AMERICAN SOCIETY OF GENE & CELL THERAPY

21ST ANNUAL MEETING

FINAL PROGRAM GUIDE

Program Guide Sponsored by

Voyager THERAPEUTICS

21st Annual Meeting
ASGCT
CHICAGO, ILLINOIS
2018
MAY 16-19

AMERICAN SOCIETY of GENE & CELL THERAPY

Chicago, IL, USA – Hilton Chicago
www.asgct.org #ASGCT2018

ASGCT-0318-208

Voyager THERAPEUTICS

ONE-TIME DELIVERY. BENEFITS FOR A LIFETIME.

Voyager's mission is to develop and deliver life-changing therapies to people around the world living with severe neurological diseases. This begins with a disciplined commitment to discovery, research, development and manufacturing, and extends to our thoughtful engagement with patients, healthcare providers, caregivers and advocacy organizations.

ASGCT 2018

ORAL PRESENTATIONS:

- **Alzheimer's Disease: from Genetics to Pathogenesis to Gene Therapy** (May 16, 8-8:30 a.m., Session: Gene Therapy for Alzheimer's and Related Disorders)
- **Pharmacology and Safety of VY-HTT01, an AAV miRNA Gene Therapy Targeting Huntington for the Treatment of Huntington's Disease** (May 16, 11:15 a.m., Session: RNA Virus Vectors and Small RNA Therapy)
- **AADC gene therapy for advanced Parkinson's disease: Interim Results of a Phase 1b Trial** (May 16, 5:00 p.m., Session: Translational and Clinical Progress in Neurological Disease)
- **Safety and Increased Transduction Efficiency in the Adult Nonhuman Primate Central Nervous System with Intravenous Delivery of Two Novel Adeno-Associated Virus Capsids** (May 18, 4:30 p.m., Session: Capsid Engineering)
- **Rescue of Central and Peripheral Neurological Phenotype in a Mouse Model of Friedreich's Ataxia by Intravenous Delivery of AAV Frataxin with a Novel Capsid** (May 18, 5:30 p.m., Session: Preclinical Approaches in Gene transfer to the Central Nervous System)

POSTER PRESENTATIONS:

- **Biophysical and In Vitro Comparability Analysis of an AAV Vector Produced by the Baculovirus/Sf9 System and HEK Triple Transfection System** (P100, May 16)
- **Intraoperative MRI-Guided Delivery of AAV2-hAADC for Parkinson's Disease: Role of Volume of Infusion in Putaminal Gene Expression** (P271, May 16)
- **Distribution and Transduction of Multiple rAAV Serotype/Mutant Vectors in the Non-Human Primate Brain after Intracisternal Injection by an AAV DNA/RNA Barcoding Library** (P384, May 17)
- **In Vivo Selection of CNS-Specific Novel AAV Capsid Variants by Directed Evolution** (P393, May 17)
- **Selection of an AAV Gene Therapy Targeting SOD1 for the Treatment of SOD1-ALS** (P555, May 17)
- **Evaluation of a new pan-AAV Affinity Resin for rAAV Purification and cGMP Manufacturing** (P631, May 17)
- **Insect Media Evaluation for Cell Growth and rAAV Production in an Sf9-Baculovirus Manufacturing System** (P640, May 17)
- **Evaluation of a Biological Potency Assay for an AAV2.AADC Vector Used in the Treatment of Parkinson's Disease** (P916, May 18)
- **Comparison of Bioreactors and Scale-up of Sf9-Baculovirus Systems for rAAV Production** (P942, May 18)
- **Development and Evaluation of High Throughput Scale-down Models for rAAV Chromatographic Separations for Application in cGMP Manufacturing** (P941, May 18)

www.voyagertherapeutics.com

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Thank you to Voyager Therapeutics

for their generous support in sponsoring

the ASGCT 21st Annual Meeting Final Program Guide.

A WELCOME FROM HELEN E. HESLOP, MD

Dear Colleagues,

On behalf of the American Society of Gene & Cell Therapy (ASGCT), it is my pleasure to welcome you to the ASGCT 21st Annual Meeting and the first one we have held in Chicago. I am honored to join you and thousands of our colleagues from around the globe who share in our Society's mission of advancing knowledge, awareness, and education to advance the discovery and clinical application of gene and cell therapies for alleviation of human disease.

This scientific and educational program reflects the rapid advancements that have occurred in gene and cell therapy over the past year. Our invited faculty includes nearly 150 investigators in our field, presenting cutting edge research in the 23 Scientific Symposia and overviews of important concepts and technologies in the eight Education Sessions. In addition, join me in welcoming former ASGCT President and current President of Spark Therapeutics, Dr. Kathy High, who will present the George Stamatoyannopoulos Lecture on Thursday following the presentation of the ASGCT Public Service Award to Fondazione Telethon and Francesca Pasinelli. On Friday, Outstanding Achievement Award recipient Dr. Jean Bennett will give the Outstanding Achievement Award presentation followed by Dr. Nick Restifo in the Presidential Symposium. We are also thrilled to have more than 1,000 submitted abstracts featuring groundbreaking scientific and clinical advances across the breadth of our field that will be presented as oral presentations or at poster sessions.

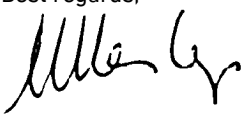
On Friday evening, I am delighted to invite you to join the ASGCT leadership and me in celebrating our 21st year as a Society. The reception takes place in striking Stanley Hall of the Field Museum of Natural History just a few blocks from the Hilton, featuring live music, dancing, drinks, and hors d'oeuvres amid dinosaurs and fighting African Elephants.

Be sure to visit our Exhibit Hall to find out about the products and services offered by almost 100 partner companies, via conversations at their booths and more formal presentations in the Tools & Technologies Forum. While in the Exhibit Hall, visit the Employment Center to discover exciting new career opportunities.

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.

Lastly, thank you for attending our Society's Annual Meeting. I sincerely hope you enjoy the science, technology, networking, and camaraderie that we have built together!

Best regards,



Helen E. Heslop, MD
President, ASGCT

Download ASGCT Annual Meeting

Mobile App

core-apps

Navigate the 2018 event like a pro with the ASGCT Annual Meeting mobile app, powered by Core-apps.

With the mobile app, you can:

- Stay organized with up-to-the-minute Exhibitor, Speaker, and Event information
- Receive important real-time communications from Show Organizers
- Build a personalized schedule and bookmark exhibitors
- Take notes and download event handouts and presentations
- Rate the sessions you attend and comment on them, too
- Find attendees and connect with your colleagues through Friends
- Stay in-the-know and join in on social media
- Share your event photos and experiences with the Activity Feed
- Find Chicago Local Places
- And much, much more!

Downloading the App is Easy!

Search "ASGCT App" in the Apple App Store or Google Play Store to download.

You can also download the app by visiting http://app.core-apps.com/asgct2018 or by scanning this QR code.

The Wireless Network name will be **Hilton Chicago Meeting.** The access code will be **ASGCT2018**.

Questions About Using the App?

Contact support@core-apps.com or visit the onsite ASGCT registration desk!

Sponsored by

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AMERICAN SOCIETY OF GENE & CELL THERAPY

MISSION AND VISION

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

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

ABSTRACT VOLUME - *MOLECULAR THERAPY*

All abstracts accepted for presentation at the ASGCT 21st Annual Meeting have been published in the May supplement of *Molecular Therapy*. Attendees are able to access a copy of the supplement online at the *Molecular Therapy* website and have access to the abstracts through the Mobile App.

ADMISSION

Official name badges will be required for admission to all ASGCT sessions. All Annual Meeting attendees receive a name badge with their registration bag. Name badges should be worn at all times inside the Hilton, as badges will be used to control access to sessions and activities. Attendees are cautioned against wearing their name badges while away from the hotel as badges may draw unwanted attention to your status as visitors to Chicago.

BUSINESS CENTER (FEDEX OFFICE)

The Business Center (FedEx Office) is located on the Lobby Level of the Hilton Chicago.

BUSINESS MEETING

Join the Society's leadership for a business meeting and continental breakfast.
Saturday, May 19
7:30 am – 8:00 am

A light continental breakfast will be offered at 7:15 am

CAMERA/RECORDING POLICY

Attendees are prohibited from using flash photography or otherwise distracting the presenters or members of the audience.

Your attendance at ASGCT events implies your permission for images captured during these events to be used for the purposes of ASGCT archival and promotional material and publications and waives your rights for compensation or ownership of these images.

CELL PHONES & MOBILE DEVICES

For the courtesy of all presenters and attendees, please ensure that cell phones and other mobile devices are turned off or silenced during all sessions as they may interfere with session audio.

CONCIERGE DESK

Chicago offers a wide variety of activities and restaurants to meet all tastes and budgets. A Concierge Desk is located in the Main Lobby of the Hilton Chicago Hotel, with Hilton staff who can help you with your city needs and reservations.

DISCLOSURE

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

EDUCATIONAL METHODS AND MATERIALS

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

EDUCATIONAL OBJECTIVES

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

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

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

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

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

EVALUATION METHOD

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

NEEDS

Clinical gene transfer has become increasingly complex due to ongoing developments in the fields of gene and cell therapy itself, 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 field of gene and cell therapy.

DATES

The ASGCT 21st Annual Meeting will begin on the morning of Wednesday, May 16, 2018 and continue through 12:00 PM on Saturday, May 19, 2018. Exhibits will be open Wednesday, May 16 through Friday, May 18.

EMPLOYMENT CENTER

At the 21st Annual Meeting, there will be a forum for academic institutions and companies to promote open positions to meeting attendees. If you have open positions, please bring multiple copies of your job posting to the meeting. Similarly, if you are seeking a job, please bring multiple copies of your résumé to submit. ASGCT will not take responsibility for résumés sent to the ASGCT office in response to a job posting. If you would like more information about this service, please contact Samantha Kay at skay@asgct.org. The Employment Center will be located in the Exhibit Hall.

EXHIBITS

The Exhibit hall is located in Stevens Salon C & D of the Hilton Chicago. The companies, organizations and institutions exhibiting at the ASGCT Annual Meeting provide the latest information on products and services directly related to your professional needs as well as recruit for open positions. An Exhibit Hall Social will be held on Wednesday, Thursday and Friday evenings and a coffee social will be offered on Thursday and Friday in the exhibit hall. All participants are urged to allow adequate time daily to visit the exhibits, as they are an integral part of the success of the meeting.

EXHIBIT HALL HOURS

Exhibits will be open Wednesday, May 16 through Friday, May 18, 2018.

Wednesday, May 16

Welcome Reception & Poster Session	5:30 pm – 7:30 pm

Thursday, May 17

Coffee Social and Oral Poster Session	10:00 am – 10:45 am
Coffee Social	3:00 pm – 3:45 pm
Networking Reception, Poster Session and Tools & Technologies Forum	5:15 pm – 7:30 pm

Friday, May 18

Coffee Social and Oral Poster Session	10:00 am – 10:45 am
Coffee Social	3:15 pm – 4:00 pm
Networking Reception, Poster Session and Tools & Technologies Forum	5:45 pm – 7:45 pm

GUEST ATTENDANCE

ASGCT asks registered attendees to refrain from taking children, spouses, or guests to any educational session or functions offered at the 21st Annual Meeting.

INTERNET ACCESS

Wireless Internet access will be available in all public areas and in meeting rooms at the Hilton Chicago Hotel for all meeting attendees.

The Wireless Network name will be **Hilton Chicago Meeting**. The access code will be **ASGCT2018**.

LEAD RETRIEVAL

A lead retrieval system has been made available to all exhibitors of the ASGCT 21st Annual Meeting. Exhibitors may ask to scan attendee name badges with a hand held scanner in order to obtain attendee contact information.

LOCATION

The 21st Annual Meeting will be held at the Hilton Chicago Hotel, 720 S Michigan Ave, Chicago, IL 60605.

Exhibits will take place in Stevens Salon C & D.

Posters will take place in Stevens Salon C & D.

Registration will be located at the Registration Desk in the 8th St. Entrance on the Lobby Level of the Hilton Chicago Hotel.

MATERIALS DISTRIBUTION

Please refrain from distributing promotional materials throughout the meeting; these may only be distributed from an exhibit booth in the Exhibit Hall.

MOBILE APP

The full ASGCT 21st Annual Meeting program, including session schedules, faculty, exhibits, sponsors and abstracts, is available through the ASGCT Mobile App which can be accessed by searching "ASGCT App" in the Apple App Store or Google Play Store to download.

You can also download the app by visiting http://app.core-apps.com/asgct2018

NO SMOKING

Smoking is prohibited at all 21st Annual Meeting sessions and events.

POSTERS

Abstract Posters will be on display in Stevens Salon C & D.

Wednesday, May 16

Abstract Poster Session I

7:30 am – 12:00 pm	Poster Setup by Authors
12:00 pm – 5:30 pm	Poster Viewing (authors present from 5:30 pm – 7:30 pm)
5:30 pm – 7:30 pm	Welcome Reception, Exhibits, and Poster Session I
7:30 pm – 8:00 pm	Authors Remove Posters

Thursday, May 17

Abstract Poster Session II

7:30 am – 12:00 pm	Poster Setup by Authors
12:00 pm – 5:15 pm	Poster Viewing (authors present from 5:15 pm – 7:30 pm)
5:15 pm – 7:30 pm	Networking Reception, Exhibits, and Poster Session II
7:30 pm – 8:00 pm	Authors Remove Posters

Friday, May 18

Abstract Poster Session III

7:30 am – 11:45 am	Poster Setup by Authors
11:45 am – 5:45 pm	Poster Viewing (authors present from 5:45 pm – 7:45 pm)
5:45 pm – 7:45 pm	Networking Reception, Exhibits, and Poster Session III
7:45 pm – 8:15 pm	Authors Remove Posters

CALL4POSTERS® PICK-UP SCHEDULE

Once again this year the ASGCT arranged with Learner's Digest International to provide poster authors with the opportunity to create their poster online using the Call4Posters® service. Poster presenters who took advantage of this service may pick-up their pre-ordered poster at the ASGCT Registration Desk during the following hours.

Tuesday, May 15	7:00 am – 7:00 pm
Wednesday, May 16	7:00 am – 6:30 pm
Thursday, May 17	7:00 am – 6:45 pm
Friday, May 18	7:00 am – 7:00 pm

PRESS

Members of the working media may register for the 21st Annual Meeting in the Press Room – Room PDR 1. Interview space, computers and internet services are available for the convenience of media representatives covering the meeting. Press must register, provide credentials, and wear their press badge for admittance to ASGCT sessions. Assistance will be provided to members of the media that would like to schedule interviews.

PRESS ROOM

The Press Room is located in PDR 1.

Press Room Hours

The press room will be available by appointment only. To schedule a time to use the press room, contact Alex Wendland at awendland@asgct.org.

REGISTRATION DESK

Name badges, final programs, and abstract supplements will be distributed at the registration desk located by the 8th Street entrance on the Lobby Level of the Hilton Chicago Hotel.

Registration Desk Hours

Tuesday, May 15	7:00 am – 7:00 pm
Wednesday, May 16	7:00 am – 7:30 pm
Thursday, May 17	7:00 am – 7:30 pm
Friday, May 18	7:00 am – 7:45 pm
Saturday, May 19	7:30 am – 12:15 pm

CANCELLATION REFUND POLICY

Refund requests should have been submitted in writing to the ASGCT Executive Office by May 2, 2018.

CHICAGO INFORMATION

Please abide by the traffic signs and lights and utilize the designated crosswalks at all times.

SPEAKER READY DESK

There will be a Speaker Ready Desk near the ASGCT Registration Desk (Foyer by the 8th Street Entrance, Lobby Level) for speakers to drop off their presentations.

All speakers, including oral abstract presenters, must deliver their presentations to the Speaker Ready Desk the day before their session or at least four hours prior to their presentations. Equipment is available at the Speaker Ready Desk for faculty to review their materials. Audiovisual personnel will be available for assistance. Please mark your materials (your name, session and speaker order) so the materials can be returned to you. The Society strongly encourages faculty to pre-load presentations at the Speaker Ready Desk; those faculty who load presentations in the meeting rooms during the sessions will have that time deducted from their presentation time by the Chair.

ASGCT strongly encourages faculty presenting on Wednesday of the Annual Meeting to check-in at the Speaker Ready Desk on Tuesday, May 15 to avoid congestion.

Avoid delays and check-in early!

Speaker Ready Desk Hours

Tuesday, May 15	5:00 pm – 7:00 pm
Wednesday, May 16	7:00 am – 7:30 pm
Thursday, May 17	7:00 am – 7:30 pm
Friday, May 18	7:00 am – 7:45 pm
Saturday, May 19	7:30 am – 10:00 am

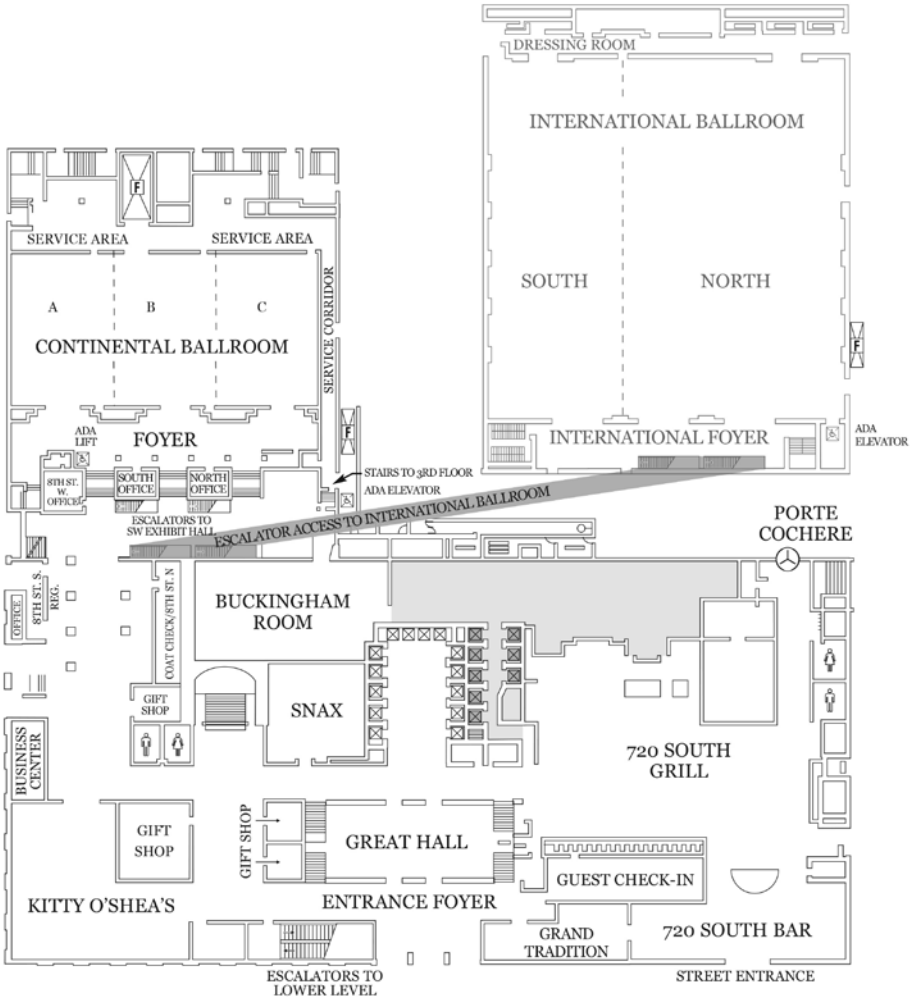
SPECIAL ACCESSIBILITY NEEDS

If you require special accommodations under the ADA in order to fully participate in the meeting or the course, please visit the Registration Desk; ASGCT staff will be happy to assist you with your specific needs.

HOTEL FLOOR PLAN

LOBBY LEVEL

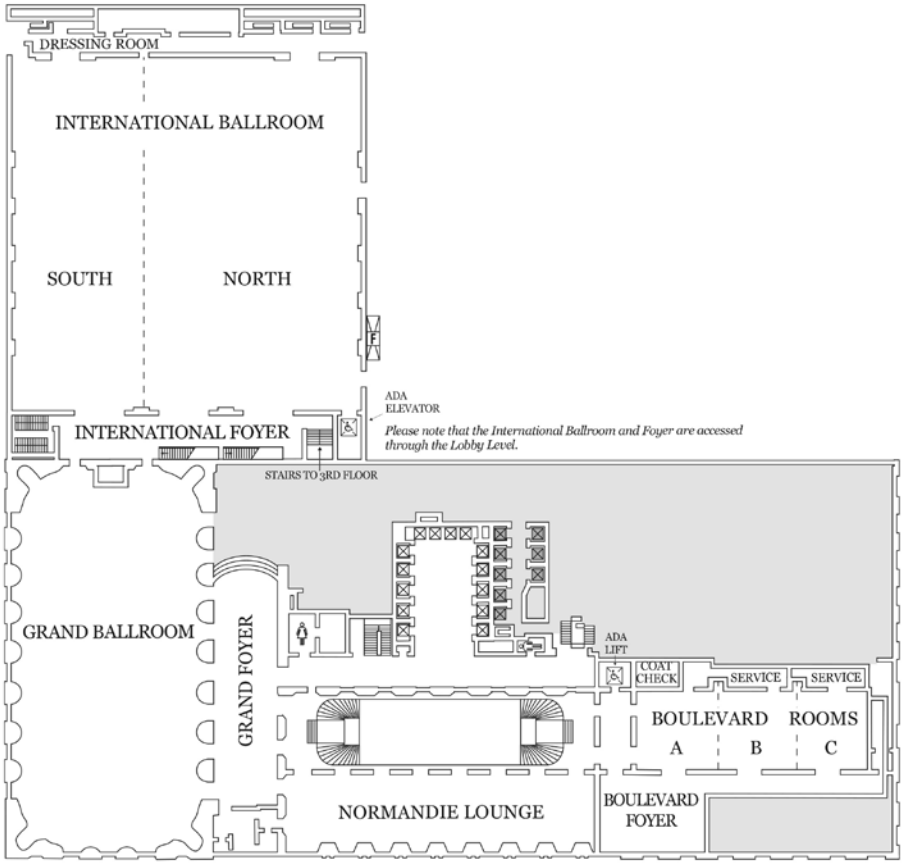
- Registration
- Pre Meeting Workshops



HOTEL FLOOR PLAN

SECOND FLOOR

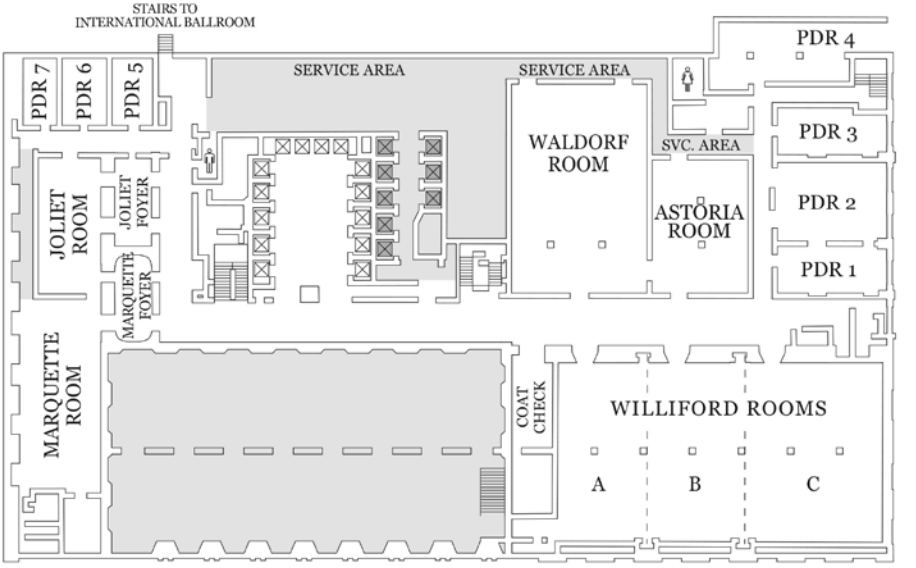
- Sessions



HOTEL FLOOR PLAN

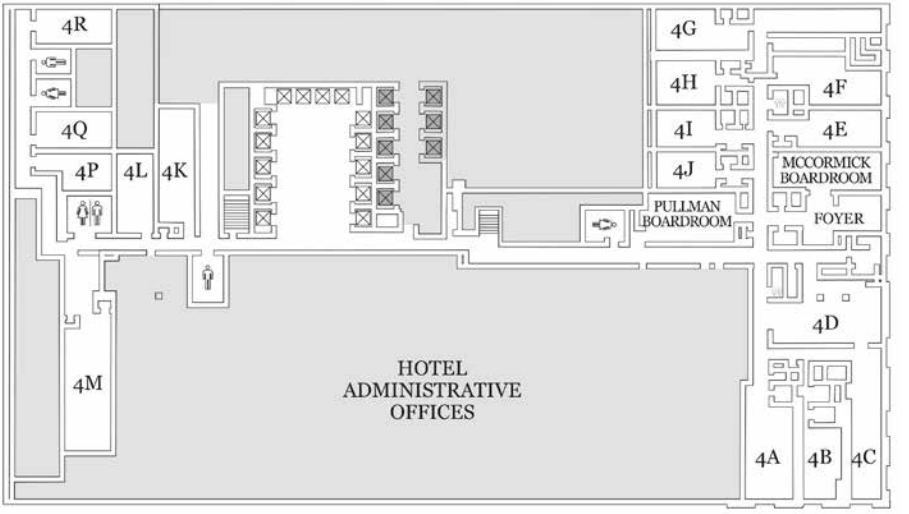
THIRD FLOOR

- Business Meeting
- Industry Sponsored Symposia
- Press Room



FOURTH FLOOR

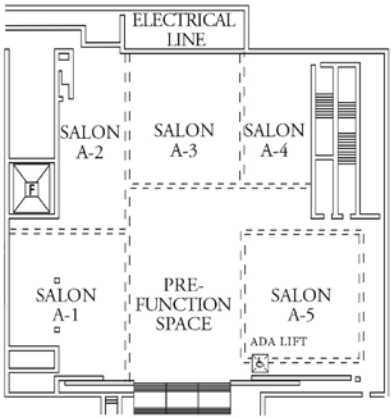
- Committee Meetings

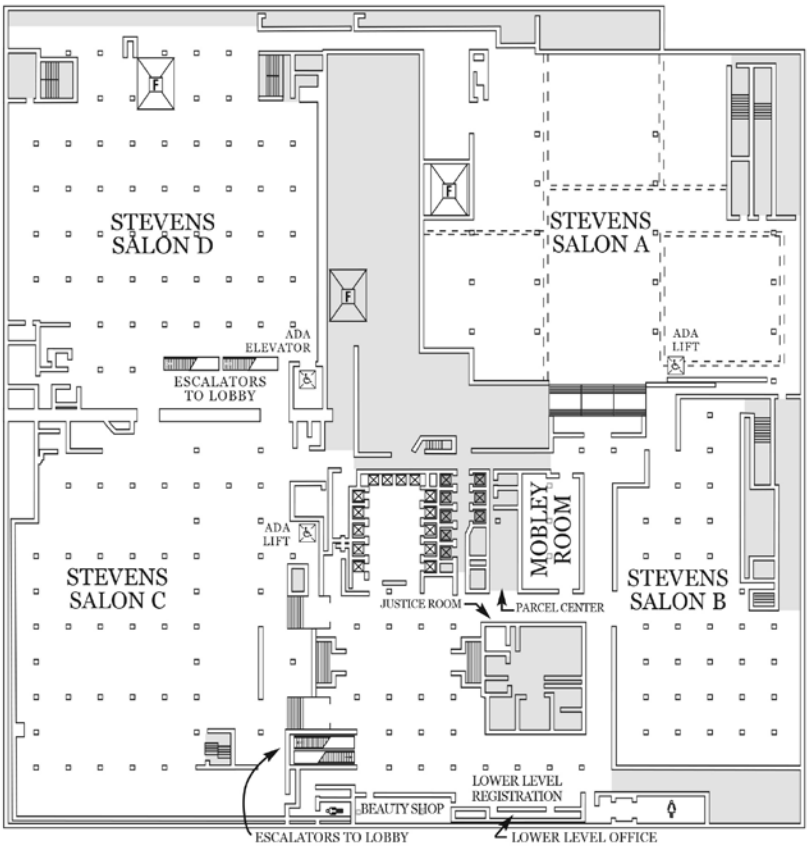


HOTEL FLOOR PLAN

LOWER LEVEL

- Exhibits/Posters
- Sessions
- Trainee Lounge
- Attendee Lounge





2018 ABSTRACT PLANNING COMMITTEE

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

ASGCT PRESIDENT
Helen E. Heslop, MD – *Baylor College of Medicine*

ASGCT PRESIDENT-ELECT
Michele P. Calos, PhD – *Stanford University School of Medicine*

ASGCT SECRETARY AND ABSTRACT CHAIR
David V. Schaffer, PhD – *University of California Berkeley*

ASGCT PROGRAM COMMITTEE MEMBER
Stephen J. Russell, MD, PhD – *Mayo Clinic*

ASGCT ABSTRACT CATEGORY CHAIRS
Barry J. Byrne, MD, PhD – *University of Florida College of Medicine*
Jean Bennett, MD, PhD - *University of Pennsylvania Scheie Eye Institute*

ABSTRACT REVIEWERS

Thank you to our 2018 abstract reviewers for their time and expertise!

RNA VIRUS VECTORS

Tal Kafri, MD, PhD - *University of North Carolina at Chapel Hill*
Carol H. Miao, PhD - *University of Washington*
Stephen J. Russell, MD, PhD - *Mayo Clinic*
Byoung Ryu, PhD - *St. Jude Children's Research Hospital*
Axel Schambach, MD, PhD - *Hannover Medical School*
Bruce E. Torbett, PhD, MSPH - *The Scripps Research Institute*

AAV VECTORS

Guangping Gao, PhD - *University of Massachusetts Medical School*
Joseph E. Rabinowitz, PhD - *Pfizer, Inc.*
David V. Schaffer, PhD - *University of California Berkeley*
Shen Shen, PhD - *Editas Medicine*
Arun Srivastava, PhD - *University of Florida College of Medicine*
Junghae Suh, PhD - *Rice University*

ADENOVIRUS AND OTHER DNA VIRUSES

Maria G. Castro, PhD - *University of Michigan School of Medicine*
David T. Curiel, MD, PhD - *Washington University Medical School*
Paola Grandi, PhD - *Oncorus*
Kah-Whye Peng, PhD - *Mayo Clinic*
Frank Tufaro, PhD - *DNAtrix*
Matthew D. Weitzman, PhD - *Children's Hospital of Philadelphia*

GENE TARGETING AND GENE CORRECTION

Paula M. Cannon, PhD - *University of Southern California*
Dirk Grimm, PhD - *University of Heidelberg*
Mark A. Kay, MD, PhD - *Stanford University School of Medicine*
Punam Malik, MD - *Cincinnati Children's Hospital Medical Center*
Matthew H. Porteus, MD, PhD - *Stanford University School of Medicine*
Andrew M. Scharenberg, MD - *Casebia Therapeutics*

OLIGONUCLEOTIDE THERAPEUTICS

Paloma H. Giangrande, PhD - *University of Iowa*
Marcin Kortylewski, PhD - *City of Hope Comprehensive Cancer Center*
Anton P. McCaffrey, PhD - *TriLink Bio Technologies*
John J. Rossi, PhD - *Beckman Research Institute City of Hope*
Laura Sepp-Lorenzino, PhD - *Alnylam Pharmaceuticals*
Marco S. Weinberg, PhD - *Vertex Pharmaceuticals*

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

Michael A. Barry, PhD - *Mayo Clinic*
Jordan J. Green, PhD - *Johns Hopkins University*
Richard Heller, PhD - *Old Dominion University*
Dexi Liu, PhD - *University of Georgia College of Pharmacy*
Chantal Pichon, PhD - *Centre de Biophysique Moleculaire*
Kevin G. Rice, PhD - *University of Iowa College of Pharmacy*
Lonnie Shea, PhD - *University of Michigan*

METABOLIC, STORAGE, ENDOCRINE, LIVER AND GASTROINTESTINAL DISEASES

Nicola Brunetti-Pierri, MD - *Telethon Institute of Genetics and Medicine*
Stephanie Cherqui, PhD - *University of California, San Diego*
Dwight Koeberl, MD, PhD - *Duke University Medical Center*
Gerald S. Lipshutz, MD - *David Geffen School of Medicine at UCLA*
Beat Thony, PhD - *University of Zurich*
Charles P. Venditti, MD, PhD - *National Human Genome Research Institute*

CARDIOVASCULAR AND PULMONARY DISEASES

Barry J. Byrne, MD, PhD - *University of Florida College of Medicine*
William Todd Cade, PT, PhD - *Washington University School of Medicine in St. Louis*
Paul Kessler, MD - *Bristol-Myers Squibb*
Carl Morris, PhD - *Solid Biosciences, LLC*
Christina A. Pacak, PhD - *University of Florida*
Xiao Xiao, PhD - *University of North Carolina at Chapel Hill*

NEUROLOGIC DISEASES (INCLUDING OPHTHALMIC AND AUDITORY DISEASES)

Alberto Auricchio, MD - *Telethon Institute of Genetics & Medicine*
Jean Bennett, MD, PhD - *University of Pennsylvania Scheie Eye Institute*
Beverly L. Davidson, PhD - *Children's Hospital of Philadelphia*
Tonia S. Rex, PhD - *Vanderbilt University Medical Center*
Miguel Sena-Esteves, PhD - *University of Massachusetts Medical School*
Luk H. Vandernberghe, PhD - *Harvard Medical School*

MUSCULO-SKELETAL DISEASES

Marinee K.L. Chuah, PhD - *Free University of Brussels, VUB*
Dongsheng Duan, PhD - *University of Missouri School of Medicine*
Renzhi Han, PhD - *The Ohio State University Wexner Medical Center*
Qi Long Lu, MD, PhD - *Carolinas Medical Center*
Louise Rodino-Klapac, PhD - *Nationwide Children's Hospital*
Kathryn R. Wagner, MD, PhD - *The Johns Hopkins School of Medicine*

CANCER - IMMUNOTHERAPY, CANCER VACCINES

Daniel J. Powell, PhD - *University of Pennsylvania*
Chiara Bonini, MD - *Fondazione Centro S. Raffaele*
Marcela V. Maus, MD, PhD - *Massachusetts General Hospital*
Rimas J. Orentas, PhD - *Seattle Children's Research Institute*
Daniel J. Powell, PhD - *University of Pennsylvania*
Barbara Savoldo, MD, PhD - *UNC Lineberger Comprehensive Cancer Center*
David M. Spencer, PhD - *Bellicum Pharmaceuticals, Inc.*

CANCER - ONCOLYTIC VIRUSES

Caroline Breitbach, PhD - *Turnstone Biologics*
Evanthia Galanis, MD, DSc - *Mayo Clinic*
Noriyuki Kasahara, MD, PhD - *University of Miami*
David Kirn, MD - *4D Molecular Therapeutics*
Liliana Maruri Avidal, PhD - *Ignite Immunotherapy*
Leonard W. Seymour, PhD - *University of Oxford*

ABSTRACT REVIEWERS

CANCER - TARGETED GENE AND CELL THERAPY

Irina V. Balyasnikova, PhD - *Northwestern University Feinberg School of Medicine*
Christine Brown, PhD - *City of Hope National Medical Center*
Balveen Kaur, PhD - *The University of Texas Health Science Center at Houston*
Katy Rezvani, MD, PhD - *MD Anderson Cancer Center*
Michel Sadelain, MD, PhD - *Memorial Sloan Kettering Cancer Center*
Richard G. Vile, PhD - *Mayo Clinic*

HEMATOLOGIC AND IMMUNOLOGIC DISEASES

Jennifer E. Adair, PhD - *Fred Hutchinson Cancer Research Center*
Alessandra Biffi, MD - *Dana-Farber/Boston Children's Cancer and Blood Disorders Center*
Jennifer L. Gori, PhD - *Editas Medicine*
Andre Larochelle, MD, PhD - *National Institutes of Health*
Harry L. Malech, MD - *NIH/NIAID Laboratory of Host Defenses*
David M. Markusic, PhD - *University of Florida*

IMMUNOLOGICAL ASPECTS OF GENE THERAPY AND VACCINES

Antonia Follenzi, MD, PhD - *University of Piemonte Orientale*
Brad E. Hoffman, PhD - *University of Florida College of Medicine*
Ann M. Leen, PhD - *Baylor College of Medicine*
Federico Mingozzi, PhD - *INSERM, UPMC, and Genethon*
Pawel J. Muranski, MD - *Columbia University Medical Center*
Lili Yang, PhD - *University of California Los Angeles*

CELL THERAPIES

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Gwendolyn K. Binder-Scholl, PhD - *Adaptimmune LLC*
Paul Gadue, PhD - *Children's Hospital of Philadelphia*
Markus Grompe, MD - *Oregon Health & Science University*
Carolyn Lutzko, PhD - *Cincinnati Children's Hospital Medical Center*
Eirini P. Papapetrou, MD, PhD - *Icahn School of Medicine at Mount Sinai*

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Maritza C. McIntyre, PhD - *Advanced Therapies Partners*
Isabelle Riviere, PhD - *Memorial Sloan-Kettering Cancer Center*
Johannes C.M. van Der Loo, PhD - *Children's Hospital of Philadelphia*
J. Fraser Wright, PhD - *Wright Consulting*

PHARMACOLOGY/TOXICOLOGY STUDIES OR ASSAY DEVELOPMENT

Giuliana Ferrari, PhD - *HSR-TIGET, Fondazione S. Raffaele*
Kathleen Meyer, MPH, PhD, DABT - *Sangamo Therapeutics*
Jakob Reiser, PhD - *Food and Drug Administration*
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Congratulations to the follow individuals for receiving an ASGCT Award!

SONIA SKARLATOS PUBLIC SERVICE AWARD

Francesca Pasinelli
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Rome, Italy

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The Outstanding Achievement Award is sponsored by Audentes Therapeutics.

AUDENTES

OUTSTANDING NEW INVESTIGATOR AWARDS

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Luk H. Vandenberghe, PhD
Harvard Medical School, Boston, MA

The Outstanding New Investigator Awards are sponsored by:

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The Excellence in Research Awards will be presented on Thursday, May 17 during the George Stamatoyannopoulos Lecture.

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SCHEDULE AT A GLANCE

WEDNESDAY, MAY 16, 2018

8:00 am – 10:00 am	Scientific Symposia Session I
10:00 am – 10:30 am	Coffee Break
10:30 am – 12:00 pm	Oral Abstract Session I
12:00 pm – 1:30 pm	Lunch Break (Lunch Not Provided)
12:00 pm – 1:30 pm	Industry Lunch Symposium – *Sponsored by Terumo BCT*
1:30 pm – 3:00 pm	Education Sessions
3:00 pm – 3:45 pm	Coffee Break
3:45 pm – 5:30 pm	Oral Abstract Session II
5:30 pm – 7:30 pm	Exhibit Hall Welcome Reception & Poster Session I – *Sponsored by Precision BioSciences*
6:00 pm – 8:00 pm	Industry Symposium – *Sponsored by Synpromics Ltd*
7:30 pm – 9:30 pm	Reconnection & Mentoring Event – *Sponsored by Biogen*

THURSDAY, MAY 17, 2018

7:00 am – 8:00 am	Industry Symposium – *Sponsored by Abeona Therapeutics*
8:00 am – 10:00 am	Scientific Symposia Session II
10:00 am – 10:45 am	Exhibit Hall Coffee Social – with Oral Poster Session
10:45 am – 12:00 pm	George Stamatoyannopoulos Lecture & Presentation of the Excellence in Research Awards & Sonia Skarlatos Public Service Award Presentation – *Sponsored by Biogen*
12:00 pm – 1:30 pm	Lunch Break (Lunch Not Provided)
12:00 pm – 1:30 pm	Industry Lunch Symposium – *Sponsored by Miltenyi Biotec Inc.*
1:30 pm – 3:00 pm	Outstanding New Investigator Symposium – *Sponsored by Burroughs Wellcome Fund (BWF)*
3:00 pm – 3:45 pm	Exhibit Hall Coffee Social
3:45 pm – 5:15 pm	Oral Abstract Session III
5:15 pm – 7:30 pm	Exhibit Hall Networking Reception & Poster Session II – with Tools and Technologies Forum – *Sponsored by YPOSKESI*
6:00 pm – 7:00 pm	New Member Reception – *Sponsored by AveXis*
7:30 pm – 9:30 pm	Industry Symposium – *Sponsored by Spark Therapeutics*

FRIDAY, MAY 18, 2018

7:00 am – 8:30 am	Industry Symposium – *Sponsored by Agilis Biotherapeutics*
8:00 am – 10:00 am	Scientific Symposia Session III
10:00 am – 10:45 am	Exhibit Hall Coffee Social – with Oral Poster Session
10:45 am – 11:45 am	Outstanding Achievement Award Lecture – *Sponsored by Audentes Therapeutics*
11:45 am – 1:15 pm	Lunch Break (Lunch Not Provided)
11:45 am – 1:15 pm	Industry Lunch Symposium – *Sponsored by MaxCyte*
1:15 pm – 3:15 pm	Presidential Symposium & Presentation of the Top Abstracts – *Sponsored by Oxford BioMedica*
3:15 pm - 4:00 pm	Exhibit Hall Coffee Social
4:00 pm – 5:45 pm	Oral Abstract Session IV
5:45 pm – 7:45 pm	Exhibit Hall Networking Reception & Poster Session III – with Tools and Technologies Forum
8:00 pm – 11:00 pm	Closing Night Reception at Field Museum – *Sponsored by University of Massachusetts Medical School*

SATURDAY, MAY 19, 2018

7:30 am - 8:00 am	Business Meeting & Coffee Break
8:00 am – 10:00 am	Scientific Symposia Session IV & Education Sessions
10:15 am - 12:15 pm	Oral Abstract Session V
12:15 pm	Annual Meeting Concludes

Trainee Lounge

7:00 am – 5:30 pm

Room: Mobley

The Trainee Lounge is reserved for Students and Trainees as a designated spot to network, grab a refreshing snack and beverage and meet other students and trainees. Members and Non-members are welcome!

Scientific Symposium 100

8:00 am - 10:00 am

Room: International Ballroom North

Immune Responses to Cell and Gene Therapies, Mechanisms, Biomarkers, and Therapeutic Interventions - Organized by the Immune Responses to Gene & Cell Therapy Committee

CO-CHAIRS: Conrad Russell Y. Cruz, MD, PhD and Oumeya Adjali, MD, PhD

SPEAKERS

8:00 am – 8:30 am

Chiara Bonini, MD. Università Vita-Salute San Raffaele, Milan, Italy, Ospedale San Raffaele Scientific Institute, Milan, Italy

TCR Gene Transfer and TCR Gene Editing

8:30 am – 9:00 am

Cameron J. Turtle, MBBS, PhD. Fred Hutchinson Cancer Research Center, Seattle, WA

Understanding Biomarkers and Outcomes of CD19 CAR-T Cell Therapy

9:00 am – 9:30 am

Luigi M. Naldini, MD, PhD. San Raffaele Telethon Institute for Gene Therapy, Milan, Italy, Vita Salute San Raffaele University, Milan, Italy

Immune Stealth Lentiviral Vectors for Liver-directed Gene Therapy for Hemophilia

9:30 am – 10:00 am

George M. Church, PhD. Harvard Medical School, Boston, MA, MIT, Cambridge, MA

Reducing the Immunogenicity of AAV through Engineering the Vector

Scientific Symposium 101

8:00 am - 10:00 am

Room: Salon A-5

Gene and Vaccine Therapies for Infectious Diseases - Organized by the Infectious Diseases and Vaccines Committee

CHAIR: Hildegard Buning, PhD

SPEAKERS

8:00 am – 8:30 am

Frank Buchholz, PhD. TU Dresden, Faculty of Medicine, Dresden, Germany

Designer-Recombinases Targeting Human Pathogenic Viruses

8:30 am – 9:00 am
Mark L. Bagarazzi, MD. Inovio Pharmaceuticals, Plymouth Meeting, PA
Phase III Trial for Treatment of Cervical Dysplasia Caused by Human Papillomavirus

9:00 am – 9:30 am
Matti Sallberg, PhD, DDS. Karolinska University Hospital Huddinge, Stockholm, Sweden
Gene and Cell Therapies for Viral Hepatitis B, C, and D Infections

9:30 am – 10:00 am
Ann M. Leen, PhD. Baylor College of Medicine - CAGT, Houston, TX
Viral Infections in Transplantation

Scientific Symposium 102

8:00 am - 10:00 am
Room: Salon A-1/2

New Developments in Physical Delivery and Vector Development - Organized by the Physical Delivery, Therapeutics & Vector Development Committee

CO-CHAIRS: Dexi Liu, PhD and Daniel Scherman, PhD

SPEAKERS

8:00 am – 8:30 am
Richard Heller, PhD. Old Dominion University, Norfolk, VA
Electroporation-mediated Gene Delivery to the Heart for Coronary Artery Disease

8:30 am – 9:00 am
Dexi Liu, PhD. University of Georgia, Athens, GA
Hydrodynamic Delivery of Genes to the Liver to Treat Obesity and Obesity-associated Hepatic Steatosis

9:00 am – 9:30 am
Maja Cemazar, PhD. Institute of Oncology Ljubljana, Ljubljana, Slovenia, Faculty of Health Sciences, University of Primorska, Izola, Slovenia
Clinical Electrochemotherapy and Gene Electrotransfer for Tumor Treatment

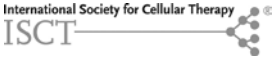
9:30 am – 10:00 am
Gadi Pelled, PhD DMD. Cedars Sinai Medical Center, Los Angeles, CA
Gene Therapy for Skeletal Tissue Repair Using Molecular Conjugates, Physical Delivery and Tissue Engineering

Scientific Symposium 103

8:00 am - 10:00 am
Room: Salon A-3, 4

Regenerative and Bionic Medicine

Co-Sponsored by the International Society for Cellular Therapy (ISCT)



CO-CHAIRS: Catherine M. Bollard, MD and Austin J. Barrett, MD

SPEAKERS

8:00 am – 8:30 am
Daniel J. Weiss, MD, PhD. University of Vermont College of Medicine, Burlington, VT
Cell Therapeutics for Pulmonary Disease

8:30 am – 9:00 am
Geoffrey Ling, MD, PhD. Johns Hopkins University School of Medicine, Baltimore, MD
Brain-Computer Interface Technologies

9:00 am – 9:30 am
Amit N. Patel, MD, MS. University of Miami, Miami, FL
Cardiac Regeneration

9:30 am – 10:00 am
Donald G. Phinney, PhD. The Scripps Research Institute-Scripps Florida, Jupiter, FL
Reconciling the Stem/Progenitor and Paracrine Functions of MSCs

Scientific Symposium 104

8:00 am - 10:00 am
Room: International Ballroom South

Stem Cells from Translation to the Clinic - Organized by the Stem Cell Committee

CO-CHAIRS: Carolyn Lutzko, PhD and Jennifer L. Gori, PhD

SPEAKERS

8:00 am – 8:30 am
Henry Klassen, MD, PhD. University of California, Irvine, Irvine, CA
Retinal Progenitor Cells for Treatment of Retinitis Pigmentosa

8:30 am – 9:00 am
Olivia Kelly, PhD. ViaCyte, Inc., San Diego, CA
Developing a Cell Therapy Combination Product for Diabetes

9:00 am – 9:30 am
Michael Helmrath, MD, MS. Cincinnati Children's Hospital Medical Center, Cincinnati, OH
***In vivo* Modeling of the Human Intestine; Looking at the Big Picture**

9:30 am – 10:00 am
Linzhao Cheng, PhD. Johns Hopkins University School of Medicine, Baltimore, MD
Genome Editing in Human Stem Cells for Blood Disease Modeling and Treatment

Scientific Symposium 105

8:00 am - 10:00 am

Room: Continental Ballroom ABC

The Universe is Expanding – Reviewing the State of the Art in Stem Cell Derived Cell Therapy - Organized by the Translational Science and Product Development Committee

CO-CHAIRS: David M. Spencer, PhD and Daniel J. Powell, Jr., PhD

SPEAKERS

8:00 am – 8:30 am
Bob Valamehr, PhD. Fate Therapeutics, Inc., San Diego, CA
Pluripotent Cell-Derived NK Cells as a Cornerstone Approach for Off-the-Shelf Cancer Immunotherapy

8:30 am – 9:00 am
Michel Sadelain, MD, PhD. Memorial Sloan Kettering Cancer Center, New York, NY
iT cells Derived from iPSCs

9:00 am – 9:30 am
Joanna E. Brewer, PhD. Adaptimmune Ltd, Oxford, United Kingdom
Gene Editing of Stem Cells for Universal Cell Therapy

9:30 am – 10:00 am
Panel Discussion

Scientific Symposium 106

8:00 am - 10:00 am

Room: Waldorf

Gene Therapy for Alzheimer's and Related Disorders

CO-CHAIRS: Ronald Crystal, MD and Nathalie Cartier-Lacave, MD

SPEAKERS

8:00 am – 8:30 am
Steven M. Paul, MD. Voyager Therapeutics, Cambridge, MA
Alzheimer's Disease: from Genetics to Pathogenesis to Gene Therapy

8:30 am – 9:00 am
Nathalie Cartier-Lacave, MD. INSERM U1169, Paris, France
Gene Therapy for Alzheimer's Disease Targeting Brain Cholesterol Metabolism

9:00 am – 9:30 am
Ronald Crystal, MD. Weill Cornell Medical College, New York, NY
Gene Therapy for APOE4 Homozygotes at High Risk for Alzheimer's Disease

9:30 am – 10:00 am
James M. Wilson, MD, PhD. University of Pennsylvania, Philadelphia, PA
Gene Therapy for Frontal Temporal Dementia

Coffee Break

10:00 am - 10:30 am

Oral Abstract Session 110

10:30 am - 12:00 pm

Room: International Ballroom North

Hematologic & Immunologic Diseases I

CO-CHAIRS: Jennifer L. Gori, PhD and Andre Larochelle, MD, PhD

PRESENTERS

10:30 am – 10:45 am

1: SPK-9001: Adeno-Associated Virus Mediated Gene Transfer for Hemophilia B - Sustained Fix Activity, Persistent Endogenous Prophylaxis and Improved Quality of Life at One Year and Beyond

Xavier M. Anguela, Spark Therapeutics, Philadelphia, PA

10:45 am – 11:00 am

2: Lentiviral Hematopoietic Stem Cells Gene Therapy for Beta-Thalassemia: Update from the Phase I/II TIGET BTHAL Trial

Samantha Scaramuzza, San Raffaele Telethon Institute for Gene Therapy, Milano, Italy

11:00 am – 11:15 am

3: Gene Editing of FOXP3 in Primary CD4+ T Cells to Generate Antigen-Specific Engineered T_{reg} Products

Logan Fisher, Seattle Children's Research Institute, Seattle, WA

11:15 am – 11:30 am

4: The Potential Use of Nanobodies Delivered *Via* AAV Vectors in the Treatment of Haemophilia

Elena Barbon, Genethon and INSERM U951, Evry, France

11:30 am – 11:45 am

5: Reconstitution of Hematopoiesis in Patients Treated with Gene Therapy for Beta-Thalassemia

Maria Rosa Lidonnici, SR-TIGET, Milan, Italy

11:45 am – 12:00 pm

6: Hematopoietic Stem Cell Gene Editing for the Treatment of Wiskott-Aldrich Syndrome

Alessia Cavazza, GOS Institute of Child Health, University College London, London, United Kingdom

Oral Abstract Session 111

10:30 am - 12:00 pm

Room: International Ballroom South

Musculo-Skeletal Diseases I

CO-CHAIRS: Dongsheng Duan, PhD and Louise Rodino-Klapac, PhD

PRESENTERS

10:30 am – 10:45 am

7: Aspiro Phase 1/2 Gene Therapy Trial in X-Linked Myotubular Myopathy (Xlmtm): Preliminary Safety and Efficacy Findings
Suyash Prasad, Audentes Therapeutics, San Francisco, CA

10:45 am – 11:00 am

8: AAV Micro-Dystrophin Therapy Ameliorates Muscular Dystrophy in Young Adult Duchenne Muscular Dystrophy Dogs for Up to Thirty Months Following Injection
Chady Hakim, NCATS/University of Missouri, Columbia, MO

11:00 am – 11:15 am

9: Dose Escalation Study of Systemically Delivered AAVrh74.MHCK7.Micro-Dystrophin in the Mdx Mouse Model of DMD
Rachael A. Potter, The Research Institute at Nationwide Children's Hospital, Columbus, OH

11:15 am – 11:30 am

10: Gene Therapy Leads to Dose-Dependent Transcriptome Remodeling and Provides Biomarkers of Therapeutic Efficacy in X-Linked Myotubular Myopathy
Jean-Baptiste Dupont, University of Washington, Department of Rehabilitation Medicine, ISCRM, Seattle, WA

11:30 am – 11:45 am

11: AAV-SERCA2a Gene Therapy Ameliorated Muscle Disease in Duchenne Muscular Dystrophy Dogs
Kasun Kodippili, University of Missouri - Columbia, Columbia, MO

11:45 am – 12:00 pm

12: A Phase 1/2 Clinical Trial of Intra-Arterial Gene Transfer of rAAVrh74.MCK.GALGT2 for DMD: Initial Safety Profile
Kevin M. Flanigan, Nationwide Children's Hospital, Columbus, OH

Oral Abstract Session 112

10:30 am - 12:00 pm
Room: Continental Ballroom ABC

Cancer - Oncolytic Viruses

CO-CHAIRS: David Kirn, MD and Liliana Maruri Avidal, PhD

PRESENTERS

10:30 am – 10:45 am

13: The Innate Immune System as a Determinant for Response to Measles Virotherapy
Cheyne Kurokawa, Mayo Clinic, Rochester, MN

10:45 am – 11:00 am

14: Combined Mesothelin-Redirected Chimeric Antigen Receptor T Cells with Cytokine-Armed Oncolytic Adenoviruses for the Treatment of Pancreatic Cancer
Keisuke Watanabe, University of Pennsylvania, Philadelphia, PA

11:00 am – 11:15 am

15: Phase I Study of Potentially "Best-in-Class" Survivin-Responsive Conditionally Replicating Adenovirus for Advanced Sarcoma Actually Demonstrates Potent and Long-Term Efficacy and High Safety
Ken-ichiro Kosai, Kagoshima University, Kagoshima, Japan

11:15 am – 11:30 am

16: Preclinical Evaluation of NIS Expressing Oncolytic Adenoviruses as a Theranostic Tool for Pancreatic Cancer
Lisa Koodie, University of Minnesota, Minneapolis, MN

11:30 am – 11:45 am

17: Oncolytic Measles Virus Replication is Enhanced in Acute Lymphoblastic Leukaemia-Associated Mesenchymal Stromal Cells Which Have Adopted a Cancer Associated Fibroblast Phenotype and Correlates with Upregulation of Matrix Metalloproteinase 1
Aditi D. Dey, University College London, London, United Kingdom

11:45 am – 12:00 pm

18: Oncolytic Viruses to Enhance BiTE and CAR Therapy of Solid Tumors
Johannes P.W. Heidbuechel, German Cancer Research Center (DKFZ) and National Center for Tumor Diseases (NCT), Heidelberg, Germany

Oral Abstract Session 113

10:30 am - 12:00 pm

Room: Salon A-1/2

Cancer - Targeted Gene & Cell Therapy I

CO-CHAIRS: Irina Balyasnikova, PhD and Balveen Kaur, PhD

PRESENTERS

10:30 am – 10:45 am

19: Monocyte-Derived IL-1 and IL-6 are Differentially Required for Cytokine Release Syndrome and Neurotoxicity by CAR-T Cells
Margherita Norelli, Università Vita-Salute San Raffaele, Milan, Italy

10:45 am – 11:00 am

20: Turbocharged CAR T Cells Stimulate Host T Cells against Prostate Cancer
Zeguo Zhao, Memorial Sloan Kettering Cancer Center, New York, NY

11:00 am – 11:15 am

21: HER2-Targeted Dual Switch CAR-T Cells Enable Post-Infusion Control of CAR-T Efficacy and Safety with Small Molecules
Matthew R. Collinson-Pautz, Bellicum Pharmaceuticals, Houston, TX

11:15 am – 11:30 am

22: Intracerebral Immunomodulation Using Genetically Engineered Mesenchymal Stem Cells Induces Long-Term Survival and Immunity in Glioblastoma
Nils Ole Schmidt, University Medical Center Hamburg-Eppendorf, Hamburg, Germany

11:30 am – 11:45 am

23: T Cell Redirection with Coupled Long Terminal-CRISPR Gene Editing Effects
Christos Georgiadis, UCL, GOS Institute of Child Health, London, United Kingdom

11:45 am – 12:00 pm

24: CD30-Redirected Chimeric Antigen Receptor T Cells Target $CD30^+$ and $CD30^-$ Embryonal Carcinoma via Antigen-Dependent and Fas/FasL Interactions
L Hong, University of North Carolina, Chapel Hill, NC

Oral Abstract Session 114

10:30 am - 12:00 pm

Room: Salon A-3, 4

RNA Virus Vectors and Small RNA Therapy

CO-CHAIRS: Byoung Y. Ryu, PhD and Paloma H. Giangrande, PhD

PRESENTERS

10:30 am – 10:45 am

25: Transient Retroviral MS2-CRISPR/Cas9 All-in-One Particles for Efficient Targeted Gene Knockout
Yvonne Knopp, Hannover Medical School, Hannover, Germany

10:45 am – 11:00 am

26: Beta-Deliverin Relieves Anti-Viral Restriction of the IFITM Proteins Thereby Enhancing VSV-G Lentiviral Vector Gene Delivery to Human Hematopoietic Stem Cells

Stosh Ozog, The Scripps Research Institute, La Jolla, CA

11:00 am – 11:15 am

27: Addressing the Impact of Vector Integration on Chromatin Architecture

Monica Volpin, San Raffaele Telethon Institute for Gene Therapy, Milan, Italy

11:15 am – 11:30 am

28: Pharmacology and Safety of VY-HTT01, an AAV miRNA Gene Therapy Targeting Huntingtin for the Treatment of Huntington's Disease

Dinah Sah, Voyager Therapeutics, Inc, Cambridge, MA

11:30 am – 11:45 am

29: AAV.U7-snRNA-Mediated Exon Skipping of the Toxic DUX4 Gene as a Promising Therapeutic Approach for Facioscapulohumeral Muscular Dystrophy

Afrooz Rashnonejad, Research Institute at Nationwide Children's Hospital, Columbus, OH

11:45 am – 12:00 pm

30: Peptide Mediated Delivery of Oligonucleotides to the CNS across the Blood-Brain Barrier

Brian Spencer, University of California, San Diego, San Diego, CA

Oral Abstract Session 115

10:30 am - 12:00 pm

Room: Salon A-5

Hematopoietic Cell Therapies

CO-CHAIRS: Daniel Bauer, MD, PhD and Christian Brendel, PhD

PRESENTERS

10:30 am – 10:45 am

31: A Novel Target for Hematopoietic Stem Cell (HSC) Gene Therapy and Editing

Stefan Radtke, Fred Hutchinson Cancer Research Center, Seattle, WA

10:45 am – 11:00 am

32: Immunotoxin-Based Conditioning Facilitates Autologous Hematopoietic Stem Cell Engraftment and Multi-Lineage Development in a Fanconi Anemia Mouse Model

Meera Srikanthan, Seattle Children's Hospital, Seattle, WA

11:00 am – 11:15 am

33: Development of a New Generation of Gene-Edited HSC to Induce Engraftment Advantage and Favour Outgrowth *In Vivo*

Claire Latroche, TIGET-San Rafaelle, Milan, Italy

11:15 am – 11:30 am

34: Towards a Mechanistic Understanding of *Ex Vivo* Hematopoietic Stem Cell Expansion for Gene Therapy

Bernhard Gentner, San Raffaele Telethon Institute for Gene Therapy, Milano, Italy

11:30 am – 11:45 am

35: Long-Term Human Protein Expression in Mice via Engraftment of Gene-Edited Human Plasma Cells

King L. Hung, Seattle Children's Research Institute, Seattle, WA

11:45 am – 12:00 pm

773: Macrophage Polarization Impacts Tunneling Nanotube Formation and Organelle Trafficking Following Hematopoietic Stem Cell Therapy for Cystinosis

Spencer Goodman, University of California, San Diego, La Jolla, CA

Industry Symposium 120

12:00 pm - 1:30 pm

Room: Waldorf

What I Wish I Knew Then: Key Learnings in Bringing a Cell/Gene Therapy from Inception to Commercialization

Sponsored by Terumo BCT

TERUMOBCT
Unlocking the Potential of Blood

MODERATOR: Regis Leonard

PANELISTS

Julie G. Allickson, PhD. Wake Forest Institute for Regenerative Medicine, Winston-Salem, NC

Scott Broughton. Cognate Bioservices, Portsmouth, NH

Salim Mamujee. Kite Pharma, a Gilead Company, Santa Monica, CA

Break

12:00 pm - 1:30 pm

Lunch Break *(On Own - Not Provided)*

Trainee Lunch Session

12:00 pm – 1:30 pm

Room: Mobley

Tips for Writing Successful Grants & Introduction to the CSR Study Section

Kee Pyon, PhD - **Introduction to the CSR Study Section**

Cary Harding, PhD - **Introduction to Grant Writing**

Sponsored by Rocket Pharma rocket pharma

Education Session 130

1:30 pm - 3:00 pm

Room: Continental Ballroom ABC

CAR-T Cell Design & Function Improvements

CHAIR: Avery D. Posey, Jr., PhD

SPEAKERS

1:30 pm – 2:00 pm
Maksim Mamonkin, PhD. Baylor College of Medicine, Houston, TX
Structure-Function Relationship in Chimeric Antigen Receptors

2:00 pm – 2:30 pm
Roderick O'Connor, PhD. University of Pennsylvania, Philadelphia, PA
Assessing the Immunometabolism of CAR-T cells

2:30 pm – 3:00 pm
Kole Roybal, PhD. University of California, San Francisco, San Francisco, CA, Parker Institute for Cancer Immunotherapy, San Francisco, CA
Synthetic Control of Therapeutic T cell Function

Education Session 131

1:30 pm - 3:00 pm
Room: Salon A-5

Challenges for Successful Non-viral Delivery of Nucleic Acids and Proteins *in vivo*

CO-CHAIRS: Assem G. Ziady, PhD and Millicent O. Sullivan, PhD

SPEAKERS

1:30 pm – 2:00 pm
Euan Ramsay, PhD. Precision NanoSystems Inc., Vancouver, BC, Canada
Overview of Next-generation Approaches for Rapid Production of Nanoparticles for Use in Gene and Cell Therapies

2:00 pm – 2:30 pm
Kevin G. Rice, PhD. University of Iowa College of Pharmacy, Iowa City, IA
Design, Formulation and Testing of Systemically Dosed Peptide and Polymer DNA and mRNA Nanoparticles for Gene Delivery

2:30 pm – 3:00 pm
Daniel G. Anderson, PhD. Massachusetts Institute of Technology, Cambridge, MA
Nucleic Acid Delivery Systems for RNA Therapy and Gene Editing

Education Session 132

1:30 pm - 3:00 pm
Room: Salon A-3, 4

Exosomes

CHAIR: Casey A. Maguire, PhD

SPEAKERS

1:30 pm – 2:00 pm
George N. Pavlakis, MD, PhD. National Cancer Institute, Frederick, MD
Exosomes/ "Scalable Purification and Tumor Targeting of Engineered Extracellular Vesicles"

2:00 pm – 2:30 pm
Chulhee Choi, MD, PhD. KAIST, Daejeon, Republic of Korea
Exosomes/ "Exosome Engineering for Delivery of Therapeutic Proteins: Principles and Applications"

2:30 pm – 3:00 pm
Jason Howitt, PhD. Florey Institute of Neuroscience and Mental Health, Melbourne, Australia, Swinburne University of Technology, Melbourne, Australia
Exosomes/ "Engineering Exosomes for Use as Therapeutic Agents"

Education Session 133

1:30 pm - 3:00 pm
Room: International Ballroom South

Immunogenicity

CHAIR: Andrew M. Scharenberg, MD

SPEAKERS

1:30 pm – 2:00 pm
Roland W. Herzog, PhD. University of Florida, Gainesville, FL
Immunogenicity of AAV Vectors and Transgenes

2:00 pm – 2:30 pm
Dwight Koeberl, MD, PhD. Duke University Medical Center, Durham, NC
Inducing Immune Tolerance with Liver-directed Expression by AAV

2:30 pm – 3:00 pm
Carol H. Miao, PhD. University of Washington, Seattle, WA, Seattle Children's Research Institute, Seattle, WA
Regulatory T-cells for Inducing Tolerance to FVIII

Education Session 134

1:30 pm - 3:00 pm
Room: Salon A-1/2

Oncolytics

CO-CHAIRS: Daniel J. Powell, Jr., PhD and TBD

SPEAKERS

1:30 pm – 2:00 pm
Evanthia Galanis, MD, DSc. Mayo Clinic, Rochester, MN
Measles Virus Based Oncolytic Virotherapy and Immunovirotherapy

2:00 pm – 2:30 pm
Joseph C. Glorioso, III, PhD. University of Pittsburgh, Pittsburgh, PA
Design and Translational Application of HSV Gene Vectors in Nervous System Diseases

2:30 pm – 3:00 pm
Leonard W. Seymour, PhD. University of Oxford, Oxford, United Kingdom
Oncolytic Viruses for Targeted Immunotherapy

Education Session 135

1:30 pm - 3:00 pm
Room: International Ballroom North

The Basics of Genome Editing

CHAIR: Thomas Wechsler, PhD

SPEAKERS

1:30 pm – 2:00 pm
Elizabeth Garner, PhD. Caribou Biosciences, Inc., Berkeley, CA
Editing Nucleases and DNA Repair

2:00 pm – 2:30 pm
Paula M. Cannon, PhD. University of Southern California, Los Angeles, CA
Genome Editing: a Path to Clinic

2:30 pm – 3:00 pm
Silvia Camporesi, PhD. King's College London, London, United Kingdom
Bioethics of Genome Editing

Coffee Break

3:00 pm - 3:45 pm

Oral Abstract Session 140

3:45 pm - 5:30 pm
Room: International Ballroom North

Advances in CRISPR/Cas Technologies

CO-CHAIRS: Paula M. Cannon, PhD and Shondra Pruett-Miller, PhD

PRESENTERS

3:45 pm – 4:00 pm
37: Development of Orthogonal Cas9-Cas9 Fusion Proteins and Their Potential Application as β-Hemoglobinopathy Therapeutics
Scot A. Wolfe, University of Massachusetts Medical School, Worcester, MA

4:00 pm – 4:15 pm
38: RNA Secondary Structure Increases the Specificity of Class 2 CRISPR Effectors
D Dewran Kocak, Duke University, Durham, NC

4:15 pm – 4:30 pm
39: An Enhanced CRISPR-Cas12a Variant to Improve Genome and Epigenome Editing Efficacy
Benjamin P. Kleinstiver, Massachusetts General Hospital, Charlestown, MA

4:30 pm – 4:45 pm
40: Multiplex Human T Cell Engineering by Cas9 Base Editor Technology
Beau Webber, University of Minnesota, Minneapolis, MN

4:45 pm – 5:00 pm

41: Efficient Genome Editing in Primary Human T, B and HSCs Using Baboon Envelope Gp Pseudotyped Viral Derived "Nanoblades" Loaded with Cas9/sgRNA Ribonucleoproteins

Els Verhoeyen, INSERM, Lyon, France

5:00 pm – 5:15 pm

42: *In Vivo* CRISPR-Cas Genome Editing with No Detectable Off-Target Mutations

Maggie L. Bobbin, MGH, Harvard Medical School,Boston, MA

5:15 pm – 5:30 pm

43: Enhanced Genome Editing Using Split *Staphylococcus Aureus* Cas9 Delivered in Double-Stranded Adeno-Associated Viral Vectors

Carolin C. Schmelas, University Hospital Heidelberg, Heidelberg, Germany

Oral Abstract Session 141

3:45 pm - 5:30 pm

Room: International Ballroom South

Hematologic & Immunologic Diseases II

CO-CHAIRS: Jennifer E. Adair, PhD and David M. Markusic, PhD

PRESENTERS

3:45 pm – 4:00 pm

44: Selective HSC-Ablation Using CD117 Antibody-Drug-Conjugates Enables Safe and Effective Murine and Human Hematopoietic Stem Cell Transplantation

Agnieszka Czechowicz, Stanford University School of Medicine, Stanford, CA

4:00 pm – 4:15 pm

45: Liver-Directed Gene Therapy for Hemophilia B with Immune Stealth Lentiviral Vectors

Michela Milani, San Raffaele-Telethon Institute for Gene Therapy, Milan, Italy

4:15 pm – 4:30 pm

46: *In Vivo* HSC Transduction in Mobilized Mice with Subsequent *In Vivo* Selection Results in Efficient Expression of Human Gamma Globin in Peripheral Blood Erythrocytes

Hongjie Wang, University of Washington, Seattle, WA

4:30 pm – 4:45 pm

47: Safe and Effective Platelet-Targeted Gene Therapy of Hemophilia A Enabled Using Non-Genotoxic, Antibody-Drug-Conjugate Conditioning

Qizhen Shi, Medical College of Wisconsin, Blood Research Institute, Children's Research Institute, Milwaukee, WI

4:45 pm – 5:00 pm

48: Restoration of PLT Structure and Function in Wiskott-Aldrich Syndrome Patients after Gene Therapy Treatment

Lucia Sereni, San Raffaele Hospital, Milano, Italy

5:00 pm – 5:15 pm

49: Successful Hematopoietic Stem Cell Mobilization and Apheresis Collection Using Plerixafor Alone for Sickle Cell Disease Gene Therapy
Erica B. Esrick, Boston Children's Hospital, Boston, MA

5:15 pm – 5:30 pm

50: BTK Lentiviral Therapy of XLA Patient Stem Cells Restores B Cell Development and Function in Humanized Mice
Swati Singh, Seattle Children's Research Institute, Seattle, WA

Oral Abstract Session 142

3:45 pm - 5:30 pm
Room: Continental Ballroom ABC

AAV Biology

CO-CHAIRS: Shen S. Shen, PhD and Junghae Suh, PhD

PRESENTERS

3:45 pm – 4:00 pm

51: RNF121 is a Key Transcriptional Regulator of AAV Genome Expression
Victoria J. Madigan, UNC Chapel Hill, Chapel Hill, NC

4:00 pm – 4:15 pm

52: Mapping and Engineering Functional Domains of the Assembly Activating Protein of Adeno-Associated Viruses
Longping V. Tse, UNC Chapel Hill, Chapel Hill, NC

4:15 pm – 4:30 pm

53: A Novel Class of Clade C AAV Capsids Isolated from the Human Population Exhibit Greater Than Ten-Fold Higher Packaging Efficiencies Than AAV2
Meiyu Xu, University of Massachusetts Medical School, Worcester, MA

4:30 pm – 4:45 pm

54: Interactions of AAV-2 with Its Cellular Receptor, AAVR, Visualized by *Cryo*-Electron Microscopy
Nancy L. Meyer, Oregon Health and Science University, Portland, OR

4:45 pm – 5:00 pm

55: Capsid-Glycan Receptor Interactions Influence AAV Transport across the Blood-Brain Barrier
Blake H. Albright, University of North Carolina at Chapel Hill, Chapel Hill, NC

5:00 pm – 5:15 pm

56: Systematic Functional Characterization of the AAV Capsid Fitness Landscape
Eric Kelsic, Harvard Medical School, Boston, MA

5:15 pm – 5:30 pm

57: A Proximity-Based Proteomics Approach to Identify Cellular Proteins Interacting with AAV Assembly Activating Protein (AAP)
Swapna Kollu, Oregon Health & Science University, Portland, OR

Oral Abstract Session 143

3:45 pm - 5:30 pm
Room: Salon A-1/2

Translational and Clinical Progress in Neurological Disease

CO-CHAIRS: Alberto Auricchio, MD and Beverly L. Davidson, PhD

PRESENTERS

3:45 pm – 4:00 pm

58: AVXS-101 Phase 1 Gene Therapy Clinical Trial in SMA Type 1: Event-Free Survival and Achievement of Developmental Milestones
Jerry R. Mendell, Center for Gene Therapy Nationwide Children's Hospital, Columbus, OH

4:00 pm – 4:15 pm

59: Dose Escalation Gene Therapy Trial in Children with AADC Deficiency
Krystof Bankiewicz, University of California San Francisco, San Francisco, CA

4:15 pm – 4:30 pm

60: Gene Therapy Improves Cerebral White Matter Microstructures in Patients with Aromatic L-Amino Acid Decarboxylase Deficiency
Wuh-Liang Hwu, National Taiwan University Hospital, Taipei, Taiwan

4:30 pm – 4:45 pm

61: First Gene Supplementation Therapy for CNGA3-Linked Achromatospia
Stylianos Michalakis, Ludwig-Maximilians-University Munich, Munich, Germany

4:45 pm – 5:00 pm

62: Immune Blockade in CNS Gene Therapy Improves Safety and Clinical Outcome
Manuela Corti, University of Florida, Gainesville, FL

5:00 pm – 5:15 pm

63: *AADC* Gene Therapy for Advanced Parkinson's Disease: Interim Results of a Phase 1b Trial
Brendon Boot, Voyager Therapeutics, Cambridge, MA

5:15 pm – 5:30 pm

64: First-In-Human Intrathecal Gene Transfer Study for Giant Axonal Neuropathy: Interim Analysis of Efficacy and Review of Safety
Diana Bharucha-Goebel, National Institutes of Health/ NINDS, Bethesda, MD

Oral Abstract Session 144

3:45 pm - 5:30 pm
Room: Salon A-3, 4

Cell Therapies

CO-CHAIRS: Markus Grompe, MD and Carolyn Lutzko, PhD

PRESENTERS

3:45 pm – 4:00 pm
65: hESC-Derived Striatal Cells Generated in a 3D Hydrogel Promote Recovery in a Huntington's Disease Mouse Model
Maroof Adil, UC Berkeley, Berkeley, CA

4:00 pm – 4:15 pm
66: CRISPR-Mediated Genetic Engineering of Human Mesenchymal Stromal Cells for Therapeutic Protein Delivery in Chronic Wounds
Waracharee Srifa, Stanford University, Stanford, CA

4:15 pm – 4:30 pm
67: Delivery of Zinc Finger Artificial Transcription Factors Using Engineered Mesenchymal Stem Cells Results in Re-Activation of UBE3A Throughout the Mouse Brain
Peter Deng, UC Davis Medical Center, Sacramento, CA

4:30 pm – 4:45 pm
68: Functionally-Relevant Morphological Profiling Using Visne Reveals Emergent Morphological Subpopulations of IFN-γ-Stimulated MSCs That Predict Immunosuppression
Ross Marklein, US Food and Drug Administration, Silver Spring, MD

4:45 pm – 5:00 pm
69: Surface Mobility and Cluster Formation of Various Melanoma Associated Antigens Modulates Car T Cell Activation
Arpad Szoor, University of Debrecen, Debrecen, Hungary

5:00 pm – 5:15 pm
70: Improving Functional Maturation of Human Pluripotent Stem Cells Derived Cardiomyocytes through Metabolic Understanding
Margarida Serra, iBET, Oeiras, Portugal

5:15 pm – 5:30 pm
71: Highly Efficient Chondrogenic Differentiation of Human iPSCs and Purification via a Reporter Allele Generated by CRISPR-Cas9 Genome Editing
Shaunak S. Adkar, Duke University, Durham, NC

Oral Abstract Session 145

3:45 pm - 5:30 pm
Room: Salon A-5

Oligonucleotide Therapeutics

CO-CHAIRS: Anton P. McCaffrey, PhD and Shambhavi Shubham, PhD

PRESENTERS

3:45 pm – 4:00 pm

72: Systemic Delivery of PPMO Results in Widespread Muscle Delivery and Efficacy in Mice and Non-Human Primates for the Treatment of Duchenne Muscular Dystrophy
Marco A. Passini, Sarepta Therapeutics, Inc., Cambridge, MA

4:00 pm – 4:15 pm

73: Inhibition of Extracellular Histones in Sepsis-Induced Cardiovascular Function
Ofonime Udofot, University of Iowa, Iowa City, IA

4:15 pm – 4:30 pm

74: Rna Inhibitors of Nuclear Proteins Implicated in Multiple Organ Dysfunction Syndrome
Kevin T. Urak, University of Iowa, Iowa City, IA

4:30 pm – 4:45 pm

75: Targeted Delivery of miR-146a Mimic or Antisense Oligonucleotides as a Potential Therapeutic Approach to Modulate NF-κB Signaling in Cancer and Autoimmune Diseases
Yu-Lin Su, City of Hope Beckman Research Center, Duarte, CA

4:45 pm – 5:00 pm

76: Artificial microRNA Silences C9ORF72 Variants *In Vivo* and Decreases the Toxic Dipeptides in BAC Transgenic Mouse Model
Gabriela Toro Cabrera, UMASS Med, Worcester, MA

5:00 pm – 5:15 pm

77: RNA-Based Combination Therapy for Diabetic Wound Healing
Brian H. Johnston, SomaGenics, Santa Cruz, CA

5:15 pm – 5:30 pm

78: Modulation of Ldlr and Cholesterol by Transcriptional Silencing of the Long Non-Coding Rna Bm450697
Roslyn M. Ray, City of Hope, Beckman Research Institute, Duarte, CA

Exhibit Hall Welcome Reception & Poster Session I

5:30 pm - 7:30 pm
Room: Stevens Salon C & D

Sponsored by Precision BioSciences

Industry Symposium 150

6:00 pm - 8:00 pm
Room: Waldorf

"Next Generation Gene Medicine – Leveraging synthetic promoters in product design"

Sponsored by Synpromics Ltd

Synpromics
the leader in gene control

Welcome Remarks
David Venables, PhD. Chief Executive Officer, Synpromics

Introduction - Symposium/Panelists
Sarah Haecker Meeks, PhD. Vice President, Business Development, Synpromics

Scientific Presentations
Michael Roberts, PhD. Chief Scientific Officer, Founder, Synpromics
Joel Schneider, PhD. Chief Technology Officer, Solid Bio
Jude Samulski, PhD. Founder, AskBIO
Francois Du Plessis, Scientist - Vector Development, uniQure
Albert Seymour, PhD. Chief Scientific Officer, Homology Medicines
Mark S. Shearman, PhD. Chief Scientific Officer, Applied Genetic Technologies Corporation

Panel Discussion
Moderated by Sarah Haecker Meeks, PhD

Networking Reception and Refreshments

Supporting Standards Development for Regenerative Medicine Therapies

6:30 pm – 9:00 pm
Room: Salon A-5

Sponsored by the Standards Coordinating Body and Nexight Group

This workshop, part of a larger project funded by the FDA, offers stakeholders in the regenerative medicine (RM) field with an opportunity to provide input into the development of processes and tools to be used in improving the identification, prioritization and feasibility of standards needs for RM therapies.

Reconnection and Mentoring Event

7:30 pm - 9:30 pm
Room: Stevens Salon B

Sponsored by Biogen

Biogen 40 YEARS

Trainee Lounge

7:00 am – 5:15 pm

Room: Mobley

The Trainee Lounge is reserved for Students and Trainees as a designated spot to network, grab a refreshing snack and beverage and meet other students and trainees. Members and Non-members are welcome!

Industry Symposium 2

7:00 am – 8:00 am

Room: Waldorf

Assessing Neurodevelopment in Neurodegenerative Diseases: The Use and Interpretation of Cognitive Scales

Sponsored by Abeona Therapeutics

7:00 am - 7:25 am
Use and interpretation of cognitive scales in neurodegenerative diseases

7:25 am - 7:45 am
Fireside chat with Maria Escolar, moderated by Barry Byrne

7:45 am – 8:00 am
Audience Q&A

SPEAKERS IN THIS SYMPOSIUM:

Barry Byrne, MD, PhD
Maria Escolar, MD
Tim Miller, PhD

Scientific Symposium 200

8:00 am - 10:00 am

Room: International Ballroom South

CARs: Getting Your License, New CAR Models, and Commercialization - Organized by the Bio Industry Liaison Committee

CO-CHAIRS: Mark L. Bonyhadi, PhD and Gwendolyn K. Binder-Scholl, PhD

SPEAKERS

8:00 am – 8:30 am
Carl H. June, MD. University of Pennsylvania, Philadelphia, PA
History of CAR Approval and Next Generation CARs

8:30 am – 9:00 am
Richard A. Morgan, PhD. bluebird bio, Cambridge, MA
New and Improved CARs

9:00 am – 9:30 am
TBD
Commercializing CAR Therapy

9:30 am – 10:00 am
Panel Discussion

Scientific Symposium 201

8:00 am - 10:00 am
Room: International Ballroom North

Clinical Trials Spotlight I

CO-CHAIRS: Stephen J. Russell, MD, PhD and Marcela V. Maus, MD, PhD

SPEAKERS

8:00 am – 8:20 am
Donald B. Kohn, MD. University of California, Los Angeles, Los Angeles, CA
340: Gene Therapy for X-Linked Chronic Granulomatous Disease

8:20 am – 8:40 am
J.Y. Tang. Stanford University, Stanford, CA
341: Phase I / II Clinical Trial for Recessive Dystrophic Epidermolysis Bullosa Using EB-101 (COL7A1 Gene-Corrected Autologous Keratinocytes)

8:40 am – 9:00 am
Prasad S. Adusumilli, MD. Memorial Sloan Kettering Cancer Center, New York, NY
342: A Phase I Clinical Trial of Malignant Pleural Disease Treated with Regionally Delivered Autologous Mesothelin-Targeted CAR T Cells: Safety and Efficacy - A Preliminary Report

9:00 am – 9:20 am
Carlos A. Ramos, MD. Baylor College of Medicine, Houston, TX
343: Autologous Tgfβ-Resistant HPV-16/18 E6/E7-Specific T Lymphocytes with or without Lymphodepletion for the Treatment of HPV-Associated Cancers

9:20 am – 9:40 am
Asha Das. Tocagen Inc. San Diego, CA
344: Treatment of Recurrent HGG Patients with the Retroviral Replicating Vector Toca 511 and Toca FC Gives Durable Responses and Survival Lasting 3 Years or Longer: Immune Mechanisms and Molecular Analyses of Tumors

9:40 am – 10:00 am
Evanthia Galanis, MD, DSc. Mayo Clinic, Rochester, MN
345: Phase I/II Trial Oof Intratumoral and Resection Cavity Administration of an Edmonston Oncolytic Measles Virus (MV) Derivative Expressing the Human Carcinoembryonic Antigen (CEA) in Patients with Recurrent Glioblastoma

Scientific Symposium 202

8:00 am - 10:00 am
Room: Salon A-3, 4

Do Current Pricing Strategies Assure Fair Access to the Values of Gene-based Therapies? - Organized by the Ethics and Government Relations Committees

CO-CHAIRS: Rachel Salzman, DVM and Theodore Friedmann, MD

SPEAKERS

8:00 am – 8:15 am
Eric Auger, MA. Putnam Associates, Boston, MA
Economic and Commercial Bases for Pricing of Genetic Therapies

8:15 am – 8:30 am
TBD

8:30 am – 8:45 am
Maria-Grazia Roncarolo, MD. Stanford University School of Medicine, Stanford, CA
Development and Marketing of Gene Therapies for Rare Genetic Disease

8:45 am – 9:00 am
Carl H. June, MD. University of Pennsylvania, Philadelphia, PA
Sticker Shock: How to Purchase a CAR

9:00 am – 9:15 am
Wendy White, Wendy White Consulting, Oak Park, IL
Patient Issues and Opportunities for Fair Access to Gene-based Therapies in Rare Disease

9:15 am – 10:00 am
Panel Discussion

Scientific Symposium 203

8:00 am - 10:00 am
Room: Salon A-1/2

Applications of Disease-specific Delivery Systems - Organized by the Nanoagents & Synthetic Formulations Committee

CO-CHAIRS: Chantal Pichon, PhD and Rajagopal Ramesh, PhD

SPEAKERS

8:00 am – 8:30 am
Angela K. Pannier, PhD. University of Nebraska-Lincoln, Lincoln, NE
Design and Assembly of Nano-Microparticles for Oral Gene Delivery Applications, including DNA Vaccination and Treatment of Gastrointestinal Tract Diseases

8:30 am – 9:00 am
Christopher M. Jewell, PhD. University of Maryland, College Park, MD, Department of Veterans Affairs, VA Baltimore Medical Center, Baltimore, MD
Harnessing Biomaterials to Control the Local Lymph Node Environment for Immunotherapy

9:00 am – 9:30 am
Daniel J. Siegwart, PhD. UT Southwestern Medical Center, Dallas, TX
Design of Non-viral Delivery Carriers for CRISPR/Cas Gene Editing

9:30 am – 10:00 am
Niren Murthy, PhD. UC Berkeley, Berkeley, CA
Gold Nanoparticles for CRISPR-Cas9 Delivery

Scientific Symposium 204

8:00 am - 10:00 am
Room: Continental Ballroom ABC

Novel Delivery Agents for the Achievement of Neuroprotection in the Brain, the Eye and the Ear - Organized by the Neurologic & Ophthalmic Gene & Cell Therapy Committee

CO-CHAIRS: Steven J. Gray, PhD and Stephen G. Kaler, MD

SPEAKERS

8:00 am – 8:30 am
David V. Schaffer, PhD. University of California Berkeley, Berkeley, CA
New AAVs for Central Nervous System Targeting

8:30 am – 9:00 am
Casey A. Maguire, PhD. Massachusetts General Hospital, Charlestown, MA
New AAVs for Sensory Organ Targeting

9:00 am – 9:30 am
Richard J. Price, PhD. University of Virginia, Charlottesville, VA
MR Image-Guided Transfection of the CNS with Focused Ultrasound and Brain-Penetrating Nanoparticles

9:30 am – 10:00 am
Muna I. Naash, PhD. University of Houston, Houston, TX
Developing Non-viral/Nanoparticle Gene Delivery for Retinal Degeneration

Scientific Symposium 205

8:00 am - 10:00 am
Room: Salon A-5

Novel Treatment Approaches for Respiratory and Gastrointestinal Diseases Organized by the Respiratory & GI Tract Committee

CO-CHAIRS: Katherine J. Excoffon, PhD and Liudmila Cebotaru, RN, MD, JD, LLM

SPEAKERS

8:00 am – 8:30 am
Joseph Zabner, MD. University of Iowa, Iowa City, IA
AAV Mediated Gene Transfer to Airway Stem Cells

8:30 am – 9:00 am
Frank McKeon, PhD. The University of Houston, Houston, TX
Regenerative Strategies for Chronic Lung Conditions

9:00 am – 9:30 am
Liudmila Cebotaru, RN, MD, JD, LLM. Johns Hopkins University, Baltimore, MD
A Preclinical Study in Rhesus Macaques for Cystic Fibrosis to Assess Gene Transfer and Transduction by AAV1 and AAV5 with a Dual-Luciferase Reporter System

9:30 am – 10:00 am
Mark Donowitz, MD. Johns Hopkins Un SOM, Baltimore, MD
Human mini-guts

Exhibit Hall Coffee Social – with Oral Poster Session

10:00 am - 10:45 am
Room: Stevens Salon C & D

Oral Poster Session

CO-CHAIRS: Luca Biasco, PhD and Martin J. Hicks, PhD

SPEAKERS

10:00 am – 10:15 am
Jerusha Naidoo, PhD. University of California San Francisco, San Francisco, CA
Widespread Transduction and Spread of a Modified AAV2 Capsid in the Non Human Primate Central Nervous System

10:15 am – 10:30 am
Dan Wang, PhD. University of Massachusetts Medical School, Worcester, MA
Bi-Allelic cleavage by Cas9 Causes Translocation between Homologous Chromosomes -Therapeutic Opportunities and Mechanisms in Non-Dividing and Dividing Cells

10:30 am – 10:45 am
Liujiang Song, PhD. University of North Carolina, Chapel Hill, NC
AAV Gene Therapy in a Canine Model of MPS1 Prevents and Reverses Corneal Blindness

Plenary Session 210

10:45 am - 12:00 pm
Room: International Ballroom North & South

George Stamatoyannopoulos Lecture & Presentation of the Excellence in Research Awards & Sonia Skarlatos Public Service Award Presentation

Sponsored by Biogen

CHAIR: Helen E. Heslop, MD

SPEAKER

Katherine A. High, MD. Spark Therapeutics, Philadelphia, PA
Turning Genes into Medicines: Therapeutics for the New Millenium

Industry Symposium 220

12:00 pm - 1:30 pm
Room: Waldorf

The Latest Advances in Cell Therapy Technologies

Sponsored by Miltenyi Biotec Inc.

12:00 pm – 12:10 pm
CHAIR: Boro Dropulic, PhD

SPEAKERS

12:10 pm – 12:35 pm
Els Verhoeyen, PhD. INSERM, Lyon, France
Alternative Lentiviral pseudotypes allow novel HSC, T and B cell based cell therapies

12:35 pm – 1:00 pm
Alan Smith, PhD. Bellicum Pharmaceuticals, Houston, TX
Overcoming the Challenges in Developing Gene-Modified T Cell Products

1:00 pm – 1:25 pm
Boro Dropulic, PhD. Lentigen Technology Inc., Gaithersburg, MD
Automated and Decentralized Manufacturing of CAR-T cells

Break

12:00 pm - 1:30 pm

Lunch Break *(On Own - Not Provided)*

Trainee Lunch Session

12:00 pm – 1:30 pm

Room: Mobley

Mock Review

Sponsored by Juno Therapeutics

Paula Cannon, PhD – **Mock Reviewer**
Kevin Morris, PhD – **Mock Reviewer**
Cary Harding, PhD – **Introduction to Grant Writing**
Jill Morris, PhD – **Chair Person**
Kee Pyon, PhD – **Program Officer**

Plenary Session 230

1:30 pm - 3:00 pm

Room: International Ballroom North & South

Outstanding New Investigator Symposium

Sponsored by Burroughs Wellcome Fund (BWF)

CHAIR: Michele P. Calos, PhD

SPEAKERS

1:30 pm – 2:15 pm
Luca Biasco, PhD. Dana-Farber/Boston Children's Cancer and Blood Disorders Center, Boston, MA
Clonal Tracking of Genetically Engineered Hematopoietic Cells in Humans

2:15 pm – 3:00 pm
Luk H. Vandenberghe, PhD. Grousbeck Gene Therapy Center, Boston, MA
On the Shoulders of Giants: Building Vectors and Gene Therapies

Exhibit Hall Coffee Social

3:00 pm - 3:45 pm
Room: Stevens Salon C & D

Oral Abstract Session 240

3:45 pm - 5:15 pm
Room: International Ballroom North

Hematologic & Immunologic Diseases III, Cancer-Targeted Gene & Cell Therapy II

CO-CHAIRS: Alessandra Biffi, MD and Richard G. Vile, PhD

PRESENTERS

3:45 pm – 4:00 pm

346: Towards Breaking Immune Tolerance of Tumors by Transplantation of Hematopoietic Stem and Progenitor Cells Engineered to Express Interferons in Their Tumor-Infiltrating Myeloid Progeny
Adele Mucci, San Raffaele Telethon Institute for Gene Therapy (SR-Tiget), Milan, Italy

4:00 pm – 4:15 pm

347: Gene Therapy for Chronic Eosinophilic Leukemia
Odelya E. Pagovich, Weill Cornell Medical College, New York, NY

4:15 pm – 4:30 pm

348: Toward a Hematopoietic Stem Cell-Based Prophylactic Immuno-Gene Therapy Approach for Cancer
Chang Li, University of Washington, Seattle, WA

4:30 pm – 4:45 pm

349: Lentiviral Gene Therapy for Severe Wiskott-Aldrich Syndrome - Longer Term Follow Up London Experience in 6 Patients
Claire Booth, UCL GOS Institute of Child Health, London, United Kingdom

4:45 pm – 5:00 pm

350: SPK-8011: Preliminary Results from a Phase 1/2 Trial of Investigational Gene Therapy for Hemophilia Confirm Transgene Derived Increases in FVIII Activity That Are Persistent and Stable beyond Eight Months
Xavier M. Anguela, Spark Therapeutics, Philadelphia, PA

5:00 pm – 5:15 pm

351: Human Mesenchymal Stem Cells Genetically Engineered to Express Alpha-1 Anti-Trypsin (apceth-201) Confer a Long-Term Survival Benefit in a Lethal, Haplo-Identical Mouse Model of Graft-Vs-Host-Disease
Ulf Geumann, apceth Biopharma GmbH, Munich, Germany

Oral Abstract Session 241

3:45 pm - 5:15 pm

Room: International Ballroom South

Musculo-Skeletal Diseases II

CO-CHAIRS: Renzhi Han, PhD and Qi Long Lu, MD, PhD

PRESENTERS

3:45 pm – 4:00 pm

352: Progressive Myopathy in a New Mouse Model of Facioscapulohumeral Muscular Dystrophy (FSHD) Facilitates Development of Targeted Molecular Therapies

Carlee R. Giesige, Research Institute at Nationwide Childrens Hospital, Columbus, OH

4:00 pm – 4:15 pm

353: Pre-Clinical Data and a Planned Phase One Human Trial of AAV.IL-1Ra-Mediated Gene Therapy for Osteoarthritis

Christopher Evans, Mayo Clinic, Rochester, MN

4:15 pm – 4:30 pm

354: BB-301: A Single "Silence and Replace" AAV-Based Vector for the Treatment of Oculopharyngeal Muscular Dystrophy (OPMD)

D. Suhy, Benitec Biopharma, Hayward, CA

4:30 pm – 4:45 pm

355: Identification of Novel AAV Capsids for Skeletal Muscle Gene Transfer by *In Vivo* Selection in Humanized Mice

Jennifer C. Gifford, University of Massachusetts Medical School, Worcester, MA

4:45 pm – 5:00 pm

356: AAV Therapy Attenuates Respiratory Dysfunction in a Novel Rat Model of Pompe Disease

Bhuvna Mahajan, University of Florida, Gainesville, FL

5:00 pm – 5:15 pm

357: Systemic Dose Escalation Study of Alpha-Sarcoglycan Provides Functional Improvement in *SGCA*$^{-/-}$ Mouse Model of LGMD2D

Danielle Griffin, The Research Institute at Nationwide Childrens Hospital, Columbus, OH

Oral Abstract Session 242

3:45 pm - 5:15 pm
Room: Continental Ballroom ABC

Gene Editing, Gene Delivery and Vector Design

CO-CHAIRS: Johannes C.M. van Der Loo, PhD and J. Fraser Wright, PhD

PRESENTERS

3:45 pm – 4:00 pm

358: Probing Capsid Mosaics Formation in Adeno-Associated Virus Library Preparations
Simon Pacouret, Grousbeck Gene Therapy Center, Schepens Eye Research Institute, Massachusetts Eye and Ear Infirmary, Harvard Medical School, Boston, MA

4:00 pm – 4:15 pm

359: Heterogeneic Genome Encapsidation of rAAV-CRISPR/Cas9 Vectors Underscores Potential Limitations for Promising *In Vivo* Gene-Editing Platforms
Phillip W. L. Tai, University of Massachusetts, Medical School, Worcester, MA

4:15 pm – 4:30 pm

360: Deep Search: Next-Gen Strategies for Accelerating AAV Capsid Engineering
Eric Kelsic, Harvard Medical School, Boston, MA

4:30 pm – 4:45 pm

361: Compositional Control of pDNA/lPEI Nanoparticles Using Flash Nanocomplexation to Improve *In Vivo* Transfection Efficiency and Biocompatibility
Yizong Hu, Johns Hopkins University School of Medicine, Baltimore, MD

4:45 pm – 5:00 pm

362: CRISPR/Cas9-Based Whole Genome Screenings of Packaging Cells to Identify Cellular Factors for Enhanced rAAV Production
Alexander Brown, University of Massachusetts Medical School, Worcester, MA

5:00 pm – 5:15 pm

363: A High-Throughput Method of Constructing and Screening Short Synthetic Gene Regulatory Elements
Elizabeth Ferrick-Kiddie, Oregon Health & Science University, Portland, OR

Oral Abstract Session 243

3:45 pm - 5:15 pm
Room: Salon A-1/2

Preclinical Approaches in Gene Therapy for Neurosensory Disorders

CO-CHAIRS: Jean Bennett, MD, PhD and Miguel Sena-Esteves, PhD

PRESENTERS

3:45 pm – 4:00 pm

364: Allele-Specific Deafness Gene Disruption through Discrimination of a Single Base Change by *S. aureus*Cas9^{KKH} Prevents Progressive Hearing Loss after AAV Mediated Gene Delivery
Bence Gyorgy, Harvard Medical School, Boston, MA

4:00 pm – 4:15 pm

365: A Single Neonatal Injection of PHP.B-AAV9-*Clrn1* Rescues Hearing in a Model of Usher Syndrome Type IIIa

Bence Gyorgy, Harvard Medical School, Boston, MA

4:15 pm – 4:30 pm

366: Peripheral Gene Therapeutic Rescue of an Olfactory Ciliopathy Induces Central Neural Plasticity and Restores Odor Guided Behavior

Cedric R. Uytingco, University of Florida, College of Medicine, Gainesville, FL

4:30 pm – 4:45 pm

367: Gene Augmentation Therapy in a Large Animal Model of *PDE6A*-Retinitis Pigmentosa Rescues Rod Function and Promotes Cone Survival Long-Term

Laurence M. Occelli, VMC Michigan State University, East Lansing, MI

4:45 pm – 5:00 pm

368: Effect of Inducible VEGF-Trap Expression on CNV Formation in a Murine Model of Wet AMD Following Intravitreal Administration of a Capsid Mutant rAAV-Riboswitch Vector

Chris A. Reid, Medical College of Wisconsin, Milwaukee, WI

5:00 pm – 5:15 pm

369: Hypoxia-Regulated, Cell-Specific Angiostatic Gene Therapy for Choroidal Neovascularization (CNV)

Manas R. Biswal, University of Florida College of Medicine, Gainesville, FL

Oral Abstract Session 244

3:45 pm - 5:15 pm

Room: Salon A-3, 4

Immune Cell Therapies

CO-CHAIRS: Gwendolyn K. Binder-Scholl, PhD and Justin Eyquem, PhD

PRESENTERS

3:45 pm – 4:00 pm

370: Restraining Macrophages Alleviates CAR T Cell-Induced Cytokine Release Syndrome and Informs Novel Therapeutic Interventions

Theodoros Giavridis, MSKCC, New York, NY

4:00 pm – 4:15 pm

371: Development of an Allogeneic Nkt Cell Platform for Off-The-Shelf Cancer Immunotherapy

Jingling Jin, Baylor College of Medicine, Houston, TX

4:15 pm – 4:30 pm

372: A Phase I Clinical Trial with *Ex Vivo* Expanded Recipient Regulatory T Cells in Living Donor Kidney Transplants

Jessica H. Voss, Northwestern Feinberg School of Medicine, Chicago, IL

4:30 pm – 4:45 pm

373: Control of Human T-Cell Expansion by Chemically-Induced Signal Complexes

Li-Jie Wang, Seattle Children's Research Institute, Seattle, WA

4:45 pm – 5:00 pm

374: T Rapa Cells as Vehicles for Delivery of Therapeutic Cargo
Murtaza S. Nagree, University of Toronto, Toronto, ON, Canada

5:00 pm – 5:15 pm

375: Scalable Generation of iPSC-Derived Macrophages Displaying *In Vivo* Plasticity
Mania Ackermann, Hannover Medical School (MHH), Hannover, Germany

Oral Abstract Session 245

3:45 pm - 5:15 pm
Room: Salon A-5

Therapeutic Intervention Using Tissue Directed Non-Viral Gene Transfer

CO-CHAIRS: Richard Heller, PhD and Chantal Pichon, PhD

PRESENTERS

3:45 pm – 4:00 pm

376: Evaluating *In Vivo* Electroporation as a Method of Gene Transfer for Cardiac Gene Therapy
Jacob Hoffman, Northwestern University, Chicago, IL

4:00 pm – 4:15 pm

377: Targeted Homology Directed Repair in Blood Stem and Progenitor Cells with Highly Potent CRISPR Nanoformulations
Reza Shahbazi, Fred Hutchinson Cancer Research Center, Seattle, WA

4:15 pm – 4:30 pm

378: Kidney-Directed Hydrodynamic Injection of *Slc3a1 piggyBac* Transposon Lowers Urinary Cystine in a Mouse Model of Cystinuria Type I
Lauren E. Woodard, Department of Veterans Affairs, Nashville, TN

4:30 pm – 4:45 pm

379: Amphiphilic Triblock Copolymer Promotes Delivery of a Mini-Dystrophin Plasmid DNA in Skeletal Muscles of *Mdx* Rodents Following Hydrodynamic Limb Vein Injection
Yann Le Guen, INSERM, Brest, France

4:45 pm – 5:00 pm

380: Rescue of Coat Color Phenotypes in Mutant Mice Using the Gonad Method
Masato Ohtsuka, Tokai University, Isehara, Japan

5:00 pm – 5:15 pm

381: Intramuscular Electroporation of DNA-Based Monoclonal Antibodies in Sheep: A Translational Model for Clinical Antibody Gene Transfer
Kevin Hollevoet, KU Leuven - University of Leuven, Leuven, Belgium

Exhibit Hall Networking Reception & Poster Session II – including Tools and Technologies Forum

5:15 pm - 7:30 pm

Room: Stevens Salon C & D

Sponsored by YPOSKESI YPOSKESI

Tools and Technologies Forum

CO-CHAIRS: Nicole Faust, PhD and Janet Benson, PhD, DABT

SPEAKERS

5:15 pm – 5:30 pm
Alaina C. Schlinker, PhD. Fresenius Kabi USA LLC, Lake Zurich, IL
Immunomagnetic Selection Preparation Using the Lovo Cell Processing System

5:30 pm – 5:45 pm
Duncan Griffiths. Malvern, Westborough, MA
Advances in Particle Titer and Formulation Stability Characterization Tools

5:45 pm – 6:00 pm
Marco Schmeer, PhD. PlasmidFactory GmbH & Co. KG, Bielefeld, Germany
Minicircles for AAV and CAR-T

6:00 pm – 6:15 pm
Juan C. Ramirez, PhD. VIVEbiotech, Donostia-San Sebastián, Spain
Lentiviral Vectors: Safety and Manufacturing Related Aspects

6:15 pm – 6:30 pm
Ken Rando. BioSpherix, Ltd., Parish, NY
Cost Effective Scale-Up/Scale-Out Gene Therapy Manufacturing

6:30 pm – 6:45 pm
Yonatan Levinson, MSc. Lonza, Walkersville, MD
Bioreactors for Cell Therapy Products: from Lab to Production Plant

6:45 pm – 7:00 pm
Jonathan Mitchell, PhD. Imanis Life Sciences, Rochester, MN
***In Vivo*. *Ex Vivo*. One Translational Technology for Tracking Gene Therapies**

7:00 pm – 7:15 pm
Sandra Merino. Alfa Wassermann BV, Woerden, Netherlands
Ultracentrifugation Applications in Gene and Cell Therapy Research

7:15 pm – 7:30 pm
William Tan, PhD. GenScript Synthetic Biology Services, Piscataway, NJ
Synthetic DNA Applications Beyond Molecular Cloning

New Member Reception

6:00 pm – 7:00 pm
Room: Stevens Salon B
Sponsored by AveXis

Industry Symposium 250

7:30 pm - 9:30 pm
Room: Waldorf

Advances with AAV Gene Therapy

Sponsored by Spark Therapeutics

Topic 1: **Overcoming Immune Response to AAV** (Federico Mingozzi, PhD)
Topic 2: **Gene Therapy for Pompe Disease** (Sean Armour, PhD)
Topic 3: **Regulatory review and approval for Luxturna, the first gene therapy treating a genetic disease** (Jim Wang, PhD, MBA)

Trainee Lounge

7:00 am – 5:45 pm

Room: Mobley

The Trainee Lounge is reserved for Students and Trainees as a designated spot to network, grab a refreshing snack and beverage and meet other students and trainees. Members and Non-members are welcome!

Industry Symposium 3

7:00 am – 8:30 am

Room: Waldorf

Rare Inherited Disorders of Pediatric Neurotransmitter Synthesis – Insights into Diagnosis and Clinical Experience

Sponsored by Agilis Biotherapeutics

agilis™

7:00 am – 7:05 am
Paul Hwu, MD, PhD
Welcome and Introduction

7:05 am – 7:25 am
Toni Pearson, MD
Clinical Features of Dopamine/Serotonin Related Diseases

7:25 am – 7:45 am
Keith Hyland, PhD
Diagnostic Interventions for Neurotransmitter Disorders

7:45 am – 8:05 am
Paul Hwu, MD, PhD
The Natural History of Aromatic L-Amino Acid Decarboxylase Deficiency

8:05 am – 8:20 am
Lisa Flint, Managing Director
AADC D Patient Journey Experience

8:20 am – 8:30 am
Q&A
MODERATOR: Paul Hwu, PhD

Scientific Symposium 300

8:00 am - 10:00 am
Room: International Ballroom South

Tipping Point: Gene Therapy Entering the Market - Organized by the Clinical Trials and Regulatory Affairs Committee

CO-CHAIRS: Lawrence S. Lamb, Jr., PhD and Marcela V. Maus, MD, PhD

SPEAKERS

8:00 am – 8:30 am
Bruce L. Levine, PhD. University of Pennsylvania, Philadelphia, PA
Regulatory and Technical Lessons Learned in Transfer of Gene Therapy Products from Academia to Industry

8:30 am – 9:00 am
Brian K. Kaspar, PhD. AveXis, Inc., Bannockburn, IL
Commercial Manufacturing Process/Licensure/Process/Validation Issues and Challenges in the Face of Promising Phase 1 data

9:00 am – 9:30 am
Stuart Bunting, PhD. BioMarin Pharmaceutical Inc., Novato, CA
HemA Program Preclinical Development FVIII Gene Therapy Translation Study

9:30 am – 10:00 am
Daniel Takefman, PhD. Spark Therapeutics, Inc., Philadelphia, PA
From Clinical Development to FDA Approval of LUXTURNA (voretigene neparvovec-rzyl): a Regulatory Perspective

Scientific Symposium 301

8:00 am - 10:00 am
Room: Continental Ballroom ABC

Gene and Cell Therapies for Neurometabolic Diseases - Organized by the Gene & Cell Therapy of Genetic and Metabolic Diseases Committee

CO-CHAIRS: Federico Mingozzi, PhD and Dwight Koeberl, MD, PhD

SPEAKERS

8:00 am – 8:30 am
Cary O. Harding, MD. OHSU, Portland, OR
Update on Phase I/II Clinical Trial for Ornithine Transcarbamylase (OTC) Deficiency Using AAV-mediated Gene Delivery

8:30 am – 9:00 am
Kevin M. Flanigan, MD. Nationwide Children's Hospital, Columbus, OH
Update on Phase I/II Gene Transfer Clinical Trial of Systemic Gene Transfer of scAAV9.U1a.hSGSH for MPSIIIA (Sanfilippo Syndrome)

9:00 am – 9:30 am
Steven J. Gray, PhD. University of Texas Southwestern Medical Center, Dallas, TX
Optimization of AAV-mediated MeCP2 Gene Transfer for the Treatment of Rett Syndrome

9:30 am – 10:00 am
Marc Tardieu, MD, PhD. Hopitaux universitaires Paris Sud, Le Kremlin-Bicetre, France
Update on Phase I/II Clinical Trial of Intracerebral Administration of rAAV2/5hNAGLU Vector in Children with MPSIIIB

Scientific Symposium 302

8:00 am - 10:00 am
Room: International Ballroom North

Advances in Genome Editing in HSCs - Organized by the Hematologic and Immunologic Gene and Cell Therapy Committee

CO-CHAIRS: David M. Markusic, PhD and Andre Larochelle, MD, PhD

SPEAKERS

8:00 am – 8:30 am
Tony Ho, MD. CRISPR Therapeutics, Cambridge, MA
Induction of HbF to Treat β-hemoglobinopathies- Bringing CRISPR to the Clinic

8:30 am – 9:00 am
Nancy Maizels, PhD. University of Washington, Seattle, WA
Gene Correction at Targeted DNA Nicks

9:00 am – 9:30 am
Paula Rio, PhD. CIEMAT, Madrid, Spain
Gene Editing in Fanconi Anemia Hematopoietic Stem and Progenitor Cells

9:30 am – 10:00 am
Suk See De Ravin, MD, PhD. National Institutes of Health, NIAID, Bethesda, MD
Gene Editing Approaches for Treatment of X-linked Chronic Granulomatous Disease

Scientific Symposium 303

8:00 am - 10:00 am
Room: Salon A-3, 4

From Multinational Trials Towards Global Gene Therapy Products: Approval Process, Manufacturing, Distribution, Follow-up and Ethics - Organized by the International Committee

CO-CHAIRS: Axel Schambach, MD, PhD and Evangelia Yannaki, MD

SPEAKERS

8:00 am – 8:30 am
Alexandria Petrusich. bluebird bio, Cambridge, MA
Thalassemia Clinical Trials

8:30 am – 9:00 am
Cliona M. Rooney, PhD. Baylor College of Medicine, Houston, TX
Working with Industry to Develop Cellular Therapies for Cancer

9:00 am – 9:30 am
Alessandro Aiuti, MD, PhD. San Raffaele Telethon Institute for Gene Therapy (SR-TIGET), San Raffaele Scientific Institute, Milan, Italy, Pediatric Immunohematology and Bone Marrow Transplantion Unit, San Raffaele Scientific Institute, Milan, Italy
Gene Therapy for ADA-SCID Patients: Strimvelis™ as a Successful Model for the Development of Advanced Therapies for Ultra-rare Diseases

9:30 am – 10:00 am
Jonathan Kimmelman, PhD. McGill University, Montreal, QC, Canada
Ethics: from Clinical Trials to Products

Scientific Symposium 304

8:00 am - 10:00 am
Room: Salon A-1/2

New Directions and Clinical Trials for Muscle and Skeletal Disorders - Organized by the Musculo-Skeletal Gene & Cell Therapy Committee

CO-CHAIRS: Michele P. Calos, PhD and John T. Gray, PhD

SPEAKERS

8:00 am – 8:30 am
April Pyle, PhD. UCLA, Eli and Edythe Broad Stem Cell Center, Los Angeles, CA
CRISPR-Cas9 Deletion Strategy that Targets the Majority of DMD Patients in hiPSC-derived Muscle Cells

8:30 am – 9:00 am
Frank Barry, PhD. Regenerative Medicine Institute, National University of Ireland Galway, Ireland, University Health Network Toronto Western Hospital, Toronto, ON, Canada
Adipose Stromal Cell Therapy for Osteoarthritis of the Knee: Clinical Progress and Challenges

9:00 am – 9:30 am
John T. Gray, PhD. Audentes Therapeutics, Inc., San Francisco, CA
The ASPIRO Trial for X-Linked Myotubular Myopathy: Carefully Taking Systemic AAV Treatments to the Next Level

9:30 am – 10:00 am
Manuela Corti, PT, PhD. University of Florida, Gainesville, FL
Readministration of AAV vector in Pompe Disease

Scientific Symposium 305

8:00 am - 10:00 am
Room: Salon A-5

Next Generation RNA-targeted Therapeutics - Organized by the Oligonucleotide and RNAi Therapeutics Committee

CO-CHAIRS: Paloma H. Giangrande, PhD and Marcin Kortylewski, PhD

SPEAKERS

8:00 am – 8:30 am
Paolo Martini, PhD. Moderna, Cambridge, MA
Messenger RNA as a Treatment for Inherited Metabolic Disorders

8:30 am – 9:00 am
Samuel I. Gunderson, PhD. Rutgers University, Piscataway, NJ
Long-lasting Tumor Regression when Silencing KRAS in Human Pancreatic Cancer Xenografts with U1 Adaptor Oligonucleotide-peptide Conjugates

9:00 am – 9:30 am
Nagy A. Habib, MD, ChM, FRCS. Imperial College of London, London, United Kingdom
Short Activating RNAs. MTL-CEBPa has a Broad Range of Liver Disease Models and Encouraging Early Clinical Data in a Phase 1 Trial in HCC

9:30 am – 10:00 am
Anna M. Krichevsky, PhD. Brigham & Women's Hospital / HMS, Boston, MA
Targeting Onco-microRNA Dependence of Malignant Glioma by Gene Editing

Exhibit Hall Coffee Social – with Oral Poster Session

10:00 am - 10:45 am
Room: Stevens Salon C & D

Oral Poster Session

CO-CHAIRS: Luca Biasco, PhD and Martin J. Hicks, PhD

SPEAKERS

10:00 am – 10:15 am
Chun-Yu Chen, PhD. Seattle Children's Research Institute, Seattle, WA
Treatment of Hemophilia A Using Factor VIII Messenger RNA Lipid Nanoparticle

10:15 am – 10:30 am
Makan Khoshnejad. The Wistar Institute, Philadelphia, PA
Engineering of DNA-Encoded PCSK9 Monoclonal Antibodies as Novel Lipid-Lowering Therapeutics

10:30 am – 10:45 am
Rita Milazzo, PhD. San Raffaele Telethon Institute for Gene Therapy, Milan, Italy
Strategies to Enhance and Modulate Hematopoietic Stem Cell Contribution to Brain Myeloid Cell/Microglia Turnover

Plenary Session 310

10:45 am - 11:45 am
Room: International Ballroom North & South

Outstanding Achievement Award Lecture

Sponsored by Audentes Therapeutics

AUDENTES

CHAIR: Guangping Gao, PhD

SPEAKER

Jean Bennett, MD, PhD. University of Pennsylvania Scheie Eye Institute, Philadelphia, PA
Seeing the Light with Retinal Gene Therapy: From Fantasy to Reality

Industry Symposium 320

11:45 am - 1:15 pm
Room: Waldorf

Therapeutically-Relevant Approaches to Cellular Engineering

Sponsored by MaxCyte

MaxCyte®

Any Cell. Any Molecule. Any Scale.®

Late-Breaking Scientific Presentation by Dr. Matthew Cooper from Washington University in St. Louis

Keynote: TED-Style Talk by Dr. David DiGiusto from Stanford University

Break

11:45 am - 1:15 pm
Lunch Break *(On Own - Not Provided)*

Trainee Lunch Session

11:45 am – 1:15 pm
Room: Mobley

Industry Interactions & Managing IP

Sponsored by Spark Therapeutics

Spark THERAPEUTICS

Michael Moore, PhD - Northwestern University Tech Transfer Office

Federico Mingozzi, PhD - Spark Therapeutics

Presidential Symposium 330

1:15 pm - 3:15 pm

Room: International Ballroom North & South

Presidential Symposium & Presentation of the Top Abstracts

Sponsored by Oxford BioMedica

CHAIR: Helen E. Heslop, MD

SPEAKERS

1:20 pm – 2:15 pm
Nicholas Restifo, MD. National Cancer Institute, Bethesda, MD
Fundamentals of T cell-based Immunotherapy

Presentation of Top Abstracts

2:15 pm – 2:30 pm
Megan S. Keiser, PhD. The Children's Hospital of Philadelphia, Philadelphia, PA
Translating RNAi for Huntington's Disease: Intra-Putaminal Delivery of AAV2/1. Mihds1 for Comprehensive Dosing, Biodistribution, Silencing and Safety in a Non-Human Primate

2:30 pm – 2:45 pm
Adele Ricciardi. Yale University, New Haven CT
In Utero Gene Correction Mediated by PNA-Nanoparticles

2:45 pm – 3:00 pm
Juan A. Bueren, PhD. CIEMAT/CIBERER, Madrid, Spain
Engraftment and Phenotypic Correction of Hematopoietic Stem Cells in Non-Conditioned Fanconi Anemia Patients Treated with Ex Vivo Gene Therapy

3:00 pm – 3:15 pm
Olivier Humbert, PhD. Fred Hutchinson Cancer Research Center, Seattle, WA
Transplantation and Persistence of CRISPR/Cas9 -Edited Hematopoietic Stem and Progenitors Cells for the Reactivation of Fetal Hemoglobin in Nonhuman Primates

Exhibit Hall Coffee Social

3:15 pm - 4:00 pm
Room: Stevens Salon C & D

Oral Abstract Session 340

4:00 pm - 5:45 pm
Room: International Ballroom North

Innovations in CAR-T Therapies

CO-CHAIRS: Daniel J. Powell, Jr., PhD and David M. Spencer, PhD

PRESENTERS

4:00 pm – 4:15 pm
645: A Simple Protein-Based Method for Generation of 'Off the Shelf' Allogeneic Chimeric Antigen Receptor T-Cells
Paul M. Maciocia, UCL, London, United Kingdom

4:15 pm – 4:30 pm
646: Engineered Trans-Presentation of IL-15 Enhances Therapeutic Efficacy of GD2-Specific Car NKT Cells in a Xenogenic Model of Neuroblastoma
Bin Liu, Baylor College of Medicine, Houston, TX

4:30 pm – 4:45 pm
647: Targeting CD19-Negative Relapsed B-Acute Lymphoblastic Leukemia Using Trivalent CAR T Cells
Kristen Fousek, Baylor College of Medicine, Houston, TX

4:45 pm – 5:00 pm
648: Immunosuppressive Population of Tumor-Associated Macrophages Expresses FRβ and Can Be Depleted with Specific CAR T-Cells to Control Ovarian Cancer Progression
Alba Rodriguez-Garcia, University of Pennsylvania, Philadelphia, PA

5:00 pm – 5:15 pm
649: Enhancing the Potency and Specificity of Tumor-Directed T Cells
Juan F. Vera, Baylor College of Medicine, Houston, TX

5:15 pm – 5:30 pm
650: Optimization of CD28-Costimulated CARs for Increased Anti-Tumor Activity and Persistence
Sonia Guedan, University of Pennsylvania, Philadelphia, PA

5:30 pm – 5:45 pm
651: Adoptive Cell Therapy with April Trimer Chimeric Antigen Receptor Shows Increased Anti-Tumor Efficiency against Multiple Myeloma
Andrea Schmidts, Massachusetts General Hospital Cancer Center, Charlestown, MA

Oral Abstract Session 341

4:00 pm - 5:45 pm

Room: International Ballroom South

Cardiovascular and Pulmonary Diseases

CO-CHAIRS: Glen Banks, PhD and Xiao Xiao, PhD

PRESENTERS

4:00 pm – 4:15 pm

652: Gene Therapy Improves Lifespan and Cardiac Function in a Rat Model of Pompe Disease

Lauren S. Duncanson, University of Florida, Gainesville, FL

4:15 pm – 4:30 pm

653: AAV-Mediated *TAZ* Gene Replacement Restores Cardioskeletal Function and Improves Aberrant Proteomic Profiles in Barth Syndrome

Silveli Suzuki Hatano, University of Florida, Gainesville, FL

4:30 pm – 4:45 pm

656: Protease-Activatable Adeno-Associated Virus Vectors for Cardiac Disease Applications

Mitchell Brun, Rice University, Houston, TX

4:45 pm – 5:00 pm

144: CAR-Tregs to Treat Heart Disease

Robert D. Schwab, University of Pennsylvania, Philadelphia, PA

5:00pm – 5:15 pm

654: Second Generation Gene Therapy for α1-Antitrypsin Deficiency Using a Genetically Modified α1-Antitrypsin Transgene Resistant to Oxidation

Meredith L. Sosulski, Weill Cornell Medical College, New York, NY

5:15 pm – 5:30 pm

657: *In Vivo* Genome Editing of PCSK9 in Macaque Liver Leads to a Stable Reduction in Serum Cholesterol

Lili Wang, University of Pennsylvania, Philadelphia, PA

5:30 pm – 5:45 pm

658: Development and Optimization of a PCSK9-Specific Meganuclease That Mediates Long-Term LDL Reduction in Non-Human Primates

Janel Lape, Precision BioSciences, Durham, NC

Oral Abstract Session 342

4:00 pm - 5:45 pm

Room: Continental Ballroom ABC

Capsid Engineering

CO-CHAIRS: Aravind Asokan, PhD and Phillip WL Tai, PhD

PRESENTERS

4:00 pm – 4:15 pm

659: Characterization of a Protease-Activatable Adeno-Associated Virus Vector for Disease-Targeted Gene Delivery

Annicka Evans, Rice University, Houston, TX

4:15 pm – 4:30 pm

660: Engineering Cell Type Specific Delivery Vectors for Noninvasive Modulation of Brain Circuits and Behaviors

Nicholas Flytzanis, California Institute of Technology, Pasadena, CA

4:30 pm – 4:45 pm

661: Safety and Increased Transduction Efficiency in the Adult Nonhuman Primate Central Nervous System with Intravenous Delivery of Two Novel Adeno-Associated Virus Capsids

Dinah Sah, Voyager Therapeutics, Cambridge, MA

4:45 pm – 5:00 pm

662: Engineering an All-In-One Light Activatable Adeno-Associated Virus Vector for Tunable Gene Delivery

Esther J. Lee, Rice University, Houston, TX

5:00 pm – 5:15 pm

663: Characterization of a Novel Serine/Threonine Motif in the N-Terminal Region of Adeno-Associated Virus

Jessica Tong, Rice University, Houston, TX

5:15 pm – 5:30 pm

664: Engineering Adeno-Associated Virus with a Self-Peptide for Immune-Avoidance

Tawana M. Robinson, Rice University, Houston, TX

5:30 pm – 5:45 pm

665: Harnessing the Activatable Peptide Display Behavior of Adeno-Associated Virus for Delivery of Peptides

Nicole N. Thadani, Rice University, Houston, TX

Oral Abstract Session 343

4:00 pm - 5:45 pm

Room: Salon A-1/2

Preclinical Approaches in Gene transfer to the Central Nervous System

CO-CHAIRS: Shannon E. Boye, PhD and TBD

PRESENTERS

4:00 pm – 4:15 pm

666: Rett Syndrome Gene Therapy Improves Survival and Ameliorates Behavioral Phenotypes in MeCP2 Null

Brian K. Kaspar, AveXis, Inc., Bannockburn, IL

4:15 pm – 4:30 pm

667: From Bench to Bedside: Gene Therapy for Batten (CLN6) Disease

Shibi Likhite, Nationwide Children's Hospital, Columbus, OH

4:30 pm – 4:45 pm

668: A Novel Brain-Directed Hematopoietic Stem Cell (HSC) Gene Therapy Approach Provides Unique Therapeutic Benefit to the Mouse Model of Infantile Neuronal Ceroid Lipofuscinosis (CLN1)

Marco Peviani, Dana-Farber/Boston Children's Cancer and Blood Disorders Center, Boston, MA

4:45 pm – 5:00 pm

669: AAV-Mediated CYP46A1 Gene Therapy as a Strategy to Counteract Huntington's Disease

Nathalie Cartier, INSERM/CEA UMR1169, MIRCEN CEA and Université Paris-Sud, Université Paris Saclay, Fontenay aux Roses, France

5:00 pm – 5:15 pm

670: Virally-Mediated Dopamine Autoreceptor Expression Blocks Levodopa-Induced Dyskinesia Development by Inhibiting False Neurotransmission of Serotonin Neurons

Rhyomi C. Sellnow, Michigan State University, Grand Rapids, MI

5:15 pm – 5:30 pm

671: Preclinical Development of Gene Therapy for Niemann Pick Disease Type A

Lluis Samaranch, UCSF, San Francisco, CA

5:30 pm – 5:45 pm

672: Rescue of Central and Peripheral Neurological Phenotype in a Mouse Model of Friedreich's Ataxia by Intravenous Delivery of AAV Frataxin with a Novel Capsid

Martin Goulet, Voyager Therapeutics, Cambridge, MA

Oral Abstract Session 344

4:00 pm - 5:45 pm

Room: Salon A-3, 4

Metabolic, Storage, Endocrine, Liver and Gastrointestinal Diseases

CO-CHAIRS: Dwight Koeberl, MD, PhD and Charles Venditti, MD, PhD

PRESENTERS

4:00 pm – 4:15 pm

673: Engineering the Hematopoietic System for Lysosomal Storage Disorders: Correction of Mucopolysaccharidosis Type I Using Genome-Edited, Human Hematopoietic Stem and Progenitor Cells

Natalia Gomez-Ospina, Stanford, Stanford, CA

4:15 pm – 4:30 pm

674: Efficacy and Safety of Long-Term Prophylaxis in Severe Hemophilia A Dogs Following Liver Gene Therapy Using AAV Vectors: A 10 Year Follow-Up Report

Denise E. Sabatino, The Children's Hospital of Philadelphia, Philadelphia, PA

4:30 pm – 4:45 pm

675: A Phase 1 / 2 Clinical Trial of Systemic Gene Transfer of rAAV9.CMV. hNAGLU for MPS IIIB: Safety, Tolerability, and Preliminary Evidence of Biopotency

Kevin M. Flanigan, Nationwide Children's Hospital, Columbus, OH

4:45 pm – 5:00 pm

676: Correction of Advanced Pompe Phenotype in Mice with AAV Liver Gene Transfer of Secretable GAA

Francesco Puzzo, Genethon, Evry, France

5:00 pm – 5:15 pm

677: Molecular Characterization of Hematopoietic System Reconstitution in Metachromatic Leukodystrophy Patients Following Hematopoietic Stem Cell Gene Therapy

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

5:15 pm – 5:30 pm

678: Therapeutic Effect of Neonatal Gene Transfer and Bone Marrow Transplantation in Neuronopathic Gaucher Disease

Dao Pan, Cincinnati Children's Hospital Medical Center, Cincinnati, OH

5:30 pm – 5:45 pm

679: Isolation of an Adeno-Associated Virus 8 Variant with Improved Properties for Liver Gene Therapy

Qiang Wang, University of Pennsylvania, Philadelphia, PA

Oral Abstract Session 345

4:00 pm - 5:45 pm

Room: Salon A-5

Preclinical Pharmacology and Toxicology Studies and Assessment of Gene Therapy in Large Animal Models

CO-CHAIRS: Kevin G. Rice, PhD and Gabor Veres, PhD

PRESENTERS

4:00 pm – 4:15 pm

680: Evaluation of Tolerability and Immunogenicity of EDIT-101 Following Subretinal Injection in Non-Human Primate

Haiyan Jiang, Editas Medicine, Cambridge, MA

4:15 pm – 4:30 pm

681: Aberrant Clonal Hematopoiesis of the Erythroid and Myeloid Lineages in a Lentivirally Barcoded Rhesus Macaque

Diego A. Espinoza, Perelman School of Medicine, University of Pennsylvania, Philadelphia, PA

4:30 pm – 4:45 pm

682: Long-Term Efficacy and Safety Evaluation of the Administration of AAV9-Sulfamidase to the CSF of Dogs: 5-Year-Follow Up

Sara Marcó, Universitat Autònoma de Barcelona, Bellaterra, Spain

4:45 pm – 5:00 pm

683: AAV-Associated Axonopathy of the Spinal Cord and Peripheral Nerves in Laboratory Animals: a Retrospective and Prospective Study

Elizabeth L. Buza, University of Pennsylvania, Philadelphia, PA

5:00pm – 5:15 pm

684: pH-Sensitive Multifunctional Lipid ECO Plasmid DNA Nanoparticles as Efficient Non-Viral Gene Therapy for Stargardt's Disease

Da Sun, Case Western Reserve University, Cleveland, OH

5:15 pm – 5:30 pm

685: Assessments of Liver-Targeted Hydrodynamic Gene Delivery in Hemophiliac Dogs

Kenya Kamimura, Niigata University, Niigata, Japan

5:30 pm – 5:45 pm

686: A Minimally Invasive Procedure for Ultrasound-Mediated Nonviral Gene Delivery to Liver in a Porcine Model

Dominic M. Tran, Seattle Children's Research Institute, Seattle, WA

Exhibit Hall Networking Reception & Poster Session III – including Tools and Technologies Forum

5:45 pm - 7:45 pm

Room: Stevens Salon C & D

Tools and Technologies Forum

CO-CHAIRS: S. Kaye Spratt, PhD and TBD

SPEAKERS

5:45 pm – 6:00 pm

Klaus Kühlcke, PhD. BioNTech Innovative Manufacturing Services GmbH, Idar-Oberstein, Germany

GMP Manufacturing of Transiently Produced Gamma Retroviral Vectors

6:00 pm – 6:15 pm

Stephen D. Rodriguez. VGXI Inc., The Woodlands, TX

Overcoming the Challenges of Large Scale Production of Viral Vector Plasmids

6:15 pm – 6:30 pm

Marian McKee, PhD. MilliporeSigma, Billerica, MA

Improving Accuracy: Droplet Digital PCR for Quantitation of Viral Vectors Used in Cell and Gene Therapy

6:30 pm – 6:45 pm

Haifeng Chen, PhD. Virovek, Inc., Hayward, CA

Three-Phase Partitioning Coupled with Density Gradient Centrifugation for Large Scale AAV Purification

6:45 pm – 7:00 pm

Andy Kokaji, PhD. STEMCELL Technologies, Vancouver, BC, Canada

Scientists Helping Scientists: Tools and Reagents for your Cell Therapy Research

7:00 pm – 7:15 pm

Ken Hoffman. Cygnus Technologies, LLC, Southport, NC

Bioprocess Impurity Analysis in the Developing Fields of Cell & Gene Therapy

7:15 pm – 7:30 pm

Yoshinari Miyata, PhD. PhoenixBio USA Corporation, New York, NY

PXB-Mouse: a Humanized Liver Chimeric Mouse Model for the Study of Oligonucleotide and Gene Therapies

Closing Night Reception

8:00 pm - 11:00 pm

Field Museum – Ticketed Event

Sponsored by University of Massachusetts Medical School

University of Massachusetts Medical School

Shuttle Bus Transportation

ASGCT will provide free shuttle transportation for the reception at the Field Museum during the 21st Annual Meeting on Friday, May 18, 2018. Shuttle buses will leave from the 8th Street Entrance of the Hilton Chicago Hotel.

Meeting Times

Friday, May 18, 2018 – 7:30 pm Departure (Running continuously until 11:30 pm)

Trainee Lounge

7:00 am – 12:15 pm
Room: Mobley
The Trainee Lounge is reserved for Students and Trainees as a designated spot to network, grab a refreshing snack and beverage and meet other students and trainees. Members and Non-members are welcome!

Business Meeting

7:30 am - 8:00 am
Room: Waldorf

Coffee Break

7:30 am - 8:00 am

Education Session 400

8:00 am - 9:30 am
Room: Salon A-5

AAV Vectors

CHAIR: Aravind Asokan, PhD

SPEAKERS

8:00 am – 8:30 am
Shannon E. Boye, PhD. University of Florida, Gainesville, FL
AAV Vectors in the Eye

8:30 am – 9:00 am
Miguel Sena-Esteves, PhD. University of Massachusetts Medical School, Worcester, MA
AAV Gene Therapy to CNS: Past, Present and Future

9:00 am – 9:30 am
Ian E. Alexander, BMedSci, MBBS, PhD, FRACP, HGSACG, FAHMS. Sydney Children's Hospitals Network, Westmead, Australia, Children's Medical Research Institute, Westmead, Australia
AAV Vectors in the Liver

Education Session 401

8:00 am - 9:30 am
Room: Salon A-1

RNA Therapeutics

CO-CHAIRS: Richard P. Harbottle, PhD and Patrick Schmidt, PhD

SPEAKERS

8:00 am – 8:30 am
Patrick Schmidt, PhD. National Center for Tumor Diseases, Heidelberg, Germany
RNA CARt Immunotherapy

8:30 am – 9:00 am
Anastasia Khvorova, PhD. University of Massachusetts Medical School, Worcester, MA
Oligonucleotide Therapeutics: Technology Evolution for Clinically Relevant Tissue Delivery

9:00 am – 9:30 am
Jonathan Finn, PhD. Intellia Therapeutics, Cambridge, MA
Lipid nanoparticle based RNA delivery: at the intersection of Chemistry and Immunology

Scientific Symposium 402

8:00 am - 10:00 am
Room: International Ballroom South

Advances and Future Directions for Gene Therapy of Cancer in 2018 - Organized by the Cancer Gene & Cell Therapy Committee

CO-CHAIRS: Saar Gill, MD, PhD and Masato Yamamoto, MD, PhD

SPEAKERS

8:00 am – 8:30 am
Saar Gill, MD, PhD. University of Pennsylvania, Philadelphia, PA
Engineered T cells for Cancer Therapy in 2018

8:30 am – 9:00 am
Stephen P. Schoenberger, PhD. La Jolla Institute for Allergy and Immunology, La Jolla, CA, UCSD Moores Cancer Center, La Jolla, CA
Functional Identification of Tumor Neoantigens

9:00 am – 9:30 am
Matthias Stephan, MD, PhD. Fred Hutchinson Cancer Research Center, Seattle, WA
In situ Programming of Therapeutic T cells Using Synthetic Nanoparticles

9:30 am – 10:00 am
Klaus Cichutek, PhD. Paul-Ehrlich-Institut, Langen, Germany
Development and Regulation of Immunotherapy

Scientific Symposium 403

8:00 am - 10:00 am
Room: Salon A-2

Emerging Discovery in Cardiovascular Diseases - Organized by the Cardiovascular Gene & Cell Therapy Committee

CO-CHAIRS: Manfred Boehm, MD and Matthew L. Springer, PhD

SPEAKERS

8:00 am – 8:30 am
Michael A. Laflamme, MD, PhD. Toronto General Research Institute, University of Toronto, Toronto, ON, Canada
Pluripotent Stem Cells for Heart Regeneration

8:30 am – 9:00 am
Thierry Pedrazzini, PhD. University of Lausanne Medical School, Lausanne, Switzerland
Enhancer-associated Long Noncoding RNAs in Cardiac Specification and Differentiation

9:00 am – 9:30 am
Mauro Giacca, MD, PhD. ICGEB, Trieste, Italy
Small RNAs for Cardiac Gene Editing and Regeneration

9:30 am – 10:00 am
Matthew L. Springer, PhD. University of California, San Francisco, San Francisco, CA
Decrease in B-Lymphocyte Levels by Advanced Aged or Myocardial Infarction Reduces the Efficacy of Autologous Bone Marrow Cell Therapy

Scientific Symposium 404

8:00 am - 10:00 am
Room: Continental Ballroom ABC

Clinical Trials Spotlight II

CO-CHAIRS: Robin R. Ali, PhD and Masafumi Onodera, MD, PhD

SPEAKERS

8:00 am – 8:30 am
Brian P. Sorrentino, MD. St. Jude Children's Research Hospital, Memphis, TN
SCID Immunodeficiency Trial

8:30 am – 9:00 am
Robert H. I. Andtbacka, MD, CM. Huntsman Cancer Institute, Salt Lake City, UT
Melanoma Skin Trial

9:00 am – 9:30 am
Savita Rangarajan, MD; FRCP; FRCPath. University Hospitals Southampton NHS Foundation Trust, Southampton, United Kingdom
Haemophilia A Gene Therapy Finally Promises Cure

9:30 am – 10:00 am
Discussion/Questions and Answers

Scientific Symposium 405

8:00 am - 10:00 am
Room: International Ballroom North

Cutting-Edge Genome Editing Technologies and Applications - Organized by the Genome Editing Committee

CO-CHAIRS: J. Keith Joung, MD, PhD and Toni Cathomen, PhD

SPEAKERS

*8:*00 am – 8:30 am
Prashant Mali, PhD. UC San Diego Jacobs School of Engineering, La Jolla, CA
Therapeutic Strategies via CRISPR-Cas: New Approaches and New Challenges

8:30 am – 9:00 am
David Guay, PhD. Feldan Therapeutics, Quebec, QC, Canada
Novel Peptide Technology to Deliver CRISPR Ribonucleoproteins

9:00 am – 9:30 am
Alexander Marson, MD, PhD. University of California, San Francisco, San Francisco, CA
Reprogramming Human T Cell Circuitry

9:30 am – 10:00 am
Amy Wagers, PhD. Harvard University, Cambridge, MA
Dystrophin Correction and Targeting Satellite Cells *in vivo*

Scientific Symposium 406

8:00 am - 10:00 am
Room: Salon A-3, 4

New Directions in Viral Gene Delivery - Organized by the Viral Gene Transfer Vectors Committee

CO-CHAIRS: Junghae Suh, PhD and H. Trent Spencer, PhD

SPEAKERS

8:00 am – 8:30 am
Luk H. Vandenberghe, PhD. Harvard Medical School, Boston, MA
Ancestral AAV Capsid Reconstruction: Interrogating Viral Evolution to Inform Rational AAV Design

8:30 am – 9:00 am
Hildegard Buning, PhD. Hannover Medical School, Hannover, Germany
Optimizing AAV Vectors - Capsid Engineering and Beyond

9:00 am – 9:30 am
Bobby Gaspar, MD, PhD. UCL Great Ormond Street Institute of Child Health, London, United Kingdom
LV-mediated Clinical Trials in Immunodeficiencies

9:30 am – 10:00 am
Kah-Whye Peng, PhD. Mayo Clinic, Rochester, MN
Noninvasive Imaging Technologies to Monitor Biodistribution, Activity, and Toxicity of Viruses and Cell Therapies

Break

10:00 am - 10:15 am

Oral Abstract Session 410

10:15 am - 12:15 pm

Room: International Ballroom North

Gene Targeting and Gene Correction

CHAIR: Adi Barzel, PhD and Punam Malik, MD

PRESENTERS

10:15 am – 10:30 am

948: Efficient *In Vivo* Selection of Gene-Targeted Hepatocytes Using Acetaminophen-Induced Liver Toxicity

Sean Nygaard, Oregon Health and Science University, Portland, OR

10:30 am – 10:45 am

949: Designed Zinc Finger Protein Transcription Factors for Single-Gene Regulation Throughout the Central Nervous System

Bryan J. Zeitler, Sangamo Therapeutics, Inc, Richmond, CA

10:45 am – 11:00 am

950: Long-Term Evaluation of Genome Editing Outcomes for Duchenne Muscular Dystrophy

Christopher Nelson, Duke University, Durham, NC

11:00 am – 11:15 am

951: AAV-Vector Integration into CRISPR-Induced Double-Stranded Breaks

Bence Gyorgy, Harvard Medical School, Boston, MA

11:15 am – 11:30 am

952: Non-Viral Delivery of ZFN mRNA Enables Highly Efficient *In Vivo* Genome Editing of Multiple Therapeutic Gene Targets

Anthony Conway, Sangamo Therapeutics, Richmond, CA

11:30 am – 11:45 am

953: Improved Genome Editing through Inhibition of the FANCM Pathway

Gustavo de Alencastro, Stanford University, Stanford, CA

11:45 am – 12:00 pm

954: rAAV-Mediated Nuclease-Assisted Vector Integration (rAAV-NAVI) Promotes Highly Efficient and Stable Transgene Expression in Somatic Tissues

Alexander Brown, University of Massachusetts Medical School, Worcester, MA

12:00 pm – 12:15 pm

955: Direct *In Vivo* Transduction of Mobilized CD34 HSPCs with Adenoviral Vectors in Non-Human Primates

Kevin G. Haworth, Fred Hutchinson Cancer Research Center, Seattle, WA

Oral Abstract Session 411

10:15 am - 12:15 pm
Room: International Ballroom South

AAV Vectorology

CO-CHAIRS: Chen Ling, PhD and Dan Wang, PhD

PRESENTERS

10:15 am – 10:30 am
956: Structure-Function Characterization of Non-Primate AAV Capsids for Their Usage as Therapeutic Gene Delivery Vectors
Mario Mietzsch, University of Florida, Gainesville, FL

10:30 am – 10:45 am
960: Cell-Specific Responses Following Intravenous Administration of AAV Vectors: Liver Single Cell Transcriptome Analysis of Cell Type-Specific Transgene Expression and Cell Type-Specific Vector-Mediated Transcriptome Dysregulation
Detu Zhu, Weill Cornell Medical College, New York, NY

10:45 am – 11:00 am
958: Wild-Type and Recombinant AAV Mitochondrial Integration and Trafficking
Jessika Ceiler, National Center for Tumor Diseases (NCT) and German Cancer Research Center (DKFZ), Heidelberg, Germany

11:00 am – 11:15 am
959: A Novel rAAV-amiRNA Platform Enables Potent *In Vivo* Gene Silencing and a Ten-Fold Enhancement of On-Target Specificity over Conventional shRNA Vectors
Jun Xie, Umass Medical School, Horae Gene Therapy Center, Worcester, MA

11:15 am – 11:30 am
957: Post-Transcriptional Fine-Tuning of AAV Vector Gene Expression for Hemophilia A Gene Therapy
Claire Domenger, Heidelberg University Hospital, Heidelberg, Germany

11:30 am – 11:45 am
961: Hypothermia Improves AAV Entry into CNS across BBB and Enhances ALS Survival by Delivering BDNF
Bin Xiao, University of North Carolina, Chapel Hill, NC

11:45 am – 12:00 pm
962: Targeting Muscle Satellite Cells with Adeno-Associated Viral Vectors
Jennifer Kwon, Duke University, Durham, NC

12:00 pm – 12:15 pm
963: Development of a Novel Recombinant Adeno-Associated Virus Production System Using Human Bocavirus 1 Helper Genes
Zekun Wang, University of Kansas Medical Center, Kansas City, KS

Oral Abstract Session 412

10:15 am - 12:15 pm
Room: Continental Ballroom ABC

Advancements in T Cell-Based Therapies

CO-CHAIRS: Marcela V. Maus, MD, PhD and Rimas J. Orentas, PhD

PRESENTERS

10:15 am – 10:30 am
108: Novel Phosphorylation Sites in the CD28 Costimulatory Domain Shape CAR-T Cell Function
Maria C. Ramello, Moffitt Cancer Center, Tampa, FL

10:30 am – 10:45 am
965: Effective Antitumor Responses in the Absence of Toxicity in Pancreatic Ductal Adenocarcinoma Models by Targeting B7-H3 via Chimeric Antigen Receptor T Cells
Hongwei Du, University of North Carolina at Chapel Hill, Chapel Hill, NC

10:45 am – 11:00 am
966: Car T Cells Secreting an Immune Checkpoint Blockade scFv Have Enhanced Anti-Tumor Efficacy
Sarwish Rafiq, Memorial Sloan Kettering Cancer Center, New York, NY

11:00 am – 11:15 am
967: Early Signs of Clinical Activity in Aml Patients Receiving NKG2D CAR T Cell Therapy in the Absence of Pre-Conditioning Chemotherapy: An Alternative Strategy to CAR T Cell Therapy
Eytan Breman, Celyad, Mont St Guibert, Belgium

11:15 am – 11:30 am
968: Highly Efficient and Specific Multiplexed Gene Editing in T Cells Using Enhanced Zinc-Finger Nucleases (Zfns) Enables Strategic Engineering of Allogeneic T Cell Immunotherapies
Sumiti Jain, Sangamo Therapeutics, Richmond, CA

11:30 am – 11:45 am
969: Armored Glypican-3-Specific CAR T Cells for the Immunotherapy of Hepatocellular Carcinoma
Sai A. Batra, Baylor College of Medicine, Houston, TX

11:45 am – 12:00 pm
970: Vector Integration and Efficacy of CD19-Directed CAR T Cell Therapy in ALL and CLL
Christopher L. Nobles, Perelman School of Medicine, University of Pennsylvania, Philadelphia, PA

12:00 pm – 12:15 pm
971: Cancer Immunotherapy with APOBEC3B-Induced Heteroclitic Library Tumor Cell Vaccines and Immune Checkpoint Blockade
Richard Vile, Mayo Clinic, Rochester, MN

Oral Abstract Session 413

10:15 am - 12:15 pm

Room: Salon A-1

Adenovirus Vectors and Other DNA Virus Vectors, Technical Advances in CNS Gene Therapy

CO-CHAIRS: Paola G. Grandi, PhD and Shannon E. Boye, PhD

PRESENTERS

10:15 am – 10:30 am

972: HDAd5/35++-Mediated Targeted Integration in HSCs of AAVS1 Transgenic Mice Results in Efficient Transgene Marking in Peripheral Blood Mononuclear Cells

Chang Li, University of Washington, Seattle, WA

10:30 am – 10:45 am

973: Molecular Evolution of the Next Generation of Bocaviral Vectors

Julia Fakhiri, Heidelberg University Hospital, Heidelberg, Germany

10:45 am – 11:00 am

974: A Human In Vitro Model to Study Adenoviral Receptors and Virus-Cell Interactions

Anja Ehrhardt, Witten/Herdecke University, Witten, Germany

11:00 am – 11:15 am

975: Novel Small Molecules That Enhance Adenovirus Transduction of the Airway Epithelium

Hannah W. Shows, Wright State University, Dayton, OH

11:15 am – 11:30 am

976: Allele-Specific RNA Interference: Precision Gene Therapy for Dominant Forms of Charcot-Marie-Tooth Disease

Kathryn H. Morelli, The Jackson Laboratory, Bar Harbor, ME

11:30 am – 11:45 am

977: Intravenous Injection of a Mito Targeted AAV9 Delivering Wild Type Human ATP6 Reversed Paralysis and Prevented Death in a Transgenic Mouse Model of Leigh Syndrome Caused by a T8993G Mutation in Subunit 6 of Mitochondrial ATP Synthase

Huijun Yuan, University of Miami, Miami, FL

11:45 am – 12:00 pm

978: Systemic Administration of AAV-PHP.B in Cats Does Not Increase CNS Transduction over AAV9

Ana Rita Batista, University of Massachusetts Medical School, Worcester, MA

12:00 pm – 12:15 pm

979: Correction of GM2 Gangliosidosis in Neonatal, Juvenile, and Adult Sandhoff Mice Using a Bicistronic Hexosaminidase Vector

Karlaina J.L. Osmon, Queen's University, Kingston, ON, Canada

Oral Abstract Session 414

10:15 am - 12:15 pm

Room: Salon A-2

Liver Monogenic Diseases: Genome Editing, AAV Vectors, and Cell Therapy

CO-CHAIRS: Gerald S. Lipshutz, MD and Beat Thony, PhD

PRESENTERS

10:15 am – 10:30 am

980: Targeted Integration of *MUT* into the *Albumin* Locus Using a Promoterless AAV Vector (Generide™) Confers a Hepatocellular Growth Advantage in Mice with Methylmalonic Acidemia

Randy J. Chandler, National Institutes of Health, Bethesda, MD

10:30 am – 10:45 am

981: Bezafibrate Enhances AAV Gene Therapy in Glycogen Storage Disease Type Ia

Hye Ri Kang, Duke University, Durham, NC

10:45 am – 11:00 am

982: Conditional Disruption of Hepatic Carbamoyl Phosphate Synthetase 1 in Mice Results in Hyperammonemia without Orotic Aciduria and Can Be Corrected by *In Vivo* Liver-Directed Gene Therapy

Suhail Khoja, University of California Los Angeles, Los Angeles, CA

11:00 am – 11:15 am

983: Effective Treatment of a Novel Immunocompromised Conditional Arginase-Deficient Mouse Model with Human Hepatocyte Transplantation

Brian Truong, University of California, Los Angeles, Los Angeles, CA

11:15 am – 11:30 am

984: Successful *In Vivo* Editing of the OTC Locus in Primary Human Hepatocytes Xenografted into the FRG Mouse Liver

Anais K. Amaya, Children's Medical Research Institute, The University of Sydney and The Sydney Children's Hospitals Network, Westmead, Australia

11:30 am – 11:45 am

985: Successful CRISPR/Cas9-Mediated Gene Editing in Murine Phenylketonuria (PKU)

Daelyn Y. Richards, Oregon Health and Science University, Portland, OR

11:45 am – 12:00 pm

986: Development and Characterization of a Humanized Porcine Model of Phenylketonuria

Kari Allen, Mayo Clinic, Rochester, MN

12:00 pm – 12:15 pm

987: Anc80 and AAV8 Vectors Mediate Equivalent Long-Term Hepatic Correction of Methylmalonyl-CoA Mutase Deficiency in a Murine Model of Methymalonic Acidemia (MMA)

Lina Li, NIH, Bethesda, MD

Oral Abstract Session 415

10:15 am - 12:15 pm
Room: Salon A-3, 4

Cell Engineering and Clinical Trials

CO-CHAIRS: Robert M. Kotin, PhD and Isabelle Riviere, PhD

PRESENTERS

10:15 am – 10:30 am
988: 3CAR: Gene Edited Anti-CD3 Chimeric Antigen Receptor T Cells
Jane Rasaiyaah, UCL Great Ormond Street Institute of Child Health, London, United Kingdom

10:30 am – 10:45 am
989: Transposon-Modified T-Lymphocytes for Sustained Erythyopoietin Delivery *In Vivo*
Richard ONeil, Vanderbilt University Medical Center, Nashville, TN

10:45 am – 11:00 am
990: Closed System Manufacturing of Expanded and Activated Gamma/Delta T Cells as Preemptive Immunotherapy in Haploidentical Hematopoietic Cell Transplantation: a Phase I Trial
Samantha L. Youngblood, University of Alabama at Birmingham, Birmingham, AL

11:00 am – 11:15 am
991: Phase 1 Feasibility and Safety Study of Lentiviral Gene-Modified Epidermal Grafts for Netherton Syndrome
Wei-Li Di, UCL GOS Institute of Child Health, London, United Kingdom

11:15 am – 11:30 am
992: Production of Good Manufacturing Practice Compliant Gene Engineered Autologous Fibroblasts for Recessive Dystrophic Epidermolysis Bullosa
Farhatullah Syed, University College London, London, United Kingdom

11:30 am – 11:45 am
993: Efficient Clinical Scale CRISPR/Cas9-Mediated Editing of Plerixafor-Mobilized Hematopoietic Stem and Progenitor CD34+ Cells for Treatment of Sickle Cell Disease
Hui Yu, CRISPR Therapeutics, Cambridge, MA

11:45 am – 12:00 pm
994: Genome-wide Integration Profile of a Lentiviral Vector Carrying the Human *FAH* Gene in Human Hepatocytes
Zeji Du, Mayo Clinic, Rochester, MN

12:00 pm – 12:15 pm
995: Changing Cell Manufacturing Processes during Clinical Trials: A Success Story
Tom Spencer, Novartis Pharmaceuticals Corporation, East Hanover, NJ

Oral Abstract Session 416

10:15 am - 12:15 pm
Room: Salon A-5

Immunological Aspects of Gene Therapy and Vaccines

CO-CHAIRS: Antonia Follenzi, MD, PhD and Federico Mingozzi, PhD

PRESENTERS

10:15 am – 10:30 am

996: FVIII Expression Driven by Its Native Promoter by Lentiviral Vectors Allowed Sustained Long-Term FVIII Expression Blunting Immune Responses in Hemophilic Mice
Rosella Fama, University of Piemonte Orientale 'A. Avogadro', Novara, Italy

10:30 am – 10:45 am

997: *In Vivo* Activation of Dendritic Cells by AAV Vectors and NK Cell-Independent Cross-Priming of CD8+ T Cells
Jamie Shirley, University of Florida, Gainesville, FL

10:45 am – 11:00 am

998: Modeling and Modulating Anti-Transgene Immune Response in *Ex-Vivo* Gene Therapy for Mucopolysaccharidosis Type-I (MPS-I)
Giorgia Squeri, SR-TIGET, Milan, Italy

11:00 am – 11:15 am

999: Review of CSF and Peripheral Immune Responses Following Intrathecal Gene Transfer for Giant Axonal Neuropathy
Dimah Saade, Neuromusuclar and Neurogenetic Disorders of Childhood Section, Neurogenetics Branch, NINDS, NIH, Bethesda, MD

11:15 am – 11:30 am

1000: Reversal of Multiple Sclerosis in Multiple Mice Models
Geoffrey D. Keeler, University of Florida College of Medicine, Gainesville, FL

11:30 am – 11:45 am

1001: Engineering AAV Vectors to Evade Innate Immune and Inflammatory Responses
Ying Kai Chan, Harvard University, Boston, MA

11:45 am – 12:00 pm

1002: Therapeutic Factor IX (FIX) Activity after Single Treatment with AMT-060 (AAV5-FIX) in Hemophilia B Patients with Pre-Existing Anti-AAV5 Humoral Immunity
Anna Majowicz, uniQure, Amsterdam, Netherlands

12:00 pm – 12:15 pm

1003: Intradermal Delivery of a Synthetic, Consensus DNA Vaccine against Zika Virus, GLS-5700, is Highly Immunogenic in Humans Inducing Humoral and Cellular Immunity That is Maintained for over One Year
Emma L. Reuschel, Wistar Institute, Philadelphia, PA

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BOOTH NUMBER: 315

The Gene Therapy Program is a complex R&D organization with the internal capacity to perform gene transfer studies from basic research through phase 1/2 proof-of-concept human trials. An array of core laboratories, a manufacturing facility, and regulatory affairs teamwork in synergy to support the advancement of breakthrough discoveries.

Gene Tools, LLC

1001 Summerton Way
Philomath, OR 97370
Phone: (541) 929-7840
Fax: (541) 929-7841
E-mail: zli@gene-tools.com
Website: www.gene-tools.com

BOOTH NUMBER: 716

Gene Tools manufactures Morpholino oligos for blocking translation, modifying splicing or inhibiting miRNA activity. Morpholinos are used in cell cultures, embryos or, as Vivo-Morpholinos, in adult animals. Morpholinos are effective, specific, stable and non-toxic. Backed by Ph.D.-level customer support, Gene Tools designs and synthesizes Morpholinos and offers cytosolic delivery options.

GenesisBPS

465 Route 17 South
Ramsey, NJ 07446
Phone: (201) 708-1400
Fax: (201) 708-1104
E-mail: mrosenfeld@genesisbps.com
Website: www.genesisbps.com

BOOTH NUMBER: 208 b

GenesisBPS is a global manufacturer and supplier of precision equipment to clinical laboratories, life science customers, blood collection and cellular therapy organizations. Our product portfolio represents quality and consistency in a world of ever-changing requirements for collection, processing, safe storage and transport.

GeneWerk GmbH

Im Neuenheimer Feld 582
Heidelberg 69120
Germany
Phone: +49 6221 42790-10
Fax: +49 6221 42790-12
E-mail: annette.deichmann@genewerk.de
Website: www.genewerk.de

BOOTH NUMBER: 707

GeneWerk specializes in vector safety and integration site analysis for gene therapy, gene editing and immunotherapy. We offer a wide variety of custom-tailored services to determine fusion sequences adjacent to known DNA or RNA fragments in minimal tissue samples. Our goal is to help our clients move towards safer therapies.

Genezen Laboratories, Inc.

1075 Foster Rd.
Iowa City, IA 52245
Phone: (317) 822-8330
E-mail: bvincent@genezenlabs.com
Website: www.genezenlabs.com

BOOTH NUMBER: 620

Genezen offers contract viral vector production and testing services; including clinical trial patient sample testing through the expertise of the nation's leading academic vector production and research laboratories.

GenoSafe

1 rue de l'internationale
Evry 91000
France
Phone: 01 69 47 11 57
E-mail: ahuguenot@genosafe.com
Website: www.genosafe.com

BOOTH NUMBER: 613

GenoSafe is a CRO providing analytical testing Under GLP, GMP and GCP compliance. Its scientific fields of expertise are biomolecular, virology, immunology. Working for the leaders in Gene and Cell therapy, GenoSafe has been involved from preclinical research to clinical studies and Quality Control testing. GenoSafe's clients are biotechs, pharmaceuticals, public/private research laboratory and Universities/Hospitals.

GenScript USA Inc.

860 Centennial Ave
Piscataway, NJ 08854
Phone: (732) 885-9188
Fax: (732) 210-0262
E-mail: david.lin@genscript.com
Website: www.GenScript.com

BOOTH NUMBER: 513

GenScript is a leading biotech company providing life science services and products to scientists all over the world. With gene, peptide, protein, antibody, CRISPR, and preclinical antibody drug development services, we are recognized as a leading biotech company specializing in fundamental life science, translational biomedical, and pharmaceutical research.

iBET & GENIBET

Apartado 12
Oeiras 2781-901
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Phone: 00 351 21 4411277
E-mail: info@ibet.pt
Website: www.ibet.pt

BOOTH NUMBER: 612

iBET & GenIbet Biopharmaceuticals is your Contract Development and Manufacturing Organization of choice, offering highly specialized microbial, cell culture and viral propagation process development and cGMP manufacturing services. This, combined with in-house fill and finish capabilities, gives our clients the opportunity to go from bench to clinic in one facility.

Imanis Life Sciences

221 First Ave SW
Suite 102
Rochester, MN 55902
Phone: (507) 218-2559
E-mail: support@imanislife.com
Website: www.imanislife.com

BOOTH NUMBER: 705

Imanis Life Sciences specializes in noninvasive reporter gene imaging of virus, gene, and cell therapies. We provide multi-reporter gene cell lines, oncolytic viruses, lentiviral

vectors, and antibodies. We take pride in offering high-quality research services for virus engineering, custom cell line generation, evaluation of oncolytic viruses, and cell tracking with reporter genes. Our vision is the widespread adoption of noninvasive imaging in preclinical and clinical research.

Insperity

9399 W. Higgins Road, Suite 925
Rosemont, IL 60018
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Fax: (866) 366-1017
E-mail: jim.deady@insperity.com
Website: www.insperity.com

BOOTH NUMBER: 511

Insperity has been in business since 1986, and helped to form what is now the Certified Professional Employer Industry. Our mission is to help Companies Succeed so Communities Prosper. We offer Business Performance solutions that delivers administrative relief in key areas: Health Care, Payroll, Human Capital Management, Workers Compensation Insurance, plus an array of other Human Resource Solutions that can be delivered in a multitude of service options.

Johns Hopkins Univ. Center for Biotechnology Education

9601 Medical Center Drive
Rockville, MD 20850
Phone: (301) 294-7159
E-mail: biotechnology@jhu.edu
Website: http://advanced.jhu.edu/academics/centers/
center-for-biotechnology-education/

BOOTH NUMBER: 510

The Johns Hopkins Center for Biotechnology Education offers programs available 100% online or onsite and part or full time. Recently, the Center has developed the concentration Regenerative and Stem Cell Technologies. The program features hands-on and theoretical skillsets that translate across regenerative medicine, cell therapy, gene therapy and tissue engineering.

Juno Therapeutics, Inc.

400 Dexter Ave. N
Suite 1200
Seattle, WA 98109
Phone: (206) 582-1600
Website: www.junotherapeutics.com

BOOTH NUMBER: 715

Juno Therapeutics is building a fully integrated biopharmaceutical company focused on re-engaging the body's immune system to revolutionize the treatment of cancer. Juno is developing cell-based cancer immunotherapies based on chimeric antigen receptor and high-affinity T cell receptor technologies to genetically engineer T cells to recognize and kill cancer.

KBI Biopharma

1101 Hamlin Road
Durham, NC 27704
Phone: (919) 943-8572
E-mail: bnewsom@kbibiopharma.com
Website: www.kbibiopharma.com

BOOTH NUMBER: 611

KBI Biopharma, Inc. is a biopharmaceutical Contract Development and Manufacturing Organization that accelerates the development of innovative discoveries into life-changing biological products. From early-stage to academic/non-profit organizations, to many of the world's largest pharmaceutical companies, KBI has served 250+ clients globally to accelerate and optimize their drug development programs.

Kite

2400 Broadway
Santa Monica, CA 90404
Phone: (424) 238-1880
E-mail: efaust@kitepharma.com
Website: www.kitepharma.com

BOOTH NUMBER: 607

Kite, a Gilead Company, is a biopharmaceutical company based in Santa Monica, California. Kite is engaged in the development of innovative cancer immunotherapies. The company is focused on chimeric antigen receptor and T cell receptor engineered cell therapies. For more information on Kite, please visit www.kitepharma.com.

Gilead Sciences, Inc. is a research-based biopharmaceutical company that discovers, develops and commercializes innovative

medicines in areas of unmet medical need. We strive to transform and simplify care for people with life-threatening illnesses around the world. Gilead's portfolio of products and pipeline of investigational drugs includes treatments for HIV/AIDS, liver diseases, cancer, inflammatory and respiratory diseases, and cardiovascular conditions. For more information on Gilead, please visit www.gilead.com.

Lonza AG/Ltd

8830 Biggs Ford Road
Walkersville, MD 21793
Phone: (800) 638-8174
Fax: (301) 845-4024
E-mail: custom@lonza.com
Website: www.pharma.lonza.com

BOOTH NUMBER: 709 & 711

Lonza is a global leader in contract development & manufacturing to the pharma & biotech industry, with recognized, reliable, high-quality services, leveraging a global capacity, innovative technology platforms and extensive experience in the cell and gene therapy field.

Lovelace Biomedical

2425 Ridgecrest Dr. SE
Albuquerque, NM 87108
Phone: (505) 348-9457
E-mail: jbenson@lovelacebiomedical.org
Website: www.lovelacebiomedical.org

BOOTH NUMBER: 704

Lovelace Biomedical, the commercially-focused CRO arm of the Lovelace Respiratory Research Institute, has extensive experience performing GLP and non-GLP safety and efficacy studies of gene and cell therapies. Services include animal models of disease, pre-clinical safety assessments, biodistribution and qPCR in large and small animals.

Malvern Panalytical

117 Flanders Road
Westborough, MA 01581
Phone: (508) 768-6400
Fax: (580) 768-6403
E-mail: sales.us@malvern.com
Website: www.malvern.com

BOOTH NUMBER: 609

Malvern Panalytical is a leader in analytical characterization, creating expert solutions for the challenges associated with maximizing productivity, developing better quality products and getting them to market faster. We provide superior, customer-focused solutions and services which deliver tangible economic impact through chemical, biophysical and structural analysis. www.malvernpanalytical.com

Mary Ann Liebert, Inc.

140 Huguenot Street
New Rochelle, NY 10801
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E-mail: jgatti@liebertpub.com
Website: www.liebertpub.com

BOOTH NUMBER: 712

Mary Ann Liebert Inc. is the proud publisher of cutting-edge, peer-reviewed journals were first in their field including Human Gene Therapy, HGT Methods, & HGT Clinical Development, as well as the newly launched CRISPR Journal. These publications provide unprecedented access to global research and applications critical to the evolution of the gene therapy field! Visit us @ booth #712 to get free access.

MaxCyte

22 Firstfield Road, Suite 110
Gaithersburg, MD 20878
Phone: (301) 944-1700
Fax: (301) 944-1703
E-mail: info@maxcyte.com
Website: www.maxcyte.com

BOOTH NUMBER: 301

MaxCyte's non-viral delivery platform allows for engineering of nearly all cell types, including human primary cells, with any molecule, at any scale for use in drug discovery and development, biomanufacturing, gene editing, cell therapy, and immuno-oncology. Its consistency and minimal cell disturbance facilitate rapid, clinical and commercial grade cell engineering.

MilliporeSigma

80 Ashby Rd.
Bedford, MA 01730
Phone: (800) 225-3384
Website: www.emdmillipore.com

BOOTH NUMBER: 209

MilliporeSigma, a life science leader, is a business of Merck KGaA, Darmstadt, Germany. Our BioReliance® services offering in support of cell and gene therapy includes cGMP Manufacturing services for viral gene therapy, biosafety testing services for cell and gene therapy products, and gene editing technologies.

Miltenyi Biotec Inc.

6125 Cornerstone Court, East
San Diego, CA 92121
Phone: (800) 367-6227
Fax: (858) 535-2166
E-mail: macs@miltenyibiotec.com
Website: www.miltenyibiotec.com

BOOTH NUMBER: 307 & 309

Miltenyi Biotec's mission is to improve scientific understanding and medical progress. By developing innovative products that address the processes that are necessary for the study of immuno-oncology we help researchers advance science in these key areas as well as help clinicians to make the concept of cellular therapy a reality.

MPI Research

54943 North Main Street
Mattawan, MI 49071
Phone: (269) 668-3336
Fax: (269) 668-4151
E-mail: info@mpiresearch.com
Website: www.mpiresearch.com

BOOTH NUMBER: 110

MPI Research is a leading early stage drug development CRO dedicated to bringing safer and more effective treatments to the world. With a commitment to responsiveness, integrity, trust, and teamwork, MPI Research consistently delivers high-quality services to Sponsors across the globe. Find out more at www.mpiresearch.com.

National Gene Vector Biorepository (NGVB)

980 West Walnut Street
Bldg. R3 Room C650
Indianapolis, IN 46202
Phone: (317) 274-4519
E-mail: lrubin@iupui.edu
Website: https://www.ngvbcc.org

BOOTH NUMBER: 706

The National Gene Vector Biorepository (NGVB) is a NIH/NHLBI funded resources that provides support for clinical gene therapy trials and pre-clinical research. The NGVB assists investigators in meeting FDA required testing including post-trial monitoring for replication competent virus and insertion site analysis. The NGVB will also store samples collected in pharmacology or toxicology studies under FDA GLP requirements. In addition, the NGVB will store clinical post-trial samples, final product reserves, and back-up master cell banks. For preclinical work, the NGVB maintains a reagent repository comprising cell lines, AAV plasmids, vectors and other items that can be searched on our website. Finally, we maintain an online pharm/tox database containing detailed summaries of gene therapy toxicology studies submitted to the FDA. If a studies proves relevant to your clinical study, we can facilitate a FDA letter of cross reference. Go to https://www.ngvbcc.org or come and see us in Booth 706 for more information.

Nature Technology Corp.

4701 Innovation Dr.
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Website: www.natx.com

BOOTH NUMBER: 603

NTC is a developer of safe and effective nucleic acids based gene therapeutics, DNA vaccines, and associated technologies, including antibiotic-free selection (RNA-OUT), regulatory compliance, viral vector retrofitting, and best in class Nanoplasmidstm. NTC provides custom design, synthesis, manufacturing and tech transfer, resulting in rapid development of APIs, ready for preclinical testing.

New England Biolabs
240 County Road
Ipswich, MA 01938
Phone: (978) 380-7352
E-mail: hornblower@neb.com
Website: www.neb.com
BOOTH NUMBER: 505

For 40 years New England Biolabs, Inc. has led the industry in the discovery and production of molecular biology reagents. In addition to products for genomic research, NEB continues to expand its product offerings into areas related to PCR, gene expression, sample preparation for next generation sequencing, cellular analysis, epigenetics and RNA analysis.

NOVASEP
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Boothwyn, PA 19061
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Website: www.novasep.com
BOOTH NUMBER: 108

Ology Bioservices
13200 NW Nano Court
Alachua, FL 32615
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Website: www.ologybio.com
BOOTH NUMBER: 115

Ology Bioservices, Inc. is a biologics-focused CDMO serving both government and commercial clients. Ology Bio's capabilities include process development/optimization, cGMP manufacturing, analytical development and testing, and full regulatory support of proteins, antibodies, viral vaccines, and gene therapies. Ology Bio provides expertise from preclinical through FDA licensure, and offers proprietary cell lines for antibodies and vaccines.

OriGen Biomedical
7000 Burleson Rd. Bldg. D.
Austin, TX 78744
Phone: (512) 474-7278
Fax: (512) 617-1503
E-mail: r.patino@origenbio.com
Website: www.origen.com
BOOTH NUMBER: 500

At OriGen Biomedical we manufacture a full range of cryopreservation products including bags for cell and tissue storage. We have CE-marked, sterile DMSO solutions available in syringe and vial configurations and OriGen has FEP cell culture bags in many standard sizes. Custom products can be designed to meet your needs.

Oxford BioMedica
Windrush Court, Transport Way
Oxford, Oxfordshire OX4 6LT
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Phone: +44 1865 783000
E-mail: j.slingsby@oxfordbiomedica.co.uk
Website: www.oxfordbiomedica.co.uk
BOOTH NUMBER: 509

Oxford BioMedica has more than 20 years of experience in the field of gene and cell therapy and was the first organisation to administer lentiviral vectors directly to humans. Our established LentiVector® gene delivery platform and associated technical capabilities help us design, develop and produce gene and cell-based medicines for ourselves and for our partners.

Oxford Genetics
210 Broadway St. #201
Cambridge, MA 02139
Phone: (508) 596-3428
E-mail: award@oxgene.com
Website: www.oxfordgenetics.com
BOOTH NUMBER: 216

Oxford Genetics leverages their unique position in rational design, standardized constructs and automated screening capabilities. Integration of these pillars ensure reproducibility and quality of your early phase development. Oxford Genetics delivers purpose-designed packaging and producer cell lines, highly efficient viral vector platforms, and an expertise in construct design.

Packgene Biotech, LLC
1 Innovation Drive, Three Biotech
Worcester, MA 01605
Phone: (774) 312-1463
E-mail: info@packgene.com
Website: www.packgene.com
BOOTH NUMBER: 619

Packgene Biotech is a fast-growing high-quality rAAV packaging service

provider, committed to meet the vast needs from global clients including biotechnology/ pharmaceutical companies, and academic research laboratories. With advanced manufacture and purification technology, we deliver in a timely manner and help scientists speed up drug development process and accelerate research progresses.

Pall Life Sciences

20 Walkup Dr.
Westborough, MA 01581
Phone: (516) 644-6151
E-mail: carol_dellicicchi@pall.com
Website: www.pall.com

BOOTH NUMBER: 616 & 618

Pall meets the demanding needs of customers discovering, developing and producing biologics, vaccines, cell and gene therapy products & classic pharmaceuticals. Pall is a leading provider of continuous bioprocessing, integrated automated systems and single-use solutions to pharmaceutical and biotechnology companies - from bioreactors, through downstream purification, to formulation and filling.

Paragon Bioservices, Inc.

801 West Baltimore St.
Suite 302
Baltimore, MD 21201
Phone: (410) 975-4050
E-mail: sales-support@paragonbioservices.com
Website: www.paragonbioservices.com

BOOTH NUMBER: 608 & 610

Paragon Bioservices is an industry-leading, private-equity backed contract development and manufacturing organization. Paragon aims to build strong client partnerships with the world's best biotech and pharma companies, focusing on transformative technologies, including gene therapies (AAV), next-generation vaccines, oncology immunotherapies (oncolytic viruses and CAR-T cell therapies), therapeutic proteins, and other complex biologics.

Penn Vector Core

125S, 31st Street
Suite 2000
Gene Therapy Program, Perelman School of Medicine, University of Pennsylvania
Philadelphia, PA 19104
Phone: (215) 573-0633
E-mail: vector@mail.med.upenn.edu
Website: www.med.upenn.edu/gtp/vectorcore/

BOOTH NUMBER: 313

Penn Vector Core is a state-of-the-art viral vector production facility for investigators both within and external to the University of Pennsylvania. Our main focus at this time is to make various serotypes of high quality research grade AAV viral vectors for gene transfer/Vaccine studies at the preclinical and basic research level.

PeproTech, Inc.

5 Crescent Avenue
Rocky Hill, NJ 08553-0275
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E-mail: Hklemens@peprotech.com
Website: www.peprotech.com

BOOTH NUMBER: 602

PeproTech creates the building blocks of your life science research by manufacturing high-quality products that advance scientific discovery and human health. PeproTech manufactures Recombinant Cytokines, Animal-Free Recombinant Cytokines, Monoclonal Antibodies, Affinity Purified Polyclonal Antibodies, Affinity Purified Biotinylated Polyclonal Antibodies, ELISA Development Kits, Cell Culture Media Products and GMP Cytokines.

Phacilitate

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Fulham
London SW6 3JW
United Kingdom
Phone: 020 7067 1867
E-mail: team@phacilitate.co.uk
Website: www.phacilitate.co.uk

BOOTH NUMBER: 518

In a disruptive, cutting edge industry no single person or entity has all of the answers. By fostering the community and bringing together a diverse range of expertise,

Phacilitate is creating the world's most trusted online community. A cooperative networking of partnerships and relationships will bring our industry closer to achieving the ultimate goal of improving patient care, developing commercially viable and curative treatments. We believe in the power of partnerships.

PhoenixBio Co., Ltd.

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Japan
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Fax: +81-82-431-0017
E-mail: rpbd2.pxbusa@phoenixbiousa.com
Website: www.phoenixbio.co.jp/em/

BOOTH NUMBER: 109

PhoenixBio produces the PXB-Mouse®, the world's most widely used humanized liver chimeric mouse model for preclinical drug development. With up to 95% of the liver replaced with human hepatocytes, PXB-mice express human genes and offer excellent human translatability in DMPK/Tox and liver disease areas, including oligonucleotide therapeutics with liver targets.

PlasmidFactory GmbH & Co. KG

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Fax: +49 521 299 7355
E-mail: info@plasmidfactory.com
Website: www.plasmidfactory.com

BOOTH NUMBER: 720

PlasmidFactory is a biopharmaceutical company founded in 2000 in Bielefeld, Germany. It has since developed into an international company. In addition to its In-Stock products and custom manufacturing of plasmid and minicircle DNA, PlasmidFactory focuses its R&D efforts on its core competencies in the production, analysis, application, and storage of DNA.

Polyplus-transfection

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Fax: +33-3-90-40-61-81
E-mail: vkedinger@polyplus-transfection.com
Website: www.polyplus-transfection.com

BOOTH NUMBER: 207

Polyplus-transfection provides qualified and GMP-grade transfection reagents for clinical trials in the fields of Gene and Cell therapy. Polyplus' state-of-the-art reagents are effective to deliver most nucleic acids, including DNA and siRNA in vitro and *in vivo*. Moreover, Polyplus offers qualified reagents dedicated to bioproduction needs such as GMP virus production.

Powell Gene Therapy Center

1200 Newell Drive
Gainesville, FL 32610
Phone: (352) 273-5522
E-mail: nclement@peds.ufl.edu
Website: http://powellcenter.med.ufl.edu

BOOTH NUMBER: 710

The Powell Gene Therapy Center at the University of Florida is an academic leader in the field of Gene Therapy, with a 20-year track record in Manufacturing, Toxicology studies and FDA-approved Clinical trials. It offers translational services for gene and cell therapies to promote bench-to-clinic research for the treatment of genetic diseases.

Precision NanoSystems Inc.

50-655 West Kent Ave. North
Vancouver, British Columbia V6P 6T7
Canada
Phone: (888) 618-0031
E-mail: vleung@precision-nano.com
Website: www.precisionnanosystems.com

BOOTH NUMBER: 519

Precision NanoSystems Inc (PNI) provides solutions for the discovery, development and manufacture of transformative nanomedicines. Nanomedicines are essential to delivering targeted and personalized medicines, but traditional manufacturing has impeded their translation. PNI's solutions, including NanoAssemblr technology, accelerate development of innovative medicines by enabling rapid, reproducible, and scalable nanoparticle manufacturing.

PROGEN Biotechnik GmbH

Maassstr. 30
Heidelberg 69123
Germany
Phone: 49622182780
E-mail: info@progen.com
Website: www.progen.com

BOOTH NUMBER: 718

Since 1983, PROGEN has been an established manufacturer and supplier of premium antibodies, IVDs, and reagents for the global life science research community. The activities of the DIN EN ISO 13485 certified company focus on the development of ELISA tests for the determination of AAV titers in gene therapy. www.progen.com

Proteintech Group, Inc.

5400 Pearl Street
Rosemont, IL 60018
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E-mail: jeff@ptglab.com
Website: https://www.ptglab.com

BOOTH NUMBER: 117

Puresyn, Inc.

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Phone: (610) 640-0800, ext. 24
Fax: (610) 640-0808
E-mail: leslie@puresyn.com
Website: www.puresyn.com

BOOTH NUMBER: 310

Puresyn, Inc. is focused on and dedicated to providing manufacturing services capable of producing high quality DNA which can be used for many applications including GMP manufacture of gene therapy products. These services include the following:

Contract Plasmid DNA Production Services
TransfectionReady – small scale, fast and economical
ResearchReady – large scale for research applications
INDReady™ – for further manufacture of GMP products

Puresyn, Inc. is well known for our rapid customer service, efficient processing times and superior quality products and services.

Puresyn incorporates a stringent Quality system and is committed to providing our collaborators with products and services of the highest quality. In order to meet that goal, all Puresyn functions are performed under the umbrella of our quality system. Puresyn maintains high standards to ensure that products meet or exceed our established specifications and those of our collaborators.

To inquire about a collaboration with us please call 610-640-0800.

Sangamo Therapeutics

501 Canal Blvd.
Suite F
Richmond, CA 94804
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E-mail: thawks@sangamo.com
Website: www.sangamo.com

BOOTH NUMBER: 703

Sangamo Therapeutics, Inc. is focused on translating ground-breaking science into genomic therapies that transform patients' lives using the Company's industry leading platform technologies in genome editing, gene therapy, gene regulation and cell therapy. For more information about Sangamo, visit www.sangamo.com.

Sartorius Stedim Biotech

August Spindler Strasse 11
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BOOTH NUMBER: 108 b

SIRION Biotech GmbH

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Martinsried/Planegg 82152
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Website: www.sirion-biotech.com

BOOTH NUMBER: 721

SIRION Biotech provides custom engineering and production services of viral vectors (AAV, Lentivirus, Adenovirus) for research and development in the life sciences and industry. Their unique LentiBOOSTTM transduction

enhancer is used in Lentivirus-based clinical trials to improve hematopoietic gene- and CAR-T cell therapies.
www.sirion-biotech.com

SNBL USA, Ltd.
6605 Merrill Creek Pkwy.
Everett, WA 98203
Phone: (877) 311-2501
E-mail: info@snblusa.com
Website: https://snbl.com/
BOOTH NUMBER: 621
SNBL USA is a nonclinical contract research organization renowned for its diverse range of safety assessment services and NHP expertise. With programs in toxicology, radiation biology, exploratory research, pathology and bioanalysis, our teams of scientists are committed to the advancement of novel therapeutics, devices and medical breakthroughs.

Spark Therapeutics
3737 Market Street
Philadelphia, PA 19104
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E-mail: info@sparktx.com
Website: www.sparktx.com
BOOTH NUMBER: 406

St. Jude Children's Research Hospital
262 Danny Thomas Place
Memphis, TN 38105
Phone: (901) 595-2750
E-mail: postdoc@stjude.org
Website: www.stjude.org/postdoc
BOOTH NUMBER: 617
Non-profit research institution with 240 faculty, where basic research is rapidly translated into groundbreaking treatments for cancer, non-malignant hematological disorders, and other life-threatening diseases. Consistently ranked on FORTUNE magazine's "100 Best Companies to Work For" list. Postdoctoral opportunities are available in areas including gene therapy, cell therapy, and cancer immunology.
postdoc@stjude.org
www.stjude.org/postdoc

STEMCELL Technologies Inc.
1618 Station St.
Vancouver, BC V6A 1B6
Canada
Phone: (604) 675-7575
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Website: www.stemcell.com
BOOTH NUMBER: 303
STEMCELL Technologies provides reagents to take cell therapy research from bench to bedside with T cell isolation, activation and expansion products. STEMCELL's collaboration with GE Healthcare aims to give researchers the confidence of a path to the clinic with cGMP-grade T cell reagents. Learn more at www.stemcell.com/t-cell-therapy

Syd Labs, Inc.
19 Erie Drive
Natick, MA 01760
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Fax: (617) 606-5019
E-mail: message@sydlabs.com
Website: www.sydlabs.com
BOOTH NUMBER: 520
Syd Labs, Inc. provides the CRO services of lentivirus, retrovirus, and AAV production, cell line engineering, gene editing, CAR-T, CAR-NK, CAR-T, and related quality control and functional assays in addition to various molecular biology, cell biology, and antibody-related services and products. All services are done in Massachusetts and California.

Synpromics Ltd
Roslin Innovation Centre
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Website: www.synpromics.com
BOOTH NUMBER: 517
Synpromics is the leader in gene control, improving human health by enabling safer, more effective cell and gene medicines through proprietary genomics, bioinformatics and intelligent data-driven design. The company has developed PromPT®, its multi-dimensional bioinformatics database that enables product-specific promoter design and selection empowering the next generation of cell and gene based medicines and bioprocessing applications.

Synthego Corporation

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E-mail: info@synthego.com
Website: www.synthego.com

BOOTH NUMBER: 107 b

Synthego is a leading provider of genome engineering solutions, including software and synthetic RNA kits designed for CRISPR genome editing. With next-generation informatics and machine learning, Synthego's vision is to enable precise, automated, rapid and cost-effective research for every scientist.

Terumo BCT

10811 W. Collins Avenue
Lakewood, CO 80215
Phone: (303) 513-2989
E-mail: celltherapy.orders@terumobct.com
Website: www.terumobct.com

BOOTH NUMBER: 201

Terumo BCT is a global leader in blood component, therapeutic apheresis and cellular technologies. We believe in the potential of blood to do even more for patients than it does today. This belief inspires our innovation and strengthens our collaboration with customers.

Thermo Fisher Scientific

35 Wiggins Ave.
Bedford, MA 01730
Phone: (781) 430-1645
E-mail: bp@thermofisher.com
Website: www.thermofisher.com/poros

BOOTH NUMBER: 419 & 421

Thermo Fisher Scientific supplies innovative solutions for the world's pharmaceutical and biopharmaceutical industries. With applications that span the drug development process – from drug discovery through large-scale commercial production - we provide a broad range of products and services. POROS™ and CaptureSelect™ chromatography resins offer high performance polish and unprecedented affinity chromatography solutions, enabling purification of the most complex biomolecules.

ThermoGenesis Corp.

2711 Citrus Road
Rancho Cordova, CA 95742
Phone: (916) 858-5119
E-mail: mlloyd@thermogenesis.com
Website: www.thermogenesis.com

BOOTH NUMBER: 417

ThermoGenesis Corporation, a wholly owned subsidiary of Cesca Therapeutics, is a pioneer and market leader in the development and commercialization of automated technologies for cell-based therapeutics and bioprocessing. The division's automated functionally-closed cell processing and cryopreservation systems provide researchers, physicians and cell-based manufacturers with solutions for Clinical Biobanking, Point-of-Care and Immuno-Oncology.

TriLink BioTechnologies

9955 Mesa Rim Road
San Diego, CA 92121
Phone: (858) 546-0004
E-mail: marketing@trilinkbiotech.com
Website: www.trilinkbiotech.com

BOOTH NUMBER: 507

Since 1996 TriLink has offered cutting edge services to researchers in the fields of gene therapy, nucleoside chemotherapy, oligonucleotide therapy and diagnostics. Our scientists and technicians have decades of collective experience in synthesizing modified nucleosides, nucleotides, mRNA and oligonucleotides for research, diagnostics and therapeutic applications.

Vectalys

Canal Biotech II
Toulouse 31400
France
Phone: 561287075
E-mail: sales@vectalys.com
Website: https://www.vectalys.com

BOOTH NUMBER: 615

Vectalys is a biotechnology company expert in lentiviral tools manufacturing. Since 2005, Vectalys develops high quality lentiviral particles for efficient transduction of delicate cells and *in vivo* tissues, without cytotoxicity. FlashCell, Vectalys' spin off, has been created to develop therapeutical programs (gene editing, immunotherapy, gene therapy) based on lentiviral tools.

VGXI, Inc.

2700 Research Forest Drive
Suite 180
The Woodlands, TX 77381
Phone: (281) 466-3790
E-mail: cfranco@vgxii.com
Website: www.vgxii.com

BOOTH NUMBER: 105

VGXI is a leading provider of plasmid DNA manufacturing services for DNA vaccines and gene therapies. With over 15 years of experience supporting clinical trials worldwide, the company has an outstanding track record of success in manufacturing challenging plasmid products under cGMP conditions with exceptional yield, quality, and prompt delivery.

Vibalogics GmbH

Zeppelinstr. 2
Cuxhaven 27472
Germany
Phone: +49 4721 565 400
E-mail: experts@vibalogics.com
Website: www.vibalogics.com

BOOTH NUMBER: 719

Vibalogics is a CMO offering process development, cGMP manufacturing and fill/finish of products based on viruses and live bacteria for companies involved in the development of vaccines, gene and oncolytic viral therapies. We offer a full range of development and manufacturing services, excellent quality and transparent customer experience.

Vigene Biosciences

9430 Key West Ave
Rockville, MD 20850
Phone: (301) 251-6638
E-mail: orders@vigenebio.com
Website: www.vigenebio.com

BOOTH NUMBER: 409

Vigene Biosciences' mission is Excellence in Gene Delivery. To achieve its mission Vigene is taking a two-pronged approach. For biomedical research, Vigene is developing, manufacturing, and distributing state-of-the-art AAV, lentivirus and adenovirus based reagents including Janelia Research Campus AAV Biosensors, and AAV alpha-synuclein vectors for Parkinsons Disease, developed in partnership with The Michael J Fox Foundation. On the cGMP clinical product and service side, Vigene is combining new production technologies with regulatory compliant cGMP production to meet the needs and expectations of clinical material clients. Headquartered in Rockville, MD, Vigene is a leader of both research grade and cGMP grade AAV, lentivirus and adenovirus, serving over 1000 academic, biotech and pharmaceutical labs.

Vineti

633 Howard Street
San Francisco, CA 94105
Phone: (415) 704-8730
E-mail: april.lynch@vineti.com
Website: https://vineti.com

BOOTH NUMBER: 116

Vineti creates innovative digital technology to drive the automation, production, and delivery of 21st-century medicine. The company combines leading software expertise with deep, first-hand industry experience in developing and commercializing personalized therapies to develop a cloud-based platform that ensures quality, scale, security, efficiency, traceability, and safety. Vineti is based in San Francisco, California.

Virovek, Inc.

22429 Hesperian Blvd.
Hayward, CA 94541
Phone: (510) 887-7121
Fax: (510) 887-7178
E-mail: hchen@virovek.com
Website: www.virovek.com

BOOTH NUMBER: 210

Virovek is a services company specializing in large scale AAV production and technology licensing. With our patented technologies, we provide:

Custom-made AAVs from 1E+13vg to 1E+17vg scales full services starting from gene cloning to purification and formulation. Toxin-gene containing AAVs as gene therapy vector for cell ablation and cancer therapies.

VIVEbiotech
Paseo Mikeletegi 81
San Sebastián Gipuzkoa 20009
Spain
Phone: +34 943 30 85 68
E-mail: nelizalde@vivebiotech.com
Website: www.vivebiotech.com

BOOTH NUMBER: 604

VIVEbiotech is a company fully specialized in lentiviral vectors with two areas of expertise:
GMP CDMO: working with European and USA-based companies, our main aim is adapting to technical and calendar-related requirements.

Innovation: advanced producer cell lines development, new pseudo-typing strategies and the stable non-integrative lentiviral vector (LENTISOMA) among others.

Waisman Biomanufacturing
1500 Highland Ave.
Madison, WI 53705
Phone: (608) 262-9547
Fax: (608) 263-5725
E-mail: info@gmpbio.org
Website: www.gmpbio.org

BOOTH NUMBER: 202

Waisman Biomanufacturing specializes in manufacturing a wide range of biotherapeutics for human Phase I/II clinical trials including viral vector gene therapeutics, plasmid DNA, and many other products. Services include process development through cGMP production with fill/finish capabilities and full quality system support including CMC support for IND filings.

Wilson Wolf Corp.
33 5th Avenue NW
Suite 800
Saint Paul, MN 55112
Phone: (651) 628-9259
Fax: (651) 628-9507
E-mail: info@wilsonwolf.com
Website: www.wilsonwolf.com

BOOTH NUMBER: 407

Wilson Wolf developed "G-Rex" technology to create the most practical cell production platform possible for Adoptive Cell Therapy. G-Rex devices save material and labor while simultaneously shortening production time. Stop by our booth to learn about our scale up and scale out plans in addition to customized options for specialized requirements.

Yecuris
15055 SW Sequoia Pkwy, Suite 130
Portland, OR 97224
Phone: (503) 352-4663
Fax: (503) 352-4811
E-mail: johnbial@yecuris.com
Website: www.yecuris.com

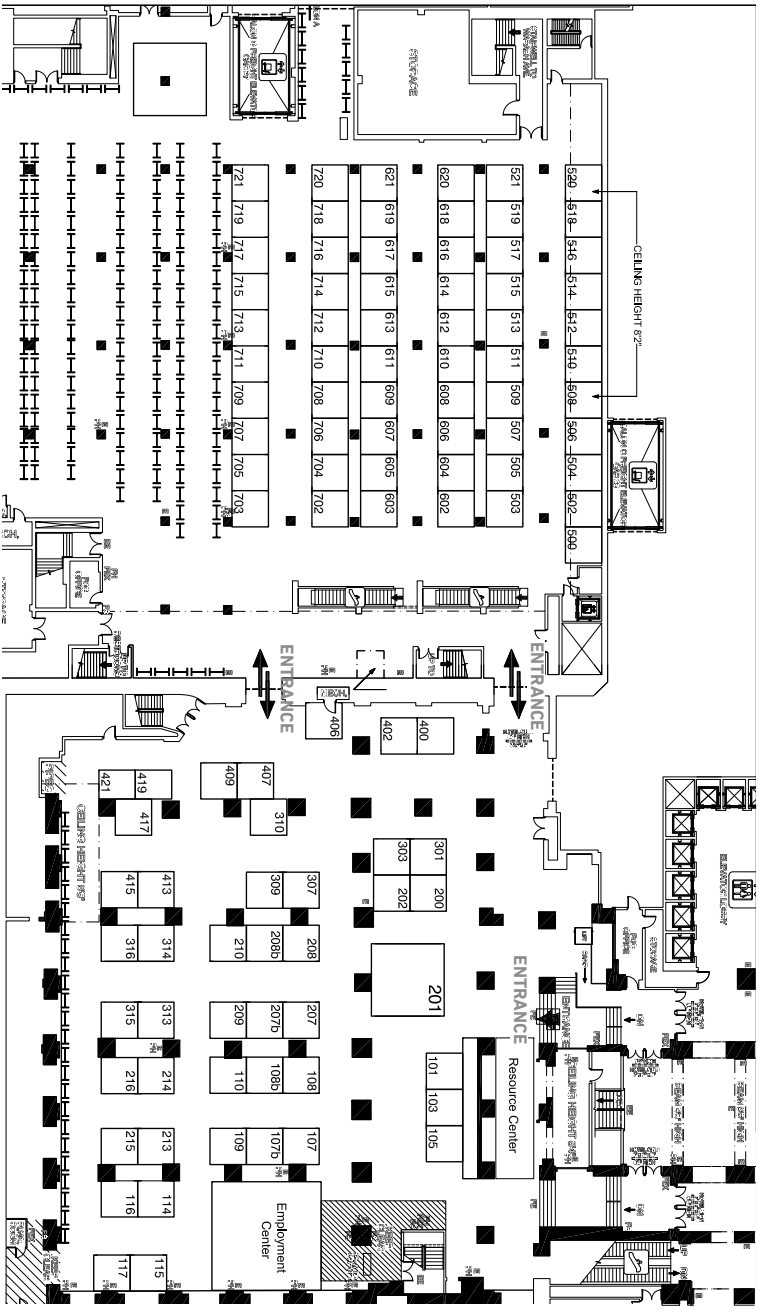
BOOTH NUMBER: 215

Yecuris helps researchers validate their approach with human relevance. We offer a complete platform, from in vitro development & optimization to *in vivo* validation. Whether you're building innovative gene & cell therapies or testing your small molecule for metabolism or toxicology, researchers have trusted the FRG® KO platform for over a decade.

EXHIBIT HALL FLOOR PLAN

ASGCT 21ST ANNUAL MEETING

May 16-19, 2018 • Hilton Chicago - Stevens Salons C & D



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ASGCT 21st Annual Meeting
Chicago • May 16-19, 2018

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ASGCT
22nd ANNUAL
MEETING
WASHINGTON, DC
April 29 – May 2, 2019
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AMERICAN SOCIETY OF GENE & CELL THERAPY
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