EMCN CAR-NK Cell Therapy (SENTI-202) for Precise Targeting of AML** *Brian Garrison, PhD, Senti Biosciences*
5:00–5:15 PM	**845: FT536: A First-of-Kind, Off-the-Shelf CAR-iNK Cell Product Candidate for Solid Tumors Designed to Specifically Target MICA/B Stress Proteins and Overcome Mechanisms of Tumor Evasion** *John Goulding, PhD, Fate Therapeutics Inc.*
5:15–5:30 PM	**846: IFNalpha by *In Vivo*-engineered Macrophages Abates Liver Metastases and Triggers Counter Regulatory Responses Limiting Efficacy** *Thomas Kerzel, SR-TIGET*

SCHEDULE (ALL TIMES LISTED IN ET)

WEDNESDAY, MAY 18, 2022

ORAL ABSTRACT SESSIONS

SALON G
Cell-based Cancer Immunotherapies II
Co-chairs: Masato Yamamoto, MD, PhD, University of Minnesota and Irina Balyasnikova, PhD, Northwestern University Feinberg School of Medicine

3:45–4:00 PM	**847: Characterization of the Transcriptomic and T-cell Receptor (TCR) Clonal Heterogeneity of Tumor-Infiltrating Lymphocyte (TIL) Therapy Infusion Products by Single-Cell Sequencing and Correlative Analyses With Clinical Efficacy in Patients With Advanced Cutaneous Melanoma** *Jinzhou Yuan, PhD, Instil Bio, Inc*
4:00–4:15 PM	**848: Dual CD33/CLL-1 Targeted CAR T-cells for Treatment of Acute Myeloid Leukemia** *Kevin Kowal, Washington University School of Medicine*
4:15–4:30 PM	**849: Off-the-shelf Natural Killer Cells Derived from HIPSC via Genetic Modifications** *Luhan Yang, PhD, Qihan Biotech*
4:30–4:45 PM	**850: Targeting the C Domain of Tenascin C with CAR T-cells for the Immunotherapy of Pediatric Brain and Solid Tumors** *Elizabeth Wickman, St. Jude Graduate School*
4:45–5:00 PM	**851: A Phase 1 Dose Escalation Study of GCC19CART a Novel CoupledCAR® Therapy for Subjects With Metastatic Colorectal Cancer** *Jiuwei Cui, MD, PhD, The First Bethune Hospital of Jilin University*
5:00–5:15 PM	**852: Discovery of a Novel C07:02-Resticted Epitope on MAGE-A1 and Pre-clinical Development of an Enhanced TCR T-cell Therapy Candidate for the Treatment of Solid Tumors** *Gavin MacBeath, PhD, TScan Therapeutics*
5:15–5:30 PM	**853: Asymmetric cell division for fate induction of chimeric antigen receptor (CAR) T-cells** *Christoph Ellebrecht, MD, University of Pennsylvania*

SCHEDULE (ALL TIMES LISTED IN ET)

WEDNESDAY, MAY 18, 2022

ORAL ABSTRACT SESSIONS

ROOM 206
Engineered Cell Therapies
Co-chairs: Bakhos Tannous, PhD, Harvard Medical School and Massachusetts General Hospital
and M Graca Almeida-Porada, MD, PhD, Wake Forest School of Medicine

3:45–4:00 PM	**854: Primary Human Hepatocytes, Genetically Engineered ex vivo to be Hypoimmunogenic, Can Rescue a Model of Metabolic Liver Disease** *Fei Yi, Ambys Medicines*
4:00–4:15 PM	**855: Kinase p38 Regulates Macrophage Adaptation in the Lung Upon Pulmonary Macrophage Transfer** *Ariane Nguyen M. Sc., Hannover Medical School*
4:15–4:30 PM	**856: *Ex Vivo* Transduced Macrophages Engraft in the Lung Following Transplantation and Produce Therapeutic Levels of Secreted Proteins** *Nora Clarke, PhD, Imperial College London*
4:30–4:45 PM	**857: Scalable Generation and Tailored Design of Human iPSC-macrophages for Novel Immunotherapies Targeting Bacterial Infections** *Mania Ackermann, Hannover Medical School*
4:45–5:00 PM	**858: Bioprinting of Organotypic Aggregates of Hepatocytes and Mesenchymal Cells as a Platform for Liver Cell Therapy** *Christopher Dickman, Aspect Biosystems*
5:00–5:15 PM	**859: iPSC-Derived Monocytes Generate Functional M1 and M2 Macrophages with Enhanced Cytokine Secretion and Tumor Cell-Killing Activity** *Ian Hay, Factor Bioscience*
5:15–5:30 PM	**860: Transplantation of Gene Edited Upper Airway Basal Stem Cells in Immunocompromised Mice Using Fibrinogen Based Scaffolds** *Sriram Vaidyanathan, PhD, Stanford University*

SCHEDULE (ALL TIMES LISTED IN ET)

WEDNESDAY, MAY 18, 2022

ORAL ABSTRACT SESSIONS

ROOM 201
Vector Manufacturing and Engineering 3: Improving Vector Design and System Performance
Chair: Christopher Doering, PhD, Emory University

3:45–4:00 PM	**861: Size-Optimized and Shelf-Stable Plasmid DNA Particles for Production of Viral Vectors** *Yizong Hu, Johns Hopkins University*
4:00–4:15 PM	**862: The Primate Selective Transduction of rAAV-LK03 Vectors is Related to Variation in Histone and Histone Post-translational Modifications on the Viral Genome in the Host Nucleus** *Adriana Gonzalez-Sandoval, PhD, Stanford University*
4:15–4:30 PM	**863: Development and Characterization of Highly Optimized Monoclonal Producer Cell Lines (PCLs) for the Treatment of CDKL5 Deficiency Disorder (CDD)** *Laurie Tran, MSc, Ultragenyx Pharmaceutical*
4:30–4:45 PM	**864: Optimized Human Regulatory Sequences Achieve Targeted Expression in CNS and Decreased Liver Expression in Mice** *Annie Tanenhaus, Encoded Therapeutics*
4:45–5:00 PM	**865: Development of Next Generation Vaccine Platform with Self-amplifying mRNAs** *Anitha Thomas, PhD PMP, Precision NanoSystems*
5:00–5:15 PM	**866: Long Term Stability Profiles of AAV Vectors at Ambient Temperature within a Film Matrix** *Maria Croyle, PhD, UT-Austin College of Pharmacy*
5:15–5:30 PM	**867: Combination of Advanced Plasmid Design, Transfection Reagent and Design of Experiment (DOE) Achieves High-yield, High-quality and Potent AAV Vectors in Scalable Suspension HEK293 Cells** *Jing Liao, PhD, LogicBio Therapeutics*

SCHEDULE (ALL TIMES LISTED IN ET)

WEDNESDAY, MAY 18, 2022

ORAL ABSTRACT SESSIONS

ROOM 204
Pharmacology/Toxicology Studies or Assay Development I
Co-chairs: Heikki Turunen, PhD, Dyno Therapeutics and Eva Andres-Mateos, MD, PhD, Atsena Therapeutics S, Inc

3:45–4:00 PM	**868: Circulating Neurofilament Light Chain as a Promising Biomarker of AAV-induced Dorsal Root Ganglia Toxicity in Nonclinical Toxicology Species** *Kelly Fader, PhD, Pfizer Inc*
4:00–4:15 PM	**869: Development of Probe-Based qPCR Assays for the Detection of Replication-Competent Lentiviral Particles** *Menna Ahmed, ProtaGene CGT GmbH*
4:15–4:30 PM	**870: Sonication Linker Mediated-PCR (SLiM-PCR), an Efficient Method for Quantitative Retrieval of Vector Integration Sites** *Fabrizio Benedicenti, SR-TIGET*
4:30–4:45 PM	**871: CDMS Analysis of DNA Released From rAAV Gene Therapy Vectors** *Benjamin Draper, PhD, Megadalton Solutions*
4:45–5:00 PM	**872: The Safety and Biodistribution Profiles of Systemically Delivered Oncolytic Adenovirus in Pigs** *Margarita Romanenko, PhD, University of Minnesota*
5:00–5:15 PM	**873: Electrophysiology and Soluble Biomarker as Translational Tools to Monitor Adeno-Associated-Virus Related Ganglionopathy** *Juliette Hordeaux, University of Pennsylvania*
5:15–5:30 PM	**874: Application of Single-Cell Transcriptomics to Assess rAAV-Delivered Transgene Expression** *Richard Lamontagne, University of Pennsylvania*

SCHEDULE (ALL TIMES LISTED IN ET)

WEDNESDAY, MAY 18, 2022

HALL D
Networking Reception & Poster Session III
5:30–6:30 PM

INTERNATIONAL SPY MUSEUM
Networking Event—Closing Night Reception
8:00–11:00 PM

Join us for a night of fun during the Closing Night Reception at Washington, D.C.'s **International Spy Museum!** Enjoy the museum exhibits, light appetizers, and drinks.
You can purchase a ticket ($50 per person) for the reception before or after you **register for the meeting.** Space is limited!

zolgensma®
(onasemnogene abeparvovec-xioi)
suspension for intravenous infusion

The one-time-only dose to stop SMA progression

ZOLGENSMA is a gene therapy for pediatric patients less than 2 years of age with spinal muscular atrophy (SMA), that is delivered as a single-dose, 1-hour intravenous infusion[1]

Event-free survival

91% (20/22) of patients were alive and free of permanent ventilation at the 14-months-of-age study visit, **a primary endpoint**, and at 18 months of age[2,a-c]

Motor milestones achieved

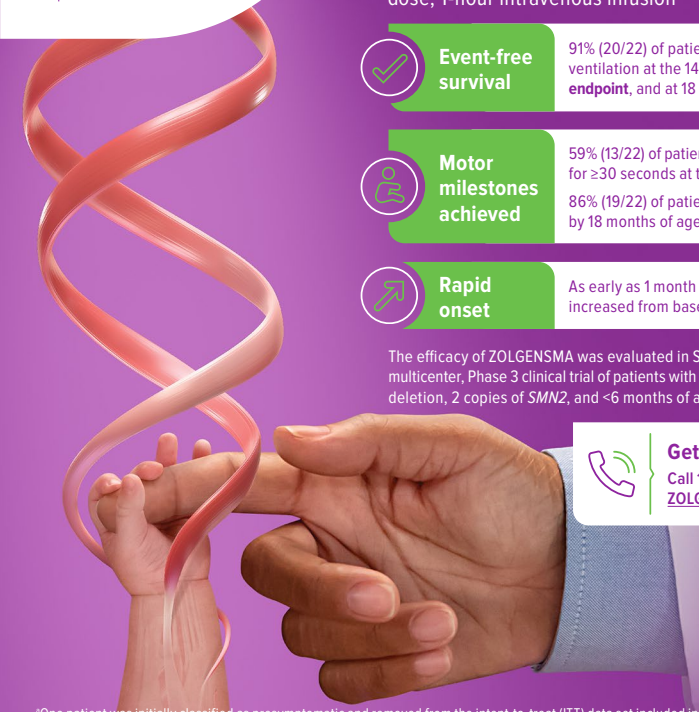
59% (13/22) of patients achieved the ability to sit without support for ≥30 seconds at the 18-month study visit, **a primary endpoint**[2,a]

86% (19/22) of patients achieved one or more motor milestones by 18 months of age[2,a]

Rapid onset

As early as 1 month post infusion, CHOP INTEND scores increased from baseline by a mean of 6.9 points (N=22)[2,a]

The efficacy of ZOLGENSMA was evaluated in STR1VE, a completed, open-label, single-arm, multicenter, Phase 3 clinical trial of patients with SMA Type 1 (genetically confirmed bi-allelic *SMN1* deletion, 2 copies of *SMN2*, and <6 months of age at symptom onset and treatment; N=22).[1,a,b]



Get started with ZOLGENSMA today:
Call 1-855-441-GENE (4363) or learn more at ZOLGENSMA-hcp.com

1800+ PATIENTS TREATED AS OF DECEMBER 2021[3,d]

[a]One patient was initially classified as presymptomatic and removed from the intent-to-treat (ITT) data set included in the Prescribing Information. The patient was later confirmed to be symptomatic at baseline and included in the final ITT analysis.[2]

[b]One patient died at age 7.8 months due to respiratory failure, which was considered unrelated to treatment. One patient withdrew consent at 11.9 months of age; this patient required permanent ventilation at 11.0 months prior to withdrawal of consent. One patient discontinued participation at the age of 18.0 months, before the month 18 end-of-study visit, due to an adverse event of respiratory distress, which was considered unrelated to treatment.[2]

[c]Event is defined as death or the need for permanent ventilatory support consisting of ≥16 hours of respiratory assistance per day continuously for ≥14 days in the absence of an acute reversible illness, excluding perioperative ventilation.[1]

[d]Treated globally in clinical trials, managed access programs, and commercially.

Indication and Important Safety Information

Indication

ZOLGENSMA is an adeno-associated virus vector-based gene therapy indicated for the treatment of pediatric patients less than 2 years of age with spinal muscular atrophy (SMA) with bi-allelic mutations in the *survival motor neuron 1* (*SMN1*) gene.

Limitations of Use

The safety and effectiveness of repeat administration or the use in patients with advanced SMA (e.g., complete paralysis of limbs, permanent ventilator dependence) has not been evaluated with ZOLGENSMA.

Important Safety Information

BOXED WARNING: Acute Serious Liver Injury and Acute Liver Failure

Acute serious liver injury, acute liver failure, and elevated aminotransferases can occur with ZOLGENSMA. Patients with preexisting liver impairment may be at higher risk. Prior to infusion, assess liver function of all patients by clinical examination and laboratory testing (e.g., hepatic aminotransferases [aspartate aminotransferase (AST) and alanine aminotransferase (ALT)], total bilirubin, and prothrombin time). Administer a systemic corticosteroid to all patients before and after ZOLGENSMA infusion. Continue to monitor liver function for at least 3 months after infusion.

WARNINGS AND PRECAUTIONS

Thrombocytopenia

Transient decreases in platelet counts, some of which met the criteria for thrombocytopenia, were typically observed within the first two weeks after ZOLGENSMA infusion. Monitor platelet counts before ZOLGENSMA infusion and on a regular basis for at least 3 months afterwards.

Thrombotic Microangiopathy

Cases of thrombotic microangiopathy (TMA) were reported approximately 1 week after ZOLGENSMA infusion. Obtain baseline creatinine and complete blood count before ZOLGENSMA infusion. Following infusion, monitor for thrombocytopenia as well as other signs and symptoms of TMA. Consult a pediatric hematologist and/or pediatric nephrologist immediately to manage if clinically indicated.

Elevated Troponin-I

Increases in cardiac troponin-I levels were observed following ZOLGENSMA infusion. Monitor troponin-I before ZOLGENSMA infusion and on a regular basis for at least 3 months afterwards.

ADVERSE REACTIONS

The most commonly observed adverse reactions (incidence ≥5%) in clinical studies were elevated aminotransferases and vomiting.

Please see Brief Summary of Prescribing Information on the adjacent page.

References: 1. ZOLGENSMA [prescribing information]. Bannockburn, IL: Novartis Gene Therapies, Inc; 2021. **2.** Data on file. AveXis, Inc. 2020. **3.** Data on file. Novartis Gene Therapies, Inc. 2022.

NOVARTIS

For US healthcare professionals only.
03/2022 US-ZOL-22-0047

ZOLGENSMA® (onasemnogene abeparvovec-xioi) **Rx Only**

Suspension for intravenous infusion
Brief Summary of the Full Prescribing Information.
See Full Prescribing Information.

BOXED WARNING: ACUTE SERIOUS LIVER INJURY AND ACUTE LIVER FAILURE

- **Acute serious liver injury, acute liver failure, and elevated aminotransferases can occur with ZOLGENSMA.**
- **Patients with preexisting liver impairment may be at higher risk.**
- **Prior to infusion, assess liver function of all patients by clinical examination and laboratory testing (e.g., hepatic aminotransferases [aspartate aminotransferase (AST) and alanine aminotransferase (ALT)], total bilirubin, and prothrombin time). Administer systemic corticosteroid to all patients before and after ZOLGENSMA infusion. Continue to monitor liver function for at least 3 months after infusion.**

INDICATIONS AND USAGE

ZOLGENSMA is an adeno-associated virus vector-based gene therapy indicated for the treatment of pediatric patients less than 2 years of age with spinal muscular atrophy (SMA) with bi-allelic mutations in the *survival motor neuron 1* (*SMN1*) gene.

Limitation of Use: The safety and effectiveness of repeat administration of ZOLGENSMA or the use in patients with advanced SMA (e.g., complete paralysis of limbs, permanent ventilator dependence) has not been evaluated.

DOSAGE AND ADMINISTRATION

For single-dose intravenous infusion only.

The recommended dosage of ZOLGENSMA is 1.1×10^{14} vector genomes per kilogram (vg/kg) of body weight.

- Administer ZOLGENSMA as an intravenous infusion over 60 minutes.
- Postpone ZOLGENSMA in patients with concurrent infections until the infection has resolved. Clinical signs or symptoms of infection should not be evident at the time of ZOLGENSMA administration.
- Starting one day prior to ZOLGENSMA infusion, administer systemic corticosteroids equivalent to oral prednisolone at 1 mg/kg of body weight per day for a total of 30 days. At the end of the 30-day period of systemic corticosteroid treatment, check liver function by clinical examination and by laboratory testing. For patients with unremarkable findings, taper the corticosteroid dose gradually over the next 28 days. If liver function abnormalities persist, continue systemic corticosteroids (equivalent to oral prednisolone at 1 mg/kg/day) until findings become unremarkable, and then taper the corticosteroid dose gradually over the next 28 days or longer if needed. Do not stop systemic corticosteroids abruptly. If liver function abnormalities continue to persist ≥ 2 × ULN after the 30-day period of systemic corticosteroids, consult a pediatric gastroenterologist or hepatologist.

WARNINGS AND PRECAUTIONS

Acute Serious Liver Injury, Acute Liver Failure or Elevated Aminotransferases

Acute serious liver injury, acute liver failure and elevated aminotransferases can occur with ZOLGENSMA. Hepatotoxicity (which may be immune-mediated), generally manifested as elevated ALT and/or AST levels and at times as acute serious liver injury or acute liver failure, has been reported with ZOLGENSMA use. In order to mitigate potential aminotransferase elevations, administer systemic corticosteroid to all patients before and after ZOLGENSMA infusion. Immune-mediated hepatotoxicity may require adjustment of the corticosteroid treatment regimen, including longer duration, increased dose, or prolongation of the corticosteroid. Patients with preexisting liver impairment or acute hepatic viral infection may be at higher risk of acute serious liver injury/acute liver failure. Patients with ALT, AST, or total bilirubin levels (except due to neonatal jaundice) > 2 × ULN have not been studied in clinical trials with ZOLGENSMA. The risks and benefits of infusion with ZOLGENSMA in patients with preexisting liver impairment should be weighed carefully against the risks of not treating the patient. Although in the clinical trials and in postmarketing experience, asymptomatic aminotransferase elevations were very commonly reported, in the managed access program and in the postmarketing setting, cases of acute serious liver injury and acute liver failure have been reported. Some patients have experienced elevations in ALT and AST > 20 × ULN, prolonged prothrombin time and have been symptomatic (e.g., vomiting, jaundice), which resolved with the use of prednisolone, sometimes requiring prolonged duration and/or a higher dose. If acute serious liver injury or acute liver failure is suspected, consult a pediatric gastroenterologist or hepatologist. Prior to ZOLGENSMA infusion, assess liver function by clinical examination and laboratory testing (hepatic aminotransferases [AST and ALT], total bilirubin level, and prothrombin time). Continue to monitor liver function for at least 3 months after ZOLGENSMA infusion (weekly for the first month, and then every other week for the second and third months, until results are unremarkable).

Thrombocytopenia

Transient decreases in platelet counts, some of which met the criteria for thrombocytopenia, were typically observed within the first two weeks after ZOLGENSMA infusion. Monitor platelet counts before ZOLGENSMA infusion and on a regular basis afterwards (weekly for the first month; every other week for the second and third months until platelet counts return to baseline).

Thrombotic Microangiopathy

Cases of thrombotic microangiopathy (TMA) were reported approximately one week after ZOLGENSMA infusion in the post-marketing setting. TMA is characterized by thrombocytopenia, microangiopathic hemolytic anemia, and acute kidney injury. Concurrent immune system activation (e.g., infections, vaccinations) was identified in some cases. Monitor platelet counts, as well as signs and symptoms of TMA, such as hypertension, increased bruising, seizures, or decreased urine output. In case these signs and symptoms occur in the presence of thrombocytopenia, further diagnostic evaluation for hemolytic anemia and renal dysfunction should be undertaken. If clinical signs, symptoms and/or laboratory findings consistent with TMA occur, consult a pediatric hematologist and/or pediatric nephrologist immediately to manage TMA as clinically indicated.

Elevated Troponin-I

Increases in cardiac troponin-I levels (up to 0.176 mcg/L) were observed following ZOLGENSMA infusion in clinical trials. The clinical importance of these findings is not known. However, cardiac toxicity was observed in animal studies. Monitor troponin-I before ZOLGENSMA infusion and on a regular basis for at least 3 months afterwards (weekly for the first month, and then monthly for the second and third months until troponin-I level returns to baseline). Consider consultation with a cardiologist, if troponin elevations are accompanied by clinical signs or symptoms.

ADVERSE REACTIONS

The safety data described in this section reflect exposure to ZOLGENSMA in four open-label studies conducted in the United States, including one completed clinical trial, two ongoing clinical trials, and one ongoing observational long-term follow-up study of the completed trial. A total of 44 patients with SMA received intravenous infusion of ZOLGENSMA, 41 patients at or above the recommended dose, and 3 patients at a lower dose. The patient population ranged in age from 0.3 months to 7.9 months at the time of infusion (weight range 3.0 kg to 8.4 kg). The most frequent adverse reactions (incidence ≥ 5%) observed in the 4 studies were elevated aminotransferases* 27.3% (12/44) and vomiting 6.8% (3/44).

*Elevated aminotransferases include elevation of alanine aminotransferase (ALT) and/or aspartate aminotransferase (AST). In the completed clinical trial, one patient (the first patient infused in that study) was enrolled prior to the protocol amendment instituting administration of prednisolone before and after ZOLGENSMA infusion.

One patient in an ongoing non-United States clinical trial initially presented with respiratory insufficiency 12 days after ZOLGENSMA infusion and was found to have respiratory syncytial virus (RSV) and parainfluenza in respiratory secretions. The patient had episodes of serious hypotension, followed by seizures, and was found to have leukoencephalopathy (brain white matter defects) approximately 30 days after ZOLGENSMA infusion. The patient died after withdrawal of life support 52 days after ZOLGENSMA infusion.

Immunogenicity

In ZOLGENSMA clinical trials, patients were required to have baseline anti-AAV9 antibody titers of ≤ 1:50, measured using an enzyme-linked immunosorbent assay (ELISA). Evidence of prior exposure to AAV9 was uncommon. The safety and efficacy of ZOLGENSMA in patients with anti-AAV9 antibody titers above 1:50 have not been evaluated. Perform baseline testing for the presence of anti-AAV9 antibodies prior to ZOLGENSMA infusion. Retesting may be performed if anti-AAV9 antibody titers are reported as > 1:50.

Following ZOLGENSMA infusion, increases from baseline in anti-AAV9 antibody titers occurred in all patients. In the completed clinical trial, anti-AAV9 antibody titers reached at least 1:102,400 in every patient, and titers exceeded 1:819,200 in most patients. Re-administration of ZOLGENSMA in the presence of high anti-AAV9 antibody titer has not been evaluated.

DRUG INTERACTIONS

Where feasible, adjust a patient's vaccination schedule to accommodate concomitant corticosteroid administration prior to and following ZOLGENSMA infusion. Certain vaccines, such as MMR and varicella, are contraindicated for patients on a substantially immunosuppressive steroid dose (i.e., ≥ 2 weeks of daily receipt of 20 mg or 2 mg/kg body weight of prednisone or equivalent). Seasonal RSV prophylaxis is not precluded.

USE IN SPECIAL POPULATIONS

Pediatric Use

Administration of ZOLGENSMA to premature neonates before reaching full-term gestational age is not recommended, because concomitant treatment with corticosteroids may adversely affect neurological development. Delay ZOLGENSMA infusion until the corresponding full-term gestational age is reached. There is no information on whether breastfeeding should be restricted in mothers who may be seropositive for anti-AAV9 antibodies. The safety of ZOLGENSMA was studied in pediatric patients who received ZOLGENSMA infusion at age 0.3 to 7.9 months (weight range 3.0 kg to 8.4 kg). The efficacy of ZOLGENSMA was studied in pediatric patients who received ZOLGENSMA infusion at age 0.5 to 7.9 months (weight range 3.6 kg to 8.4 kg).

Hepatic Impairment

ZOLGENSMA therapy should be carefully considered in patients with liver impairment. Cases of acute serious liver injury and acute liver failure have been reported with ZOLGENSMA in patients with preexisting liver abnormalities. In clinical trials, elevation of aminotransferases was observed in patients following ZOLGENSMA infusion.

PATIENT COUNSELING INFORMATION

See the ZOLGENSMA Full Prescribing Information for the Patient Counseling Information.

Please visit ZOLGENSMA-HCP.com for Full Prescribing Information, including Boxed Warning.

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Novartis Gene Therapies, Inc.
2275 Half Day Road, Suite 200
Bannockburn, IL 60015 USA

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zolgensma®
(onasemnogene abeparvovec-xioi)
suspension for intravenous infusion

NOVARTIS

Bannockburn, IL 60015 11/2021 US-ZOL-19-0202 V4

SCHEDULE (ALL TIMES LISTED IN ET)

THURSDAY, MAY 19, 2022

7:00 AM–12:00 PM REGISTRATION HOURS—EAST SALON

SCIENTIFIC SYMPOSIA

ROOM 201
Effective Regulatory Interactions
Co-chairs: Megan Zoschg-Canniere, PhD, Spark Therapeutics and Kit Shaw, PhD, Dana-Farber Cancer Institute

8:00–8:10 AM	**ASGCT Survey on Sponsor Communication Challenges** *Megan Zoschg-Canniere, PhD, Spark Therapeutics*
8:10–8:25 AM	**FDA-Sponsor Communications** *Wilson Bryan, MD, Food and Drug Administration*
8:25–8:40 AM	**Key Proposals Within PDUFA to Address Communication Challenges** *Khushboo Sharma, PhD, Biotechnology Innovation Organization*
8:40–8:55 AM	**Optimizing Early FDA Interactions (Pre-IND)** *Aron Stein, PhD, Intellia Therapeutics*
8:55–9:10 AM	**Strategies for Effective Late-stage FDA Communications** *Anita Freed, PhD, Pfizer*
9:10–9:45 AM	**Panel Discussion**

SCHEDULE (ALL TIMES LISTED IN ET)

THURSDAY, MAY 19, 2022

SCIENTIFIC SYMPOSIUM

BALLROOM B
Emerging Clinical and Translational Safety Topics in Cell and Gene Therapy
Co-Chair: Megan Hoban, PhD, bluebird bio and TK

8:00–8:25 AM	**Advanced Vectors for *In Vivo* CAR T-cell Generation** *Christian J. Buchholz, PhD, Paul Erhlich Institut, Germany*
8:25–8:50 AM	**MDS/AML After Curative Therapies for Sickle Cell Disease** *Courtney Fitzhugh, MD, National Institutes of Health*
8:50–9:15 AM	**Product-derived Lymphoma Following Infusion of PiggyBac Modified CD19 Chimeric Antigen Receptor T-cells** *Kenneth Micklethwaite, MD, PhD, Westmead Hospital*
9:15–9:40 AM	**Roadmap for Determining Vector Involvement in Hematologic Malignancy** *Melissa Bonner, PhD, bluebird bio*

SCHEDULE (ALL TIMES LISTED IN ET)

THURSDAY, MAY 19, 2022

SCIENTIFIC SYMPOSIUM

BALLROOM A

Finding a "Cure": The Promise and Perils of Gene Therapy, *In Utero* and Beyond

Co-chairs: Randy J. Chandler, PhD, National Institutes of Health and Stephanie Cherqui, PhD, UC San Diego

8:00–8:25 AM	***In Utero* Gene Editing for Congenital Diseases** *William Peranteau, MD, Children's Hospital of Philadelphia*
8:25–8:50 AM	**Gene-based Therapeutics Operating Under Natural Regulation and Targeting Common Causes of Rare Diseases** *Dan Wang, PhD, University of Massachusetts Chan Medical School*
8:50–9:15 AM	**HSC Gene Therapy Applications in Neurometabolic and Neurodegenerative Disorders** *Alessandra Biffi, MD, DFCI/Boston Children's Cancer and Blood Disorders Center*
9:15–9:40 AM	**X^{on}a Drug-induced Splicing Switch for Regulated Control of Gene Therapies** *Alejandro Mas Monteys, PhD, University of Philadelphia; Children's Hospital of Philadelphia*

SCHEDULE (ALL TIMES LISTED IN ET)

THURSDAY, MAY 19, 2022

SCIENTIFIC SYMPOSIA

ROOM 207
Global Experience With COVID-19 Vaccines
Co-chairs: Shan Lu, MD, PhD, University of Massachusetts Chan Medical School and David B. Weiner, PhD, Wistar Institute

8:00–8:25 AM — **A Gene-based Vaccine Platform Leads to Durable Immunity**
Luk Vandenberghe, PhD, ciendias bio, Grousbeck Gene Therapy Center, Mass Eye and Ear, Harvard & The Broad Institute of Harvard and MIT

8:25–8:50 AM — **Duration of Immunity, Long-term Prospects to Control COVID**
Alessandro Sette, Dr.Biol.Sci, La Jolla Institute for Immunology

8:50–9:15 AM — **Prospect for Universal COVID Vaccine**
Steven L. Zeichner, MD, PhD, University of Virginia

9:15–9:40 AM — **Community-acquired Immunity Against SARS-CoV-2 Prevents Hospitalization, Severe Disease and Death Against Delta and Omicron Variants Better Than Vaccine-induced Immunity—At a High Cost for Many**
Gary P. Kobinger, PhD, Galveston National Laboratory (GNL), UT Medical Branch at Galveston

SALON G
What are the Most Pressing Issues of the Future?
Chair: Rayne Rouce, MD, Baylor College of Medicine

8:00–9:45 AM — **Panel Discussion**
Sarwish Rafiq, PhD, Emory University
Li Ou, PhD, Capsida Biotherapeutics
Alisha Gruntman, DVM, PhD, Tufts Cummings School of Veterinary Medicine; University of Massachusetts Chan Medical School
Avery D. Posey, Jr, PhD University of Pennsylvania Perelman School of Medicine

SCHEDULE (ALL TIMES LISTED IN ET)

THURSDAY, MAY 19, 2022

SCIENTIFIC SYMPOSIA

ROOM 204
Cutting Edge Gene and Cell Therapy Research in Europe, in Memory of Dr. Manfred Schmidt
Co-chairs: Juan A. Bueren, PhD, CIEMAT and Hildegard Büning, PhD, Hannover Medical School

8:00–8:26 AM	**Manfred's Legacy: Assaying the Pharmacodynamics of Insertional Gene Therapy** *Christof von Kalle, PhD, Berlin Institute of Health (Charite)*
8:26–8:52 AM	**How Integration Site Analysis Impacted the Design of Safer Vectors and Clinical Trials** *Axel Schambach, MD, PhD, Hannover Medical School*
8:52–9:18 AM	**DNA Integration in Therapeutic Gene Modification: History and Current Challenges** *Frederic D. Bushman, PhD, University of Pennsylvania School of Medicine*
9:18–9:44 AM	**Next-generation Genotoxicity Testing and Clonal Tracking Methodologies** *Eugenio Montini, PhD. San Raffaele Telethon Institute for Gene Therapy*

SCHEDULE (ALL TIMES LISTED IN ET)

THURSDAY, MAY 19, 2022

ORAL ABSTRACT SESSIONS

BALLROOM C
Clinical Trials Spotlight Symposium
Chair: Maria Grazia Roncarolo, MD, Stanford University

8:00–8:15 AM	**1188: Interim Results from an Ongoing Phase 1/2 Study of Lentiviral-Mediated *Ex Vivo* Gene Therapy for Pediatric Patients with Severe Leukocyte Adhesion Deficiency-I (LAD-I)** *Donald B. Kohn, MD, UCLA*
8:15–8:30 AM	**1189: Safety and Efficacy of SARS-COV-2-specific T-cells as Adoptive Immunotherapy for High-risk COVID-19 Patients: A Phase I/II, Randomized Clinical Trial** *Evangelia Yannaki, George Papanikolaou Hospital*
8:30–8:45 AM	**1190: Autologous Cell & Gene Therapy for the Therapeutic Targeting of Immune Payloads to the Solid Tumor Microenvironment: Preliminary Results of the TEM-GBM Study** *Bernhard Gentner, MD, PhD, SR-TIGET*
8:45–9:00 AM	**1191: Lentiviral Gene Therapy with Low Dose Busulfan for Infants With X-Linked Severe Combined Immune Deficiency (XSCID) results in the Development of a Normal and Sustained Immune System: Interim Results of an Ongoing Phase I/II Clinical Study** *Ewelina Mamcarz, MD, St. Jude Children's Research Hospital*
9:00–9:15 AM	**1192: Stable Hemostatic Correction and Improved Hemophilia-Related Quality of Life: Final Analysis From the Pivotal Phase 3 HOPE-B Trial of Etranacogene Dezaparvovec** *Steven Pipe, University of Michigan*
9:15–9:30 AM	**1193: IGNITE DMD Study of SGT-001 Microdystrophin Gene Therapy for Duchenne Muscular Dystrophy: Long-Term Outcomes and Biomarker Update** *Carl Morris, PhD, Solid Biosciences*
9:30–9:45 AM	**1194: The OPTIC Study of Intravitreal Gene Therapy With ADVM-022 for Neovascular AMD (nAMD): the Role of Neutralizing Antibodies** *Szilard Kiss, MD, Weill Cornell Medical College*

SCHEDULE (ALL TIMES LISTED IN ET)

THURSDAY, MAY 19, 2022

ORAL ABSTRACT SESSIONS

BALLROOM A
Novel AAV Capsids for the Brain, Eye and Kidney
Co-chairs: Nicole Paulk, PhD, UC San Francisco and Andrew Steinsapir, Deerfield Management

10:15–10:30 AM	**1195: Machine-Guided Design Reveals AAV Variants Efficient in Transducing NHP Retina After Intravitreal Delivery** *Heikki Turunen, Dyno Therapeutics*
10:30–10:45 AM	**1196: A Newly Evolved AAV Variant Enables Potent Gene Transfer in Kidneys of Multiple Species** *Alan Rosales, BS, MS, Duke University*
10:45–11:00 AM	**1197: A Promising New Family of Peptide-modified AAV Capsids for Gene Delivery to the Central Nervous System in Non-human Primates** *Ken Chan, PhD, Broad Institute of MIT and Harvard*
11:00–11:15 AM	**1198: Directed Evolution of AAV9 Peptide Display Libraries Identifies a Family of Cross-Species Variants With Enhanced Brain Tropism in Non-Human Primates and Mice Following Systemic Administration** *Tyler Moyer, PhD, Voyager Therapeutics*
11:15–11:30 AM	**1199: Novel AAV Capsids for Intravitreal Delivery: Identifying and Characterizing Novel AAV Variants in Non-Human Primates** *Karen Guerin, PhD, Vedere Bio II*
11:30–11:45 AM	**1200: Identification and Characterization of an AAV9-Based Engineered Capsid Variant Capable of Mediating Enhanced Transcription in the Central Nervous System of Non-Human Primates and Rodents** *April Giles, PhD, REGENXBIO*
11:45 AM–12:00 PM	**1201: Fit4Function: A Machine Learning-guided Approach for Systematic Multi-trait AAV Capsid Engineering** *Fatma-Elzahraa Eid, PhD, Broad Institute of MIT and Harvard*

SCHEDULE (ALL TIMES LISTED IN ET)

THURSDAY, MAY 19, 2022

ORAL ABSTRACT SESSIONS

BALLROOM C
New Technologies for AAV Gene Therapy
Co-chairs: Phillip Tai, PhD, UMass Chan Medical School and Aravind Asokan, PhD, Duke University School of Medicine

10:15–10:30 AM	**1202: Effects of Complement Component 1 (C1) Inhibition on AAV-based Gene Transfer Efficacy and Immunogenicity in Mice** *Nikki Ross, PhD, Spark Therapeutics*
10:30–10:45 AM	**1203: Inducible Gene Expression for Gene Therapy: Design and Exemplification of Powerful, Small, Modular and Tightly Controlled Regulatable Promoters** *Graham Whyteside, PhD, Asklepios Biopharmaceutical Inc*
10:45–11:00 AM	**1204: Inclusion of AAV Empty Capsids can Increase Expression in Certain Contexts *In Vitro* and *In Vivo*** *Julie Crudele, PhD, University of Washington*
11:00–11:15 AM	**1205: Disease-responsive Therapy for the Treatment of Myotonic Dystrophy** *Ellie Carrell, PhD, Children's Hospital of Philadelphia*
11:15–11:30 AM	**1206: Preclinical Gene Reactivation of the X-linked Cdkl5 Gene using Dual Adeno-associated Virus Mediated CRISPR/dCas9 Epigenetic Editing** *Julian Halmai, PhD, MS, UC Davis*
11:30–11:45 AM	**1207: Characterization of Optimized Dual AAV-MYO7A Vectors for the Treatment of Usher Syndrome (USH1B) in *Myo7a*$^{-/-}$ Mice and NHP** *Kaitlyn Calabro, PhD, University of Florida*
11:45 AM–12:00 PM	**1208: Differential Histopathological and Proteomics Changes in Dorsal Root Ganglia (DRG) of *Cynomolgus Macaques* Following Intrathecal (IT) Delivery of "Empty" AAV9 Capsids, and AAV9 carrying Transcriptionally Active or Inert Cargo** *Ghiabe Guibinga, PhD, Novartis Institutes for BioMedical Research (NIBR)*

SCHEDULE (ALL TIMES LISTED IN ET)

THURSDAY, MAY 19, 2022

ORAL ABSTRACT SESSIONS

BALLROOM B
AAV Vectors—Clinical Studies
Co-chairs: Kathryn Wagner, MD, PhD, F. Hoffmann-La Roche and Christian Mueller, PhD, Sanofi Genzyme

10:15–10:30 AM	**1209: A Phase 2 Trial Evaluating Safety and Efficacy of Delandistrogene Moxeparvovec in Duchenne Muscular Dystrophy** *Teji Singh, MD, PhD, Sarepta Therapeutics, Inc.*
10:30–10:45 AM	**1210: Evaluation of Total Binding Antibodies Against rAAVrh74 in Patients with Duchenne Muscular Dystrophy** *Natalie Goedeker, CPNP, Washington University School of Medicine*
10:45–11:00 AM	**1211: Phase 1 Study of Gene Therapy in Late-onset Pompe Disease: Initial 104-Week Experience** *Edward Smith, MD, Duke University School of Medicine*
11:00–11:15 AM	**1212: Sustained Efficacy and Safety at Week 52 and up to Three Years in Adults With Glycogen Storage Disease Type iA (GSDIa): Results From a Phase 1/2 Clinical Trial of DTX401, an AAV8-mediated, Liver-directed Gene Therapy** *Rebecca Riba-Wolman, MD, University of Connecticut*
11:15–11:30 AM	**1213: Rationally Designed Cardiotropic AAV Capsid Demonstrates 30 Fold Higher Efficiency in Human vs Porcine Heart** *Tugba Guven-Ozkan, PhD, AskBio Biopharmaceutical*
11:30–11:45 AM	**1214: Safety, β-Sarcoglycan Expression, and Functional Outcomes From Systemic Gene Transfer of rAAVrh74. MHCK7.hSGCB in LGMD2E/R4** *Eric Pozsgai, Sarepta Therapeutics, Inc.*

SCHEDULE (ALL TIMES LISTED IN ET)

THURSDAY, MAY 19, 2022

ORAL ABSTRACT SESSIONS

ROOM 202
Delivery Technologies and *In Vivo* Gene Editing
Co-chairs: TJ Cradick, PhD, Excision BioTherapeutics, Inc and Daniela Cesana, PhD, SR-TIGET

10:15–10:30 AM	**1215: Durable Silencing of Pcsk9 by *In Vivo* Hit-and-run Epigenome Editing** *Martino Alfredo Cappelluti, IRCCS San Raffaele Scientific Institute*
10:30–10:45 AM	**1216: AAV-CRISPR/Cas9 Gene Editing is Therapeutic in a Novel, Humanized Mouse Model of *GUCY2D*-associated Cone Rod Dystrophy (CORD6)** *Russell Mellen, PhD, University of Florida*
10:45–11:00 AM	**1217: A Large Scale Exon Editing Solution for Treating ABCA4 Related Retinopathies** *Robert Bell, PhD, Ascidian Therapeutics*
11:00–11:15 AM	**1218: Highly Efficient and Safe *In Vivo* HSC Base Editing for the Treatment of Hemoglobinopathies** *Chang Li, PhD, University of Washington*
11:15–11:30 AM	**1219: Dual Editing and *in vivo* Selection at the *HBG* Promoter Using *MGMT-P140K* Cassette** *Mason Berger, Seattle Children's Research Institute*
11:30–11:45 AM	**1220: *In Vivo* Expansion Of Gene-targeted Hepatocytes Through Inhibition Of An Essential Gene** *Marco De Giorgi, PhD, Baylor College of Medicine*
11:45 AM–12:00 PM	**1221: Intravitreal Delivery of AAV2 - Exon-specific U1snRNA corrects *ELP1* Splicing and Rescues Retinal Degeneration in a Mouse Model of Familial Dysautonomia** *Anil Chekuri, PhD, Massachusetts Eye and Ear Infirmary*

SCHEDULE (ALL TIMES LISTED IN ET)

THURSDAY, MAY 19, 2022

ORAL ABSTRACT SESSIONS

ROOM 201
Breakthroughs in Neuromuscular and Hearing Disorders
Co-chairs: Scott Q. Harper, PhD, Ohio State University and Rachel M. Bailey, PhD, UT Southwestern

10:15–10:30 AM	**1222: CYP46A1 as a Relavant Target to Treat ALS Pathology Independent From its Origin** *Françoise PIGUET, PhD, Neurogencell, ICM*
10:30–10:45 AM	**1223: Gene Therapy for ALS by Specifically Overexpressing a Pleiotropic Chronokine, Secreted α-Klotho, in Skeletal Muscles** *Sergi Verdés, PhD Student*
10:45–11:00 AM	**1224: AAV-9 Mediated Delivery of RNA Targeting Systems Eliminates Hexanucleotide Repeat Expansions in *C9ORF72* ALS/FTD Models** *Jeannie Chew, PhD, Locanabio*
11:00–11:15 AM	**1225: TFRC-Targeted GAA Delivered as Gene Therapy Treats CNS and Muscle in Pompe Disease Model Mice** *Maria Praggastis, PhD, Regeneron*
11:15–11:30 AM	**1226: Preclinical Development of an AAV-based Gene Therapy (OTO-825) for Congenital Hearing Loss Due to GJB2 Deficiency** *Phillip Uribe, PhD, Otonomy*
11:30–11:45 AM	**1227: Adult AAV Gene Therapy Rescues Auditory Function in a Mouse Model of Human TMPRSS3 Recessive Deafness DFNB8/10** *Wan Du, MD, PhD, Harvard Medical School/Mass Eye and Ear*
11:45 AM–12:00 PM	**1228: A Knockdown and Replacement Strategy for the Treatment of Charcot Marie Tooth Type 2A** *Eileen Workman, University of Pennsylvania*

SCHEDULE (ALL TIMES LISTED IN ET)

THURSDAY, MAY 19, 2022

ORAL ABSTRACT SESSIONS

SALON G
Ophthalmic and Auditory Diseases
Co-chairs: Hemant Khanna, PhD, IVERIC Bio and Mariacarmela Allocca, PhD, Editas Medicine

10:15–10:30 AM	**1229: A Mutation-Independent CRISPR/Cas9-based 'Knockout and Replace' Strategy to Treat Rhodopsin-Associated Autosomal Dominant Retinitis Pigmentosa** *Chi-Hsiu Liu, PhD, Editas Medicine, Inc.*
10:30–10:45 AM	**1230: Development of an AAV-based Gene Therapy for the Ocular Phenotype of Friedreich's Ataxia** *Siddhant Gupte, University of Florida*
10:45–11:00 AM	**1231: SPVN06, a Novel Mutation-independent AAV-based Gene Therapy, Dramatically Reduces Vision Loss in the rd10 Mouse Model of Rod-cone Dystrophy and is Well Tolerated in a 1-month Pilot Safety Monkey Study** *Florence Lorget, SparingVision*
11:00–11:15 AM	**1232: Reduced Ocular Inflammation and Improved GFP Expression in Rabbits with Controlled Release of Adeno-Associated Virus from Degradable Hydrogel Implants** *Steven Lu, PhD, Ocular Therapeutix*
11:15–11:30 AM	**1233: Nonclinical *In Vivo* Expression, Durability of Effect, Biodistribution/Shedding, and Safety Evaluations Support Clinical Development of AK-OTOF (AAVAnc80-hOTOF Vector) for *OTOF*-mediated Hearing Loss** *Ann Hickox, PhD, Akouos, Inc.*
11:30–11:45 AM	**1234: Novel Capsid LSV1 has a Unique 3D Structure at the Loop Substitution Area - Confers Superior Retinal Transduction from Intravitreal Injection** *Cameron Baker, PhD, Adverum*
11:45 AM–12:00 PM	**1235: Development of Dual-PCDH15 AAV Gene Therapy for Usher Syndrome Type 1F Deafness and Blindness** *Maryna Ivanchenko, PhD, MD, Harvard Medical School*

SCHEDULE (ALL TIMES LISTED IN ET)

THURSDAY, MAY 19, 2022

ORAL ABSTRACT SESSIONS

ROOM 207
Cell-Based Cancer Immunotherapies III
Co-chairs: Saad Kenderian, MD, Mayo Clinic and Michael Milone, MD, PhD, University of Pennsylvania

10:15–10:30 AM	**1236: Optimized CD70-Targeted CAR Secreting a CD33-Targeted Bispecific T-cell Engager Overcomes Antigen Heterogeneity for Acute Myeloid Leukemia** *Mark Leick, MD, Massachusetts General Hospital Cancer Center*
10:30–10:45 AM	**1237: Safety Lead-in of Ph2 AML Study Results Using Zedenoleucel** *Mythili Koneru, MD, PhD, Marker Therapeutics*
10:45–11:00 AM	**1238: N-glycosylation Inhibition Hinders Immunosuppressive Activity of Tumor Microenvironment Cells and Improves CAR T Cell Efficacy** *Camilla Sirini, San Raffaele Scientific Institute*
11:00–11:15 AM	**1239: CAR T That Targets MUC1 Transmembrane Cleavage Product has Increased Persistence and Kills Low Antigen Cells** *Cynthia Bamdad, PhD, Minerva Biotechnologies*
11:15–11:30 AM	**1240: CD4 CAR T Cells Drive Extensive CD8 CAR T-cell Expansion, Leading to Severe Cytokine Release Syndrome** *Camilla Bove, Innovative Immunotherapies Unit, IRCCS San Raffaele Scientific Institute*
11:30–11:45 AM	**1241: Targeting Tumors and the Tumor Microenvironment with Banana Lectin Expressing T-cells** *Mary Kathryn K. McKenna, PhD, Baylor College of Medicine*
11:45 AM–12:00 PM	**1242: Preclinical Activity and Safety of UB-VV100, A Novel Lentiviral Vector Product Designed for Selective and Effective *In Vivo* Engineering of Therapeutic Anti-CD19 CART Cells for B-Cell Malignancies** *Alissa Brandes, PhD, Umoja Biopharma*

SCHEDULE (ALL TIMES LISTED IN ET)

THURSDAY, MAY 19, 2022

ORAL ABSTRACT SESSIONS

ROOM 204
Cell Therapies for Hematological Disorders
Co-chairs: Andre Larochelle, MD, PhD, NIH
and Punam Malik, MD, PhD, Cincinnati Children's Hospital Medical Center

10:15–10:30 AM	**1243: *Ex Vivo* Generated ProTcell™ Product Exhibits Fate Plasticity Between T and NK Lineages: An Opportunity for Innate and Adaptive Cell Therapy Strategies** *Pierre Gaudeaux, MS, Smart Immune*
10:30–10:45 AM	**1244: Hypoimmunemouse Primary Pancreatic Islet Cells Survive and Functionally Rescue Allogeneic Diabetic Mic** *Sonja Schrepfer, MD, PhD, Sana Biotechnology Inc*
10:45–11:00 AM	**1245: FVIII-Expressing Human Placental Cells Engraft in Multiple Organs and Provide FVIII Protein While Evading Induction of FVIII Inhibitors When Administered to Juvenile Sheep** *Brady Trevisan, Wake Forest Institute for Regenerative Medicine*
11:00–11:15 AM	**1246: Modeling Clinical Scale, Efficacious CRISPR-edited HSPC Therapies in Nonhuman Primates** *Jason Murray, PhD, Fred Hutchinson Cancer Research Center*
11:15–11:30 AM	**1247: Engineering T-cells To Prevent Acute Graft-Versus-Host Disease And Leukemia Relapse Following Allogeneic Stem Cell Transplantation** *Feiyan Mo, Baylor College of Medicine*
11:30–11:45 AM	**1248: Postnatal Boosting With FVIII-Expressing Human Placental Cells Supports Immune Tolerance was Induced During Prenatal Exposure, and is BTLA-Mediated** *Martin Rodriguez, BS, Wake Forest Institute for Regenerative Medicine*
11:45 AM–12:00 PM	**1249: Diphtheria Toxin Based Bivalent Anti-cMPL Immunotoxin Effectively and Safely Depletes Rhesus Hematopoietic Stem and Progenitor Cells** *Daisuke Araki, Cellular and Molecular Therapeutics Branch, NHLBI, NIH*

SCHEDULE (ALL TIMES LISTED IN ET)

THURSDAY, MAY 19, 2022

ORAL ABSTRACT SESSIONS

ROOM 206

Cell Therapy Product Engineering, Development or Manufacturing

Co-chairs: Isabelle Riviere, PhD, Memorial Sloan-Kettering Cancer Center and Maksim Mamonkin, PhD, Baylor College of Medicine

10:15–10:30 AM	**1250: Improved Anti-tumor Potency of CAR Gamma Delta T-cells Expanded with Human Platelet Lysate** *Norihiro Watanabe, PhD, Baylor College of Medicine*
10:30–10:45 AM	**1251: Base Editors as a Singular Platform for Polyfunctional Multiplex Engineering of T-cells for Cancer Immunotherapy** *Walker Lahr, University of Minnesota*
10:45–11:00 AM	**1252: Allogeneic CAR T Cells Derived From Younger Donor T Cells Have More Desirable T-cell Phenotype and Better *In Vitro* Functionality** *Meng-Yin Lin, Allogene Therapeutics*
11:00–11:15 AM	**1253: Enhanced CAR T-cell Generation by CD8-LV Through Alleviating Antiviral Mechanisms with mTOR Inhibitors** *Filippos Charitidis, MSc, Paul Ehrlich Institut*
11:15–11:30 AM	**1254: Generation of Off-the-shelf Allogeneic Hypoimmune Tregs** *Ivayla Gyurova, PhD, Sana Biotechnology*
11:30–11:45 AM	**1255: Dysfunctional Immune Synapses Restrain Anti DIPG Activity of CAR T-cells** *Jorge Ibanez, PhD, St. Jude Children's Research Hospital*
11:45 AM–12:00 PM	**1256: mRNA-based Gene Editing in Primary Human Muscle Stem Cells** *Christian Stadelmann, Muscle Research Unit, Experimental and Clinical Research Center,*

SCHEDULE (ALL TIMES LISTED IN ET)

THURSDAY, MAY 19, 2022

ORAL ABSTRACT SESSIONS

ROOM 102
Pharmacology/Toxicology Studies or Assay Development II
Co-chairs: Angela Lynch, PhD, ToxPlus Consulting, LLC and Eva Andres-Mateos, PhD, Atsena Therapeutics S, Inc

10:15–10:30 AM	**1257: A GLP-compliant Toxicology and Biodistribution Study of ADVM-062 (AAV.7m8-L-opsin), a Novel Gene Therapy Product Being Developed as a Potential Single Intravitreal Administration for the Treatment of Blue Cone Monochromacy** *Kelly Hanna, MS, Adverum Biotechnologies*
10:30–10:45 AM	**1258: Visium CytAssist: A Novel Platform for Spatial Transcriptomic Analysis of FFPE Sections Mounted on Standard Glass Slides** *Hardeep Singh, Senior Scientist, 10x Genomics*
10:45–11:00 AM	**1259: Development of an AAV-Based Gene Therapy for Children With Congenital Hearing Loss Due to Otoferlin Deficiency (DB-OTO)** *Orion Keifer Jr, MD, PhD, Decibel Therapetics*
11:00–11:15 AM	**1260: Long-read Sequencing and Multiplex ddPCR for Viral Vector Genome Integrity Identification** *David Dobnik, PhD, National Institute of Biology*
11:15–11:30 AM	**1261: Toxicity of Frataxin Overexpression in Nonhuman Primates Treated with Intravenous and MRI-guided Intracerebellar Infusion of an AAV Vector** *Christian Hinderer, University of Pennsylvania*
11:30–11:45 AM	**1262: ASC618, a Second Generation of FVIII Gene Therapy for Hemophilia A, Exhibits Major Transduction and Transgene Expression in the Target Liver Tissues: Results of IND-enabling Pharmacokinetics Studies in Mice and Non-Human Primates** *Chengtao Yang, ASC Therapeutics*
11:45 AM–12:00 PM	**1263: Safety and Biodistribution of VTX-801, an AAV3B Gene Therapy Vector, in Healthy Cynomolgus Monkeys** *Blanche Tamarit, Vivet Therapeutics*

ABSTRACT REVIEWERS

A1 - RNA Virus Vectors

Review Chair: Patricia Devaux, PhD – *Mayo Clinic*

Alessandra Biffi, MD – *DFCI/BCH Cancer and Blood Disorders Center*
Douglas Jolly, PhD – *Abintus Bio Inc.*
John Tisdale, MD – *NIH, NHLBI*
Andrew Wilber, PhD – *Southern Illinois University School of Medicine*
Mario Amendola, PhD – *Genethon*

A2 - AAV Vectors—Virology and Vectorology

Review Chair: Nicole Paulk, PhD – *UC San Francisco*

Daniel Lipinski, MSc,DPhil – *Medical College of Wisconsin*
Anna Maurer, PhD – *UC Berkeley*
Amanda Dudek, PhD – *Stanford University*
Rafael Yáñez-Muñoz, PhD – *Royal Holloway University of London*
Isabelle Richard, PhD – *Genethon*

A3 - AAV Vectors—Preclinical and Proof-of-Concept Studies

Review Chair: Heather Gray-Edwards, DVM, PhD – *UMass Chan Medical School*

Brian Bigge, PhD – *University of Manchester*
Philip Tai, PhD – *UMass Chan Medical School*
Allison Bradbury, PhD – *Nationwide Children's Hospital*
Juliette Hordeaux , DVM, PhD, DECVP – *University of Pennsylvania*
Rita Batista, PhD – *UMass Chan Medical School*

A4 - AAV Vectors—Clinical Studies

Review Chair: Kathryn Wagner, MD, PhD – *F. Hoffmann-La Roche*

Carolyn Lutzko, PhD – *Cincinnati Childrens Hospital Medical Center*
Katherine High, MD – *Ask Bio*
Chris Mueller, PhD – *Sanofi Genzyme*
Ana Buj Bello, MD, PhD – *Genethon*
Gallia Levy, MD, PhD – *Spark Therapeutics*

B - Gene Targeting and Gene Correction

Review Chair: Maria Grazia Roncarolo, MD – *Stanford University*

Paula Cannon, PhD – *University of Southern California*
TJ Cradick, PhD – *Excision BioTherapeutics*
Mara Pavel-Dinu, PhD – *Stanford University*
Giuliana Ferrari, PhD – *HSR-TIGET, Fondazione S. Raffaele*
Luca Biasco, PhD – *AVROBIO*

C - Oligonucleotide Therapeutics

Review Chair: Paloma Giangrande, PhD – *Wave Life Sciences*

Marcin Kortylewski, PhD – *City of Hope Comprehensive Cancer Center*
Michelle Hastings, PhD – *Rosalind Franklin University*
Janaiah Kota, PhD – *Indiana University School of Medicine*
Shen Shen, PhD – *Vertex Pharmaceuticals*
Mark Behlke, MD, PhD – *IDT*

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

Review Chair: Carol Miao, PhD – *Seattle Children's Research Institute*

Dexi Liu, PhD – *University of Georgia*
Julie Champion (Synthetic), PhD – *Georgia Tech School of Chemical and Biomolecular Engineering*
Kenya Kamimura (Physical), MD, PhD – *Niigata University*
Richard Heller (Physical), PhD – *University of South Florida*
David Dean (Physical), PhD – *University of Rochester*
Fan Yuan (Synthetic), PhD – *Duke University*

E1 - Metabolic, Storage, Endocrine, Liver and Gastrointestinal Diseases

Review Chair: Nicola Brunetti-Pierri, MD – *Telethon Institute of Genetics and Medicine*

Gloria Gonzalez-Aseguinolaza, PhD – *Vivet Therapeutics*

Charles Venditti, MD, PhD – *NIH*

Stephanie Cherqui, PhD – *UC San Diego*

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

Andres Muro, PhD – *International Centre for Genetic Engineering and Biotechnology (ICGEB)*

E2 - Cardiovascular and Pulmonary Diseases

Review Chair: Uta Griesenbach, PhD – *Imperial College Faculty of Medicine*

Kate Excoffon (Respiratory), PhD – *Spirovant Sciences, Inc.*

Patrick Sinn, PhD – *University of Iowa*

Margaret Sleeper (Cardio), VMD, DACVIM – *University of Florida*

Mai ElMallah, MD – *Duke University Medical School*

Gerry McLachlan, PhD – *University of Edinburgh*

E3 - Neurologic Diseases

Review Chair: Allison Bradbury – *Ohio State University*

Kathrin Meyer, PhD – *Nationwide Children's Hospital*

Miguel Sena-Esteves, PhD – *UMass Chan Medical School*

Kleopas Kleopa, MD, PhD – *Cyprus Institute of Neurology and Genetics*

Laura Ferraiuolo, PhD – *Sheffield University*

Gad Vatine, PhD – *University of Negev*

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

E4 - Ophthalmic and Auditory Diseases

Review Chair: Luk Vandenberghe, PhD – *Harvard University*

Litia Carvalho, PhD – *MGH*
Deniz Dalkara, PhD – *Institute de lab vision, Paris*
Hemant Khanna, PhD – *University of Massachusetts*
Yen-Fu Cheng, MD, PhD – *Veterans Hospital Taipei*
Casey Maguire, PhD – *Massachusetts General Hospital*

E5 - Musculo-skeletal Diseases

Review Chair: Dongsheng Duan, PhD – *University of Missouri School of Medicine*

Annemieke Aartsma-Rus, PhD – *Leiden University Medical Center*
Bruce Smith, VMD, PhD – *Auburn University*
Louise Rodino-Klapac, PhD – *Sarepta Therapeutics*

E6 - Cancer - Immunotherapy, Cancer Vaccines

Review Chair: Chiara Bonini, MD – *Universita Vita Salute San Raffaele*

Monica Casucci, PhD – *San Raffaele Scientific Institute*
Ann Leen, PhD – *Baylor College of Medicine - CAGT*
Barbara Savoldo, MD, PhD – *UNC Lineberger Comprehensive Cancer Center*
Michael Hudecek, MD – *University Hospital Wurzburg*
Sarwish Rafiq, PhD – *Emory University*

E7 - Cancer—Oncolytic Viruses

Review Chair: Melissa Kotterman, PhD – *4D Molecular Therapeutics (4DMT)*

Marta Alonso, PhD – *University Hospital of Navarra*
Evanthia Galanis, MD – *Mayo Clinic*
Deepak Verma, PhD – *Johns Hopkins University*
Paola Grandi, PhD, MBA – *CG Oncology*
Sunil S. Raikar, MD – *Emory University*

E8 - Cancer—Targeted Gene and Cell Therapy

Review Chair: Michael Milone, MD, PhD – *University of Pennsylvania*

Masato Yamamoto, MD, PhD – *University of Minnesota*

Daniel Abate-Daga, PhD H – *Lee Moffitt Cancer Center and Research Institute*

Barbra Sasu, PhD – *Allogene*

Saad Kenderian, MB, ChB – *Mayo Clinic*

Irina Balyasnikova, PhD – *Northwestern University Feinberg School of Medicine*

E9 - Hematologic and Immunologic Diseases

Review Chair: Juan Bueren, PhD – *CIEMAT/CIBERER/Fundacion Jiminez Diaz*

Bernhard Gentner, MD, PhD – SR-TIGET

Donald Kohn, MD – *UCLA*

Anne Galy, PhD – *GENETHON*

Claire Booth, MBBS, PhD – *UCL Great Ormond Street Institute of Child Health*

F - Immunological Aspects of Gene Therapy and Vaccines

Review Chair: Allison Keeler-Klunk, PhD – *UMass Chan Medical School*

Julie Crudele, PhD – *University of Washington*

Gwladys Gernoux, PhD – *Nantes University*

Chengwen Li, MD, PhD – *UNC Chapel Hill*

Oumeya Adjali, MD, PhD – *INSERM U1089*

Ying Kai Chan, PhD – *Harvard University*

G - Cell Therapies

Review Chair: Punam Malik, MD, PhD – *Cincinnati Childrens Hospital Medical Center*

Grace Almeida-Porada, MD, PhD – *Wake Forest of Medicine*

Andre Larochelle, MD, PhD – *NIH*

Jan Nolta, PhD – *UC Davis*

Alice Tarantal, PhD – *UC Davis*

Stefano Rivella, PhD – *Children's Hospital of Philadelphia*

H1 - Vector Product Engineering, Development or Manufacturing

Review Chair: H. Trent Spencer, PhD – *Emory University School of Medicine*

Johannes van der Loo, PhD – *Children's Hospital of Philadelphia*
John Gray, PhD – *Vertex Pharmaceuticals*
Anindya Dasgupta, PhD – *Expression Manufacturing, LLC*
Kerry Dooriss, PhD – *City of Hope Comprehensive Cancer Center*

H2 - Cell Therapy Product Engineering, Development or Manufacturing

Review Chair: Adrian Gee, PhD – *Baylor College of Medicine*

Maksim Mamonkin, PhD – *Baylor College of Medicine*
Daniela Bischof, PhD – *Indiana University School of Medicine*
Katy Rezvani, MD, PhD – *MD Anderson Cancer Center*
Isabelle Riviere, PhD – *Memorial Sloan-Kettering Cancer Center*
Edwin Horwitz, MD, PhD – *Emory University School of Medicine*

H3 - Pharmacology/Toxicology Studies or Assay Development

Review Chair: Eva Andres-Mateos, PhD – *Atsena Therapeutics, Inc.*

Angela Lynch, PhD – *ToxPlus Consulting, LLC*
Sarah Wassmer, PhD – *Gene Therapy Directorate*
Heikki Turunen, PhD – *Dyno Therapeutics*
Linda Couto, PhD – *Atsena Therapeutics, Inc.*
Caner Gunaydin, PhD

I - Gene and Cell Therapy Trials in Progress

Review Chair: Kevin Flanigan, MD – *Nationwide Children's Hospital*

Diana Bharucha-Goebel, MD – *NIH/NINDS and Children's National Hospital*
Barry J. Byrne, MD, PhD – *University of Florida*
Helen E. Heslop, MD, DSc – *Baylor College of Medicine*
Carsten Bonnemann, MD – *NINDS/NIH*

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