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• TCRab+ T-cell/CD19+ B-cell depleted hematopoietic grafts in combination with JSP191 to treat Fanconi anemia

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

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

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

I would like to extend a warm welcome to Michel Sadelain, M.D., Ph.D., and Robert L. Martuza, M.D., who will present the George Stamatoyannopoulos Memorial Lecture on Wednesday and the Presidential Symposium on Thursday, respectively. I would also like to take this opportunity to congratulate Drs. Carl June, Michel Sadelain, Larry Corey, Kathleen Neuzil, Marcela Maus, Benjamin Kleinstiver, Natalia Gomez-Ospina, Annarita Miccio, and Jerry Mendell on the awards they will so deservedly receive.

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

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

Finally, I would like to thank everyone involved in the planning and execution of our Annual Meeting, especially the Program Committee, Scientific and Education Committees, abstract reviewers, staff, and all of our volunteers.

Their sustained support, dedication, and hard work throughout the year now comes to fruition in what is sure to be a spectacular meeting.

Sincerely,

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

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

Continuing Medical Education
The ASGCT Board of Directors decided that the Society will not offer CME credit for the 24th Annual Meeting.

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

Education Methods + Materials
Educational Objectives
At the conclusion of the activity, the participant should be able to:

- Provide advice to patients who inquire about the potential of gene and cell therapy or the availability of open clinical trials, based on their exposure to the current clinical trials in gene and cell therapies.

- Better instruct their students in medical school and other health venues using the state-of-the-art basic science and clinical trials data presented at the meeting.

- Use the latest advances in gene and cell therapy to enhance their research mission, as physician scientists conducting basic and clinical research.

- Demonstrate improved regulatory compliance in conducting gene and cell therapy clinical trials, through exposure to NIH and FDA faculty during the educational program.

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

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

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

Dates
The ASGCT 24th Annual Meeting will begin on the morning of Tuesday, May 11, 2021 and continue through 2 p.m. (ET) on Friday, May 14, 2021. Exhibits will be open 24/7, Tuesday, May 11 through Friday, May 14. All content will be available to registrants on-demand for 30 days following the conclusion of the meeting.
Thank you to the following individuals for serving on the ASGCT Program Committee and helping with the overall development of the ASGCT 24th Annual Meeting.

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

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

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

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

**ASGCT President Elect**
Beverly L. Davidson, Ph.D. – Children’s Hospital of Philadelphia

**ASGCT Secretary + Abstract Chair**
Terence R. Flotte, MD – University of Massachusetts Medical School

**ASGCT Program Committee Members**
Helen Heslop, Ph.D. – Baylor College of Medicine
Hans-Peter Kiem – Fred Hutchinson Cancer Research Center
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JOIN US AT OUR ASGCT ANNUAL MEETING SHOWCASE SESSION

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

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

Presented by Ly Nguyen-Jatkoe, PhD
Executive Director, Americas
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24th Annual Virtual Meeting
OUTSTANDING ACHIEVEMENT AWARD

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

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

The Outstanding Achievement Award is Sponsored by:

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SONIA SKARLATOS PUBLIC SERVICE AWARD

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

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

OUTSTANDING NEW INVESTIGATOR AWARDS

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

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

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

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

The Outstanding New Investigator Award is Sponsored by:

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Congratulations to the Following Individuals for Receiving an ASGCT Award!

**JERRY MENDELL AWARD FOR TRANSLATIONAL SCIENCE:**

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

**EXCELLENCE IN RESEARCH AWARDS**

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

*The Excellence in Research Awards are sponsored by:*
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GeneWerk
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  - www.milttenyibiotec.com
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- **Mission Bio**
  - South San Francisco, CA
  - www.missionbio.com
  - Mission Bio helps unravel the complexity of cancer with the Tapestry Single-cell Multi-omics Platform. Discover how Tapestry enables accurate characterization of your cell and gene therapy candidates for more effective therapeutic strategies.

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  - www.mogrify.co.uk
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- **myriade**
  - Paris, France
  - www.myriadelab.com/en
  - VIDEODROP by Myriade makes it possible to measure the Size & Concentration of lentivirus & adenovirus in the range of 70 nm & 10 microns, in real time (40s), in a single drop (5µL), without labelling, without purification if required on viscous samples

- **Nordmark Biochemicals**
  - Uetersen, Germany
  - www.nordmark-pharma.de/en/home/
  - Nordmark Biochemicals offers products for cell isolation, including Collagenase NB 6 GMP Grade for stem cell isolation and animal-free Collagenase AF-1 GMP Grade. We provide translational - research and GMP Grade-collagenases for research to clinic.

- **National Gene Vector Biorepository (NGVB) & NHLBI Primate Center for Gene Therapy**
  - Indianapolis, IN
  - www.ngvbc.org
  - NHLBI funds 2 gene therapy resources: The NGVB provides reagents, archiving and testing services (NGVBCC.org). The PCGT assists in evaluating the safety and efficiency of gene transfer strategies (pcgt.ucdavis.edu). Services are free when eligible!

- **National Organization for Rare Disorders (NORD)**
  - Danbury, CT
  - www.rarediseases.org
  - NORD is a patient advocacy organization dedicated to individuals with rare diseases. NORD and its members are committed to the identification, treatment, and cure of rare disorders through education, advocacy, research, and patient services.

- **Mission Bio**
  - South San Francisco, CA
  - www.missionbio.com
  - Mission Bio helps unravel the complexity of cancer with the Tapestry Single-cell Multi-omics Platform. Discover how Tapestry enables accurate characterization of your cell and gene therapy candidates for more effective therapeutic strategies.

- **myriade**
  - Paris, France
  - www.myriadelab.com/en
  - VIDEODROP by Myriade makes it possible to measure the Size & Concentration of lentivirus & adenovirus in the range of 70 nm & 10 microns, in real time (40s), in a single drop (5µL), without labelling, without purification if required on viscous samples
ANNUAL MEETING EXHIBITORS

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

Novartis & Novartis Medical
Deerfield, IL

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

PerkinElmer
Waltham, MA
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PerkinElmer is a global leader committed to innovating for a healthier world. Our dedicated team of 8,000 employees worldwide are passionate about providing customers with an unmatched experience as they help solve critical issues.

PHC Corporation of North America
Wood Dale, IL
www.phchd.com/us/biomedical

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

Polyplus-transfection
Illkirch, France
www.polyplus-transfection.com

Polyplus-transfection is the leading supplier of a key component for viral vectors manufacturing for Gene & Cell Therapy. We provide GMP-grade transfection reagents, a scientific & regulatory support and in vitro & in vivo transfection reagents.

Pall Corporation
Westborough, MA
www.pall.com/en/biotech.html

Bioprocessing, integrated automated end-to-end systems and single-use solutions for gene and cell therapies. Bioreactors through downstream purification and buffer management, to transportation, automated freeze - thawing, to formulation & filling.

Porton Biologics
Suzhou, China
www.portonbio.com

Porton Biologics Ltd. is located in China. As a subsidiary of Porton Pharma Solutions Ltd, Portonbio provides Gene and Cell Therapy CDMO services for global innovators from the early stage to commercial production.
Virtual Exhibit Hall open 24hrs online. Exhibitors available:
Tues: 10:30a-12p, 2-3:30p, 5:15-6:45p | Wed. & Thr.: 10:45a-12:15p, 2-3:30p, 5:15-6:45p | Fri.: 10:45a-12:15p

**Precision For Medicine**
Bethesda, MD
www.precisionformedicine.com

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

**Sarepta Therapeutics**
Cambridge, MA
www.sarepta.com/

At Sarepta, we are working with urgency to develop breakthrough therapies to treat genetic diseases.

Currently, we have more than 40 investigational therapies in various stages of development—many already in late-stage clinical trials.

**Sartorius**
Goettingen, Germany
www.sartorius.com

BIA Separations develops and manufactures CIM® monolithic chromatographic columns for purification and analysis of large biomolecules.

Biological Industries is a leading supplier for cell culture media development and manufacturing. Part of Sartorius

**ScaleReady**
Saint Paul, MN
www.scaleready.com

ScaleReady is a joint venture between Bio-Techne, Fresenius Kabi, and Wilson Wolf. Bringing together proven tools and technologies for cell culture, cell activation, gene editing, and cell processing.

**SCIEX**
Framingham, MA
www.sciex.com/applications/pharma-and-biopharma/gene-therapy-research

SCIEX CE and LC-MS technology can benefit your laboratory with flexible workflows to get the answers you need. Discover ways to accelerate your gene therapy and oligo development by getting the right answers through precise analytics you can trust.

**PROGEN**
Heidelberg, Germany
www.progen.com

As exclusive manufacturer of AAV antibodies & ELISAs, PROGEN provides high-quality tools for gene therapy R&D. We offer AAV ELISAs for quantification of different serotypes & specific antibodies to study assembly, capsid formation & neutralization.

**Refeyn**
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REFEYN | Introducing mass photometry, a revolutionary new technology for analysing biomolecules.
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www.unchainedlabs.com
Here's the deal. We’re all about helping researchers break free from tools that just don’t cut it. Unleashing problem-tackling products that make a huge difference in the real science they do every day. Check out our epic gene therapy tools!

Ultragenyx Pharmaceutical Inc.
Novato, CA
www.ultragenyx.com/
Ultragenyx is a biopharmaceutical company committed to bringing to patients novel products for the treatment of serious rare and ultra-rare genetic diseases. The company has built a diverse portfolio of approved therapies and product candidates.

Touchlight AAV
San Sebastián, Spain
www.touchlightaav.com
Touchlight AAV is advancing gene therapy by offering its synthetic, linear, double stranded DNA as the new industry standard for transfection-based AAV production.

SIRION Biotech
Martinsried/Planegg, Germany
www.sirion-biotech.com
SIRION Biotech is Europe’s leading supplier of viral vector technologies (AAV, LV, AV). Our viral vector know-how enables engineering and manufacture of a new generation of optimized clinically compliant vectors for late preclinical applications.

Terumo Blood and Cell Technologies
Lakewood, CO
www.terumobct.com/cell-therapy-technologies
Terumo Blood and Cell Technologies, a global leader in blood component, therapeutic apheresis and cellular therapy technologies, believes in the potential of blood and cells to do even more for patients than they do today.

Solentim
Wimborne, United Kingdom
www.solentim.com
Solentim is the trusted global leader in workflows for antibody and cell-based therapies. Our assurance rich technologies enable the isolation, growth and characterization of high value cells while our data driven platform enables smarter decisions.

Spectradyne
Signal Hill, CA
www.nanoparticleanalyzer.com
Spectradyne LLC, has developed a nanoparticle analyzer based on Resistive Pulse Sensing, which overcomes the limitations found with light scattering-based methods with respect

Ultradex
pharmaceutical
Ultragenyx Pharmaceutical Inc.
Novato, CA
www.ultragenyx.com/
Ultradex is a biopharmaceutical company committed to bringing to patients novel products for the treatment of serious rare and ultra-rare genetic diseases. The company has built a diverse portfolio of approved therapies and product candidates.

Unchained Labs
Pleasanton, CA
www.unchainedlabs.com
Here's the deal. We're all about helping researchers break free from tools that just don’t cut it. Unleashing problem-tackling products that make a huge difference in the real science they do every day. Check out our epic gene therapy tools!
Virtual Exhibit Hall open 24hrs online. Exhibitors available:
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**VectorBuilder.com**

**VectorBuilder**  
Chicago, IL  
www.en.vectorbuilder.com

As a global pioneer in custom DNA vectors and recombinant viruses, VectorBuilder’s revolutionary online-to-offline (O2O) platform provides a powerful one-stop solution to all the vector and virus needs in the life sciences.

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www.viralgenvc.com

Viralgen is gene therapy CDMO, specializing in AAV therapeutics, created to help broaden the access to life-saving therapeutics and contribute to the advancement of health and human welfare around the world.

**VERTEX**

**Vertex Pharmaceuticals**  
Boston, MA  
www.vrtx.com

Vertex is a global biotechnology company that invests in scientific innovation to create transformative medicines for people with serious diseases, including cystic fibrosis, hemoglobinopathies, type 1 diabetes, Duchenne muscular dystrophy and more.

**VGXI, Inc.**  
The Woodlands, TX  
www.vgxii.com

VGXI is a leading contract manufacturer of DNA-based pharmaceuticals with nearly 20 years experience providing high quality GMP products to clinical trials worldwide. Uses include DNA vaccines, immunotherapies, and cell & gene therapy applications.

**Vigene Biosciences**  
Rockville, MD  
www.vigenebio.com

Vigene Biosciences is a leader in viral vector-based gene delivery for life science research, gene therapy and cell therapy applications. Vigene features integrated plasmid and viral vector production and analytical service offerings.

**VRL Laboratories**  
San Antonio, TX  
www.vrl.net

For more than 30 years, VRL has been in business as a diagnostic virus laboratory. Our knowledgeable staff and the experience it represents ensures our clients, reliable results and fast turn around times.

**Wyatt Technology**  
Santa Barbara, CA  
www.wyatt.com

Wyatt Technology offers unique solutions for gene therapy products including AAV, adenovirus, lentivirus and LNP-RNA. Our products quantify critical quality attributes such as identity, titer, purity and payload content (Vg/Cp or RNA fraction).

**YECURIS™**

**Yecuris**  
Portland, OR  
www.yecuris.com

Humanized FRG mice have helped gene therapy groups through optimization, verification of target specificity & evaluation of off-target effects in a human-relevant setting. When speed & accuracy are critical to your success, we’re here to help.
TUESDAY, MAY 11, 2021

<table>
<thead>
<tr>
<th>Time</th>
<th>Digital Abstract Presentations</th>
<th>Exhibit Hall</th>
<th>Education Sessions</th>
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</table>
| ALL DAY ACCESS|                                | Connect with Exhibitors: | • Gene Therapy for Hematologic Disorders  
Co-Chairs: John Tisdale, M.D. and Matthew Porteus, M.D., Ph.D.  
• Predictive Animal Models for Preclinical Testing of Gene Immunotherapies  
Co-Chairs: Renata Stripecke, Ph.D. and Satiro De Oliveira, M.D.  
• Stem Cell Expansion  
Chair: Mitchell Horwitz, M.D.  
Scientific Symposia  
• Cutting Edge Gene and Cell Therapy Research in Japan  
(Organized by JSGCT)  
Co-Chairs: Noriyuki Kasahara, M.D., Ph.D. and Takafumi Nakamura, Ph.D.  
• Gene Therapy Development Challenges and Opportunities in Low- and Middle-Income Countries (Organized by the Global Outreach Committee)  
Co-Chairs: Kenneth Cornetta, M.D. and Jayandharan Rao, Ph.D.  
• Genome Editing - Clinical and Preclinical Updates (Organized by the Genome Editing Committee)  
Co-Chairs: Benjamin Kleinstiver, Ph.D. and Angela Whatley, Ph.D.  
• Payment Policies for Non-Policy Specialists: Joining the Conversation (Organized by the Commercialization Committee)  
Co-Chairs: Mark Skinner, J.D. and Jeremy Allen  
• Recent Advances and Future Directions of Gene and Cellular Therapies in Immune Oncology (Organized by the Cancer Gene and Cell Therapy Committee)  
Co-Chairs: Robert Sobol, M.D. and Katy Rezvani, M.D., Ph.D.  

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<tr>
<th>Time</th>
<th>Chat Lounge Networking</th>
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<td>9 - 10 AM</td>
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10 - 11:45 AM

SPECIAL SYMPOSIUM
• COVID-19: Vaccines to the Rescue
# Schedule AT A GLANCE

**ALL TIMES LISTED IN EDT**

## TUESDAY, MAY 11, 2021

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<th>Time</th>
<th>Event</th>
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<tr>
<td>10:30 - 12 PM</td>
<td><strong>EXHIBITOR SHOWCASES</strong>&lt;br&gt;• Catalent Cell &amp; Gene Therapy - 10:30-11:15&lt;br&gt;• STEMCELL Technologies - 10:30-11:15&lt;br&gt;• Aldevron - 11:15-12&lt;br&gt;• MilliporeSigma - 11:15-12</td>
</tr>
<tr>
<td>12 - 1 PM</td>
<td><strong>FIRESIDE CHAT</strong>&lt;br&gt;Jennifer Doudna, Ph.D., UC Berkeley</td>
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<tr>
<td>1:30 - 3:30 PM</td>
<td><strong>MENTOR MEET-UP EVENT</strong>&lt;br&gt;Sponsored by Caribou Biosciences, Dyno Therapeutics, Rocket Pharmaceuticals, and Terumo Blood and Cell Technologies</td>
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<tr>
<td>2:00 - 3:30 PM</td>
<td><strong>INDUSTRY SPONSORED SYMPOSIA</strong>&lt;br&gt;• Corning Incorporated&lt;br&gt;• Maxcyte, Inc.&lt;br&gt;• Sarepta Therapeutics, Inc.&lt;br&gt;• Terumo Blood and Cell Technologies</td>
</tr>
<tr>
<td>3:30 - 5:15 PM</td>
<td><strong>EDUCATION SESSIONS</strong>&lt;br&gt;• AAV Vectors from Basic Biology to Clinical Application and Back&lt;br&gt;  <em>Co-Chairs: Hildegard Buning, Ph.D., and Alberto Auricchio, M.D.</em>&lt;br&gt;  3:30 PM - 5:15 PM&lt;br&gt;• Gene Therapy in Cancer&lt;br&gt;  <em>Co-Chairs: Rayne Rouce, M.D. and Renata Stripecke, Ph.D.</em>&lt;br&gt;  3:30 PM - 5:15 PM&lt;br&gt;• In Vivo Gene Editing&lt;br&gt;  <em>Co-Chairs: Juliana Alvarez Argote, M.D. and Blythe Sather, Ph.D.</em>&lt;br&gt;  3:30 PM - 5:15 PM&lt;br&gt;• Issues in gene therapy: Considerations for efficient development and access&lt;br&gt;  <em>Chair: John Tisdale, M.D.</em>&lt;br&gt;  3:30 PM - 5:15 PM</td>
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<tr>
<th>Time</th>
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<tr>
<td>3:30 - 5:15 PM</td>
<td><strong>PLENARY SESSION</strong></td>
<td>• Career Development Award Presentations</td>
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<tr>
<td>5:30 - 7:30 PM</td>
<td><strong>ABSTRACT SESSIONS</strong></td>
<td>• Advances in Ex Vivo Modified Cell Therapies&lt;br&gt;  <em>Co-Chairs: Adrian Gee, Ph.D. and Joseph Gold, Ph.D.</em>&lt;br&gt;  5:30 PM - 7:15 PM</td>
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<td>• Cancer - Oncolytic Viruses&lt;br&gt;  <em>Co-Chairs: Paola Grandi, Ph.D. and Melissa Kotterman, Ph.D.</em>&lt;br&gt;  5:30 PM - 7:15 PM</td>
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<td>• Delivery Technologies and CRISPR for Therapeutics&lt;br&gt;  <em>Co-Chairs: Nicole Gaudelli, Ph.D. and Alejandro Chavez, M.D., Ph.D.</em>&lt;br&gt;  5:30 PM - 7:00 PM</td>
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<td>• Development of AAV Capsid Variants&lt;br&gt;  <em>Co-Chairs: Deep Bhattacharya, Ph.D. and Christine Le Bec, Ph.D.</em>&lt;br&gt;  5:30 PM - 7:15 PM</td>
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<td>• Gene Therapy for Inborn Errors of Metabolism&lt;br&gt;  <em>Co-Chairs: Giuseppe Ronzitti, Ph.D. and Gloria Gonzalez-Aseguinolaza, Ph.D.</em>&lt;br&gt;  5:30 PM - 7:15 PM</td>
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<td>• Genetic Blood and Immune Disorders&lt;br&gt;  <em>Co-Chairs: Denise Sabatino, Ph.D. and Cyndi Dunbar, M.D.</em>&lt;br&gt;  5:30 PM - 7:15 PM</td>
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<td>• Musculo-Skeletal Diseases&lt;br&gt;  <em>Co-Chairs: Olivier Danos, Ph.D. and Rita Perlingeiro Ph.D.</em>&lt;br&gt;  5:30 PM - 7:15 PM</td>
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<td>• Novel AAV Capsids for Brain, Eye and Muscle Tissues&lt;br&gt;  <em>Co-Chairs: Nicole Paulk, Ph.D. and Daniel Lipinski, D.Phil.</em>&lt;br&gt;  5:30 PM - 7:15 PM</td>
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<td>• Preclinical Gene Therapy for Neurologic Diseases I&lt;br&gt;  <em>Co-Chairs: Ana Rita Batista, Ph.D. and Martin Hicks, Ph.D.</em>&lt;br&gt;  5:30 PM - 7:15 PM</td>
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<tr>
<th>Time</th>
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<th>Networking Roulette</th>
<th>Tools and Technology Forum I</th>
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<tr>
<td>5:15 - 6:45 PM</td>
<td>- Precision for Medicine</td>
<td>- Sponsored by Teknova</td>
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<td>- Thermo Fisher Scientific</td>
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<td>5:15 - 6:15 PM</td>
<td><strong>NETWORKING ROULETTE</strong></td>
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<tr>
<td>5:15 - 7 PM</td>
<td><strong>TOOLS AND TECHNOLOGY FORUM I</strong></td>
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## ALL TIMES LISTED IN EDT

### WEDNESDAY, MAY 12, 2021

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

**WEDNESDAY, MAY 12, 2021**

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<td>10 - 11:45 AM</td>
<td><strong>SPECIAL SYMPOSIUM</strong>&lt;br&gt;• Jerry Mendell Award for Translational Science Symposium&lt;br&gt;Supported by Dr. Suku and Ann Nagendran</td>
</tr>
<tr>
<td>10:45 - 12:15 PM</td>
<td><strong>EXHIBITOR SHOWCASES</strong>&lt;br&gt;• Unchained Labs - 10:45 AM - 11:30 AM&lt;br&gt;• Wyatt Technology - 10:45 AM - 11:30 AM&lt;br&gt;• Bio-Rad Laboratories - 11:30 AM - 12:15 PM&lt;br&gt;• Halo Labs - 11:30 AM - 12:15 PM</td>
</tr>
<tr>
<td>12:15 - 2 PM</td>
<td><strong>GEORGE STAMATOYANNOPOULOS MEMORIAL LECTURE AND PRESENTATION OF THE EXCELLENCE IN RESEARCH AWARDS</strong>&lt;br&gt;Sponsored by REGENXBIO</td>
</tr>
<tr>
<td>2 - 3:30 PM</td>
<td><strong>INDUSTRY SPONSORED SYMPOSIA</strong>&lt;br&gt;• Cytiva&lt;br&gt;• Miltenyi Biotec&lt;br&gt;• Vertex Pharmaceuticals Inc.&lt;br&gt;• Voyager Therapeutics</td>
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<tr>
<td>2 - 3 PM</td>
<td><strong>CHAT LOUNGE NETWORKING</strong></td>
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<tr>
<td>3:30 - 5:15 PM</td>
<td><strong>PLENARY SESSION</strong>&lt;br&gt;Outstanding New Investigator Symposium&lt;br&gt;Sponsored by BURROUGHS WELLCOME FUND</td>
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<tr>
<td>5:15 - 7:15 PM</td>
<td><strong>ABSTRACT SESSIONS</strong>&lt;br&gt;• AAV Biology, Engineering, Immunology and Animal Modeling&lt;br&gt;Co-Chairs: Allison Bradbury, Ph.D. and Miguel Sena-Esteves, Ph.D.&lt;br&gt;5:30 PM - 7:15 PM&lt;br&gt;• CAR Modified Cellular Therapies&lt;br&gt;Co-Chairs: Maria-Grazia Roncarolo, M.D. and Pietro Genovese, Ph.D.&lt;br&gt;5:30 PM - 7:00 PM&lt;br&gt;• Gene Therapies for Hemoglobinopathies&lt;br&gt;Co-Chairs: John Chapin, M.D. and Pankaj Mandal, Ph.D.&lt;br&gt;5:30 PM - 7:15 PM</td>
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<td>Time</td>
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<tr>
<td>5:15 - 7:15 PM</td>
<td><strong>ABSTRACT SESSIONS</strong></td>
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<td></td>
<td>• Immune Responses to AAV Vectors</td>
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<td>Co-Chairs: Ying Kai Chan, Ph.D. and Manish Muhuri, Ph.D.</td>
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<td>5:30 - 7:00 PM</td>
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<td>• Novel Factors in AAV Transduction and AAV Genomes</td>
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<td>Co-Chairs: David Markusic, Ph.D. and Amanda Dudek, Ph.D.</td>
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<td>5:30 PM - 7:15 PM</td>
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<td>• Preclinical Gene Therapy for Neurologic Disorders II</td>
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<td>Co-Chairs: Gwladys Gernoux, Ph.D. and Juliette Hordeaux, D.V.M., Ph.D.</td>
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<td>5:30 PM - 7:00 PM</td>
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<td>• Synthetic/Molecular Conjugates and Physical Methods for Delivery</td>
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<td>Co-Chairs: Angela Pannier, Ph.D. and Kenya Kamimura, M.D., Ph.D.</td>
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<td>• Targeted Gene and Cell Therapy for Cancer</td>
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<td>Co-Chairs: Michael Milone, M.D., Ph.D. and Hernando Lopez-Bertoni, Ph.D.</td>
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<td>5:30 PM - 7:15 PM</td>
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<td></td>
<td>• Upstream Process Development for AAV Vector Production</td>
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<td>Co-Chairs: Sanford Boye and Laura Adamson-Small, Ph.D.</td>
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<td>5:30 PM - 7:15 PM</td>
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<td>5:15 - 6:45 PM</td>
<td><strong>INDUSTRY SYMPOSIA</strong></td>
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<td>• 908 Devices</td>
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<td>• Pall Corporation</td>
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<td>5:15 - 6:15 PM</td>
<td><strong>NETWORKING ROULETTE</strong></td>
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<tr>
<td>5:15 - 7:00 PM</td>
<td><strong>TOOLS AND TECHNOLOGY FORUM II</strong></td>
</tr>
</tbody>
</table>

**WEDNESDAY, MAY 12, 2021**
### THURSDAY, MAY 13, 2021

<table>
<thead>
<tr>
<th>Time</th>
<th>Event</th>
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<tbody>
<tr>
<td>ALL DAY ACCESS</td>
<td><strong>DIGITAL ABSTRACT PRESENTATIONS</strong></td>
</tr>
<tr>
<td></td>
<td><strong>EXHIBIT HALL</strong></td>
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<td>Connect with Exhibitors:</td>
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<tr>
<td></td>
<td>10:45 AM - 12:15 PM</td>
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<td>2:00 PM - 3:30 PM</td>
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<td>5:15 PM - 6:45 PM</td>
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<tr>
<td>9 - 10 AM</td>
<td><strong>CHAT LOUNGE NETWORKING</strong></td>
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<tr>
<td>10 - 11:45 AM</td>
<td><strong>EDUCATION SESSIONS</strong></td>
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<tr>
<td></td>
<td>• Career and Workforce Development Issues in Gene and Cell Therapy</td>
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<td></td>
<td><em>Co-Chairs: Juliana Alvarez Argote, M.D. and Rayne Rouce, M.D.</em></td>
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<td>10:00 AM - 11:45 AM</td>
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<td></td>
<td>• Gene Therapies for Liver Diseases</td>
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<td><em>Chair: Nuria Morral, Ph.D.</em></td>
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<td>10:00 AM - 11:45 AM</td>
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<td></td>
<td>• Therapeutic Applications of EVs: From Diagnostics to Drug Delivery</td>
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<td></td>
<td><em>(Organized by the Nanoagents &amp; Synthetic Formulations Committee)</em></td>
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<td><em>Co-Chairs: Rajagopal Ramesh, Ph.D. and Assem Ziady, Ph.D.</em></td>
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<td>10:00 AM - 11:45 AM</td>
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<td><strong>SCIENTIFIC SYMPOSIA</strong></td>
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<td></td>
<td>• International Focus on Stem Cell Gene Therapy</td>
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<td><em>(Organized by the International Committee)</em></td>
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<td><em>Co-Chairs: Toni Cathomen, Ph.D. and Alessandro Aiuti, M.D., Ph.D.</em></td>
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<td>10:00 AM - 11:45 AM</td>
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<td>• Racial Justice in the Gene Therapy Field</td>
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<td><em>(Organized by the Ethics &amp; Diversity and Inclusion Committees)</em></td>
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<td><em>Co-Chairs: Rayne Rouce, M.D. and David Segal, Ph.D.</em></td>
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<td>10:15 AM - 11:45 AM</td>
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<td>• Reporter Gene Imaging And The 3 Rs In Cell And Gene Therapy</td>
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<td><em>Co-Chairs: Stephen Russell, M.D., Ph.D.</em></td>
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<td>10:00 AM - 11:45 AM</td>
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<td>• Vaccine Development: Successes and Emerging Challenges</td>
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<td><em>(Organized by the Infectious Diseases and Vaccines Committee)</em></td>
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<tr>
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<td><em>Co-Chairs: David Weiner, Ph.D. and Sterghios Moschos, Ph.D.</em></td>
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|          |    10:00 AM - 11:45 AM
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<thead>
<tr>
<th>Time</th>
<th>Event</th>
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<tbody>
<tr>
<td>10 - 11:45 AM</td>
<td><strong>SCIENTIFIC SYMPOSIA</strong>&lt;br&gt;• Vector Manufacturing and Downstream Processing&lt;br&gt;  <em>Co-Chairs: Anne Galy, Ph.D. and Boro Dropulic, Ph.D.</em>&lt;br&gt;  10:00 AM - 11:45 AM&lt;br&gt;• Viral Vector Safety: A Renewed Focus on Vector Safety and Innate Immune Responses to Leading Viral Vectors (Organized by the Translational Science Committee)&lt;br&gt;  <em>Co-Chairs: H. Trent Spencer, Ph.D. and Nicole Paulk, Ph.D.</em>&lt;br&gt;  10:00 AM - 11:45 AM</td>
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<td>10:45 - 12:15 PM</td>
<td><strong>EXHIBITOR SHOWCASES</strong>&lt;br&gt;• BIA Separations now a Sartorius company - 10:45 AM - 11:30 AM&lt;br&gt;• Bristol Myers Squibb - 10:45 AM - 11:30 AM&lt;br&gt;• Brooks Life Sciences GENEWIZ Inc. - 11:30 AM - 12:15 PM&lt;br&gt;• Solentim - 11:30 AM - 12:15 PM</td>
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<td>12:15 - 2 PM</td>
<td><strong>OUTSTANDING ACHIEVEMENT AWARD LECTURE AND PRESENTATION OF THE SONIA SKARLATOS PUBLIC SERVICE AWARD</strong>&lt;br&gt;Sponsored by <strong>AskBio</strong></td>
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<td>2 - 3:30 PM</td>
<td><strong>INDUSTRY SPONSORED SYMPOSIA</strong>&lt;br&gt;• Charles River Laboratories&lt;br&gt;• FUJIFILM Diosynth Biotechnologies&lt;br&gt;• GenScript&lt;br&gt;• Precision NanoSystems Inc.</td>
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<td>2 - 3 PM</td>
<td><strong>CHAT LOUNGE NETWORKING</strong></td>
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<tr>
<td>3:30 - 5:15 PM</td>
<td><strong>PLENARY SESSION</strong>&lt;br&gt;Presidential Symposium and Presentation of Top Abstracts&lt;br&gt;Sponsored by <strong>MODALIS</strong></td>
</tr>
</tbody>
</table>
THURSDAY, MAY 13, 2021

ABSTRACT SESSIONS

• AAV Therapies for Neurological and Sensory Diseases  
  Co-Chairs: Phillip Tai, Ph.D. and Lluis Samaranch, Ph.D.  
  5:30 PM - 7:15 PM

• Advances in Cellular and Immunotherapies  
  Co-Chairs: Rayne Rouce, M.D., and Daniel Bauer, M.D., Ph.D.  
  5:30 PM - 7:15 PM

• CAR-Based Cancer Gene Therapy  
  Co-Chairs: Monica Casucci, Ph.D. and Daniel Abate-Daga, Ph.D.  
  5:30 PM - 7:15 PM

• Cardiovascular and Pulmonary Gene Therapy  
  Judith Greengard, Ph.D. and Mai ElMallah, M.D.  
  5:30 PM - 7:00 PM

• Clinical Trials and Advanced Preclinical Studies for Neurologic Diseases  
  Co-Chairs: Heather Gray-Edwards, D.V.M., Ph.D. and Patricia Dickson, M.D.  
  5:30 PM - 7:15 PM

• Downstream Process of Vector Manufacturing  
  Co-Chairs: Chris Morrison, Ph.D. and Eric Horowitz, Ph.D.  
  5:30 PM - 7:15 PM

• Immunotherapy and Vaccines  
  Co-Chairs: Matt Gardner, Ph.D. and Allison Keeler-Klunk, Ph.D.  
  5:30 PM - 7:15 PM

• New Gene Editing Technologies and Applications  
  Co-Chairs: Alexis Komor, Ph.D. and T.J. Cradick, Ph.D.  
  5:30 PM - 7:15 PM

• Novel AAV Biology and Platform Technologies  
  Co-Chairs: Lauren Woodard, Ph.D. and Anna Maurer, Ph.D.  
  5:30 PM - 7:15 PM

5:15 - 6:45 PM  

INDUSTRY SPONSORED SYMPOSIA

• Dyno Therapeutics

• L7 Informatics, Inc.

5:15 - 6:15 PM  

NETWORKING ROULETTE

5:15 - 7:00 PM  

TOOLS AND TECHNOLOGY FORUM III
## FRIDAY, MAY 14, 2021

### ALL DAY ACCESS

**DIGITAL ABSTRACT PRESENTATIONS**

**EXHIBIT HALL**

Connect with Exhibitors:

10:45 AM - 12:15 PM

### 9 - 10 AM

**CHAT LOUNGE NETWORKING**

### 10 - 11:45 AM

#### SCIENTIFIC SYMPOSIA

- **Hot Topics and Remaining Challenges in RNAi and Oligonucleotide Therapy for 2021** (Organized by the Oligonucleotide and RNAi Therapeutics Committee)
  
  *Chair: Paloma Giangrande, Ph.D.*
  
  10:00 AM - 11:45 AM

- **New Advances in Physical Gene Delivery and Nucleic Acid Vectorology** (Organized by the Physical Delivery, Therapeutics & Vector Development Committee)
  
  *Co-Chairs: Loree Heller, Ph.D. and Carol Miao, Ph.D.*
  
  10:00 AM - 11:45 AM

- **Newborn Screening: Innovative Policies and Technologies to Eliminate the Diagnostic Odyssey** (Organized by the Government Relations Committee)
  
  *Co-Chairs: Diane Berry, Ph.D. and Philip Reilly, M.D., J.D.*
  
  10:00 AM - 11:45 AM

- **Race to Respiratory Therapies for COVID-19** (Organized by the Respiratory and GI Tract Gene and Cell Therapy Committee)
  
  *Chair: Amy Ryan, Ph.D.*
  
  10:00 AM - 11:45 AM

- **RNA Therapies for Neurologic and Ophthalmic Disorders** (Organized by the Neurologic & Ophthalmic Gene and Cell Therapy Committee)
  
  *Co-Chairs: Kourous Rezaei, M.D. and Jason Shepherd, Ph.D.*
  
  10:00 AM - 11:45 AM

- **Safety and Efficacy of Body-Wide Therapy for Musculo-Skeletal Diseases** (Organized by the Musculo-Skeletal Gene & Cell Therapy Committee)
  
  *Co-Chairs: Jyoti Jaiswal, Ph.D. and Christina Pacak, Ph.D.*
  
  10:00 AM - 11:45 AM

- **Vaccine Nanotechnology for Rapid Response Applications** (Organized by the Nanoagents and Synthetic Formulations Committee)
  
  *Co-Chairs: Jordan Green, Ph.D. and Julie Champion, Ph.D.*
  
  10:00 AM - 11:45 AM
FRIDAY, MAY 14, 2021

10:45 - 11:30 AM
EXHIBITOR SHOWCASES
- 10x Genomics
- Informa Pharma Intelligence

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

ALL TIMES LISTED IN EDT
24th Annual Virtual Meeting

TUESDAY, MAY 11, 2021

ALL DAY

DIGITAL ABSTRACT PRESENTATIONS

EXHIBIT HALL

Be sure to connect with exhibit booth staff during listed times

9:00 AM - 10:00 AM

CHAT LOUNGE NETWORKING

10:00 AM - 11:45 AM

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

10:00 AM - 10:35 AM
Gene Addition Therapy For Hematologic Disorders: The Long Road Towards The Cure
Giuliana Ferrari, Ph.D., SR-TIGET, Scientific Institute San Raffaele

10:35 AM - 11:10 AM
Gene Disruption Through Gene Editing For The Treatment Of Hematologic Disorders
Daniel Bauer, M.D., Ph.D., Boston Children’s Hospital

11:10 AM - 11:45 AM
Base Editing To Treat Blood Disease Without Double-Strand DNA Breaks
David Liu, Ph.D., Broad Institute, Harvard University, and HHMI

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

10:00 AM - 10:35 AM
Creating Non-Human Primate Models Of Neurodegenerative Disease
Jodi McBride, Oregon National Primate Research Center

10:35 AM - 11:10 AM
Utilization Of An In Vivo Pmc Humanized Mouse Model For Determining Bispecific Antibody Related Cytokine Release Syndrome
James Keck, The Jackson Laboratory

11:10 AM - 11:45 AM
Testing Human CAR-T Cells And Oncolytic Virus In Mice Challenged With Human Tumors
Amanda Rosewell Shaw, Baylor College of Medicine
Developing groundbreaking gene therapies for patients with rare diseases.

AGTC, a clinical-stage biotechnology company, is now conducting research studies exploring potentially life-changing treatments for rare genetic eye disorders, including the Clarity clinical trials, in patients with achromatopsia, and the Scenic clinical trials, in patients with XLRP. To learn more, visit agtc.com.
TUESDAY, MAY 11, 2021

10:00 AM - 11:45 AM

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

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

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

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

11:18 AM - 11:45 AM  
**Treatment Strategies for Refractory Gastroesophageal Cancer Using Oncolytic HerPEs G47Δ**  
Kotaro Sugawara, M.D., Ph.D., Institute of Medical Science, The University of Tokyo
TUESDAY, MAY 11, 2021

10:00 AM - 11:45 AM

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

10:00 AM - 10:20 AM  Overview Of Limitations And Progress In Gene Therapy Development In Low- And Middle-Income Countries
Kenneth Cornetta, M.D., Indiana University

10:20 AM - 10:40 AM  Regulatory Pathways For Gene And Cell Therapies In Brazil
João Batista Silva Junior, ANVISA

10:40 AM - 11:00 AM  Challenges And Opportunities For Gene Therapy Development For Hemophilia In South Africa
Johnny Mahlangu, M.B.Ch.B., M.Med., University of Witwatersrand

11:00 AM - 11:20 AM  Opportunities For Clinical Trials Of Indigenously Developed Gene Therapies In India
Rahul Purwar, Ph.D., Indian Institute of Technology Bombay

11:20 AM - 11:45 AM  Panel Discussion

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

10:00 AM - 10:26 AM  In Vivo CRISPR Base Editing Of PCSK9 In Primates And Durable Cholesterol Reduction
Andrew Bellinger, M.D., Ph.D., Verve Therapeutics

10:26 AM - 10:52 AM  Development Of Systemic CRISPR-Based Therapeutics
Laura Sepp-Lorenzino, Ph.D., Intellia Therapeutics

10:52 AM - 11:18 AM  Ind Enabling Studies For Gene Correction For Sickle Cell Disease
Annalisa Lattanzi, Ph.D., Stanford University

11:18 AM - 11:45 AM  Development Of CRISPR-Enhanced Bacteriophages For The Treatment Of Urinary Tract Infections
Dave Ousterout, Ph.D., Locus Biosciences
## SCIENTIFIC SYMPOSIA

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

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<tr>
<th>Time</th>
<th>Topic</th>
<th>Speaker/Sponsor</th>
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<tbody>
<tr>
<td>10:00 AM - 10:25 AM</td>
<td><strong>Gene Therapy Payment Systems In The United States</strong></td>
<td>Beth Halpern, Hogan Lovells</td>
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<tr>
<td>10:25 AM - 10:50 AM</td>
<td><strong>Gene &amp; Cell Therapy Payment Systems In Europe</strong></td>
<td>Christian Hill, MAP BioPharma</td>
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<td>10:50 AM - 11:15 AM</td>
<td><strong>Current Drug Pricing And Payment Policy Debates: Applications To Gene Therapy</strong></td>
<td>Remy Brim, Ph.D., BGR Group</td>
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<tr>
<td>11:20 AM - 11:45 AM</td>
<td>Panel Discussion</td>
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**Homology Medicines, Inc.**

Translating **gene therapy** and **gene editing** technology into one-time treatments and potential cures to transform patients’ lives.
TUESDAY, MAY 11, 2021

10:00 AM - 11:45 AM

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

10:00 AM - 10:26 AM
Cancer Immunotherapy: Where We Are And Where We Are Going
Antoni Ribas, University of California-Los Angeles

10:26 AM - 10:52 AM
Updates On Oncolytic Viral Therapy And Combinational Approaches For Gliomas And Other Solid Tumors
Juan Fueyo, M.D., M.D. Anderson Cancer Center

10:52 AM - 11:18 AM
Next Wave Of Innovation In NK Cell Therapies For Cancer
Jeffrey Miller, University of Minnesota

11:18 AM - 11:44 AM
Novel Vaccine Technologies To Prevent And Treat Cancer
David Weiner, The Wistar Institute
TUESDAY, MAY 11, 2021

COVID-19: Vaccines to the Rescue
10:00 AM - 11:45 AM
Roundtable Discussion
- Stephen Russell, M.D., Ph.D., Mayo Clinic
- Sarah Gilbert, Ph.D., University of Oxford
- Larry Corey, M.D., Fred Hutchinson Cancer Research Center
- Gregory Poland, M.D., Mayo Clinic
- Ligia Pinto, Ph.D., Frederick National Laboratory for Cancer Research
- Philip Dormitzer, M.D., Ph.D., Pfizer

10:30 AM - 12:00 PM

EXHIBIT HALL OPEN
EXHIBITORS AVAILABLE TO CONNECT
Exhibit Hall is Accessible 24 Hours

10:30 AM - 12:00 PM

Catalent Cell & Gene Therapy – 10:30 AM - 11:15 AM
Strategies and Solutions for Gene Therapy Development and Manufacturing
Thomas VanCott, Ph.D., Catalent Cell & Gene Therapy

STEMCELL Technologies – 10:30 AM - 11:15 AM
Serum- and Feeder-Free Differentiation of Erythroid Progenitor Cells from hPSCs
Crystal Chau and Selena Hallahan, STEMCELL Technologies

Aldevron – 11:15 AM - 12:00 PM
Meeting Global Demand for Critical Biologics
Kevin Ballinger, Michelle Berg, Tom Foti, and Ken Bonnell, Aldevron

MilliporeSigma – 11:15 AM - 12:00 PM
A Platform Standard for Viral Vector Manufacturing and Commercialization
Eva Fong and Jessica Hilmo, MilliporeSigma
TUESDAY, MAY 11, 2021

12:00 PM - 1:00 PM

FIRESIDE CHAT
Jennifer Doudna, Ph.D.,
UC Berkeley

1:30 PM - 3:30 PM

MENTOR MEET-UP EVENT
Sponsored by:

Don’t Miss the Following Presentations

Tuesday, May 11th

EXHIBITOR SHOWCASE
11:15 am–12:00 pm
A Platform Standard for Viral Vector Manufacturing and Commercialization
Eva Fong, Sr. Scientist and Jessica Hilmo, Technical Leads Manager

TOOLS & TECHNOLOGY FORUM
5:15–5:30 pm
Biosafety in Gene Therapy: Applying the Latest Regulatory Guidance for RCL Testing
Leyla Diaz, Ph.D., Principal Scientist

Explore the latest developments in cell & gene therapy
Explore Today
TUESDAY, MAY 11, 2021

2:00 PM - 3:30 PM

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

2:00 PM - 2:15 PM
The Case for Scaling Up Adherent Cell Culture Systems
Todd Upton, Ph.D., Corning Life Sciences

2:15 PM - 2:45 PM
The CDMO Perspective on Viral Vector Production - Challenges and Opportunities
Plamena Kirova, Andelyn Biosciences

2:45 PM - 3:15 PM
Using a Scalable, Intensified Fixed Bed System for High-Yield Viral Vector Production
Zara Melkomian, Ph.D., Corning Life Sciences

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

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

2:00 PM - 2:10 PM
NKARTA Therapeutics
James Trager, Ph.D., NKARTA Therapeutics

2:10 PM - 2:20 PM
Genespire
Julia Berretta, Ph.D., Genespire

2:20 PM - 2:30 PM
NHLBI
John Tsdale, M.D., National Institutes of Health, NHLBI

2:30 PM - 2:40 PM
VOR Biopharma
Sadik Kassim, Ph.D., VOR Biopharma

2:40 PM - 2:50 PM
Myeloid Therapeutics
Daniel Getts, Ph.D., Myeloid Therapeutics

2:50 PM - 3:30 PM
Panel Discussion
Moderated by Sarah Haecker Meeks, Ph.D., MaxCyte, Inc.
24th Annual Virtual Meeting

TUESDAY, MAY 11, 2021

2:00 PM - 3:30 PM

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

2:00 PM - 2:05 PM
Welcome and Introduction
Louise Rodino-Klapac, Ph.D., Sarepta Therapeutics, Inc.

2:05 PM - 2:40 PM
AAV Gene Transfer Therapy: Challenges and Future Directions
Louise Rodino-Klapac, Ph.D., Sarepta Therapeutics, Inc.

2:40 PM - 3:00 PM
Duchenne Muscular Dystrophy Study 9001-102 Interim Findings
Perry Shieh, M.D., Ph.D., David Geffen School of Medicine at UCLA, University of California

3:00 PM - 3:20 PM
Limb Girdle Muscular Dystrophy Study 9003-101 Interim Findings
Erica Koenig, Ph.D., Sarepta Therapeutics, Inc.

3:20 PM - 3:30 PM
Closing Remarks & Q&A
Moderated by Louise Rodino-Klapac, Ph.D.
Panelists: Perry Shieh, M.D., Ph.D. and Erica Koenig, Ph.D.

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

2:00 PM - 2:45 PM
Optimizing Large-Scale Cell Expansion for iPS Cell-Based Applications
Fernanda Mesquita, Ph.D., Texas Heart Institute

2:45 PM - 3:30 PM
The Role of Flexible Automation in Enabling THI’s Research Initiatives
Camila Hochman-Mendez, Ph.D., Texas Heart Institute
Schedule

ALL TIMES LISTED IN EDT

TUESDAY, MAY 11, 2021

2:00 PM - 3:00 PM

CHAT LOUNGE NETWORKING

3:30 PM - 5:15 PM

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

3:30 PM - 4:05 PM
AAVs - What We Know 56 Years After Discovery
Terence R. Flotte, Ph.D., University of Massachusetts Medical School

4:05 PM - 4:40 PM
Development Pipeline - AAV Vectors for Pompe/CNS
Nathalie Cartier-Lacave, M.D., INSERM U1169

4:40 PM - 5:15 PM
Gene Therapy for Orphan Diseases
Kathrin Meyer, Ph.D., Nationwide Children’s Hospital

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

3:30 PM - 3:56 PM
Strategy and Manufacturing of Cells Expressing CARs to Fight Cancer and the Tumor Microenvironment
Cliona Rooney, Ph.D., Baylor College of Medicine

3:56 PM - 4:22 PM
Testing T Cells in Clinical Trials Against Liquid and Solid Cancer
Marcela Maus, M.D., Ph.D., Massachusetts General Hospital

4:22 PM - 4:48 PM
Oncolytic Viral Therapies: Two Knives Against Cancer
Paola Grandi, Ph.D., CG Oncology

4:48 PM - 5:15 PM
Systemic Immunogene Therapy for Cancer
Jack Roth, M.D., University of Texas M.D. Anderson Cancer Center
TUESDAY, MAY 11, 2021

3:30 PM - 5:15 PM

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

3:30 PM - 3:56 PM  **Introduction to In Vivo Gene Editing**
Paula Cannon, Ph.D., University of Southern California

3:56 PM - 4:22 PM  **In Vivo Gene Editing With the Kamicas9 Self-Inactivating System**
Nicole Deglon, Ph.D., Lausanne University Hospital (CHUV)

4:22 PM - 4:48 PM  **Gene and Epigenome Editing for Disease Therapies**
Juan Carlos Izpisua Belmonte, Ph.D., The Salk Institute for Biological Studies

4:48 PM - 5:15 PM  **Safety Issues and Solutions for In Vivo Gene Editing**
William McKillop, Ph.D., Medical College of Wisconsin

---

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

3:30 PM - 4:05 PM  **Addressing Capacity Constraints to Viral Vector Manufacturing**
Sarah Yuan, Ph.D., bluebird bio

4:05 PM - 4:40 PM  **Value and Access Considerations in Pricing Gene Therapies**
Sarah Pitluck, Spark Therapeutics

4:40 PM - 5:15 PM  **Patient Perspective: The Value of Gene Therapy**
Charles Hough
### TUESDAY, MAY 11, 2021

3:30 PM - 5:15 PM

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

<table>
<thead>
<tr>
<th>Time</th>
<th>Title</th>
<th>Speaker and Institution</th>
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</thead>
<tbody>
<tr>
<td>3:35 PM - 3:55 PM</td>
<td>Engineering Human Pluripotent Stem Cells to Produce NK Cells With Improved Anti-Tumor Activity</td>
<td>Dan Kaufman, M.D., Ph.D., University of California - San Diego</td>
</tr>
<tr>
<td>3:55 PM - 4:15 PM</td>
<td>INKt-CAR</td>
<td>Rob Negrin, M.D., Stanford University</td>
</tr>
</tbody>
</table>

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

<table>
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<tr>
<th>Time</th>
<th>Title</th>
<th>Speaker and Institution</th>
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<tbody>
<tr>
<td>3:30 PM - 3:56 PM</td>
<td>hESC-Derived Dopaminergic Neurons for Parkinson’s</td>
<td>Lorenz Studer, M.D., Memorial Sloan Kettering Cancer Center</td>
</tr>
<tr>
<td>3:56 PM - 4:22 PM</td>
<td>Clinical Data on iPSC-Derived RPE for Macular Degeneration</td>
<td>Masayo Takahashi, M.D., Ph.D., Vision Care Inc., Kobe Eye Center</td>
</tr>
<tr>
<td>4:22 PM - 4:48 PM</td>
<td>Hypoimmunogenic iPSCs</td>
<td>Sonja Schrepfer, M.D., Ph.D., Sana Biotechnology</td>
</tr>
<tr>
<td>4:48 PM - 5:15 PM</td>
<td>CRISPR Genome Editing to Generate Immune-Compatible iPSCs</td>
<td>Akitsu Hotta, Ph.D., CiRA, Kyoto University</td>
</tr>
</tbody>
</table>

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

<table>
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<tr>
<th>Time</th>
<th>Title</th>
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<tbody>
<tr>
<td>3:30 PM - 3:56 PM</td>
<td>Overview of Immune Complications In HCT</td>
<td>John Wagner, M.D., University of Minnesota</td>
</tr>
<tr>
<td>3:56 PM - 4:22 PM</td>
<td>Complement Responses in HCT</td>
<td>Eleni Gavrilaki, M.D., Ph.D., George Papanicolaou Hospital</td>
</tr>
<tr>
<td>4:22 PM - 4:48 PM</td>
<td>Engineering Tregs</td>
<td>Megan Levings, Ph.D., University of British Columbia</td>
</tr>
<tr>
<td>4:48 PM - 5:15 PM</td>
<td>Impact of Pre-Existing Transgene Product Immunity in Engraftment of Gene Modified HSC</td>
<td>H. Trent Spencer, Ph.D., Emory University School of Medicine</td>
</tr>
</tbody>
</table>
TUESDAY, MAY 11, 2021

3:30 PM - 5:15 PM

Career Development Award Presentations

3:50 PM - 4:07 PM  Improved Strategies for Site-Specific Gene Insertion Using Non-Homologous End Joining  Geoffrey Rogers, Ph.D., University of Southern California

4:24 PM - 4:41 PM  Development of Non-Viral Mechanisms of Targeting Lung Epithelial Cells for Prenatal Gene Editing in a Large Animal Model  Kshitiz Singh, Ph.D., Children’s Hospital of Philadelphia

4:41 PM - 4:58 PM  Characterizing and Overcoming the Host Response to Genome Editing Therapy  Christopher Nelson, Ph.D., University of Arkansas

4:58 PM - 5:15 PM  Engineered CAR-T Cells to Overcome Alloimmunity in Transplant Rejection  Kalpana Parvathaneni, Ph.D., University of Pennsylvania

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ALL TIMES LISTED IN EDT
TUESDAY, MAY 11, 2021

5:15 PM - 6:45 PM

EXHIBIT HALL OPEN - EXHIBITORS AVAILABLE TO CONNECT
Exhibit Hall is Accessible 24 Hours

5:30 PM - 7:30 PM

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

5:30 PM - 5:45 PM

1: Development of an Optimized Lentiviral Transduction Process for Ex Vivo CD34+ Hematopoietic Stem Cell Gene Therapy Drug Product Manufacture
Pereinder Sagoo, Ph.D., Orchard Therapeutics Europe Ltd.

5:45 PM - 6:00 PM

2: Non-Viral Integration of Large Cargo in Primary Human T Cells by CRISPR/Cas9 Guided Homology Mediated End Joining
Matthew Johnson, Ph.D., University of Minnesota

6:00 PM - 6:15 PM

3: Epigenetic Modulation of Aging to Increase CAR-T Cell Fitness
Benedetta Nicolis di Robilant, Ph.D., Dorian Therapeutics

6:15 PM - 6:30 PM

4: Effective and Efficient Intracellular Delivery Achieved with the Cell Squeeze® Technology Enables Rapid, Scaled, and Reproducible Production of Cell Therapies
David Chirgwin, SQZ Biotechnologies

6:30 PM - 6:45 PM

5: Sequential CRISPR-mediated Engineering and Clonal Banking for the Generation of Multiplexed Engineered Master Pluripotent Cell Lines for the Mass Manufacture of Off-the-Shelf Immune Cells Targeting Solid Cancers
Ramzey Abujarour, Ph.D., Fate Therapeutics

6:45 PM - 7:00 PM

6: Generation of Engineered Tregs (EngTregs) from Umbilical Cord Blood Derived CD4+ T Cells via HDR-Dependent FOXP3 Gene Editing
Shivani Patel, Seattle Children’s Research Institute

7:00 PM - 7:15 PM

7: VOR33: A Clinic-Ready CRISPR/Cas9 Engineered Hematopoietic Stem Cell Transplant for the Treatment of Acute Myeloid Leukemia
John Lydeard, Ph.D., Vor Biopharma
Cancer - Oncolytic Viruses
Co-Chairs: Paola Grandi, Ph.D. and Melissa Kotterman, Ph.D.

5:30 PM - 5:45 PM  8: Preclinical Toxicology Assessment of an Oncolytic Measles Virus Armed With H. pylori Immunostimulatory Bacterial Antigen in Preparation for a Phase I Trial in Breast Cancer Patients
Kimberly Viker, Mayo Clinic

5:45 PM - 6:00 PM  9: Validating Secreted IFNβ as an In Vivo Biomarker of Intratumoral Replication of VSV-IFNβ-NIS
Lianwen Zhang, Mayo Clinic

6:00 PM - 6:15 PM 10: Development of Novel Oncolytic Vector Based on Alternative Adenovirus Serotype 6 for Glioblastoma and Breast Cancer Therapy
Margarta Romanenko, Ph.D., University of Minnesota

6:15 PM - 6:30 PM 11: Human Cytomegalovirus Engineered for Glioma Therapy
Haifei Jiang, M.D., Ph.D., Mayo Clinic

6:30 PM - 6:45 PM 12: Virulent Velogenic Newcastle Disease Virus is More Oncolytic Than Attenuated and Lentogenic Newcastle Viruses
Ahmed Majeed Al-Shammari, Ph.D., Mustansiriyah University, Iraqi Center for Cancer and Medical Genetic Research

6:45 PM - 7:00 PM 13: Generation and Characterization of Replication-Competent Oncolytic Foamy Virus Vectors
Karol Budzik, Mayo Clinic

7:00 PM - 7:15 PM 14: An Oncolytic Adenoviral Vector Expressing an Anti-PD-L1 scFv Reduces Tumor Growth in a Melanoma Mouse Model
Maria Vitale, University of Naples
# Delivery Technologies and CRISPR for Therapeutics

Co-Chairs: Nicole Gaudelli, Ph.D. and Alejandro Chavez, M.D., Ph.D.

<table>
<thead>
<tr>
<th>Time</th>
<th>Abstract</th>
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</table>
| 5:30 PM - 5:45 PM| **15:** CRISPR/Cas9-Mediated Targeted Gene Insertion Platform Achieves Durable, Normal Human Alpha-1 Antitrypsin Protein Levels in Non-Human Primates  
Sean Burns, M.D., Intellia Therapeutics |
| 5:45 PM - 6:00 PM| **16:** Direct rAAV-Mediated In Vivo Gene Editing of Hematopoietic Stem Cells  
Ishani Dasgupta, Ph.D., University of Massachusetts Medical School |
| 6:00 PM - 6:15 PM| **17:** CRISPR-Cas9 Genome Editing of Human CD34+ Cells at Gamma-Globin Promoter to Induce Fetal Hemoglobin as Sickle Cell Disease Therapy  
Varun Katta, St. Jude Children’s Research Hospital |
| 6:15 PM - 6:30 PM| **18:** In Utero Lipid Nanoparticle Delivery of CRISPR Technology to Correct Hereditary Tyrosinemia Type 1  
Kshitiz Singh, Ph.D., Children’s Hospital of Philadelphia |
| 6:30 PM - 6:45 PM| **19:** Correction of DMD Mutations in Human iPS-Derived Muscle Cells by Single-Cut CRISPR/Cas9-Based Gene Editing  
Ziad Al Tanoury, Ph.D., Vertex Cell and Genetic Therapies |
| 6:45 PM - 7:00 PM| **20:** Cell-Based Delivery Strategies for Artificial Transcription Factors in Preclinical Animal Models  
Peter Deng, Ph.D., UC Davis |
TUESDAY, MAY 11, 2021

5:30 PM - 7:30 PM

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

5:30 PM - 5:45 PM
22: A Novel Liver-Tropic AAV Capsid sL65 Shows Superior Transduction and Efficacy in Humanized Mice and Non-Human Primates
Jing Liao, Ph.D., LogicBio Therapeutics

5:45 PM - 6:00 PM
23: Efficient Design of Optimized AAV Capsids Using Multi-Property Machine Learning Models Trained Across Cells, Organs and Species
Eric Kelsic, Ph.D., Dyno Therapeutics

6:00 PM - 6:15 PM
24: Risk-Adjusted Selection for Validation of Sequences in AAV Design Using Composite Sampling
Lauren Wheelock, Ph.D., Dyno Therapeutics

6:15 PM - 6:30 PM
25: A Comparison of Methods Used for the Determination of Full and Empty rAAV Particles
Bryan Troxell, Ph.D., StrideBio, Inc.

6:30 PM - 6:45 PM
26: A Tetracycline Enabled Self-Silencing Adenovirus (TESSA) Platform Delivers High-Quality, High-Titre, Multi-Serotype Recombinant Adeno-Associated Virus (AAV) Stocks
Maria Patricio, Ph.D., Oxgene

6:45 PM - 7:00 PM
27: Next Generation AAV Drug Products: Enhanced Stability & Clinical Ease for High Titer Preparations
Lori Karpes, Ph.D., Homology Medicines
Gene Therapy for Inborn Errors of Metabolism
Co-Chairs: Giuseppe Ronzitti, Ph.D. and Gloria Gonzalez-Aseguinolaza, Ph.D.

5:30 PM - 5:45 PM
29: Coadministration of AAV Expressing M.D.R3 (VTX-803) and ImmTOR Allows for Vector Re-Administration to Treat Progressive Familial Intrahepatic Cholestasis Type 3 (PFIC3) in Juvenile Mice
Nicholas Weber, Ph.D., Vivet Therapeutics

5:45 PM - 6:00 PM
30: Preclinical Evaluation of Combined Adeno-Associated Virus and Nanoparticle Delivery of piggyBac® Transposon System for Durable Transgene Expression in the Growing Neonatal Murine Liver
Jingjing Jiang, Ph.D., Poseida Therapeutics

6:00 PM - 6:15 PM
31: Targeting Aberrant Acylation as a Novel Approach for Treating Methylmalonic Acidemia (MMA) and Related Other Organic Acidemias
Sangho Myung, National Institutes of Health, NHGRI

6:15 PM - 6:30 PM
32: AAV Liver Gene Therapy-mediated Inhibition Of FGF23 Signaling as a Therapeutic Strategy for X-linked Hypophosphatemia
Giuseppe Ronzitti, Ph.D., Genethon

6:30 PM - 6:45 PM
33: Comparison of Gene Addition Therapy in Genetically Distinct Mouse Models of Classical Phenylketonuria
Daelyn Richards, Ph.D., Oregon Health and Science University

6:45 PM - 7:00 PM
34: AAV8 Gene Therapy as a Potential Treatment in Adults with Late-Onset Ornithine Transcarbamylase (OTC) Deficiency: Updated Results from a Phase 1/2 Clinical Trial
Cary Harding, M.D., Oregon Health and Science University

7:00 PM - 7:15 PM
35: AAV-Mediated Delivery of MiRNA-34B/C Improves Liver Fibrosis
Pasquale Piccolo, Ph.D., Telethon Institute of Genetics and Medicine
TUESDAY, MAY 11, 2021

5:30 PM - 7:30 PM

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

5:30 PM - 5:45 PM
36: Follow-Up of a Phase I/II Gene Therapy Trial in Patients with Fanconi Anemia, Subtype A
Juan Bueren, Ph.D., Ciemat/Ciberer/Iis-Fjd, Uam

5:45 PM - 6:00 PM
37: Liver Gene Therapy with Lentiviral Vectors Corrects Hemophilia A in Mice and Achieves Normal-Range Factor VIII Activity in Non-Human Primates
Michela Milani, Ph.D., San Raffaele Telethon Institute for Gene Therapy

6:00 PM - 6:15 PM
38: Towards Clinical Translation of Hematopoietic Cell Gene Editing for Treating Hyper-IgM Type 1
Valentina Vavassori, Ph.D., San Raffaele Telethon Institute for Gene Therapy; Vita-Salute San Raffaele University

6:15 PM - 6:30 PM
39: A Phase 1/2 Study of Lentiviral-Mediated Ex-Vivo Gene Therapy for Pediatric Patients with Severe Leukocyte Adhesion Deficiency-I (LAD-I): Interim Results
Donald Kohn, M.D., UCLA

6:30 PM - 6:45 PM
40: Autologous Ex Vivo Lentiviral Gene Therapy for the Treatment of ADA-SCID
Claire Booth, Ph.D., UCL Gosh Institute of Child Health

6:45 PM - 7:00 PM
41: Efficient Ex-Vivo Selection of Gene Edited Human Hematopoietic Stem/Progenitor Cells
Martina Fiumara, San Raffaele Telethon Institute for Gene Therapy; Vita-Salute San Raffaele University

7:00 PM - 7:15 PM
42: Targeted Genome Editing of Hematopoietic Stem Cells for Treating Recombination Activating Gene 1 (RAG1) Immunodeficiency
Maria Carmina Castiello, Ph.D., San Raffaele Telethon Institute for Gene Therapy
TUESDAY, MAY 11, 2021
5:30 PM - 7:30 PM

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

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

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

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

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

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

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

7:00 PM - 7:15 PM
49: Non-Genotoxic Conditioning to Increase Gene Therapy Safety in a Rare Bone Disease
Valentina Capo, Ph.D., IRCCS San Raffaele Scientific Institute; CNR-IRGB
5:30 PM - 5:45 PM  50: Endothelial-Tropic AAVs for Genetic Access to Whole-Brain Vasculature in Wild-Type Mouse Strains following Non-Invasive Systemic Delivery
Xinhong Chen, Caltech

5:45 PM - 6:00 PM  51: RNA-Driven Evolution of AAV Capsid Libraries Identifies Variants with High Transduction Efficiency in Non-Human Primate Central Nervous System
Mathieu Nonnenmacher, Ph.D., Voyager Therapeutics

6:00 PM - 6:15 PM  52: Expanding the Utility of Intravitreal AAV via a Capsid Variant That Overcomes Neutralization by Anti-AAV2 NAbs in Human Vitreous
Siddhant Gupte, University of Florida

6:15 PM - 6:30 PM  53: Breaking Thru the Human Blood Brain Barrier: Discovering AAV Vectors Targeting the Central Nervous System Using a Transwell Model
Ren Song, Ph.D., Stanford University School of Medicine

6:30 PM - 6:45 PM  54: Expanding the AAV Toolbox for Cerebellar Transduction: Identifying and Characterizing Novel Variants in Non-Human Primates and Mice
Megan Keiser, Ph.D., Children’s Hospital of Philadelphia

6:45 PM - 7:00 PM  55: Capsid Display of Cell-Penetrating Peptides Yields AAVs with Enhanced Brain Penetration in Both Rodents and Primates
Fengfeng Bei, Ph.D., Harvard Medical School

7:00 PM - 7:15 PM  56: Engineering AAV6-Based Vectors for Improved Ocular Transduction Following Intravitreal and Intracameral Injection
Sean Crosson, Ph.D., University of Florida
5:30 PM - 7:30 PM

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

5:30 PM - 5:45 PM
57: ST3GAL5 Gene Replacement in CNS Restores Gangliosides Production and Improves Survival in a Mouse Model of GM3 Synthase Deficiency
Huiya Yang, University of Massachusetts Medical School

5:45 PM - 6:00 PM
58: CRISPR/Cas9 Strategies to Treat Spinocerebellar Ataxia Type 1
Kelly Fagan, University of Pennsylvania

6:00 PM - 6:15 PM
59: Rescue of Molecular and Motor Phenotypes in CGG Knock-In Mice With CRISPR Mediated Deletion of the Trinucleotide Repeat
Carolyn Yrigollen, Ph.D., Children’s Hospital of Philadelphia

6:15 PM - 6:30 PM
60: Transthyretin Gene Therapy as a Modulator of Alzheimer’s Disease Progression
Ana Rita Batista, Ph.D., University of Massachusetts Medical School

6:30 PM - 6:45 PM
61: CRISPR/Cas9-Mediated Excision of ALS/FTD-causing Hexanucleotide Repeat Expansion in C9ORF72 Rescues Major Disease Mechanisms in Vivo and In Vitro
Katharina Meijboom, D.Phil., University of Massachusetts Medical School

6:45 PM - 7:00 PM
62: C9ORF72 Variant-Specific RNA Interference Rescues C9-ALS/FTD Molecular Hallmarks In Vivo and In Vitro
Katharina Meijboom, D.Phil., University of Massachusetts Medical School

7:00 PM - 7:15 PM
63: Restoration of Scn1a Expression after Symptom Onset in a Novel Model of Dravet Syndrome Rescues Seizures and Behavioral Alterations
Gaia Colasante, Ph.D., Ospedale San Raffaele
TUESDAY, MAY 11, 2021

5:15 PM - 6:45 PM

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

5:15 PM - 6:00 PM
**Cell Therapy**
Megan Liles, Precision for Medicine; John Khoury, Project Farma; Alex Grosvenor, Precision Value & Health; Joachim Fruebis, BlueRock Therapeutics; Osvaldo Flores, Century Therapeutics, and Sadik Kassim, VOR Biopharma

6:00 PM - 6:45 PM
**Gene Therapy**
Deborah Phippard, Precision for Medicine; Tony Khoury, Project Farma; Phil Cyr, Precision Value & Health; Tim Kelly, Asklepios Biopharmaceutical Inc. (AskBio); Ottavio Vitolo, Alcyone Therapeutics; and Steven Zelenkofske, SwanBio Therapeutics

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

5:15 PM - 5:45 PM
**Scalable, High-Titer, Simplified AAV Production in the AAV-MAX Helper Free AAV Production System**
Chao Yan Liu, Ph.D., Thermo Fisher Scientific

5:45 PM - 6:15 PM
**Benefits of Using a Media Panel to Address the Diversity of HEK293 Cell Lines**
Gino Stolfa, Ph.D., Thermo Fisher Scientific

6:15 PM - 6:45 PM
**CGT Regulatory Landscape and Virtual Inspections**
Monica Commerford, Ph.D., Thermo Fisher Scientific

5:15 PM - 6:15 PM
**Networking Roulette**
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ALL TIMES LISTED IN EDT
TUESDAY, MAY 11, 2021

5:15 PM - 7:00 PM

Tools and Technology Forum I

5:15 PM - 5:30 PM  
Biosafety in Gene Therapy: Applying the Latest Regulatory Guidance for RCL Testing  
Leyla Diaz, Ph.D., MilliporeSigma

5:30 PM - 5:45 PM  
Design, Manufacturing and Analytics of New AAV Reference Materials - A Case Study  
Jeffrey Hung, Ph.D., Vigen Biosciences

5:45 PM - 6:00 PM  
Host Cell Protein Analytics in Viral Vector Manufacturing  
Alla Zilberman, Cygnus Technologies

6:00 PM - 6:15 PM  
Mass Photometry - A New Tool to Study Biomolecules  
Gabriella Kiss, Refeyn

6:15 PM - 6:30 PM  
Transient Transfection at Large-Scale for Clinical AAV9 Vector Manufacturing  
Denis Kole, Pall Corporation

6:30 PM - 6:45 PM  
Videodrop: Rapid Characterization of Lentiviral Vectors in a Droplet for a Better Bioproduction Follow-Up  
Marie Berger, PharM.D., MYRIADE

6:45 PM - 7:00 PM  
Trends in Cell and Gene Therapy: De-risking Platform and Product Development with Mass Spectrometry  
Chen Li, BioAnalytix
WEDNESDAY, MAY 12, 2021

ALL DAY

DIGITAL ABSTRACT PRESENTATIONS
EXHIBIT HALL
Be sure to connect with exhibit booth staff during listed times

9:00 AM - 10:00 AM
CHAT LOUNGE NETWORKING

10:00 AM - 11:45 AM

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

10:00 AM - 10:26 AM
5 Steps for a Polished Grant Submission
Kelly Turner, Ph.D., Baylor College of Medicine

10:26 AM - 10:52 AM
Advice for Early-Stage Cell Therapy Professionals
Rayne Rouce, M.D., Baylor College of Medicine

10:52 AM - 11:18 AM
Communicating Your Research to Investors
Manisha Pai, Vertex Pharmaceuticals

11:18 AM - 11:45 AM
Communicating Complex Science With a Public Audience
Roxanne Khamsi, Freelance Journalist
WEDNESDAY, MAY 12, 2021

10:00 AM - 11:45 AM

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

10:00 AM - 10:26 AM  
**AAV Capsid Engineering for In Vivo Gene Therapy**  
Hildegard Buning, Ph.D., Hannover Medical School

10:26 AM - 10:52 AM  
**Dressing Viruses in Tumors’ Clothing: Cloning-Free Platforms to Trigger Tumor-Specific Immune Response**  
Vincenzo Cerullo, Ph.D., University of Helsinki

10:52 AM - 11:18 AM  
**LV-Mediated Gene Therapy of Pyruvate Kinase Deficiency**  
Jose-Carlos Segovia, Ph.D., Centro de Investigaciones Energéticas Medioambientales y Tecnológicas (CIEMAT) and Centro de Investigación Biomédica en Red de Enfermedades Raras (CIBERER)

11:18 AM - 11:45 AM  
**Liver-Directed Gene Therapy Clinical Trial for Mucopolysaccharidosis VI**  
Nicola Brunetti-Pierri, M.D., Telethon Institute of Genetics and Medicine

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

10:00 AM - 10:15 AM  
**Preparing For a Career In Industry: What New Entrants Need to Know**  
Nathaniel Berendson, GlaxoSmithKline (GSK)

10:15 AM - 10:30 AM  
**When to Spin Out: One Academic Perspective**  
Matthew Porteus, M.D., Ph.D., Stanford University School of Medicine

10:30 AM - 10:45 AM  
**Attracting, Developing, and Retaining the Best Young Academic Science Talent: A Guide for Those in Industry**  
Kate Barclay, Ph.D., UK BioIndustry Association

10:45 AM - 11:00 AM  
**Life in The Industry Matrix: Journeying Through the Industry Professional Environment**  
Freda Lewis-Hall, M.D., Pfizer (retired)

11:00 AM - 11:45 AM  
**Panel Discussion**
WEDNESDAY, MAY 12, 2021

10:00 AM - 11:45 AM

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

**10:00 AM - 10:35 AM**
**Complement Activation Following AAV Gene Delivery**
Pascal Deschatelets, Ph.D., Apellis Pharmaceuticals

**10:35 AM - 11:10 AM**
**Cytokine Storm in Response to Gene and Cell Transfer**
Stéphan Grupp, M.D., Ph.D., University of Pennsylvania Perelman School of Medicine

**11:10 AM - 11:45 AM**
**CARs and Armoured CARs: Improving CAR T Cell Therapy for Cancer**
Renier Brentjens, M.D., Ph.D., Memorial Sloan-Kettering Cancer Center

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

**10:00 AM - 10:26 AM**
**Novel Poxvirus Vectors**
Takafumi Nakamura, Ph.D., Tottori University

**10:26 AM - 10:52 AM**
**Induced Pluripotent Stem Cells Using Single-Cycle Measles Virus Vector**
Patricia Devaux, Ph.D., Mayo Clinic

**10:52 AM - 11:18 AM**
**HSV Delivery of Genetic Circuits**
Ron Weiss, Ph.D., Massachusetts Institute of Technology

**11:18 AM - 11:45 AM**
**In Vivo Hematopoietic Stem Cell Gene Therapy With Hdad5/35++ Adenovirus Vectors**
Andre Lieber, M.D. Ph.D., University of Washington
# Regulatory Lessons Learned From COVID-19: Anomaly to Precedent
*(Organized by the Regulatory Affairs Committee)*
*Co-Chairs: Kit Shaw, Ph.D. and S. Kaye Spratt, Ph.D.*

<table>
<thead>
<tr>
<th>Time</th>
<th>Title</th>
<th>Speaker(s)</th>
</tr>
</thead>
<tbody>
<tr>
<td>10:00 AM - 10:15 AM</td>
<td><strong>EMA Experience on How Emergency Measures Will Inform Flexibility for Gene Therapies</strong></td>
<td>Emer Cooke, European Medicines Agency</td>
</tr>
<tr>
<td>10:15 AM - 10:30 AM</td>
<td><strong>Clinical Trial Efficacy Assessments During the Covid-19 Pandemic</strong></td>
<td>Wilson Bryan, M.D., Food and Drug Administration</td>
</tr>
<tr>
<td>10:30 AM - 10:45 AM</td>
<td><strong>Creating a Remote Data-Collection Paradigm in a Rare Pediatric Disease: A Case Study</strong></td>
<td>Genevieve Laforet, M.D., Ph.D., Aspa Therapeutics</td>
</tr>
<tr>
<td>10:45 AM - 11:00 AM</td>
<td><strong>Incorporating Regulatory Guidance to Provide Continuity for Ongoing Clinical Trials</strong></td>
<td>Jonathan Cotlier, M.D., Science37</td>
</tr>
<tr>
<td>11:00 AM - 11:45 AM</td>
<td><strong>Panel Discussion</strong></td>
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</tr>
</tbody>
</table>

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

<table>
<thead>
<tr>
<th>Time</th>
<th>Title</th>
<th>Speaker(s)</th>
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</thead>
<tbody>
<tr>
<td>10:00 AM - 10:26 AM</td>
<td><strong>Mtm Patient Deaths</strong></td>
<td>Carsten Bonnemann, M.D., National Institutes of Health, NINDS</td>
</tr>
<tr>
<td>10:26 AM - 10:52 AM</td>
<td><strong>The Challenges of DMD Gene Therapy Clinical Trials</strong></td>
<td>Michael Binks, M.D., Pfizer Worldwide Research, Development and Medical</td>
</tr>
<tr>
<td>10:52 AM - 11:18 AM</td>
<td><strong>Adeno-Associated Virus-Related Toxicities in Nonhuman Primates</strong></td>
<td>Juliette Hordeaux, D.V.M., Ph.D., Gene Therapy Program, University of Pennsylvania</td>
</tr>
<tr>
<td>11:18 AM - 11:45 AM</td>
<td><strong>Hemophilia - Addressing Durability and Variability of Gene Therapy</strong></td>
<td>Glenn Pierce, M.D., Ph.D., Third Rock Ventures</td>
</tr>
</tbody>
</table>
WEDNESDAY, MAY 12, 2021

10:00 AM - 11:45 AM

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

10:00 AM - 10:26 AM
Gene Therapy With Cyclin-A2 in Pigs
Hina Chaudhry, M.D., Mount Sinai Hospital

10:26 AM - 10:52 AM
Gene Therapy of Myosin Binding Protein C in Hypertrophic Cardiomyopathy
Julian Stelzer, Ph.D., Case Western Reserve University

10:52 AM - 11:18 AM
Cardiac Regenerative Strategies
Eldad Tzahor, Ph.D., Weizmann Institute of Science

11:18 AM - 11:45 AM
Cardiac Phosphoinositide 3-Kinase (P110α) as a Therapeutic Target for Diabetic Cardiomyopathy - An AAV-Mediated Gene Therapy Approach
Rebecca Ritchie, Ph.D., Monash University

10:00 AM - 11:45 AM

JERRY MENDELL AWARD FOR TRANSLATIONAL SCIENCE SYMPOSIUM

Translational Science Overcomes Obstacles for Treatment of Children With Neuromuscular Disease

Supported by Dr. Suku and Ann Nagendran

Jerry Mendell, M.D., Nationwide Children’s Hospital
10:45 AM - 12:15 PM

**EXHIBIT HALL OPEN - EXHIBITORS AVAILABLE TO CONNECT**
Exhibit Hall is Accessible 24 Hours

**10:45 AM - 12:15 PM**

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

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

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

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

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

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

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

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

**12:15 PM - 2:00 PM**

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

Sponsored by
2:00 PM - 3:30 PM

**EXHIBIT HALL OPEN - EXHIBITORS AVAILABLE TO CONNECT**

Exhibit Hall is Accessible 24 Hours

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**Cytiva - Platform processing for AAV production**

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

---

**Miltenyi Biotec - Latest Advances in Cell and Gene Therapy**

**Introduction**  
Matthew Porteus, M.D., Ph.D., Stanford University School of Medicine

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

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

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

**2:00 PM - 3:30 PM**

**24th Annual Virtual Meeting**  
**Wednesday, May 12, 2021**  
**All Times Listed in EDT**

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

<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
<th>Speaker(s)</th>
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<tbody>
<tr>
<td>2:00 PM</td>
<td>Welcome and Introduction</td>
<td>Bastiano Sanna, Ph.D., Vertex Pharmaceuticals Inc.</td>
</tr>
<tr>
<td>2:05 PM</td>
<td>Reviewing the Latest Advances in Genetic and Cell Therapy Technologies</td>
<td>Bastiano Sanna, Ph.D., Vertex Pharmaceuticals Inc.</td>
</tr>
<tr>
<td>2:25 PM</td>
<td>Restoring at the Molecular Level: CRISPR/Cas9-Mediated Gene Editing Technology</td>
<td>Eric N. Olson, Ph.D., UT Southwestern Medical Center</td>
</tr>
<tr>
<td>2:45 PM</td>
<td>Replacement at the Cellular Level: Transplantable Stem Cell-Derived Technology</td>
<td>Douglas A. Melton, Ph.D., Harvard University</td>
</tr>
<tr>
<td>3:05 PM</td>
<td>Panel Discussion</td>
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</tbody>
</table>

**Voyager Therapeutics - Advancing AAV Gene Therapy for CNS Disease**

<table>
<thead>
<tr>
<th>Time</th>
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<th>Speaker(s)</th>
</tr>
</thead>
<tbody>
<tr>
<td>2:00 PM</td>
<td>Welcome and Introduction</td>
<td>Omar Khwaja, M.D., Ph.D., Voyager Therapeutics</td>
</tr>
<tr>
<td>2:05 PM</td>
<td>Voyager Therapeutics’ Commitment to Advancing AAV Gene Therapy for CNS Disease</td>
<td>Andre Turenne, Voyager Therapeutics</td>
</tr>
<tr>
<td>2:15 PM</td>
<td>Building a Better AAV Capsid for CNS Gene Therapies</td>
<td>David Schaffer, Ph.D., University of California, Berkeley</td>
</tr>
<tr>
<td>2:35 PM</td>
<td>Optimizing the Transgene for AAV CNS Gene Therapies</td>
<td>Guangping Gao, Ph.D., University of Massachusetts Medical School</td>
</tr>
<tr>
<td>2:55 PM</td>
<td>Evolving Approaches to Direct Delivery of AAV CNS Gene Therapies</td>
<td>Mark Richardson, M.D., Ph.D., Massachusetts General Hospital</td>
</tr>
<tr>
<td>3:15 PM</td>
<td>Q&amp;A – Moderated by Omar Khwaja, M.D., Ph.D.</td>
<td>Panelists: David Schaffer, Ph.D.; Guangping Gao, Ph.D.; Mark Richardson, M.D., Ph.D.</td>
</tr>
</tbody>
</table>

**Industry Sponsored Symposia**
2:00 PM - 3:00 PM
CHAT LOUNGE NETWORKING

3:30 - 5:15 PM
OUTSTANDING NEW INVESTIGATOR SYMPOSIUM

Sponsored by

3:30 PM - 3:56 PM
Mechanisms Underlying CAR T Cell Function
Marcela Maus, M.D., Ph.D., Massachusetts General Hospital

3:56 PM - 4:22 PM
Enhancing Genome Editing Technologies Using Protein Engineering
Benjamin Kleinstiver, Ph.D., Massachusetts General Hospital

4:22 PM - 4:48 PM
Using the Blood to Treat the Brain: Engineering the Hematopoietic System to Treat Non-Hematological Diseases
Natalia Gomez-Ospina, M.D., Ph.D., Stanford

4:48 PM - 5:15 PM
Gene Therapy Approaches to β-hemoglobinopathies
Annarita Miccio, Ph.D., Institut Imagine

5:15 PM - 6:45 PM
EXHIBIT HALL OPEN - EXHIBITORS AVAILABLE TO CONNECT
Exhibit Hall is Accessible 24 Hours
# 24th Annual Virtual Meeting

## Abstract Sessions

### 5:15 PM - 7:15 PM

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

<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
<th>Presenter/Institution</th>
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<tbody>
<tr>
<td>5:30 PM - 5:45 PM</td>
<td>64: A Multi-Mechanistic Anti-Angiogenic AAV Gene Therapy Product Candidate, 4D-150, for the Treatment of Wet Age-Related Macular Degeneration (wAMD) and Diabetic Macular Edema (DME): Intravitreal Biodistribution, Transgene Expression, Safety and Efficacy in Non-Human Primates</td>
<td>Peter Francis, M.D., Ph.D., 4D Molecular Therapeutics</td>
</tr>
<tr>
<td>5:45 PM - 6:00 PM</td>
<td>65: Evolving Synthetic AAV Variants for Genome Editing in Immune Cell Populations</td>
<td>Jonathan Ark, Molecular Genetics and Microbiology, Duke University</td>
</tr>
<tr>
<td>6:00 PM - 6:15 PM</td>
<td>66: Real Time Blood Brain Barrier Disruption In A Multi-Species Model</td>
<td>Ana Rita Batista, Ph.D., University of Massachusetts Medical School</td>
</tr>
<tr>
<td>6:45 PM - 7:00 PM</td>
<td>69: Thermoresponsive Polymer-AAV Nanoparticle Vectors Improved Transgene Expression on Immunized Murine Model</td>
<td>Kai Wang, Ph.D., The University of North Carolina at Chapel Hill</td>
</tr>
<tr>
<td>7:00 PM - 7:15 PM</td>
<td>70: AAV Vector Dose Dependent Redundant and Non-Redundant Roles of TLR9 and IL1R Signaling in CD8 T Cell Activation Upon Muscle Gene Transfer</td>
<td>Ning Li, Ph.D., IU School of Medicine</td>
</tr>
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WEDNESDAY, MAY 12, 2021

5:15 PM - 7:15 PM

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

5:30 PM - 5:45 PM
71: Pre-Selected CAR_T_N/SCM Outperform CARTBULK In Driving Tumor Eradication In The Absence Of Severe CRS And ICANS
Silvia Arcangeli, Ph.D., San Raffaele Hospital

5:45 PM - 6:00 PM
72: CD5 CAR T-Cells Avoid Self-Elimination by Continuously Degrading CD5 Protein
Royce Ma, Baylor College of Medicine

6:00 PM - 6:15 PM
77: Precise Targeting of AML With First-in-Class OR / NOT Logic-Gated Gene Circuits in CAR-NK Cells
Brian Garrison, Ph.D., Senti Biosciences

6:15 PM - 6:30 PM
74: Investigating the Therapeutic Efficacy of Disruption of Cell Intrinsic Checkpoint Regulator CTLA-4 in Chimeric Antigen Receptor T cells
Sangya Agarwal, University of Pennsylvania Perelman School of Medicine

6:30 PM - 6:45 PM
75: Non-Human Primate Derived CD20 CAR T Cells Elicit a Bystander Effect on CD8 but Not CD4 CAR-T Cells
Ulrike Gerdemann, M.D., Dana Farber Cancer Institute; Boston Children’s Hospital

6:45 PM - 7:00 PM
76: Enhanced Generation of T-Cell Derived Naïve Pluripotent Cells as a Renewable Cell Source for the Mass Manufacture of Off-the-Shelf CAR T Cell Therapies
Yi-Shin Lai, Ph.D., Fate Therapeutics
Gene Therapies for Hemoglobinopathies  
Co-Chairs: John Chapin, M.D. and Pankaj Mandal, Ph.D.

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

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

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

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

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

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

7:00 PM - 7:15 PM  
83: Base Editing of the -200 Region of the γ-Globin Promoters Leads to Fetal Hb Reactivation and Rescues the Sickle Cell Disease Phenotype in Primary Patient Cells  
Panagiotis Antoniou, Institut Imagine
WEDNESDAY, MAY 12, 2021

5:15 PM - 7:15 PM

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

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

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

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

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

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

**6:45 PM - 7:00 PM**
90: IL-1a and IL-1b Are Essential for Inflammasome Independent CD8+ T Cell Responses to Hepatic AAV Gene Transfer
*Sandeep Kumar, Ph.D., Indiana University*
WEDNESDAY, MAY 12, 2021

5:15 PM - 7:15 PM

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

**5:30 PM - 5:45 PM**
92: Chemical Mediated Recruitment of Epigenetic Modifiers Regulate Adeno-Associated Virus Episomal Transgene Expression
Jessica Umana, University of North Carolina - Chapel Hill

**5:45 PM - 6:00 PM**
93: The Human Silencing Hub (HUSH Complex) is a Potent Regulator of AAV Transgene Silencing
Anshuman Das, Ph.D., Duke University

**6:00 PM - 6:15 PM**
94: Effects of Sexual Dimorphism and Genetic Background on AAV Tissue Transduction in Mice Following Intravenous Administration of a Diverse Capsid Pool
Elad Firnberg, Ph.D., REGENXBIO, Inc.

**6:15 PM - 6:30 PM**
95: High Throughput Screening of Diverse Mini-Promoter Libraries Within AAV via Expression Linked Promoter Selection (ELiPS)
Kazuomori Lewis, University of California, Berkeley

**6:30 PM - 6:45 PM**
96: GMEB2 is a Conserved Cellular AAV Restriction Factor That Inhibits Transduction of Human Stem Cells
Amanda Dudek, Ph.D., Stanford University

**6:45 PM - 7:00 PM**
97: Rationally Designed Inverted Terminal Repeats Improve AAV Vector Production
Liujiang Song, Ph.D., University of North Carolina, Chapel Hill

**7:00 PM - 7:15 PM**
98: Characterization of AAV Inverted Terminal Repeats by Atomic Force Microscopy
Marianne Laugel, University of Nantes
**Preclinical Gene Therapy for Neurologic Disorders II**  
Co-Chairs: Gwiladys Gernoux, Ph.D. and Juliette Hordeaux, D.V.M., Ph.D.

**5:30 PM - 5:45 PM**  
99: A Novel Exon Specific U1 snRNA Therapeutic Strategy to Prevent Retinal Degeneration in Familial Dysautonomia  
Anil Chekuri, Ph.D., Massachusetts General Hospital Research Institute; Harvard Medical School

**5:45 PM - 6:00 PM**  
105: Efficacy of a Vectorized Anti-Tau Antibody Using Systemic Dosing of a Blood Brain Barrier Penetrant AAV Capsid in Mouse Models of Tauopathies  
Wencheng Liu, Ph.D., Voyager Therapeutics

**6:00 PM - 6:15 PM**  
101: Evolution of Modified AAV Vectors in Rhesus Macaque Cochlea  
Paul Ranum, Ph.D., The Children’s Hospital of Philadelphia

**6:15 PM - 6:30 PM**  
102: A Novel Retinal Gene Therapy Strategy for Batten Disease and Beyond  
Maura Schwartz, The Research Institute at Nationwide Children’s Hospital

**6:30 PM - 6:45 PM**  
103: Reprogramming to Recover Youthful Epigenetic Information and Restore Vision  
Yuancheng Lu, Ph.D., Harvard Medical School

**6:45 PM - 7:00 PM**  
104: Efficacious, Safe, and Stable Inhibition of Corneal Neovascularization With rAAV-KH902 in a Mouse Model of Corneal Alkali Injury  
Wenqi Su, M.D., University of Massachusetts Medical School
5:15 PM - 7:15 PM

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

5:30 PM - 5:45 PM  
106: Combinatorial Modified mRNA Induces Cardiovascular Regeneration Post Muscle Ischemic Injury  
Keerat Kaur, Ph.D., Icahn School of Medicine, Mount Sinai

5:45 PM - 6:00 PM  
107: Sustained Episomal Transgene Expression In Vivo Driven by Non-Viral DNA Delivery to Rodent Liver  
Stoil Dimitrov, M.D., Ph.D., Moderna

6:00 PM - 6:15 PM  
108: Optimization of Transcutaneous Ultrasound Mediated Gene Delivery Into Large Animals  
Megan Manson, Seattle Children’s Research Institute

6:15 PM - 6:30 PM  
109: Assembling Several mRNA Strands for Facilitating mRNA Delivery With and Without Using Carriers  
Satoshi Uchida, M.D., Ph.D., Kyoto Prefectural University of Medicine; Kawasaki Institute of Industrial Promotion

6:30 PM - 6:45 PM  
110: Development of Hydrodynamics-Based Gene Therapy for Liver Cancer  
Kenya Kamimura, M.D., Ph.D., Niigata University

6:45 PM - 7:00 PM  
111: Hematopoietic Stem and Progenitor Cells-Targeted Polymeric Nanoparticles for In Vivo Gene Therapy  
Rkia El kharrag, Ph.D., Fred Hutchinson Cancer Research Center

7:00 PM - 7:15 PM  
112: Delivery of CRISPR/Cas9 for Recovering the Expression of the Endogenous FVIII in Hemophilia A Mice  
Chun-Yu Chen, Ph.D., Seattle Children’s Hospital Research Institute
Targeted Gene and Cell Therapy for Cancer
Co-Chairs: Michael Milone, M.D., Ph.D. and Hernando Lopez-Bertoni, Ph.D.

5:30 PM - 5:45 PM
113: Gene-based Immune Reprogramming Overcomes the Immunosuppressive Microenvironment of Liver Metastases and Enables Protective T Cell Responses
Thomas Kerzel, San Raffaele Telethon Institute for Gene Therapy

5:45 PM - 6:00 PM
114: Inducible Tumor-Targeted Interferon-α Gene Therapy Inhibits Glioblastoma Multiforme in Mouse Model Without Adverse Systemic Effects
Filippo Birocchi, San Raffaele Telethon Institute for Gene Therapy

6:00 PM - 6:15 PM
115: Design and Demonstration of Potent In Vitro and In Vivo Activity for CART-ddBCMA, a BCMA-Targeted CAR-T Cell Therapy Incorporating a Non-scFv Binding Domain
Janine Buonato, Ph.D., Arcellx

6:15 PM - 6:30 PM
116: A SOX2 Engineered Epigenetic Silencer Factor Represses the Cancer Genetic Program and Eradicate Glioblastoma Development
Vania Broccoli, Ph.D., San Raffaele Scientific Institute

6:30 PM - 6:45 PM
117: Oncolytic Adeno-Immunotherapy Expressing IL-12p70 and Immune Checkpoint Blockade PD-L1 Minibody Modulates the Host Immune System to Enable HER2.CAR T-Cells to Cure Pancreatic Tumors
Amanda Rosewell Shaw, Ph.D., Baylor College of Medicine

6:45 PM - 7:00 PM
118: UCARTCS1A, an Allogeneic CAR T-Cell Therapy Targeting CS1 in Patients With Relapsed/Refractory Multiple Myeloma (RRMM): Preliminary Translational Results From a First-in-Human Phase I Trial (MELANI-01)
Krina Patel, M.D., The University of Texas M.D. Anderson Cancer Center

7:00 PM - 7:15 PM
119: A Drug-Regulated Anti-CD33 Chimeric Antigen Receptor With Potent Anti-AML Activity and a Reversible On-Off Switch
Jacob Applebaum, M.D., Ph.D., Seattle Children’s Therapeutics; University of Washington
WEDNESDAY, MAY 12, 2021

5:15 PM - 7:15 PM

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

5:30 PM - 5:45 PM
120: Co-Identification and Characterization of Host and Viral Protein Interactomes During AAV Production by Two Different Proximity Labeling Methods
*Ji Sun Lee, Ph.D., University of Massachusetts Medical School*

5:45 PM - 6:00 PM
121: The Effects of ITR Structure and Plasmid Backbone on Plasmid Stability and Yield
*Ruofan Wang, Ph.D., Vigene Biosciences*

6:00 PM - 6:15 PM
122: Increasing Gene Therapy Vector Production Using Viral Sensitizer Molecules
*Jean-Simon Diallo, Ph.D., Virica Biotech, Inc.*

6:15 PM - 6:30 PM
123: Transcriptional Response of HEK293 Cells to Clinical-Scale Recombinant Adeno Associated Virus Production by Transient Transfection
*Cheng-Han Chung, Ph.D., Pfizer, Inc.*

6:30 PM - 6:45 PM
124: Vector Engineering of pRep-Cap and pHelper Enhanced AAV Productivity by Triple Transfection in Suspension HEK293 Cells
*Bingnan Gu, Ph.D., Lonza Houston Inc.*

6:45 PM - 7:00 PM
125: High Titer rAAV Production Upon Upstream Process Development of Stable Helper-Virus Free ELEVECTA® Producer Cells
*Juliana Coronel, Ph.D., Cevec Pharmaceuticals GmbH*

7:00 PM - 7:15 PM
126: Genome-Wide CRISPR Activation Screen Reveals That SKA2 and ITPRIP Increase AAV Manufacturing via Cell Cycle Modulation
*Hyunchul Lee, D.V.M., Ph.D., University of California, Berkeley*
WEDNESDAY, MAY 12, 2021

5:15 PM - 6:45 PM

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

**Industry Symposium: Pall Corporation**

5:15 PM - 6:15 PM

Networking Roulette
**Tools and Technology Forum II**

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

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

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

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

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

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

**6:45 PM - 7:00 PM**
Automated Parallel Chromatography to Accelerate Downstream Process Development in Gene Therapy
Jana Langhoff, Tecan; Tim Schroeder, Repligen
THURSDAY, MAY 13, 2021

ALL DAY

DIGITAL ABSTRACT PRESENTATIONS

EXHIBIT HALL

Be sure to connect with exhibit booth staff during listed times

9:00 AM - 10:00 AM

CHAT LOUNGE NETWORKING

10:00 AM - 11:45 AM

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

10:00 AM - 10:26 AM

Alternate Careers in Vector Production and Consulting
Jeffrey Medin, Ph.D.,
Medical College of Wisconsin

10:26 AM - 10:52 AM

Building and Developing a Team to Make Translation Possible
Parameswaran Hari, M.D.,
Medical College of Wisconsin

10:52 AM - 11:18 AM

The Value of Diversity and Inclusion in the Scientific Workforce
Lynn Gordon, M.D., Ph.D.,
University of California Los Angeles

11:18 AM - 11:45 AM

Dealing With Research Interruptions: From Parenting to the Pandemic
Catherine Bollard, M.D., M.B.Ch.B.,
Children’s National Hospital/The George Washington University
THURSDAY, MAY 13, 2021

10:00 AM - 11:45 AM

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

10:00 AM - 10:35 AM  *Overview of Liver Gene Therapies for Inborn Errors of Metabolism*
Gloria Gonzalez-Aseguinolaza, Ph.D., Cima-Universidad de Navarra and Vivet Therapeutics

10:35 AM - 11:10 AM  *Creation and Treatment of Murine Models of Liver-Based Inherited Enzyme Deficiencies Using CRISPR/Cas9 Gene Editing Technology*
Cary Harding, M.D., Oregon Health & Sciences University

11:10 AM - 11:45 AM  *Lipid Nanoparticles for Therapeutic Gene Targeting to the Liver*
Pieter Cullis, Ph.D., University of British Columbia, Vancouver

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

10:00 AM - 10:35 AM  *Purification of Tissue Specific EVs*
Erez Eitan, Ph.D., Neurodex

10:35 AM - 11:10 AM  *Bacterial-Derived Outer Membrane Vesicles for Gene Delivery*
Angela Panier, Ph.D., University of Nebraska-Lincoln

11:10 AM - 11:45 AM  *Exosome Delivery of Sars CoV-2 Vaccines*
Linda Marban, Ph.D., Capricor
THURSDAY, MAY 13, 2021

10:00 AM - 11:45 AM

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

10:00 AM - 10:26 AM  
iPS Cell-Based Therapy for Parkinson’s Disease  
Jun Takahashi, M.D., Ph.D., Kyoto University

10:26 AM - 10:52 AM  
Combined Cell and Gene Therapy of Epidermolysis Bullosa  
Michele De Luca, M.D. University of Modena and Reggio Emilia

10:52 AM - 11:18 AM  
Stem Cell Gene Therapy for Primary Immunodeficiencies  
Claire Booth, D.Phil., UCL Great Ormond Street Institute of Child Health

11:18 AM - 11:45 AM  
Gene Therapy in Fanconi Anemia: Current Strategies to Enable the Correction of HSCs  
Paula Rio, Ph.D., Centro de Investigaciones Energéticas Medioambientales y Tecnológicas and Centro de Investigación Biomédica en Red de Enfermedades Raras (CIEMAT/CIBERER)/Instituto de Investigación Sanitaria Fundación Jiménez (IIS-FJD, UAM)

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

10:00 AM - 10:20 AM  
Addressing Racial Disparities Due to Poverty: Partnering With Biotechnology Companies  
Rob Perez, Life Science Cares

10:20 AM - 10:40 AM  
NHLBI Cure Sickle Cell Initiative: An Update  
Traci Mondoro, Ph.D., National Institutes of Health, NHLBI

10:40 AM - 11:00 AM  
The Significance of Training and Mentorship for Underrepresented Groups in the Science Workforce  
Melody Smith, M.D., Memorial Sloan Kettering Cancer Center

11:00 AM - 11:20 AM  
Supporting and Enacting Change  
Rayne Rouce, M.D., Baylor College of Medicine

11:20 AM - 11:45 AM  
Panel Discussion
**THURSDAY, MAY 13, 2021**

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

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

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

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

<table>
<thead>
<tr>
<th>Time</th>
<th>Session</th>
</tr>
</thead>
</table>
| 10:10 AM   | **Successes in Vaccine Development**  
*Ugur Sahin, M.D., Ph.D., Biontech* |
| 10:35 AM   | **Successes in Vaccine Development**  
*Tonya Villafana, Ph.D., AstraZeneca* |
| 11:00 AM   | **Blowback, Re-Emergence, and Antigenic Drift of Infectious Disease**  
*Scott Hensley, Ph.D., University of Pennsylvania* |
| 11:25 AM   | **Panel Discussion**                                                                        |
24th Annual Virtual Meeting

THURSDAY, MAY 13, 2021

10:00 AM - 11:45 AM

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

10:00 AM - 10:26 AM  Retroviral Vector Production
Isabelle Riviere, Ph.D., Memorial Sloan-Kettering Cancer Center

10:26 AM - 10:52 AM  Large Scale Production of Lentiviral Vectors
Anne Galy, Ph.D., Genethon

10:26 AM - 10:52 AM  Large Scale Production of AAV Vectors
Patrick Santambien, Ph.D., Genethon

10:52 AM - 11:18 AM  Academic Vector Production for Early Phase Clinical Studies
Johannes van der Loo, Ph.D., Children’s Hospital of Philadelphia

11:18 AM - 11:45 AM  Lentiviral Vector Manufacture
Boro Dropulic, Ph.D., CARing Cross

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

10:00 AM - 10:21 AM  A Late Gene Transcription Factor of Rhesus Cytomegalovirus Vectors is Required to Elicit MHC-II and MHC-E-Restricted CD8 T Cells and Protect Against SIV Challenge
Klaus Früh, Ph.D., Oregon Health and Science University

10:21 AM - 10:42 AM  Human Immune Response To AAV Vectors
Katherine High, M.D., Asklepios BioPharmaceuticals

10:42 AM - 11:03 AM  AAV Vectors: Are They Safe?
Arun Srivastava, Ph.D, University of Florida

11:03 AM - 11:24 AM  Safety and Use of Retroviral Vectors
Christopher Doering, Ph.D., Emory University

11:24 AM - 11:45 AM  Safety of Adenoviral Vectors: A Platform for Therapy of Genetic Diseases, Cancer, and Novel Vaccines
Dmitry Shayakhmetov, Ph.D., Emory University School of Medicine
## Schedule

**ALL TIMES LISTED IN EDT**

**THURSDAY, MAY 13, 2021**

10:45 AM - 12:15 PM

### EXHIBIT HALL OPEN - EXHIBITORS AVAILABLE TO CONNECT

Exhibit Hall is Accessible 24 Hours

<table>
<thead>
<tr>
<th>Time</th>
<th>Presentation</th>
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</thead>
</table>
| 10:45 AM - 11:30 AM | BIA Separations now a Sartorius company - Chromatin (DNA) Removal From Harvest Before an AAV Capture Step Greatly Improves Robustness, Purity, and Yield of the Overall Downstream Process  
Ales Strancar, Ph.D., BIA Separations now a Sartorius company |
| 10:45 AM - 11:30 AM | Bristol Myers Squibb - A New Treatment Option for Adults with Relapsed or Refractory (R/R) Large B-Cell Lymphoma (LBLC) After Two or More Lines of Systemic Therapy  
Krish Patel, M.D., Swedish Cancer Institute; William Mir, Bristol Myers Squibb; and Erin McCaffrey, Pharm.D., Bristol Myers Squibb |
| 11:30 AM - 12:15 PM | Brooks Life Sciences GENEWIZ Inc. - Innovative Genomics & Cold Chain Solutions for Development of Cell and Gene Therapies  
Elizabeth Louie, GENEWIZ, A Brooks Life Sciences Company; and Kathi Shea, Brooks Life Sciences |
| 11:30 AM - 12:15 PM | Solentim - Robust Workflow for Single Cell Cloning of iPSCs for Making cGMP Master Cell Banks  
Ian Taylor, Ph.D., Solentim |

12:15 PM - 2:00 PM

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

Sponsored by AskBio
THURSDAY, MAY 13, 2021

2:00 PM - 3:30 PM

EXHIBIT HALL OPEN - EXHIBITORS AVAILABLE TO CONNECT

Exhibit Hall is Accessible 24 Hours

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

2:00 PM - 2:25 PM
The Importance of Starting Material for Cell Therapies - Your CAR Depends on It
Dominic Clarke, Ph.D., HemaCare at Charles River Laboratories

2:25 PM - 2:45 PM
CAR-T Binder Discovery Simplified: Any Target, 50 Antibodies. Engineered Specifically for CAR Formats.
Sarah Ives, PSM, Distributed Bio at Charles River Laboratories

2:45 PM - 3:05 PM
In Vitro Efficacy and Safety Testing of Your Cellular Therapy - A Good CAR Inspection
Sabrina de Munnik, Ph.D., Charles River Laboratories

3:05 PM - 3:30 PM
Take Your CAR and Drive It - Charting Your Path to Market
Steven Miklasz, MSc, CBA, Charles River Laboratories

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

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

GenScript - Innovative Solutions and Approaches in Immunotherapy

2:00 PM - 2:30 PM
De Novo Design of a Self-Assembling Super Antigen: A Potential Cancer Immunotherapy via Controlled T Cell Activation
Possu Huang, Ph.D., Stanford University

2:30 PM - 3:00 PM
Library-Selected AAV Variants Can Effectively Translate to Non-Human Primates in the Spinal Cord and Cochlea
Killian Hanlon, Ph.D., Harvard Medical School; Massachusetts General Hospital

3:00 PM - 3:30 PM
Precise and Efficient Non-Viral CRISPR Gene Editing Solutions
Lumeng Ye, Ph.D., GenScript

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

2:00 PM - 2:10 PM
Genetic Medicines From Discovery to Commercial Production
Andrew Geall, Ph.D., Precision NanoSystems Inc.

2:10 PM - 2:30 PM
Effects of Polymeric and Lipid Nanoparticle Self-Amplifying RNA Formulation on Protein Expression and Vaccine Immunogenicity
Anna Blakney, Ph.D., University of British Columbia

2:30 PM - 2:50 PM
Engineering of RNA CAR T Cells With Novel Lipid Nanoparticles for Gene Delivery
Samuel Clarke, Ph.D., Precision NanoSystems Inc.

2:50 PM - 3:10 PM
Manufacturing Considerations for the Development of Lipid Nanoparticles Using Microfluidics
Yvonne Perrie, Ph.D., University of Strathclyde

3:10 PM - 3:30 PM
Round Table Discussion: Genetic Medicine Development With Lipid Nanoparticles
Chair: Vicki Stronge, Ph.D., Precision NanoSystems Inc.
Panelists: Anna Blakney, Ph.D.; Samuel Clarke, Ph.D.; Andrew Geall, Ph.D.; and Yvonne Perrie, Ph.D.
THURSDAY, MAY 13, 2021

2:00 PM - 3:00 PM

CHAT LOUNGE NETWORKING

3:30 PM - 5:15 PM

PRESIDENTIAL SYMPOSIUM AND PRESENTATION OF TOP ABSTRACTS

Sponsored by

3:30 PM - 4:15 PM

Oncolytic Immunovirotherapy: Uniting Genetic Engineering, Virology, Immunology, and Cancer Research
Robert Martuza, M.D., Harvard Medical School and Massachusetts General Hospital

4:15 PM - 4:30 PM

127: Base Editing Rescues Sickle Cell Disease in Human Hematopoietic Stem Cells and in Mice
Jonathan Yen, Ph.D., St. Jude Children’s Research Hospital

4:30 PM - 4:45 PM

128: Safety and Efficacy Results with a Single Dose of Autologous CRISPR-Cas9-Modified CD34+ Hematopoietic Stem and Progenitor Cells (HSPCs) in Transfusion-Dependent β-Thalassemia (TDT) and Sickle Cell Disease (SCD)
Tony W. Ho, M.D., CRISPR Therapeutics

4:45 PM - 5:00 PM

129: Immunostimulatory Bacterial Antigen-armed Oncolytic Measles Virotherapy Significantly Increases the Potency of Anti-PD1 Checkpoint Therapy
Eleni Panagioti, Ph.D., Mayo Clinic

5:00 PM - 5:15 PM

130: In-Vivo Engineered B Cells Retain Memory and Secrete High Titers of Anti-HIV Antibodies in Mice
Alessio David Nahmad, M.Sc., Tel Aviv University
THURSDAY, MAY 13, 2021

5:15 PM - 6:45 PM

EXHIBIT HALL OPEN - EXHIBITORS AVAILABLE TO CONNECT
Exhibit Hall is Accessible 24 Hours

5:15 PM - 7:15 PM

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

5:30 PM - 5:45 PM
131: AAV-Mediated GJB2 Gene Therapy Rescues Hearing Loss and Cochlear Damage in a Mouse Model of Congenital Hearing Loss Caused by Conditional Connexin26 Knockout
Pranav Mathur, Ph.D., Otonomy Inc.

5:45 PM - 6:00 PM
132: AAV9 Mediated Delivery of PUF RNA Targeting System Corrects Molecular and Functional Defects in a Myotonic Dystrophy Type 1 MOUSE Model
Ranjan Batra, Ph.D., Locanabio, Inc.

6:00 PM - 6:15 PM
133: Intracorneal and Sequential Contralateral Dosing of AAV-opt-ARSB Reverses MPS VI Corneal Clouding
Matthew Hirsch, Ph.D., University of North Carolina, Chapel Hill

6:15 PM - 6:30 PM
134: Efficacy and Biodistribution of Anc80-RK-hRPGRIP1 Gene Therapy in a Mouse Model of Rpgrip1 Deficiency and in Non-Human Primate
Binit Kumar, Ph.D., PTC Therapeutics Inc.

6:30 PM - 6:45 PM
135: Bicistronic AAV Gene Therapy for Tay-Sachs and Sandhoff Diseases
Toloo Taghian, Ph.D., University of Massachusetts Medical School

6:45 PM - 7:00 PM
136: Gene Therapy Rescues Cone and Rod Function in a Pre-Clinical Model of CDHR1-Associated Retinal Degeneration Through Restoration of Photoreceptor Outer Segments
Imran Yusuf, M.D., University of Oxford

7:00 PM - 7:15 PM
137: scAAV9 Gene Replacement Therapy for Epileptic SLC13A5 Deficiency
Rachel Bailey, Ph.D., University of Texas Southwestern Medical Center
THURSDAY, MAY 13, 2021

5:15 PM - 7:15 PM

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

5:30 PM - 5:45 PM
138: Dissecting the Transcriptional and Epigenetic Landscape of hiPSC-Derived Neural Stem and Progenitor Cells: Implications for Cell Therapy Approaches
Vasco Meneghini, Ph.D., San Raffaele Telethon Institute for Gene Therapy

5:45 PM - 6:00 PM
139: Receptor Engineered TRuCTregs Maintain a Regulatory Phenotype and Are Suppressive in a Murine Model of Hemophilia A
Jyoti Rana, Ph.D., IUPUI

6:00 PM - 6:15 PM
140: Functional Benefit of Mitochondrially Augmented HSPCs: Improved Engraftment and Alterations in Immune Cell Differentiation
Noa Sher, Ph.D., Minovia Therapeutics Ltd

6:15 PM - 6:30 PM
141: Memory Enriched Epstein-Barr Virus (EBV) Specific T-cells With Broader Target Antigen Repertoire For The Treatment Of EBV+ Malignancies
Sandhya Sharma, Baylor College of Medicine

6:30 PM - 6:45 PM
142: Non-Viral Engineering of CAR-NK Cells Using the TC Buster Transposon System™
Emily Pomeroy, University of Minnesota

6:45 PM - 7:00 PM
143: Adoptively Transferred, In Vitro-Generated Alloantigen-Specific Type 1 Regulatory T (Tr1) Cells Persist Long-Term In Vivo
Alma-Martina Cepika, M.D., Ph.D., Stanford University School of Medicine

7:00 PM - 7:15 PM
144: Endothelial Progenitor Cells Engineered to Overexpress Endothelial NO-Synthase May Improve Infarct Healing: Results from the Enhanced Angiogenic Cell Therapy - Acute Myocardial Infarction (ENACT-AMI) Trial
Duncan Stewart, M.D., Ottawa Hospital Research Institute
THURSDAY, MAY 13, 2021

5:15 PM - 7:15 PM

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

5:30 PM - 5:45 PM
145: Chimeric Antigen Receptor Macrophages (CAR-M) Induce Anti-Tumor Immunity and Synergize With Immune Checkpoint Inhibitors in Pre-Clinical Solid Tumor Models
Stefano Pierini, Ph.D., Carisma Therapeutics

5:45 PM - 6:00 PM
146: Bivalent CD19/CD20-Specific CAR T Cells With 4-1BB and Mutated CD28 Co-Stimulatory Domains Show Enhanced Function
Emiliano Roselli, Ph.D., Moffitt Cancer Center

6:00 PM - 6:15 PM
147: Combining IAP Inhibitors With CAR T Cell Therapy to Treat Glioblastoma
Edward Song, University of Pennsylvania

6:15 PM - 6:30 PM
148: B-CLL-Mediated Insufficient Activation Is CAR-Independent
McKensie Collins, University of Pennsylvania

6:30 PM - 6:45 PM
149: CAR Design and Expression Determine Hyper-Proliferative States in TET2 Deficient T Cells
Nayan Jain, Memorial Sloan Kettering Cancer Center

6:45 PM - 7:00 PM
150: Tumor-Responsive, Multifunctional CAR-NK Cells Cooperate With Impaired Autophagy to Infiltrate and Target Glioblastoma
Jiao Wang, Ph.D., Purdue University

7:00 PM - 7:15 PM
151: ADCLEC.syn1 Is a Novel Combinatorial CAR Platform for Enhanced Therapeutic Index in AML
Sascha Haubner, M.D., Memorial Sloan Kettering Cancer Center
THURSDAY, MAY 13, 2021

5:15 PM - 7:15 PM

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

5:30 PM - 5:45 PM
152: Systemic Hps1 Gene Augmentation Prevents Pulmonary Manifestations in a Mouse Model of Hermansky-Pudlak Syndrome
May Malicdan, M.D., Ph.D., National Institutes of Health

5:45 PM - 6:00 PM
153: Generation of a Human 3D Lung Model for Therapeutic Gene Editing in Surfactant Protein B Deficiency
Helena Meyer-Berg, University of Oxford

6:00 PM - 6:15 PM
154: Vectored Immunoprophylaxis for COVID-19 (COVIP)
Yue Du, Ph.D., University of Oxford

6:15 PM - 6:30 PM
155: Impact of Transplant Immunosuppression on In Vivo Lung-Selective CRISPR/Cas9 Therapeutics for Lung Transplantation
Kumi Mesaki, University Health Network

6:30 PM - 6:45 PM
156: First Proof-of-Concept of miQURE™ Based Gene Targeting in the Liver: Lipid Lowering and Atherosclerosis Suppression by AAV-miQURE™-Mediated ANGPTL3 Targeting
Vanessa Zancanella, Ph.D., uniQure Biopharma

6:45 PM - 7:00 PM
158: Electroporation Mediated Gene Transfer of MRCKa to the Lungs of Mice Effectively Treats Pre-Existing Acute Lung Injury
Jing Liu, University of Rochester School of Medicine and Dentistry
Clinical Trials and Advanced Preclinical Studies for Neurologic Diseases
Co-Chairs: Heather Gray-Edwards, D.V.M., Ph.D. and Patricia Dickson, M.D.

5:30 PM - 5:45 PM
159: Gene Therapy Candidate for Metachromatic Leukodystrophy (MLD): Summary of Preclinical In Vivo Data Following an Intravenous Delivery of HMI-202
Jacinthe Gingras, Ph.D., Homology Medicines Inc.

5:45 PM - 6:00 PM
160: Gene Replacement Therapy for Angelman Syndrome
Justin Percival, Ph.D., University of Pennsylvania

6:00 PM - 6:15 PM
161: An AAV-miRNA for Androgen Receptor Knockdown in Spinal and Bulbar Muscular Atrophy
Eileen Workman, Ph.D., University of Pennsylvania

6:15 PM - 6:30 PM
162: AXO-AAV-GM1 for the Treatment of GM1 Gangliosidosis: Preliminary Results From a Phase I-II Trial
Cynthia Tifft, M.D., Ph.D., National Institutes of Health, NHGRI

6:30 PM - 6:45 PM
163: AXO-Lenti-PD Gene Therapy for Parkinson’s Disease: Efficacy, Safety, and Tolerability Data from the Second Cohort in Open-Label Dose Evaluation Study SUNRISE-PD at 6 Months Post Administration
Gavin Corcoran, M.D., Sio Gene Therapies Inc.

6:45 PM - 7:00 PM
164: Safety Evaluation of IV-Administered BBP-812, an AAV9-Based Gene Therapy for the Treatment of Canavan Disease, in Mice and Juvenile Cynomolgus Macaques
David Scott, Ph.D., Aspa Therapeutics

7:00 PM - 7:15 PM
165: Gene Replacement Therapy for SURF1-Related Leigh Syndrome Using AAV9
Qinglan Ling, Ph.D., UT Southwestern
## THURSDAY, MAY 13, 2021

**5:15 PM - 7:15 PM**

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

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<tr>
<th>Time</th>
<th>Session Title</th>
<th>Presenter(s)</th>
</tr>
</thead>
<tbody>
<tr>
<td>5:30 PM - 5:45 PM</td>
<td>166: Novel Platform for Transport and Delivery of Recombinant Adeno-Associated Virus without Need for Cold Storage during Transit</td>
<td>Maria Croyle, Ph.D., University of Texas at Austin</td>
</tr>
<tr>
<td>5:45 PM - 6:00 PM</td>
<td>167: Exposing the Content of Different AAV Fractions after Ultracentrifugation</td>
<td>David Dobnik, Ph.D., National Institute of Biology</td>
</tr>
<tr>
<td>6:00 PM - 6:15 PM</td>
<td>168: Optimization of Affinity Purification for Adeno-associated Viral Vectors</td>
<td>Huiren Zhao, Amgen Inc.</td>
</tr>
<tr>
<td>6:15 PM - 6:30 PM</td>
<td>169: Characterization of rAAV Key Quality Attributes Generated From A Highly Optimized, Hela 3.0 Producer Cell Line (PCL) Production Platform</td>
<td>Nicholas Richards, Ultragenyx Pharmaceuticals, Inc.</td>
</tr>
<tr>
<td>6:30 PM - 6:45 PM</td>
<td>170: AAVX Resin Binding Site Identification via Library Screening Analysis on Novel AAV Vectors</td>
<td>Zachary Thorpe, Affinia Therapeutics</td>
</tr>
<tr>
<td>6:45 PM - 7:00 PM</td>
<td>171: Analysis of Gene Therapy Products by Charge Detection Mass Spectrometry</td>
<td>Benjamin Draper, Ph.D., Megadalton Solutions</td>
</tr>
<tr>
<td>7:00 PM - 7:15 PM</td>
<td>172: Use of SPTFF in Continuous Downstream Manufacturing of Adeno-Associate Viruses</td>
<td>Rajeshwar Chinnawar, Ph.D., Pall Biotech</td>
</tr>
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THURSDAY, MAY 13, 2021

5:15 PM - 7:15 PM

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

5:30 PM - 5:45 PM
173: A Humanized EBV Mouse Model to Evaluate the Safety/Impact of Human Treg Cell Therapy on Antiviral Immune Responses
Swati Singh, Seattle Children’s Research Institute

5:45 PM - 6:00 PM
174: Candidate Selection in BALB/c Mice towards a Single Dose AAV-based COVID19 Prophylactic Vaccine
Wenlong Dai, Ph.D., Schepens Eye Research Institute and Massachusetts Eye and Ear Infirmary

6:00 PM - 6:15 PM
175: AAV Specific CAR Regulatory T Cells Mitigate Immune Responses Against AAV Gene Therapy
Motahareh Arjomandnejad, Ph.D., University of Massachusetts Medical School

6:15 PM - 6:30 PM
176: Engineered Protein M Analogs Enhance the Ability to Suppress Vector Neutralizing Antibodies and Generate a Window for Successful Gene Delivery
Charles Askew, Ph.D., University of North Carolina, Chapel Hill

6:30 PM - 6:45 PM
177: In Vivo HSC Gene Therapy with High-Level, Erythroid-Specific Expression of a Secreted SARS-CoV-2 Decoy Receptor
Andre Lieber, M.D., Ph.D., University of Washington

6:45 PM - 7:00 PM
178: A Platform for Genome Editing of Human B Cells to Produce Single-Chain Antibody-Like Molecules That Recapitulate Antibody Functionality
Geoffrey Rogers, Ph.D., University of Southern California

7:00 PM - 7:15 PM
179: Immune Modulation Preceding AAV9-GLB1 Gene Therapy Preserves the Possibility for Re-Dosing in Children with GM1 Gangliosidosis
Precilla D’Souza, National Institutes of Health, NHGRI
New Gene Editing Technologies and Applications
Co-Chairs: Alexis Komor, Ph.D. and T.J. Cradick, Ph.D.

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

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

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

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

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

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

7:00 PM - 7:15 PM
186: Accurate Quantification of CRISPR/Cas9 Induced Large Deletions, Insertions and Chromosomal Rearrangements Using SMRT Sequencing with Unique Molecular Identifiers
So Hyun Park, Ph.D., Rice University
Novel AAV Biology and Platform Technologies  
Co-Chairs: Lauren Woodard, Ph.D. and Anna Maurer, Ph.D.

5:30 PM - 5:45 PM  
187: Effect of pH and Temperature on AAV2 Capsid Structure and Stability  
Joshua Hull, University of Florida

5:45 PM - 6:00 PM  
188: Development of a Split Rep/Cap System to Improve AAV Capsid Production  
Derek Carbaugh, Ph.D., AskBio

6:00 PM - 6:15 PM  
189: Intravenous Administration of a Barcoded and Pooled AAV Library for the Comprehensive Characterization and Comparison of Capsid Tropisms  
April Giles, Ph.D., REGENXBIO

6:15 PM - 6:30 PM  
190: AAV Capsid Property Estimation is Improved by Combining Single-Molecule ID Tags and Hierarchical Bayesian Modeling of Experimental Processes  
Kathy Lin, Ph.D., Dyno Therapeutics

6:30 PM - 6:45 PM  
191: Hydroxylation of N-acetylneuraminic Acid Influences the In Vivo Tropism of N-linked Sialic Acid-Binding Adeno-Associated Viruses AAV1, AAV5 and AAV6  
Estrella Lopez-Gordo, Ph.D., Icahn School of Medicine at Mount Sinai

6:45 PM - 7:00 PM  
192: High-Throughput Screening of AAV Productivity to Enable Rapid Capsid Characterization  
Jenny Egley, REGENXBIO

7:00 PM - 7:15 PM  
193: AAV-GPseq Analysis of Vectors from HEK293 and BEV/Sf9 Production Platforms Reveals Differential Genome Heterogeneity and Enrichment of Potential Innate Immune DNA Epitopes in Empty Capsids  
Ngoc Tam Tran, Ph.D., University of Massachusetts Medical School
THURSDAY, MAY 13, 2021

5:15 PM - 6:45 PM

Dyno Therapeutics - Building Dyno Therapeutics

5:15 PM - 5:35 PM  Dyno’s Origins: Starting a Gene Therapy Company
Q&A with co-founders: Eric Kelsic, Ph.D. and Sam Sinai, Ph.D., Dyno Therapeutics

5:35 PM - 5:59 PM  Dyno’s Science Today: Engineering Best-in-Class Capsids
Q&A with Dyno Scientists: Cem Sengel, Ph.D.; Kathy Lin, Ph.D.; Christopher Reardon; and Elina Locane, Ph.D., Dyno Therapeutics

5:59 PM - 6:21 PM  Dyno’s Culture Today: How We Work Together as a Team
Q&A with Dyno Executives: Andrea Szekely-Hill, Paige Swanson, and Tyson Bertmaring, Dyno Therapeutics

6:21 PM - 6:45 PM  Dyno’s Future: Maximizing Our Positive Impact on Patients
Q&A with Dyno R&D: Eric Kelsic, Ph.D.; Jamie Kwasnieski, Ph.D.; Sylvain Lapan, Ph.D.; and Jeffrey Gerold, Ph.D., Dyno Therapeutics

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

5:15 PM - 6:45 PM  Moderated by John Conway, 20/15 Visioneers
Speakers: Ernie Bognar, Ph.D., Gradalis; Trent Carrier, Ph.D., MBA, L7 Informatics, Inc.; Vasu Rangadass, Ph.D., L7 Informatics, Inc.; and Jeff McDaniel, L7 Informatics, Inc.
WEDNESDAY, MAY 12, 2021

5:15 PM - 6:15 PM

Networking Roulette

5:15 PM - 7:00 PM

Tools and Technology Forum III

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

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

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

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

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

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

6:45 PM - 7:00 PM
High Throughput Upstream Processing and Quality Analysis via Microfluidic Capillary Electrophoresis
James Geiger, Ph.D., PerkinElmer
FRIDAY, MAY 14, 2021

ALL DAY DIGITAL ABSTRACT PRESENTATIONS EXHIBIT HALL
Be sure to connect with exhibit booth staff during listed times

9:00 AM - 10:00 AM
CHAT LOUNGE NETWORKING

10:00 AM - 11:45 AM

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

10:00 AM - 10:26 AM
CNS Toxicity in Higher Mammals Related to AAV-Mediated RNAi Gene Therapy
Beverly Davidson, Ph.D., Children’s Hospital of Philadelphia

10:26 AM - 10:52 AM
Splice Correction and Reduction of Toxic DMPK RNA In Vitro and In Vivo Utilizing Novel Antibody Targeted Antisense Oligonucleotides
Romesh Subramanian, Ph.D., Dyne Therapeutics

10:52 AM - 11:18 AM
Discussion of N-Lorem Foundation's Mission and Progress to Create Individual Antisense Therapies for Patients in The United States With Ultra-Rare Diseases Caused by Genetic Mutations That Affect Approximately N=1-10 Patients in the World
Stanley Crooke, M.D. Ph.D., n-Lorem Foundation

11:18 AM - 11:45 AM
Opportunities and Challenges in Development of ASOs for Treatment of Neuromuscular Disease
Toby Ferguson, M.D., Ph.D., Biogen

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

10:00 AM - 10:26 AM
Electroporation to Deliver DNA Coronavirus Vaccine
Kate Broderick, Ph.D., Inovio Pharmaceuticals, Inc.

10:26 AM - 10:52 AM
RNA-Guide DNA Insertion With CRISPR-Associated Transposases
Feng Zhang, Ph.D., Massachusetts Institute of Technology

10:52 AM - 11:18 AM
mRNA as Therapeutics to Treat Rare Genetic Disorders
Paolo Martini, Ph.D., Moderna

11:18 AM - 11:45 AM
Ultrasound-Targeted Microbubble Destruction to Facilitate Gene Delivery
Mark Borden, Ph.D., University of Colorado
24th Annual Virtual Meeting

FRIDAY, MAY 14, 2021

10:00 AM - 11:45 AM

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

10:00 AM - 10:15 AM
**Newborn Screening: An Inside Look at the Landscape, Challenges, and Opportunities for Strengthening Newborn Health**
Max Bronstein, MGB Consulting

10:15 AM - 10:30 AM
**The Impact of Newborn Screening Policies for Genetic Diseases on Children and Families**
Elisa Seeger, Aiden Jack Seeger Foundation

10:30 AM - 10:45 AM
**How Will Gene and Cell Therapies Transform Newborn Screening?**
Don Bailey, Ph.D., RTI International

10:45 AM - 11:00 AM
**Screenplus: A Multi-Disorder, Multi-Tiered, Consented Pilot Newborn Screening Program**
Melissa Wasserstein, M.D., Montefiore Medical Center

11:00 AM - 11:15 AM
**Advances in Diagnostic Testing Technology: Implications for NBS**
Ryan Taft, Ph.D., Illumina

11:15 AM - 11:45 AM
**Panel Discussion**

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

10:00 AM - 10:35 AM
**Animal Models for SARS-CoV-2 Research**
Tracy Webb, D.V.M., Ph.D., Colorado State University and Danielle Adney, Ph.D., National Institutes of Health, NIAID

10:35 AM - 11:10 AM
**AAVCovid: An AAV-Based, Single Dose, Rt Stable Covid-19 Vaccine Candidate**
Luk Vandenberghe, Ph.D., Grousbeck Gene Therapy Center, Mass Eye and Ear, Harvard & The Broad Institute of Harvard and MIT

11:10 AM - 11:45 AM
**Airway Vaccine Approach SARS-Cov-2 Using RAAV**
James Wilson, M.D., Ph.D.
University of Pennsylvania
**10:00 AM - 11:45 AM**

### RNA Therapies for Neurologic and Ophthalmic Disorders (Organized by the Neurologic & Ophthalmic Gene and Cell Therapy Committee)

**Co-Chairs:** Kourosh Rezaei, M.D. and Jason Shepherd, Ph.D.

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

### Safety and Efficacy of Body-Wide Therapy for Musculo-Skeletal Diseases (Organized by the Musculo-Skeletal Gene & Cell Therapy Committee)

**Co-Chairs:** Jyoti Jaiswal, Ph.D. and Christina Pacak, Ph.D.

<table>
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<th>Time</th>
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<tbody>
<tr>
<td>10:00 AM - 10:26 AM</td>
<td>Overview of Data From Clinical Trials of AAV Gene Therapy for DMD</td>
<td>Perry Shieh, M.D., Ph.D.</td>
<td>University of California, Los Angeles</td>
</tr>
<tr>
<td>10:26 AM - 10:52 AM</td>
<td>Safety/Efficacy of Systemic Genome Editing for DMD</td>
<td>Christopher Nelson, Ph.D.</td>
<td>University of Arkansas</td>
</tr>
<tr>
<td>10:52 AM - 11:18 AM</td>
<td>Optimization Studies to Enable a Phase 1 Clinical Trial of iPSC-Derived Myogenic Progenitors for DMD</td>
<td>Rita Perlingeiro, Ph.D.</td>
<td>University of Minnesota</td>
</tr>
<tr>
<td>11:18 AM - 11:45 AM</td>
<td>rAAV Controlled Gene Delivery to Heal Cartilage Defects Combined With Biomaterials in Large Animals</td>
<td>Magali Cucchiarini, Ph.D.</td>
<td>Saarland University Medical Center</td>
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FRIDAY, MAY 14, 2021

10:00 AM - 11:45 AM

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

10:00 AM - 10:26 AM  Polyplex Based DNA Nanoparticles as Vaccines
Darrell Irvine, Ph.D., Massachusetts Institute of Technology

10:26 AM - 10:52 AM  Identifying Vital Immunogenic Epitopes in Pathogens as Therapeutic Targets
Guarav Gaiha, M.D., Ragon Institute of MGH

10:52 AM - 11:18 AM  Replicating Viral RNA as Gene Expression Platform
Jesse Erasmus, Ph.D., University of Washington

11:18 AM - 11:45 AM  Development of RNA Vaccines
Drew Weissman, Ph.D., University of Pennsylvania

10:00 AM - 11:45 AM

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

10:00 AM - 10:15 AM  194: Systemic AAV Delivery Activates the Classical Complement Pathway Leading to Thrombotic Microangiopathy
Stephanie Salabarria, BH.Sc., University of Florida

10:15 AM - 10:30 AM  195: Activity of Banked (Off-the-Shelf) CD30. CAR-Modified Epstein-Barr Virus-Specific T Cells in Patients with CD30-Positive Lymphoma
David Quach, Ph.D.

10:30 AM - 10:45 AM  196: Updated Results From HGB-206 LentiGlobin for Sickle Cell Disease Gene Therapy Study: Group C Data and Group A AML Case Investigation
John Tisdale, M.D., National Institutes of Health

10:45 AM - 11:00 AM  197: ADVM-022 Intravitreal Gene Therapy for Neovascular AM.D. - Results From the Phase 1 OPTIC Study
Szilárd Kiss, M.D., Weill Cornell Medicine
FRIDAY, MAY 14, 2021

10:00 AM - 11:45 AM

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

11:00 AM - 11:15 AM

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

11:15 AM - 11:30 AM

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

11:30 AM - 11:45 AM

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

10:45 AM - 12:15 PM

EXHIBIT HALL OPEN - EXHIBITORS AVAILABLE TO CONNECT
Exhibit Hall is Accessible 24 Hours

10:45 AM - 11:30 PM

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

Single Cell Multiomics to Accelerate Cancer Immune Cell Therapy Research

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

11:15 AM - 11:30 AM

Single Cell and Spatial Multiomics to Accelerate Cancer Immune Cell Therapy Research
Abbey Cutchin, 10x Genomics

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

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

24th Annual Virtual Meeting
## 12:15 PM - 2:00 PM

### Base Editing and Gene Editing Approaches

**Co-Chairs: Giulia Pavani, Ph.D. and Shengdar Tsai, Ph.D.**

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

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

<table>
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<tr>
<th>Time</th>
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| 12:15 PM - 12:30 PM | 208: Clinical Application of CRISPR Edited Tumor Infiltrating Lymphocytes in Gastrointestinal Cancer  
Beau Webber, Ph.D., University of Minnesota |
| 12:30 PM - 12:45 PM | 209: Single-Cell Sequencing Approach for the Discovery of Mutant IDH1 Reactive T Cell Receptors From a Glioma Vaccine Trial and an MHC-Humanized Mouse Model  
Khwab Sanghvi, German Cancer Research Center |
| 12:45 PM - 1:00 PM | 210: Epigenome Editing Enables PD1 Silencing in Primary T Cells  
Maria Silvia Roman Azcona, University of Freiburg |
| 1:00 PM - 1:15 PM | 211: mRNA Delivery of an Evolved Bispecific Single Domain Antibody to Synergize the Immune Checkpoint Blockade Therapy for Liver Malignancies  
Rihe Liu, Ph.D., University of North Carolina - Chapel Hill |
| 1:15 PM - 1:30 PM | 212: Exhausted, Tumor-Specific T Cells Can Be Exploited to Generate a Library of T-Cell Receptors for Adoptive T-Cell Therapy in Blood Malignancies  
Francesco Manfredi, M.D., San Raffaele University |
| 1:30 PM - 1:45 PM | 213: Changes in the Tumor Microenvironment in Patients with Glioblastoma Multiforme Treated with IFN-a Immune Cell & Gene Therapy (TEM-GBM_001 Study)  
Bernhard Gentner, M.D., San Raffaele Telethon Institute for Gene Therapy |
| 1:45 PM - 2:00 PM | 214: Small Molecule-Regulated Gene Circuit for Controlling Cytokine Expression in Cell Therapies  
Michelle Hung, Ph.D., Senti Bio |
FRIDAY, MAY 14, 2021

12:15 PM - 2:00 PM

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

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

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

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

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

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

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

1:45 PM - 2:00 PM
221: Efficacy of Intracerebroventricular Adeno-Associated Virus Encoding Iduronidase with Dorsal Root Ganglia-Detargeting Sequences in a Mouse Model of Mucopolysaccharidosis Type I
Juliette Hordeaux, D.V.M., Ph.D., University of Pennsylvania
FRI, MAY 14, 2021

12:15 PM - 2:00 PM

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

**12:15 PM - 12:30 PM**

222: Stabilization of Lentiviral Vector Genomic RNA during Production Using a Novel RNA Chaperone Leads to Increased Yields
Jordan Wright, Ph.D., Oxford BioMedica

**12:30 PM - 12:45 PM**

223: Knockout of Entry Receptors in Virus Producer Cells for Improved Titer and Quality BaEVRless-Pseudotyped Retroviral Particles
Denise Klatt, Ph.D., Dana Farber Cancer Institute

**12:45 PM - 1:00 PM**

224: Development of pEMBR™- An Improved Adenovirus Helper Plasmid for AAV Production
David Dismuke, Ph.D., Forge Biologics

**1:00 PM - 1:15 PM**

225: Genetically Engineering Packaging Cells to Enhance Titer and Infectivity of Lentiviral Vectors
Jiaying Han, University of California - Los Angeles

**1:15 PM - 1:30 PM**

226: Long-Term Expression Comparison of Adeno-Associated Virus (AAV) Vector Produced in HEK293 vs Sf Cell Lines
Britta Handyside, Ph.D., BioMarin Pharmaceutical Inc.

**1:30 PM - 1:45 PM**

227: Processing of Lentiviral Vectors Pseudotypes Using Anion Exchange and Affinity Chromatography
Yuefei Huang, Ph.D., U.S. Food and Drug Administration

**1:45 PM - 2:00 PM**

228: Separation of Empty Capsids from Full Capsids for AAV Gene Therapy Using a Flow through and Step Elution Approach
Danielle Ladwig, Voyager Therapeutics
FRIDAY, MAY 14, 2021

12:15 PM - 2:00 PM

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

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

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

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

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

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

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

1:45 PM - 2:00 PM
235: Intravenous AAV5 Gene Therapy with Human CYP21A1 Corrects Phenotypic Deficiencies of the 21-Hydroxylase Knockout Mouse Model and Demonstrates Durability and Safety in Non-Human Primates and Mice
Rachel Eclov, Ph.D., BridgeBio
12:15 PM - 2:00 PM

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

12:15 PM - 12:30 PM
236: Combined Transgene and Intron-Derived miRNA Therapy Reverses Motor Phenotypes in SCA1 Mice
Ellie Carrell, Ph.D., Raymond G. Perelman Center for Cellular and Molecular Therapeutics

12:30 PM - 12:45 PM
237: Combining MALDI-Based Metabolic Imaging and Molecular Analysis for Effective and Informative Assessment of Therapeutic Efficacy in Sub-Anatomical Regions of the CNS After rAAV Gene Therapy
Dominic Gessler, M.D., Ph.D., University of Massachusetts

12:45 PM - 1:00 PM
238: Evolution of Modified AAV in Rhesus Macaque Brain
Paul Ranum, Ph.D., The Children’s Hospital of Philadelphia

1:00 PM - 1:15 PM
239: Real-Time MR Tracking of AAV Gene Therapy with Enzyme-Activated MR Probes
Toloo Taghian, Ph.D., University of Massachusetts Medical School

1:15 PM - 1:30 PM
240: Inclusion of a Degron Reduces Levels of Undesired Inteins after AAV-Mediated Protein Trans-Splicing in the Retina
Patrizia Tornabene, Telethon Institute of Genetics and Medicine

1:30 PM - 1:45 PM
241: Drug-Regulated Splicing Switch for Gene Expression Control
Alex Monteys, Ph.D., University of Pennsylvania

1:45 PM - 2:00 PM
242: Targeted Gene Therapy with Engineered Systemic AAVs for the Central and Peripheral Nervous Systems Prevents Motor Coordination Phenotypes in a Mouse Model of Friedreich’s Ataxia
Acacia Hori, California Institute of Technology
**12:15 PM - 2:00 PM**

**Oligonucleotide Therapeutics**

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

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

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

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

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

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

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

1:45 PM - 2:00 PM  
**249: The 3´-tsRNAs Are Aminoacylated Further Implicating Their Role in Ribosome Biogenesis During Tissue Homeostasis and Cancer**  
Mark Kay, M.D., Ph.D., Stanford University School of Medicine
12:15 PM - 2:00 PM

**Pharmacology/Toxicology Studies or Assay Development**

**Co-Chairs:** Cristina Baricordi, Ph.D. and Carmen Unzu, Ph.D.

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

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

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

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

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

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

- **1:45 PM - 2:00 PM**
  
  **256: A GLP Safety and Biodistribution Study of AXO-Lenti-PD Manufactured via Two Processes Delivered at a Higher Volume and Flow Rate**
  
  Thomas Pack, Ph.D., Sio Gene Therapies
<table>
<thead>
<tr>
<th>Time</th>
<th>Session Title</th>
<th>Speaker(s)</th>
</tr>
</thead>
<tbody>
<tr>
<td>12:15 PM</td>
<td><strong>RNA Virus Vectors</strong></td>
<td></td>
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<tr>
<td>12:15 PM - 12:30 PM</td>
<td><strong>257: Combinatorial Relief of Multiple Innate Immune Blocks Allows Efficient Gene Engineering of Quiescent Human Hematopoietic Stem Cells</strong></td>
<td>Erika Valeri, San Raffaele Telethon Institute for Gene Therapy</td>
</tr>
<tr>
<td>12:30 PM - 12:45 PM</td>
<td><strong>258: Administration During Liver Growth Improves the Efficiency of Lentiviral Vector Based In Vivo Gene Therapy in Mice</strong></td>
<td>Francesco Starinieri, San Raffaele Telethon Institute for Gene Therapy</td>
</tr>
<tr>
<td>12:45 PM - 1:00 PM</td>
<td><strong>259: MicroRNA Detargeting Proves Superior to Genetic Attenuation for Balancing Safety and Efficacy of Oncolytic Mengovirus in Immunodeficient Glioblastoma Mouse Model</strong></td>
<td>Yogesh Suryawanshi, M.D., Ph.D., Mayo Clinic</td>
</tr>
<tr>
<td>1:00 PM - 1:15 PM</td>
<td><strong>260: CTCF-Based Chromatin Insulators and Enhancers in Lentiviral Vectors Impact Genome Topology and Vector Safety</strong></td>
<td>Monica Volpin, Ph.D., San Raffaele Telethon Institute for Gene Therapy</td>
</tr>
<tr>
<td>1:15 PM - 1:30 PM</td>
<td><strong>AAV Vectors - Clinical Studies</strong></td>
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<tr>
<td>1:15 PM - 1:30 PM</td>
<td><strong>261: AAV8-Mediated Liver-Directed Gene Therapy as a Potential Therapeutic Option in Adults with Glycogen Storage Disease Type Ia (GSDIa): Updated Phase 1/2 Clinical Trial Results</strong></td>
<td>David Rodriguez-Buritica, M.D., University of Texas McGovern Medical School</td>
</tr>
<tr>
<td>1:30 PM - 1:45 PM</td>
<td><strong>262: Safety, β-Sarcoglycan Expression, and Functional Outcomes from Systemic Gene Transfer of rAAVrh74.MHCK7.SGCB in Limb Girdle Muscular Dystrophy Type 2E/R4</strong></td>
<td>Louise Rodino-Klapac, Ph.D., Sarepta Therapeutics</td>
</tr>
<tr>
<td>1:45 PM - 2:00 PM</td>
<td><strong>263: IGNITE-DMD Phase I/II Study of SGT-001 Microdystrophin Gene Therapy for Duchenne Muscular Dystrophy</strong></td>
<td>Carl Morris, Ph.D., Solid Biosciences</td>
</tr>
</tbody>
</table>
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Giuliana Ferrari, Ph.D.
SR-TIGET, Scientific Institute San Raffaele
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<thead>
<tr>
<th>Name</th>
<th>Institution</th>
</tr>
</thead>
<tbody>
<tr>
<td>Terence R. Flotte, Ph.D.</td>
<td>University of Massachusetts Medical School</td>
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<td>Klaus Früh, Ph.D.</td>
<td>Oregon Health and Science University</td>
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<tr>
<td>Juan Fueyo, M.D.</td>
<td>MD Anderson Cancer Center</td>
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<tr>
<td>Guarav Gaiha, M.D.</td>
<td>Ragon Institute of MGH</td>
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<tr>
<td>Anne Galy, Ph.D.</td>
<td>Genethon</td>
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<tr>
<td>Eleni Gavriilaki, M.D., Ph.D.</td>
<td>George Papanicolaou Hospital</td>
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<tr>
<td>Paloma H Giangrande, Ph.D.</td>
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<td>Gloria Gonzalez-Aseguinolaza, Ph.D.</td>
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<tr>
<td>Lynn K Gordon, M.D., Ph.D.</td>
<td>University of California Los Angeles</td>
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<tr>
<td>Melanie L Graham, MPH, Ph.D.</td>
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<tr>
<td>Paola Grandi, Ph.D.</td>
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<tr>
<td>Jordan J. Green, Ph.D.</td>
<td>Johns Hopkins University</td>
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<tr>
<td>Stephan A. Grupp, M.D., Ph.D.</td>
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<tr>
<td>Beth Halpern</td>
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<tr>
<td>Cary O Harding, M.D.</td>
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<tr>
<td>Parameswaran Hari, M.D.</td>
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<tr>
<td>Loree Heller, Ph.D.</td>
<td>University of South Florida</td>
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<td>Scott Hensley, Ph.D.</td>
<td>University of Pennsylvania</td>
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<tr>
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<tr>
<td>Akitsu Hotta, Ph.D.</td>
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<td>Charles Hough</td>
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<tr>
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<tr>
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</tr>
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<tr>
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<tr>
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</tbody>
</table>
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Andrew Wilber, Ph.D. – Southern Illinois University School of Medicine
Patricia Devaux, Ph.D. – Mayo Clinic
Mario Amendola, Ph.D. – Genethon

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Lauren Woodard, Ph.D. – Vanderbilt University Medical Center

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Arthur Krieg, M.D. – Checkmate Pharmaceuticals

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

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Chair: Dexi Liu, Ph.D. – University of Georgia

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Kenya Kamimura, M.D., Ph.D. – Niigata University

METABOLIC, STORAGE, ENDOCRINE, LIVER AND GASTROINTESTINAL DISEASES

Chair: Nicola Brunetti-Pierri, M.D. – Telethon Institute of Genetics & Medicine

Kevin Strauss, M.D. – Clinic for Special Children
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Martin Hicks, Ph.D. – *Monmouth University*
Rita Batista, Ph.D. – *University of Massachusetts Medical School*

MUSCULO-SKELETAL DISEASES
Chair: **Kathryn Wagner, M.D., Ph.D.** – *F. Hoffmann-La Roche*

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Rita Perlingeiro, Ph.D. – *University of Minnesota*
Jeffrey Chamberlain, Ph.D. – *University of Washington School of Medicine*
Paul Gregorevic, Ph.D. – *The University of Melbourne*

CANCER - IMMUNOTHERAPY, CANCER VACCINES
Chair: **Chiara Bonini, M.D.** – *Università Vita Salute San Raffaele*

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CANCER - ONCOLYTIC VIRUSES
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CANCER - TARGETED GENE AND CELL THERAPY
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Saad Kenderian, M.D. – Mayo Clinic
Irina Balyasnikova, Ph.D. – Northwestern University Feinberg School of Medicine
Hernando Lopez-Bertoni, Ph.D. – Johns Hopkins School Of Medicine

HEMATOLOGIC AND IMMUNOLOGIC DISEASES
Chair: Cyndi Dunbar, M.D. – National Institutes of Health (NIH)
Juan Bueren, Ph.D. – CIEMAT/CIBERER
Alessio Cantore, Ph.D. – SR-TIGET
Denise Sabatino, Ph.D. – The Children’s Hospital of Philadelphia
John Chapin, M.D. – Takeda Therapeutics
Pankaj Mandal, Ph.D. – Food and Drug Administration

IMMUNOLOGICAL ASPECTS OF GENE THERAPY AND VACCINES
Chair: Roland Herzog, Ph.D. – Indiana University
Manish Muhuri, Ph.D. – University of Massachusetts Medical School
Kevin Morris, Ph.D. – The City of Hope
Chengwen Li, M.D., Ph.D. – UNC at Chapel Hill
Allison M. Keeler-Klunk, Ph.D. – University of Massachusetts Medical School
Ying Kai Chan, Ph.D. – Harvard University
ABSTRACT REVIEWERS

CELL THERAPIES

Chair: Jen Adair, Ph.D. – Fred Hutchinson Cancer Research Center

Daniel E. Bauer, M.D., Ph.D. – Boston Children’s Hospital/Harvard Medical School
Maria Grazia Roncarolo, M.D. – Stanford University
Jan A. Nolta, Ph.D. – University of California Davis
Rayne Rouce, M.D. – Baylor College of Medicine
Jianping Huang, M.D., Ph.D. – University of Florida

VECTOR PRODUCT ENGINEERING, DEVELOPMENT OR MANUFACTURING

Chair: J. Fraser Wright, Ph.D. – Stanford School of Medicine

Zhongya Wang, Ph.D. – Cure Genetics
Nathalie Clement, Ph.D. – University of Florida, Powell Gene Therapy Center
Eric Horowitz, Ph.D. – Akouos
Magalie Penaud-Budloo, Ph.D. – University of Nantes, INSERM UMR1089
Deep Bhattacharya, Ph.D. – Pfizer

CELL THERAPY PRODUCT ENGINEERING, DEVELOPMENT OR MANUFACTURING

Chair: Adrian Gee, Ph.D – Baylor College of Medicine

Joseph Gold, Ph.D. – City of Hope
Daniela Bischoff, Ph.D. – Indiana University School of Medicine
Bruce Levine, Ph.D. – University of Pennsylvania
Leslie Silverstein, M.D. – Boston Children’s Hospital
Peiman Hematti, M.D. – University of Wisconsin - Madison

PHARMACOLOGY/TOXICOLOGY STUDIES OR ASSAY DEVELOPMENT

Chair: Eva Andres-Mateos, Ph.D. – Akouos

Haiyan Ma, Ph.D. – Northern Biomolecular Services
Carmen Unzu, Ph.D. – Apic Bio
Bence Gyorgy, M.D., Ph.D. – GenScript USA Inc.
Jessica Sido, Ph.D. – Kytopen Corp
Cristina Baricordi, Ph.D. – AVROBIO
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