AMERICAN SOCIETY of GENE & CELL THERAPY

American Society of Gene & Cell Therapy

22nd Annual Meeting

Final Program Guide

ASGCT
22nd ANNUAL MEETING

WASHINGTON, D.C.
April 29 – May 2, 2019

Washington, D.C., USA
Washington Hilton
www.asgct.org
#ASGCT19

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 2019

ORAL PRESENTATIONS

MON • APRIL 29, 11:00 AM
Cell Specific Transdction of a Vectorized Anti-Tau Antibody Using IV Dosing of a Blood Brain Barrier Penetrant AAV Capsid in Mice
Session: Tools, Delivery and Neuro Capsids (Monroe Room)

MON • APRIL 29, 11:30 AM
Intraparenchymal Spinal Cord Delivery of AAV Gene Therapy Provides Robust SOD1 Knockdown in Large Mammal Spinal Cord for the Treatment of SOD1-ALS
Session: Tools, Delivery and Neuro Capsids (Monroe Room)

MON • APRIL 29, 11:45 AM
Targeted in vivo Biopanning of AAV Capsid Libraries Using Cell Type-Specific RNA Expression
Session: Directed Evolution of AAV Vectors I (Georgetown Room)

MON • APRIL 29, 11:45 AM
Significant reduction of huntingtin gene expression in cortex, putamen and caudate of large mammals with combined putamen and thalamus infusions of VY-HTT01, an AAV gene therapy targeting huntingtin for the treatment of Huntington's disease
Session: Gene Silencing Approaches (IBR West Room)

TUE • APRIL 30, 5:00 PM
Evaluation of Tropism and Transduction Efficiency of AAV Variants in the CNS of NHP Using DNA/RNA Barcode-seq Technology
Session: Rational Engineering of AAV Vectors II (Georgetown Room)

POSTER PRESENTATIONS

MON • APRIL 29, 5:00 PM
Multiple novel engineered AAV capsids demonstrate enhanced brain and spinal cord gene transfer after systemic administration in adult mice (P240)
Session: Neurologic Diseases (Columbia Hall)

MON • APRIL 29, 5:00 PM
Stability of rAAV Vectors: Response to Various Biochemical and Biophysical Stresses (P342)
Session: Vector and Cell Engineering, Production or Manufacturing (Columbia Hall)

TUE • APRIL 30, 5:00 PM
Variability analysis of qPCR, ddPCR and potency assays for AAV vectors: implications for future development (P448)
Session: RNA Virus Vectors (Columbia Hall)

WED • MAY 1, 5:00 PM
Development of a High Cell Density Perfusion method for Baculovirus Infected Insect Cells (BIICs) manufacturing (P726)
Session: AAV Vectors III (Columbia Hall)

WED • MAY 1, 5:00 PM
Characterization of AAV Percentage of Full Capsids and Comparability Across Platforms (P743)
Session: AAV Vectors III (Columbia Hall)

WED • MAY 1, 5:00 PM
Optimization and Evaluation of two Potency Assays for AAV based Gene Silencing programs (P745)
Session: AAV Vectors III (Columbia Hall)

WED • MAY 1, 5:00 PM
Viral Clearance for rAAV Products in a Sf9/Baculovirus Manufacturing Process (P899)
Session: Vector and Cell Engineering, Production or Manufacturing II (Columbia Hall)

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A WELCOME FROM MICHELE CALOS, PHD

Dear Colleagues,

On behalf of the American Society of Gene & Cell Therapy (ASGCT), it is my pleasure to welcome you to the ASGCT 22nd Annual Meeting in Washington, DC. 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 wonderful 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 seven Education Sessions. In addition, join me in welcoming former ASGCT President and Director of the Center for Cell Engineering at Memorial Sloan-Kettering Cancer Center, Dr. Michel Sadelain, who will present the George Stamatoyannopoulos Lecture on Tuesday. On Wednesday morning, the presentation of the Outstanding Achievement Award to Dr. John Rossi will occur followed by Dr. George Church on Wednesday afternoon presenting the Presidential lecture. We are also thrilled to share 1,000 abstracts featuring groundbreaking scientific and clinical advances across the breadth of our field that will be presented as oral presentations or at poster sessions. I look forward to the Annual Meeting program with an immense sense of excitement and pride in our field.

On Wednesday evening, I am delighted to invite you to join the ASGCT leadership and me in celebrating the Society in our closing night reception. The reception takes place in the National Portrait Gallery of the Smithsonian Institute, where we will enjoy live music and dancing as well full access to the museum's collections including the Orchids, American Presidents, and Henrietta Lacks exhibitions.

Be sure to visit our Exhibit Hall to find out about the products and services offered by more than 100 partner companies, via conversations at their booths and more formal presentations in the Tools & Technologies Forum.

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

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,

Michele Calos

Michele Calos, PhD
President, ASGCT

DOWNLOAD THE ASGCT APP

SEARCH "ASGCT" ON GOOGLE PLAY OR THE APPLE APP STORE

Navigate the 22nd Annual Meeting like a pro with all-new ASGCT mobile app, powered by TripBuilder Media:

- Build your schedule and bookmark exhibitors
- Read all abstracts presented at the Annual Meeting
- Receive up-to-the-minute updates, including room and schedule changes
- Optimize your visit with local recommendations

Sponsored by: editas MEDICINE

App support:
info@tripbuildermedia.com

STAY CONNECTED ON ASGCT WI-FI

SAVE MOBILE DATA BY LOGGING ON THROUGH COMPLIMENTARY ANNUAL MEETING WI-FI

NETWORK: ASGCT2019

PASSWORD: annualmeeting

Sponsored by: Spark THERAPEUTICS

GENERAL MEETING INFORMATION

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 22nd Annual Meeting have been published in the May supplement of *Molecular Therapy*. Attendees are able to access a copy of the supplement online at the *Molecular Therapy* website, or have access to the abstracts through the Mobile App.

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 Washington, DC.

BUSINESS CENTER (FEDEX OFFICE)

The Business Center (FedEx Office) is located in the Washington Hilton. It is open Monday – Friday from 7:00 am to 7:00 pm and on Saturday from 9:00 am – 3:00 pm. The Business Center is closed on Sundays.

BUSINESS MEETING

Join the Society's leadership for a business meeting and continental breakfast.
Thursday, May 2, 2019
7:30 am – 8:00 am
A light continental breakfast will be offered at 7:15 am
Room: Monroe

CAMERA/RECORDING POLICY

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.

GENERAL MEETING INFORMATION

CONCIERGE DESK

Washington, DC 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 Washington Hilton, with Hilton staff who can help you with your city needs and reservations.

CONTINUING MEDICAL EDUCATION

CME credit is not available for the ASGCT 22nd Annual Meeting.

DISCLOSURE

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

EDUCATIONAL METHODS AND MATERIALS

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

EDUCATIONAL OBJECTIVES

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

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

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

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

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

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.

GENERAL MEETING INFORMATION

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 22nd Annual Meeting will begin on the morning of Monday, April 29, 2019 and continue through 12:15 PM on Thursday, May 2, 2019. Exhibits will be open Monday, April 29 through Wednesday, May 1.

JOB BOARD

At the 22nd Annual Meeting there will be a physical job board for academic institutions and companies to post open positions to meeting attendees. If you have open positions, bring a printed copy of your job posting to the meeting. If you would like more information about this service, please contact Samantha Kay at skay@asgct.org. The Job Board will be located on the Terrace Level.

EXHIBITS

The Exhibit hall is located in Columbia Hall of the Washington Hilton. 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. 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 Monday, April 29th through Wednesday, May 1st.

MONDAY, APRIL 29

Exhibit Hall Hours	10:00 am – 6:00 pm
Coffee Social	10:00 am – 10:30 am
Coffee Social	3:00 pm – 3:30 pm
Welcome Reception & Poster Session I	5:00 pm – 6:00 pm

TUESDAY, APRIL 30

Exhibit Hall Hours	10:00 am – 6:00 pm
Coffee Social	10:00 am – 10:45 am
Coffee Social	3:00 pm – 3:30 pm
Networking Reception & Poster Session II	5:00 pm – 6:00 pm

WEDNESDAY, MAY 1

Exhibit Hall Hours	10:00 am – 6:00 pm
Coffee Social	10:00 am – 10:45 am
Coffee Social	3:15 pm – 3:45 pm
Networking Reception & Poster Session III	5:00 pm – 6:00 pm

GUEST ATTENDANCE

ASGCT asks registered attendees to refrain from taking children, spouses, or guests to any session or functions offered at the 22nd Annual Meeting.

GENERAL MEETING INFORMATION

INTERNET ACCESS

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

Network: **ASGCT2019**

Password: **annualmeeting**

LEAD RETRIEVAL

A lead retrieval system has been made available to all exhibitors of the ASGCT 22nd Annual Meeting. Exhibitors may ask to scan attendee name badges with a hand held scanner in order to obtain attendee contact information.

LOCATION

The 22nd Annual Meeting will be held at the Washington Hilton Hotel, 1919 Connecticut Ave NW, Washington, DC 20009.

Exhibits will take place in Columbia Hall.

Posters will take place in Columbia Hall.

Registration will be located at the Registration Desk on the Terrace Level of the Washington Hilton 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 22nd Annual Meeting program, including session schedules, faculty, exhibits, sponsors and abstracts, is available through the ASGCT Mobile App which can be accessed by searching "ASGCT" on the Apple App Store or Google Play Store to download.

NO SMOKING

Smoking is prohibited at all 22nd Annual Meeting sessions and events.

POSTERS

Abstract Posters will be on display in Columbia Hall.

MONDAY, APRIL 29

ABSTRACT POSTER SESSION I

12:00 pm – 1:00 pm	Poster Setup by Authors
1:00 pm – 5:00 pm	Poster Viewing (authors present from 5:00 pm – 6:00 pm)
5:00 pm – 6:00 pm	Welcome Reception and Poster Session I
6:00 pm – 6:30 pm	Authors Remove Posters

TUESDAY, APRIL 30

ABSTRACT POSTER SESSION II

7:30 am – 10:00 am	Poster Setup by Authors
10:00 am – 5:00 pm	Poster Viewing (authors present from 5:00 pm – 6:00 pm)
5:00 pm – 6:00 pm	Networking Reception and Poster Session II
6:00 pm – 6:30 pm	Authors Remove Posters

WEDNESDAY, MAY 1

ABSTRACT POSTER SESSION III

7:30 am – 10:00 am	Poster Setup by Authors
10:00 am – 5:00 pm	Poster Viewing (authors present from 5:00 pm – 6:00 pm)
5:00 pm – 6:00 pm	Networking Reception and Poster Session III
6:00 pm – 6:30 pm	Authors Remove Posters

CALL4POSTERS® PICK-UP SCHEDULE

Once again this year ASGCT arranged 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.

Sunday, April 28	12:00 pm – 8:00 pm
Monday, April 29	7:00 am – 6:00 pm
Tuesday, April 30	7:00 am – 6:00 pm
Wednesday, May 1	7:00 am – 7:00 pm
Thursday, May 2	Closed

PRESS

Members of the working media may register for the 22nd Annual Meeting in the Press Room – Embassy Room. 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 the Embassy Room.

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.

GENERAL MEETING INFORMATION

REGISTRATION DESK

Name badges, final programs, and registration bags will be distributed at the registration desk located on the Terrace Level of the Washington Hilton Hotel.

REGISTRATION DESK HOURS

Sunday, April 28	12:00 pm – 8:00 pm
Monday, April 29	7:00 am – 6:00 pm
Tuesday, April 30	7:00 am – 6:00 pm
Wednesday, May 1	7:00 am – 7:00 pm
Thursday, May 2	Closed

CANCELLATION REFUND POLICY

Refund requests should have been submitted in writing to the ASGCT Executive Office by April 15, 2019.

WASHINGTON, DC INFORMATION

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

SPEAKER READY DESK

The Speaker Ready Desk is located in the Coats Room on the Terrace Level of the Washington Hilton Hotel.

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 Monday of the Annual Meeting to check-in at the Speaker Ready Desk on Sunday, April 28 to avoid congestion.

Avoid delays and check-in early!

SPEAKER READY DESK HOURS

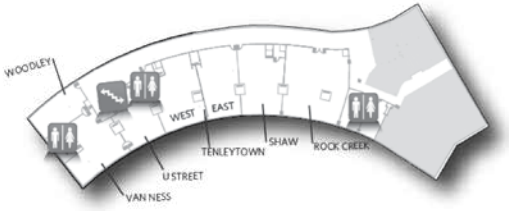
Sunday, April 28	5:00 pm – 8:00 pm
Monday, April 29	7:00 am – 6:00 pm
Tuesday, April 30	7:00 am – 6:00 pm
Wednesday, May 1	7:00 am – 7:00 pm
Thursday, May 2	7:00 am – 12:00 pm

SPECIAL ACCESSIBILITY NEEDS

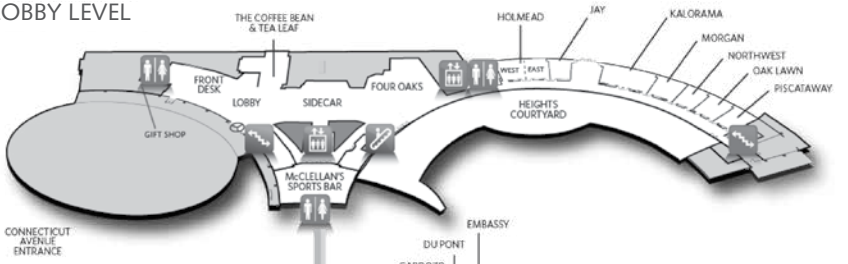
If you require special accommodations under the ADA in order to fully participate in the meeting, please visit the Registration Desk; ASGCT staff will be happy to assist you with your specific needs.

HOTEL FLOOR PLAN

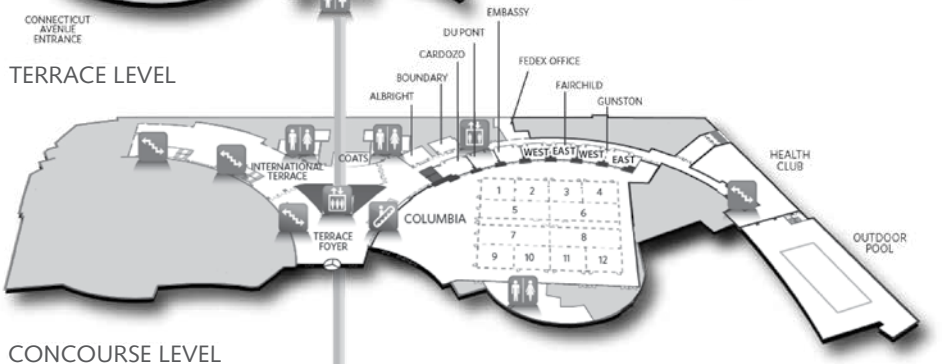
FIRST FLOOR MEETING SPACE



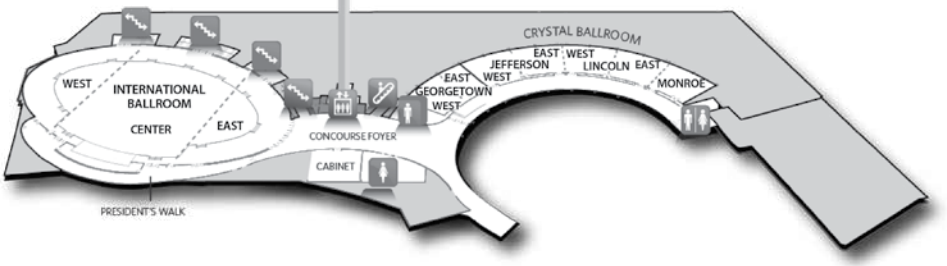
LOBBY LEVEL



TERRACE LEVEL

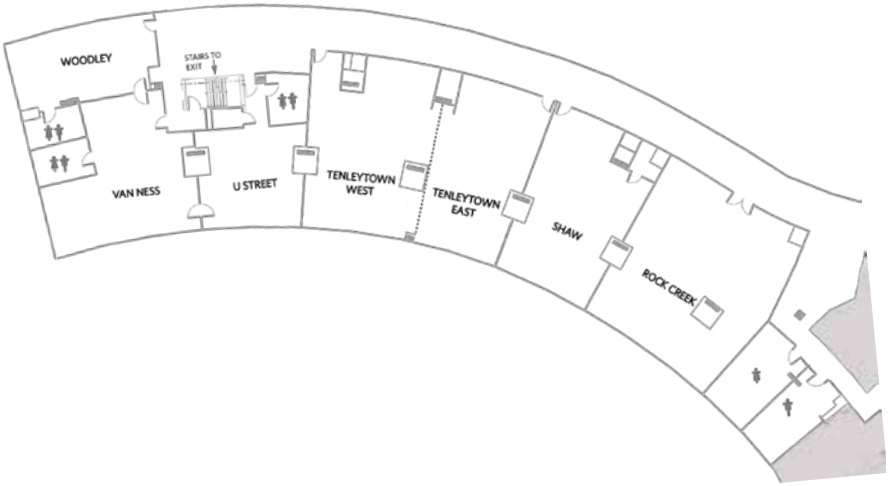


CONCOURSE LEVEL



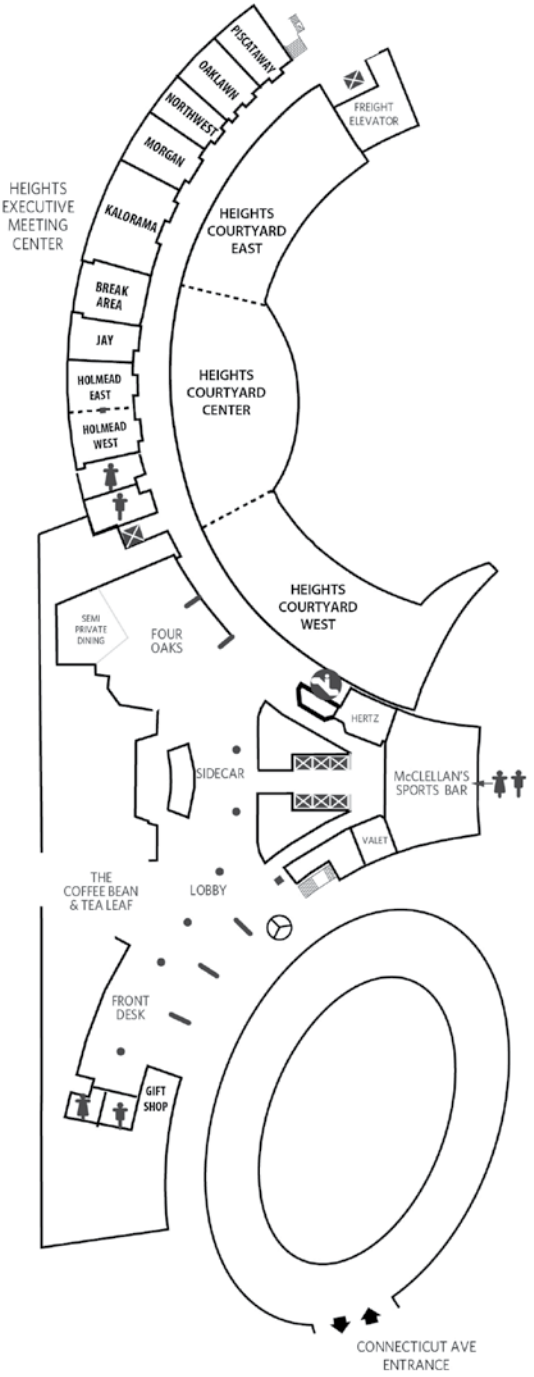
HOTEL FLOOR PLAN

FIRST FLOOR



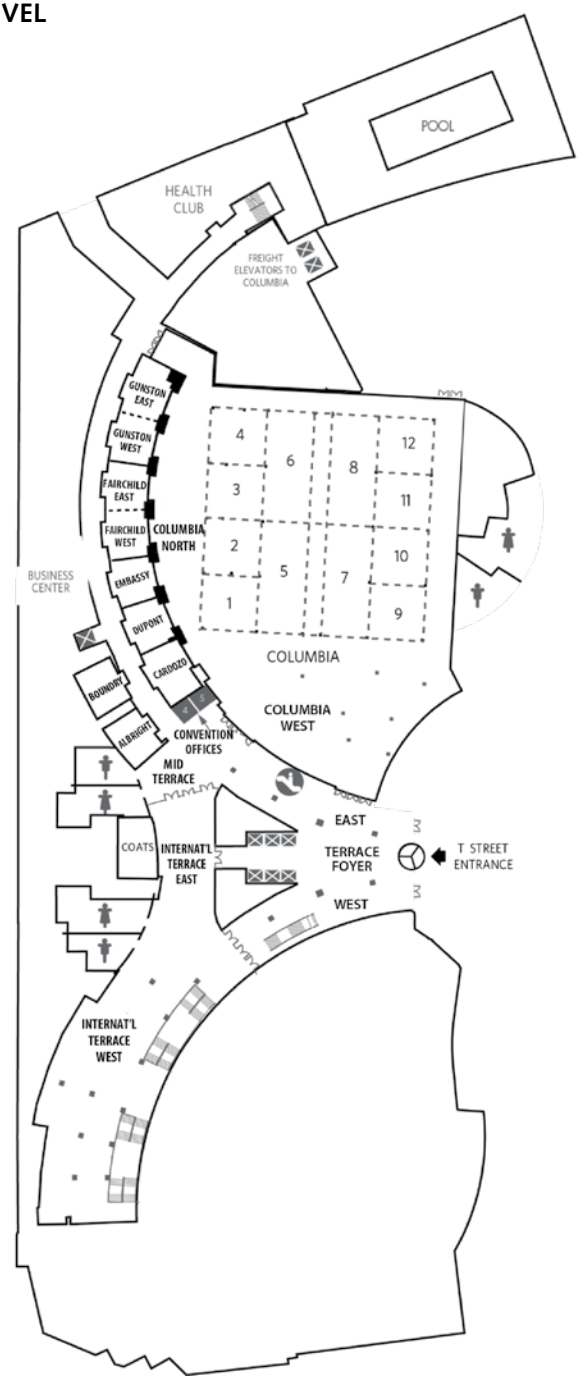
HOTEL FLOOR PLAN

LOBBY LEVEL



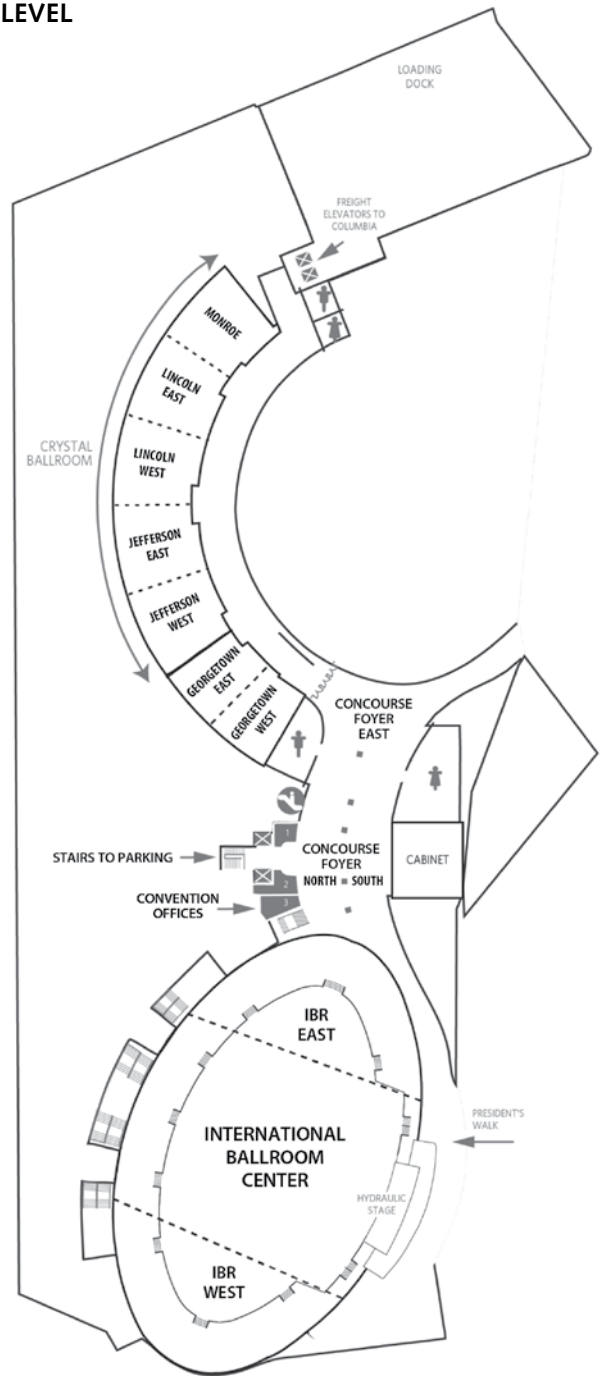
HOTEL FLOOR PLAN

TERRACE LEVEL



HOTEL FLOOR PLAN

CONCOURSE LEVEL



2019 ASGCT PROGRAM COMMITTEE

Thank you to the following individuals for serving on the ASGCT Program Committee and helping with the overall development of the ASGCT 22nd Annual Meeting.

Chair
Michele P. Calos, PhD
Stanford University School of Medicine

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

Philip D. Gregory, DPhil
bluebird bio

Markus Grompe, MD
Oregon Health & Science University

David V. Schaffer, PhD
University of California Berkeley

2019 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
Michele P. Calos, PhD – *Stanford University School of Medicine*

ASGCT President-Elect
Guangping Gao, PhD – *University of Massachusetts Medical School*

ASGCT Secretary and Abstract Chair
David V. Schaffer, PhD – *University of California Berkeley*

ASGCT Program Committee Member
Markus Grompe, MD – *Oregon Health & Science University*

ASGCT Abstract Category Chair
Beverly L. Davidson, PhD – *Children's Hospital of Philadelphia*

ABSTRACT REVIEWERS

Thank you to our 2019 abstract reviewers for their time and expertise!

RNA VIRUS VECTORS
Jennifer E. Adair, PhD - *Fred Hutchinson Cancer Research Center*
Christian Brendel, PhD - *Boston Children's Hospital/Dana-Farber Cancer Institute*
Bernhard Rudolf Gentner, MD - *Ospedale San Raffaele*
Donald B. Kohn, MD - *University of California, Los Angeles*
Axel Schambach, MD, PhD - Hannover Medical School
Bruce E. Torbett, PhD, MSPH - *The Scripps Research Institute*

AAV VECTORS
Dongsheng Duan, PhD - *University of Missouri School of Medicine*
Steven J. Gray, PhD - *University of Texas Southwestern*
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*
Jude J. Samulski, PhD - *Bamboo Therapeutics, Inc.*

ADENOVIRUS AND OTHER DNA VIRUSES
David T. Curiel, MD, PhD - *Washington University Medical School*
Imre Kovesdi, PhD - *DNATriX, Inc.*
Ko Mitani, PhD - *Saitama Medical University*
Robin J. Parks, PhD - *Ottawa Hospital Research Institute*
Dmitry M. Shayakhmetov, PhD - *Emory University*

GENE TARGETING AND GENE CORRECTION
Paula M. Cannon, PhD - *University of Southern California*
Shondra M. Miller, PhD - *St. Jude Children's Research Hospital*
Luigi M. Naldini, MD, PhD - *San Raffaele Telethon Institute for Gene Therapy*
Mark J. Osborn, PhD - *University of Minnesota*
Matthew H. Porteus, MD, PhD - *Stanford University School of Medicine*
Angela S. Whatley, PhD - *Food and Drug Administration*

OLIGONUCLEOTIDE THERAPEUTICS
Dirk Grimm, PhD - *Heidelberg University Hospital*
Shuo Gu - *National Cancer Institute*
Mark A. Kay, MD, PhD - *Stanford University School of Medicine*
John J. Rossi, PhD - *Beckman Research Institute City of Hope*
Bruce Sullenger, PhD - *Duke University Medical Center*
Paul N. Valdmanis, PhD - *University of Washington*

SYNTHETIC/MOLECULAR CONJUGATES AND PHYSICAL METHODS FOR DELIVERY OF GENE THERAPEUTICS
Joseph G. Hacia, PhD - *University of Southern California*
Richard Heller, PhD - *Old Dominion University*
Kenya Kamimura, MD, PhD - *Niigata University*
Dexi Liu, PhD - *University of Georgia College of Pharmacy*
Carol H. Miao, PhD - *University of Washington*
Kevin G. Rice, PhD - *University of Iowa College of Pharmacy*
Assem G. Ziady, PhD - *Cincinnati Children's Hospital Medical Center*

ABSTRACT REVIEWERS

METABOLIC, STORAGE, ENDOCRINE, LIVER AND GASTROINTESTINAL DISEASES

Nicola Brunetti-Pierri, MD - *Telethon Institute of Genetics and Medicine*
Randy J. Chandler, PhD, MB - *National Institutes of Health*
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*
Charles P. Venditti, MD, PhD - *National Human Genome Research Institute*

CARDIOVASCULAR AND PULMONARY DISEASES

Rishi Arora, MD - *Northwestern Medical Group*
Saumya Das, MD, PhD - *Massachusetts General Hospital*
J. Kevin Donahue, MD - *University of Massachusetts School of Medicine*
H. Kirk Hammond, MD - *UCSD and VA San Diego*
Sarah Wootton, PhD - *University of Guelph*

NEUROLOGIC DISEASES (INCLUDING OPHTHALMIC AND AUDITORY DISEASES)

Alberto Auricchio, MD - *Telethon Institute of Genetics & Medicine*
Beverly L. Davidson, PhD - *Children's Hospital of Philadelphia*
Jane Farrar, PhD - *Trinity College Dublin*
Guangping Gao, PhD - *University of Massachusetts Medical School*
Jeffrey H. Kordower, PhD - *Rush University Medical Center*
Jodi L. McBride, PhD - *Oregon Health and Science University*

MUSCULO-SKELETAL DISEASES

Carsten G. Bonnemann, MD - *NINDS/NIH*
Scott Q. Harper, PhD - *Ohio State University & Nationwide Children's Hospital*
Chunping Qiao, PhD - *REGENXBIO Inc.*
Kathryn R. Wagner, MD, PhD - *The Johns Hopkins School of Medicine*
Bing Wang, MD, PhD - *University of Pittsburgh School of Medicine*
Xiao Xiao, PhD - *University of North Carolina at Chapel Hill*

CANCER - IMMUNOTHERAPY, CANCER VACCINES

Chiara Bonini, MD - *Fondazione Centro S. Raffaele*
Maria G. Castro, PhD - *University of Michigan School of Medicine*
Ann M. Leen, PhD - *Baylor College of Medicine - CAGT*
Sarwish Rafiq, PhD - *Memorial Sloan Kettering Cancer Center*
Barbara Savoldo, MD, PhD - *UNC Lineberger Comprehensive Cancer Center*
Irene Scarfo, PhD - *Massachusetts General Hospital*

CANCER - ONCOLYTIC VIRUSES

Caroline Breitbach, PhD - *Turnstone Biologics*
Evanthia Galanis, MD, DSc - *Mayo Clinic*
Paola Grandi, PhD - *University of Pittsburgh*
Terry W. Hermiston, PhD - *Bayer HealthCare LLC*
David Kirn, MD - *4D Molecular Therapeutics*
Liliana Maruri Avidal, PhD - *Ignite Immunotherapy*

ABSTRACT REVIEWERS

CANCER - TARGETED GENE AND CELL THERAPY

Prasad S. Adusumilli, MD - *Memorial Sloan Kettering Cancer Center*
Noriyuki Kasahara, MD, PhD - *University of Miami School of Medicine*
Richard G. Vile, PhD - *Mayo Clinic*
Masato Yamamoto, MD, PhD - *University of Minnesota*
Dmitriy Zamarin, MD, PhD - *Memorial Sloan Kettering Cancer Center*

HEMATOLOGIC AND IMMUNOLOGIC DISEASES

Luca Biasco, PhD - *DFCI BCH Cancer and Blood Disorders Center*
Jennifer L. Gori, PhD - *Obsidian Therapeutics*
Suk See De Ravin, MD, PhD - *National Institutes of Health, NIAID*
Cynthia Dunbar, MD - *NIH/NHLBI Hematology Branch*
Federico Mingozzi, PhD - *INSERM, UPMC, and Genethon*
John Pasi, PhD - *Queen Mary University of London*

IMMUNOLOGICAL ASPECTS OF GENE THERAPY AND VACCINES

Brian D. Brown, PhD - *Mt. Sinai School of Medicine*
Alessio Cantore, PhD - *San Raffaele Telethon Institute for Gene Therapy*
Conrad Russell Y. Cruz, MD, PhD - *Children's National Medical Center*
Roland W. Herzog, PhD - *Indiana University*
Denise Sabatino, PhD - *The Children's Hospital of Philadelphia*
Brandon K. Wilder - *Oregon Health & Science University*

CELL THERAPIES

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Andre Larochelle, MD, PhD - *National Institutes of Health*
Jane S. Lebkowski, PhD - *Asterias Biotherapeutics, Inc.*
Carolyn Lutzko, PhD - *Cincinnati Children's Hospital Medical Center*
Jan A. Nolta, PhD - *University of California Davis*

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PHARMACOLOGY/TOXICOLOGY STUDIES OR ASSAY DEVELOPMENT

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SCHEDULE AT A GLANCE

MONDAY, APRIL 29, 2019

8:00 am – 10:00 am	Scientific Symposia Session I & Oral Abstract Session I
10:00 am – 10:30 am	Exhibit Hall Coffee Social
10:00 am – 12:00 pm	Tools and Technologies Forum
10:00 am – 6:00 pm	Exhibit Hall Open
10:30 am – 12:00 pm	Oral Abstract Session II
12:00 pm – 1:30 pm	Lunch Break (Lunch Not Provided)
1:30 pm – 3:00 pm	Education Sessions & Oral Abstract Session III
3:00 pm – 3:30 pm	Exhibit Hall Coffee Social
3:30 pm – 5:15 pm	Oral Abstract Session IV
4:00 pm – 6:00 pm	Tools and Technologies Forum
5:00 pm – 6:00 pm	Exhibit Hall Welcome Reception & Poster Session I - *Reception Sponsored by USP*
6:00 pm – 7:30 pm	Industry Evening Symposium - *Sponsored by GE Healthcare*
6:00 pm – 8:00 pm	Reconnection & Mentoring Event - *Sponsored by Rocket Pharmaceuticals Inc.*

TUESDAY, APRIL 30, 2019

8:00 am – 10:00 am	Scientific Symposia Session II & Education Session & Oral Abstract Session V
10:00 am – 10:45 am	Exhibit Hall Coffee Social
10:00 am – 6:00 pm	Exhibit Hall Open
10:45 am – 12:00 pm	George Stamatoyannopoulos Memorial Lecture & Presentation of the Excellence in Research Awards - *Sponsored by Sanofi Genzyme*
12:00 pm – 1:30 pm	Lunch Break (Lunch Not Provided)
12:00 pm – 1:30 pm	Industry Symposia - *Sponsored by MaxCyte, Miltenyi Biotec Inc., Retrogenix Limited, Terumo BCT*
1:30 pm – 3:00 pm	Outstanding New Investigator Symposium - *Sponsored by Burroughs Wellcome Fund*
3:00 pm – 3:30 pm	Exhibit Hall Coffee Social
3:30 pm – 5:15 pm	Oral Abstract Session VI
4:00 pm – 6:00 pm	Tools and Technologies Forum
5:00 pm – 6:00 pm	Exhibit Hall Networking Reception & Poster Session II
6:00 pm – 7:00 pm	New Member Reception - *Sponsored by AveXis*
6:00 pm – 7:30 pm	Industry Evening Symposium - *Sponsored by Pall Biotech*

WEDNESDAY, MAY 1, 2019

8:00 am – 10:00 am	Scientific Symposia Session III & Oral Abstract Session VII
10:00 am – 10:45 am	Exhibit Hall Coffee Social
10:00 am – 6:00 pm	Exhibit Hall Open
10:45 am – 11:45 am	Outstanding Achievement Award Lecture & Sonia Skarlatos Public Service Award Presentation - *Sponsored by Audentes Therapeutics*
11:45 am – 1:15 pm	Lunch Break (Lunch Not Provided)
11:45 am – 1:15 pm	Industry Symposia - *Sponsored by Sarepta Therapeutics, Legally Mine*
1:15 pm – 3:15 pm	Presidential Symposium & Presentation of the Top Abstracts - *Sponsored by Oxford BioMedica*
3:15 pm – 3:45 pm	Exhibit Hall Coffee Social
3:45 pm – 5:30 pm	Oral Abstract Session VIII
4:00 pm – 6:00 pm	Tools and Technologies Forum
5:00 pm – 6:00 pm	Poster Session III
6:00 pm – 7:30 pm	Industry Evening Symposium - *Sponsored by Audentes Therapeutics*
8:00 pm – 11:00 pm	Closing Night Reception - *Sponsored by University of Massachusetts Medical School*

THURSDAY, MAY 2, 2019

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

Trainee Lounge

7:00 am – 5:00 pm

Room: Fairchild

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 East

Entering the Post-RAC Era Charting the Course of Cell & Gene Therapy Drug Development for the Future - Organized by the Bio Industry Liaison Committee

CO-CHAIRS: Gwendolyn K. Binder, PhD and Marcela V. Maus, MD, PhD

SPEAKERS

8:00 AM – 8:30 AM
Katherine A. High, MD. Spark Therapeutics, Philadelphia, PA
Gene Therapy Comes of Age: Lessons from Clinical Development Programs and from Commercialization

8:30 AM – 9:00 AM
Raj Puri, MD, PhD. FDA/CBER, Silver Spring, MD
FDA Regulation of Cell and Gene Therapies: Facilitating Advanced Manufacturing

9:00 AM – 9:30 AM
Richard T. Maziarz, MD. Oregon Health & Science University, Portland, OR
The View from the Ground Floor: The Costs of Care of Gene & Cellular Therapy

9:30 AM – 10:00 AM
Panel Discussion

Scientific Symposium 101

8:00 AM - 10:00 AM

Room: International Ballroom West

Innovation in First Time in Human Study Clinical Studies - Organized by the International Committee

CO-CHAIRS: Claire Booth, MBBS, PhD and Juan A. Bueren, PhD

SPEAKERS

8:00 AM – 8:30 AM
Federico Mingozzi, PhD. Spark Therapeutics, Genethon, INSERM, Evry, France
Progress and Challenges Toward the Development of a Liver-targeted Gene Therapy for Crigler-Najjar Syndrome

8:30 AM – 9:00 AM
Christian J. Buchholz, PhD. Paul-Ehrlich-Institut, Langen, Germany
Translating Gene Therapy Medicinal Products into Early Clinical Trials

9:00 AM – 9:30 AM
Michela Gabaldo, MSc. Fondazione Telethon, Milan, Italy, SR-TIGET, San Raffaele-Telethon Institute for Gene Therapy, Milan, Italy
Perspective for Commercializing Gene Therapy, Developing and Continuing Successful Partnerships, and Ensuring Patient Access - What Type of Considerations at FTIH Stage?

9:30 AM – 10:00 AM
Philippe Duchateau, PhD. Cellectis, Paris, France
Universal Gene-Edited CAR T-Cell Immunotherapy

Scientific Symposium 102

8:00 AM - 10:00 AM
Room: Monroe

Non-viral Vectors and Immunity - Organized by the Nanoagents & Synthetic Formulations Committee

CO-CHAIRS: Chantal Pichon, PhD and Assem G. Ziady, PhD

SPEAKERS

8:00 AM – 8:30 AM
Stephen C. Hyde, PhD. University of Oxford, Oxford, United Kingdom
Phase IIb Multiple Dose, Non-Viral, Cystic Fibrosis Gene Transfer Clinical Trial

8:30 AM – 9:00 AM
Julie Champion, PhD. Georgia Tech School of Chemical and Biomolecular Engineering, Atlanta, GA
Protein Nanoparticle Engineering in Vaccine Development

9:00 AM – 9:30 AM
James A. Williams, PhD. Nature Technology Corporation, Lincoln, NE
Non-Viral Vector Design and Production

9:30 AM – 10:00 AM
Jacob J. Elmer, PhD. Villanova University, Villanova, PA
Investigating the Relationship between the Host Cell Transcriptome and Transfection Efficiency

Scientific Symposium 103

8:00 AM - 10:00 AM
Room: Georgetown

Novel Strategies for Lung and GI Tract-directed Genetic Therapies - Organized by the Respiratory & GI Tract Gene & Cell Therapy Committee

CO-CHAIRS: Maria P. Limberis, PhD and Patrick L. Sinn, PhD

SPEAKERS

8:00 AM – 8:30 AM
John F. Engelhardt, PhD. University of Iowa Carver College of Medicine, Iowa City, IA
Pharmacologic Rescue and Gene Therapy Endpoints in Ferret Models of Cystic Fibrosis

8:30 AM – 9:00 AM
Maria P. Limberis, PhD. University of Pennsylvania, Philadelphia, PA
Development of an AAV-based Prophylactic Vaccine for Influenza

9:00 AM – 9:30 AM
Paul Harmatz, MD. UCSF Benioff Children's Hospital, Oakland, CA
Clinical Trials of Genome-editing (ZFN) for MPS I and MPS II

9:30 AM – 10:00 AM
David W. Parsons, PhD. Univ of Adelaide and Women's and Children's Hospital, Adelaide, Australia
Lentivirus-mediated Gene Transfer in Stem Cells for Correction of the Cystic Fibrosis Defect in Airway

Scientific Symposium 104

8:00 AM - 10:00 AM
Room: Jefferson

Pricing, Access, and Value of Gene Therapies: What Are the Real-World Challenges and Solutions? - Organized by the Government Relations Committee

CO-CHAIRS: Francesca Cook, MPH and Timothy D. Hunt, JD

SPEAKERS

8:00 AM – 8:30 AM
Steve Miller, MD. Express Scripts, Saint Louis, MO
Real World Challenges: Considerations for Pricing and Value

8:30 AM – 9:00 AM
Michael S. Sherman, MD. Harvard Pilgrim Health Care, Wellesley, MA
Real-World Challenge: Innovative Value-Based Agreements - Opportunities and Challenges

9:00 AM – 9:30 AM
Joseph W. La Barge, JD. Spark Therapeutics, Inc., Philadelphia, PA
Real-World Challenge: High Upfront Costs for Payers; Solution: Payment Over Time, Proposed Direct to Payer Billing

9:30 AM – 10:00 AM
Mark W. Skinner, JD. Institute for Policy Advancement Ltd, Washington, DC, McMaster University, Hamilton, ON, Canada
Real-World Challenge: Establishing the Value of Gene Therapy for Patients; Solution: Patient Involvement in Core Outcomes Assessment of Comparative Effectiveness and Value for Hemophilia

Scientific Symposium 105

8:00 AM - 10:00 AM
Room: Lincoln

Clinical Trials Spotlight

CO-CHAIRS: Xandra O. Breakefield, PhD and Harry L. Malech, MD

SPEAKERS

8:00 AM – 8:20 AM

1: LentiGlobin Gene Therapy in Patients with Sickle Cell Disease: Updated Interim Results from HGB-206

John Tisdale, NHLBI/NIDDK, National Institutes of Health, Bethesda, MD

8:20 AM – 8:40 AM

2: Ex-Vivo Gene Therapy for Hurler Disease: Initial Results from a Phase I/II Clinical Study

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

8:40 AM – 9:00 AM

3: Insertion Site Analyses on Liquid Biopsies Reveal the Clonal Repertoire and Early Premalignant Expansions Hidden in Solid Tissues

Daniela Cesana, San Raffaele Telethon Institute for Gene Therapy, Milan, Italy

9:00 AM – 9:20 AM

4: Preliminary Results from Cohorts 1 and 2 of CAPtivate: A Phase 1/2 Clinical Trial of AAV8-Mediated Liver-Directed Gene Therapy in Adults with Late-Onset OTC Deficiency

George Diaz, Icahn School of Medicine at Mount Sinai, New York, NY

9:20 AM – 9:40 AM

5: Dominant Negative PD1 Armored CART Cells Induce Remission in Refractory Diffuse Large B Cell Lymphoma (DLBCL) Patients

Tong Chen, Department of Hematology, Huashan Hospital, Fudan University, Shanghai, China

9:40 AM – 10:00 AM

6: Mechanism of Action of Toca 511 and 5-FC in Animal Models and Cancer Patients

Douglas Jolly, Tocagen Inc, San Diego, CA

Oral Abstract Session 110

8:00 AM - 10:00 AM

Room: Heights Courtyard 2

Advances in Genome Editing and Hemophilia Gene Therapies

CO-CHAIRS: Federico Mingozzi, PhD and Ben Kleinstiver, PhD

8:00 AM – 8:15 AM

7: Barcoded Clonal Tracking of CRISPR-Cas9 and rAAV6-Mediated Gene Targeting in Human Hematopoietic Stem and Progenitor Cells

Daniel Dever, Stanford University, Stanford, CA

8:15 AM – 8:30 AM

8: Large-Scale CRISPR-Cas Genome-Wide Activity Profiling in Human Primary T-Cells Reveals Genetic and Epigenetic Determinants of Off-Target Effects

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

8:30 AM – 8:45 AM

9: CRISPR/Cas9-Mediated Homology Independent Targeted Integration in Human Hematopoietic Stem and Progenitor Cells

Hanan Bloomer, NHLBI/NIH, Bethesda, MD

8:45 AM – 9:00 AM

10: Human Hematopoietic Stem and Progenitor Cells that Have Undergone Nuclease-Induced Homology Directed Repair with an AAV Delivered Donor Template Exhibit Engraftment Deficiencies in Mouse Xenotransplant Studies
Madhumita Mahajan, Bluebird Bio, Cambridge, MA

9:00 AM – 9:15 AM

11: CRISPR/Cas9-Mediated Targeted Insertion of Human F9 Achieves Therapeutic Circulating Protein Levels in Mice and Non-Human Primates
Jonathan Finn, Intellia Therapeutics, Cambridge, MA

9:15 AM – 9:30 AM

12: B Cell Depletion with Anti-mCD20 Eliminates FVIII Memory B Cells in Inhibitor Positive Mice and Enhances AAV8-coF8 ITI When Combined with Rapamycin
David Markusic, IUSM, Indianapolis, IN

9:30 AM – 9:45 AM

13: Non-Genotoxic Anti-CD117 Immunotoxin Conditioning Facilitates Hematopoietic Stem Cell Transplantation Gene Therapy for Hemophilia A
Athena Russell, Emory University, Atlanta, GA

9:45 AM – 10:00 AM

14: Insertional Oncogenesis in X-CGD Patient after MFGS Retroviral Vector-Mediated Gene Therapy
Toru Uchiyama, National Center for Child Health and Development, Tokyo, Japan

Oral Abstract Session 111

8:00 AM - 10:00 AM

Room: Heights Courtyard 1

AAV Vectors and Disease Targets I

CHAIR: David Schaffer, PhD

9:15 AM – 9:30 AM

15: Regulatory and Exhausted T Cell Detection after Intramuscular AAV1 Delivery in Nonhuman Primates and Human
Gwladys Gernoux, UMass Medical School, Worcester, MA

8:15 AM – 8:30 AM

16: Recombinant Adeno-Associated Virus (rAAV) Expressing a Pan-Hemagglutinin (HA) Antibody Protects Mice Against Influenza
Renald Gilbert, National Research Council Canada, Montreal, QC, Canada

8:30 AM – 8:45 AM

17: Spinal Subpial Delivery of AAV9 Produces a Potent, Long-Lasting Block of Neuraxial Degeneration and Disease Manifestation in Adult Mice by Silencing an ALS-Causing Mutant Gene
Mariana Bravo-Hernandez, Anesthesiology, Ludwig Institute, Salk insittute, UCSD, La Jolla, CA

8:45 AM – 9:00 AM

18: Early Diagnosis and Speed to Effect in Spinal Muscular Atrophy Type 1 (SMA1)

Omar Dabbous, Avexis, Inc., Bannockburn, IL

9:00 AM – 9:15 AM

19: Preclinical Gene Therapy with scAAV9/AGA in Aspartylglucosaminuria Mice Provides Evidence for Clinical Translation

Xin Chen, UTSW Medical Center, Dallas, TX

8:00 AM – 8:15 AM

20: Positive Cohort 1 Results from the Phase 1/2 Trial with AAV8-Mediated Liver-Directed Gene Therapy in Adults with Glycogen Storage Disease Type Ia

David Weinstein, University of Connecticut and Connecticut Children's Medical Center, Hartford, CT

9:30 AM – 9:45 AM

21: Systemic AAV-Mediated Gamma-Sarcoglycan Therapy for Treatment of Muscle Deficits in LGMD2C Mice

Eric Pozsgai, The Research Institute at Nationwide Children's Hospital, Columbus, OH

Oral Abstract Session 112

8:00 AM - 10:00 AM

Room: Heights Courtyard 3

Gene Therapy for Metabolic Disorders: New Approaches

CHAIR: Gerald Lipshutz, MD

8:00 AM – 8:15 AM

23: sAAV-Mediated CPS1 Expression Rescues CPS1 Deficiency in Adult Conditional Knock Out Mice

Matthew Nitzahn, UCLA, Los Angeles, CA

8:15 AM – 8:30 AM

24: ImmTOR™ Tolerogenic Nanoparticles Enhance Transgene Expression after Both Initial and Repeat Dosing in a Mouse Model of Methylmalonic Acidemia Treated with an Anc80 AAV Vector

Petr Ilyinskii, Selecta Biosciences, Watertown, MA

8:30 AM – 8:45 AM

25: Development of a Novel AAV-Based Therapy in Combination with Tolerogenic ImmTOR Nanoparticles for a Sustained Treatment of Ornithine Transcarbamylase Deficiency

Giulia De Sabbata, International Center for Genetic Engineering and Biotechnology, ICGEB, Trieste, Italy

8:45 AM – 9:00 AM

26: Treatment of Metabolic Disorders Using Lipid Nanoparticle (LNP)-Encapsulated Messenger RNA Therapeutics (MRT)

Frank DeRosa, Translate Bio, Lexington, MA

9:00 AM – 9:15 AM

27: Liver-Directed Lipid Nanoparticle mRNA Therapy Improves Survival and Reduces Serum Branched Chain Amino Acids in a Mouse Model of Maple Syrup Urine Disease

Jenny Greig, University of Pennsylvania, Philadelphia, PA

9:15 AM – 9:30 AM

28: Salmeterol with Liver Depot Gene Therapy Reversed Biochemical and Autophagic Abnormalities of Skeletal Muscle in Pompe Disease

Sang-oh Han, Duke University Medical School, Durham, NC

9:30 AM – 9:45 AM

29: Correction of Glycogen Storage Disease Type III with an AAV Vector Encoding a Bacterial Glycogen Debranching Enzyme

Jeong-A Lim, Duke University School of Medicine, Durham, NC

9:45 AM – 10:00 AM

30: Genome Editing with CRISPR/Cas9 in a GSD Ia Canine Model

Hye Ri Kang, Duke University, Durham, NC

Exhibit Hall Coffee Social

10:00 AM - 10:30 AM

Room: Columbia Hall

Tools and Technologies Forum 1

10:00 AM – 12:00 PM

Room: Cardozo

CO-CHAIRS: Isabelle Riviere, PhD and Eduard Ayuso, DVM, PhD

SPEAKERS

10:00 AM – 10:15 AM

Clive Glover, PhD. Pall Biotech, Portsmouth, United Kingdom

Upstream Industrialized Processes for Viral Vector Production

10:15 AM – 10:30 AM

Jon Petrone, MS. Pall Biotech, Westborough, MA

Downstream Industrialized Processes for Viral Vector Production

10:30 AM – 10:45 AM

Don Healey, PhD. KBI Biopharma, The Woodlands, TX

KBI Biopharma – How we can turn your process into a product

10:45 AM – 11:00 AM

Michael L. Roberts, PhD. Synpromics Ltd, Edinburgh, United Kingdom

Mastering Gene Control to Enable the Next Generation of Gene Medicines

11:00 AM – 11:15 AM

Luca Alberici, PhD, MBA. MolMed, SpA, Milan, Italy

MolMed CDMO Services

11:15 AM – 11:30 AM

Klaus Kuehlcke, PhD. BioNTech IMFS GmbH, Idar-Oberstein, Germany

Optimised T cell Transduction based on Protransduzin

11:30 AM – 11:45 AM
Karen Hinson-Rehn. Presenting on behalf of Nordmark Biochemicals, Uetersen, Germany
Nordmark - Your Partner for Translational Collagenase

11:45 AM – 12:00 PM
Colleen Stubbs, MS. Houston Methodist, Houston, TX
Enabling Novel Therapeutics with mRNA

Exhibit Hall Open

10:00 AM - 6:00 PM
Room: Columbia Hall

Oral Abstract Session 120

10:30 AM - 12:00 PM
Room: Monroe

Tools, Delivery and Neuro Capsids

CHAIR: Lamya Shihabuddin, PhD

10:30 AM – 10:45 AM
31: A Safe and Reliable Technique for Central Nervous System Delivery of AAV Vectors via the Cisterna Magna
T Taghian, University of Massachusetts Medical School, Worcester, MA

10:45 AM – 11:00 AM
32: Highly Efficient Transduction of the Fovea Following 'Extrafoveal' Subretinal Administration of Novel AAV Vectors
Shannon Boye, University of Florida, Gainesville, FL

11:00 AM – 11:15 AM
33: Cell Specific Transduction of a Vectorized Anti-Tau Antibody Using IV Dosing of a Blood Brain Barrier Penetrant AAV Capsid in Mice
Wencheng Liu, Voyager Therapeutics, Cambridge, MA

11:15 AM – 11:30 AM
34: mGAP: A Resource to Identify Nonhuman Primate Models of Human Genetic Diseases
Betsy Ferguson, Oregon National Primate Research Center, Beaverton, OR

11:30 AM – 11:45 AM
35: Intraparenchymal Spinal Cord Delivery of AAV Gene Therapy Provides Robust SOD1 Knockdown in Large Mammal Spinal Cord for the Treatment of SOD1-ALS
Holger Patzke, Voyager Therapeutics, Cambridge, MA

11:45 AM – 12:00 PM
36: Intrathecal and Intravenous Combination Gene Therapy in the Mouse Model of Infantile Neuronal Ceroid Lipofuscinosis Extends Lifespan and Improves Behavioral Outcomes in Moderately Affected Mice
Erik Lykken, UT Southwestern, Dallas, TX

Oral Abstract Session 121

10:30 AM - 12:00 PM

Room: Lincoln

Gene Therapy in Large Animal Models

CO-CHAIRS: Kathryn Wagner, MD, PhD and Carsten Bonnemann, MD

10:30 AM – 10:45 AM

37: Safety and Biodistribution Assessment of Scaav2.5-Eqil-1RA Gene Transfer to a Large Mammalian Joint

Rachael Levings, University of Florida, Gainesville, FL

10:45 AM – 11:00 AM

38: Gene Editing Restores Dystrophin Expression in a Canine Model of Duchenne Muscular Dystrophy

Leonela Amoasii, Exonics Therapeutics, Watertown, MA

11:00 AM – 11:15 AM

39: SGT-001 Cardiac and Skeletal Muscle Microdystrophin Expression and Functional Efficacy in Preclinical Models of DMD

J Gonzalez, Solid Biosciences, Cambridge, MA

11:15 AM – 11:30 AM

40: In Vivo Correction of Dystrophin Expression in Old Dystrophic Dogs

Niclas Bengtsson, University of Washington, Seattle, WA

11:30 AM – 11:45 AM

41: Utrophin Vector Protected by Central Tolerance as Potential Cure for Muscular Dystrophy

Leon Morales, University of Pennsylvania, Philadelphia, PA

11:45 AM – 12:00 PM

42: Restoration of Dystrophin Expression by Genome Editing in the Canine X-linked Muscular Dystrophy (CXMD) with a Mutation in the N-Terminal Mutation Hotspot, a Dog Model of Duchenne Muscular Dystrophy

Rika Maruyama, University of Alberta, Edmonton, AB, Canada

Oral Abstract Session 122

10:30 AM - 12:00 PM

Room: Georgetown

Directed Evolution of AAV Vectors I

CHAIR: Phillip Tai, PhD

10:30 AM – 10:45 AM

43: Development of Efficient AAV Vectors Using iTransduce, an Expression-Based AAV Selection System

Killian Hanlon, Massachusetts General Hospital, Boston, MA

10:45 AM – 11:00 AM

44: Cell Type-Specific TRAnscription-Dependent Directed Evolution (TRADE) Identifies Novel AAV Capsids Capable of Enhanced Neuronal Transduction in Mice and Non-Human Primates

Samuel Huang, OHSU, Portland, OR

11:00 AM – 11:15 AM

45: A Novel Adeno Associated Virus Capsid Variant selected on Human Islets Shows Robust Transduction in Many Cell Types In Vitro and In Vivo
Katja Pekrun, Stanford Medical School, Stanford, CA

11:15 AM – 11:30 AM

46: Using Novel AAV Capsids to Maximize Gene Delivery Throughout the Rhesus Macaque Brain
Jodi McBride, Oregon National Primate Research Center, Beaverton, OR

11:30 AM – 11:45 AM

47: Development of Novel AAV Variants with High Retinal Transduction Efficiency
Stylianos Michalakis, Ludwig-Maximilians-University Munich, Munich, Germany

11:45 AM – 12:00 PM

48: Targeted In Vivo Biopanning of AAV Capsid Libraries Using Cell Type-Specific RNA Expression
Mathieu Nonnenmacher, Voyager Therapeutics, Cambridge, MA

Oral Abstract Session 123

10:30 AM - 12:00 PM

Room: Jefferson

Clinical Gene Therapies for Blood Diseases

CO-CHAIRS: Suk See De Ravin, MD, PhD and Brian Beard, PhD

10:30 AM – 10:45 AM

49: Gene Therapy for the Treatment of Adult and Pediatric Patients Affected by Transfusion Dependent BETA-Thalassemia
Giuliana Ferrari, San Raffaele Telethon Institute for Gene Therapy (SR-TIGET), Milano, Italy

10:45 AM – 11:00 AM

50: Gene Therapy for Sickle Cell Disease (SCD) Using RVT-1801 Lentivirus Vector and Arulite Reduced Intensity Conditioning Transplant Shows Promising Correction of the Disease Phenotype
Punam Malik, CCHMC, Cincinnati, OH

11:00 AM – 11:15AM

51: Preliminary Conclusions Obtained in Fanconi Anemia Patients Treated by Lentiviral-Mediated Gene Therapy after 2 Years of Follow-Up
Paula Rio, Centro de Investigaciones Energéticas Medioambientales y Tecnológicas and Centro de Investigación Biomédica en Red de Enfermedades Raras (CIEMAT/CIBERER), Madrid, Spain

11:15 AM – 11:30 AM

52: Genome-Wide Assessment of Lentiviral Integration Sites of Gene-Corrected Llympho-Hematopoietic Cells in FA-A Patients
Ning Wu, German Cancer Research Center and National Center for Tumor Diseases, Heidelberg, Germany

11:30 AM – 11:45 AM

53: A Diversity of Human Hematopoietic Differentiation Programs Identified through In Vivo Tracking of Hematopoiesis in Gene Therapy Patients
Emmanuelle Six, Paris Descartes–Sorbonne Paris Cité University, Imagine Institute, Paris, France

11:45 AM – 12:00 PM

54: Restoration of Cellular and Humoral Immunity by Targeted Gene Correction of T Cells as a Treatment for X-Linked Lymphoproliferative Disease (XLP1)
Benjamin Houghton, UCL Great Ormond Street Institute of Child Health, London, United Kingdom

Oral Abstract Session 124

10:30 AM - 12:00 PM

Room: International Ballroom East

Oncolytic Viruses I

CO-CHAIRS: David Kirn, MD and Noriyuki Kasahara, MD, PhD

10:45 AM – 11:00 AM

56: Phase I Clinical Trial of Intravenous Administration of VSV-IFNb-NIS in Patients with Hematologic Malignancies
Nandakumar Packiriswamy, Mayo Clinic, Rochester, MN

11:00 AM – 11:15 AM

57: A Dual Blockade of Death and Immune Checkpoints by SPG-275 Induces Complete Remissions in Treatment-Refractory Tumors
Kelvin Tsai, Stempodia Corporation, Ltd., Hillsborough, CA

11:15 AM – 11:30 AM

58: Oncolytic Virotherapy with Recombinant Measles Virus Induces Tumor Antigen-Specific T-Cell Responses
Nandakumar Packiriswamy, Mayo Clinic, Rochester, MN

11:30 AM – 11:45 AM

59: Oncolytic Measles Virotherapy Increases the Potency of aPD1 Checkpoint Therapy to Establish Long Term Antitumor Immunity in Solid Tumors
Eleni Panagioti, Mayo Clinic, Rochester, MN

11:45 AM – 12:00 PM

55: Intravenous Phase I Study of VSV-IFNb-NIS in Patients with Metastatic or Recurrent Stage III or IV Endometrial Cancer
Kah-Whye Peng, Mayo Clinic, Rochester, MN

Oral Abstract Session 125

10:30 AM - 12:00 PM

Room: International Ballroom West

Gene Silencing Approaches

CHAIR: Beverly Davidson, PhD

10:30 AM – 10:45 AM

61: Combinatorial Gene Therapy for Spinocerebellar Ataxia Type 1
Megan Keiser, The Children's Hospital of Philadelphia, Philadelphia, PA

10:45 AM – 11:00 AM

62: Evaluation of Programmable Zinc Finger Protein Transcription Factors for the Efficient Reduction of Tau in the Nonhuman Primate Brain
Bryan Zeitler, Sangamo Therapeutics, Inc, Richmond, CA

11:00 AM – 11:15 AM

63: Repression of mHTT Expression in Huntington 's Disease Mouse Models by AAV-Mediated Expression of Zinc-Finger Protein-Repressor Transgene
Vivian Choi, Shire, Cambridge, MA

11:15 AM – 11:30 AM

64: Astrocyte Transduction is Required for Rescue of Behavioral Phenotypes in the YAC128 Mouse Model with AAV-RNAi Mediated HTT Lowering Therapeutics
Lisa Stanek, Sanofi, Framingham, MA

11:30 AM – 11:45 AM

65: The New Epigenome-Editing Approach for Targeting Dysregulated SNCA Expression: Novel Target Validation for Next-Generation Drug Discovery
Ornit Chiba-Falek, Duke University Medical Center, Durham, NC

11:45 AM – 12:00 PM

66: Significant Reduction of Huntingtin Gene Expression in Cortex, Putamen and Caudate of Large Mammals with Combined Putamen and Thalamus Infusions of VY-HTT01, an AAV Gene Therapy Targeting Huntingtin for the Treatment of Huntington's Disease
Pengcheng Zhou, Voyager Therapeutics, Inc, Cambridge, MA

Oral Abstract Session 126

10:30 AM - 12:00 PM

Room: Heights Courtyard 2

CNS Disorders

CHAIR: Randy Chandler, PhD, MB

10:30 AM – 10:45 AM

67: Liver-Directed Gene Therapy Clinical Trial for Mucopolysaccharidosis Type VI
Nicola Brunetti-Pierri, TIGEM, Pozzuoli, Italy

10:45 AM – 11:00 AM

68: A Clinical Glucocerebrosidase Lentiviral Vector Corrects the Pathology and Clinical Signs in a Mouse Model for Type 1 Gaucher Disease
Stefan Karlsson, Lund University, Lund, Sweden

11:00 AM – 11:15 AM

69: Gene Editing to Treat Both Tay-Sachs and Sandhoff Diseases
Li Ou, University of Minnesota, Minneapolis, MN

11:15 AM – 11:30 AM

70: Sustained Long-Term Neurological Correction Following Haematopoietic Stem Cell Gene Therapy in Mucopolysaccharidosis IIIB Mice

Stuart Ellison, University of Manchester, Manchester, United Kingdom

11:30 AM – 11:45 AM

71: Infant Macaques Exhibit Safe and Sustained Expression of Human Iduronidase after Receiving an Intrathecal Cervical Injection of Adeno-Associated Virus 9

Juliette Hordeaux, University of Pennsylvania, Philadelphia, PA

11:45 AM – 12:00 PM

72: Systemic mRNA Therapy as a Treatment for the Inherited Metabolic Liver Disorder Arginase Deficiency

Brian Truong, UCLA, Los Angeles, CA

Oral Abstract Session 127

10:30 AM - 12:00 PM

Room: Heights Courtyard 1

Development of Manufacturing Processes for Cell Based Therapies

CHAIR: Carolyn Lutzko, PhD

10:30 AM – 10:45 AM

73: Combining CD5-Targeted Chimeric Antigen Receptor Engineering and Genetic Editing of TGF-BR2 for the Treatment of T-Cell Hematologic Malignancies

Rafet Basar, MD Anderson Cancer Center, Houston, TX

10:45 AM – 11:00 AM

74: Single Day CAR Manufacturing Platform Using mRNA and Flow Electroporation Technology

Robert Keefe, MaxCyte Inc, Gaithersburg, MD

11:00 AM – 11:15 AM

75: Titratable and Reversible Regulation of Therapeutic Proteins in Cell and Gene Therapies Using FDA Approved Drugs and a Modular Protein Stabilization Platform

Vipin Suri, Obsidian Therapeutics, Cambridge, MA

11:15 AM – 11:30 AM

76: 3D Microculture Platform Enables Advanced, High-Throughput Screening for Differentiation of hPSC-Derived Cell Therapies

Riya Muckom, UC Berkeley, Berkeley, CA

11:30 AM – 11:45 AM

77: Establishing cGMP Manufacturing of CRISPR/Cas9-Edited Human CAR T Cells

Susan Zabierowski, Memorial Sloan Kettering Cancer Center, New York, NY

11:45 AM – 12:00 PM

78: Reprogramming Human Immune Responses Using Ex Vivo MSC Bioreactor Therapy

Rita Barcia, Sentien Biotechnologies, Lexington, MA

Oral Abstract Session 128

10:30 AM - 12:00 PM

Room: Heights Courtyard 3

Pulmonary Gene Therapy

CO-CHAIRS: Alisha Gruntman, DVM, PhD and Sarah Wootton, BSc, PhD

10:30 AM – 10:45 AM

79: Assessment of a Second Generation, Oxidation Resistant Gene Therapy for Alpha 1-Antitrypsin Deficiency
Katie Stiles, Weill Cornell Medical College, New York, NY

10:45 AM – 11:00 AM

80: Lentivirus Gene Therapy for Autoimmune Pulmonary Alveolar Proteinosis
Helena Palau, Imperial College and UK CF Gene Therapy Consortium, London, United Kingdom

11:00 AM – 11:15 AM

81: Gene Correction of Cystic Fibrosis Mutations In Vitro and In Vivo Mediated by PNA Nanoparticles
Alexandra Piotrowski-Daspit, Yale University, New Haven, CT

11:15 AM – 11:30 AM

82: Delivery of AAV-CFTR to Bronchial Epithelial Cells from Cystic Fibrosis Patients Augments Functional Recovery of Chloride Conductance
Shen Lin, Talee Bio, Inc, Philadelphia, PA

11:30 AM – 11:45 AM

83: Systemic in Utero Gene Editing as a Treatment for Cystic Fibrosis
Adele Ricciardi, Yale University, New Haven, CT

11:45 AM – 12:00 PM

84: Development of Genetic Tool for Testing Cftr Gene Targeting in Pigs
Zhichang (Peter) Zhou, University of Toronto, Toronto, ON, Canada

Break

12:00 PM - 1:30 PM

Lunch Break (On Own - Not Provided)

Education Session 130

1:30 PM - 3:00 PM

Room: International Ballroom West

AAV Vectors

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

SPEAKERS

1:30 PM – 2:00 PM

Steven J. Gray, PhD. University of Texas Southwestern Medical Center, Dallas, TX
Basics of AAV Gene Therapy

2:00 PM – 2:30 PM
Dirk Grimm, PhD. Heidelberg University Hospital, Heidelberg, Germany
Next-generation AAV Vectors

2:30 PM – 3:00 PM
Anne Galy, PhD. Genethon, Evry, France
Immunological Considerations of rAAV Gene Therapy

Education Session 131

1:30 PM - 3:00 PM
Room: International Ballroom East

Adoptive T cell Therapy

CHAIR: Catherine M. Bollard, MD

SPEAKERS

1:30 PM – 2:00 PM
Catherine M. Bollard, MD. The George Washington University, Washington, DC
Multi-antigen Specific T cells

2:00 PM – 2:30 PM
Stephen Gottschalk, MD. St Jude Children's Research Hospital, Memphis, TN
CAR T cells

2:30 PM – 3:00 PM
Steven Rosenberg, MD, PhD. National Cancer Institute, Bethesda, MD, National Institutes of Health, Bethesda, MD
Cellular Immunotherapy for Solid Tumors

Education Session 132

1:30 PM - 3:00 PM
Room: Monroe

Induced Pluripotent Stem Cells

CO-CHAIRS: Blythe D. Sather, PhD and Jizhong Zou, PhD

SPEAKERS

1:30 PM – 2:00 PM
Michael A. Laflamme, MD, PhD. McEwen Stem Cell Institute, Toronto, ON, Canada
Pluripotent Stem Cells for Heart Disease

2:00 PM – 2:30 PM
Andre Larochelle, MD, PhD. National Institutes of Health, Bethesda, MD
Generation of Hematopoietic Stem and Progenitor Cells from iPSCs

2:30 PM – 3:00 PM
Bob Valamehr, PhD. Fate Therapeutics, Inc., San Diego, CA
Pluripotent Cell-Derived T and NK Cells as a Cornerstone Approach for Off-the-Shelf Cancer Immunotherapy

Education Session 133

1:30 PM - 3:00 PM

Room: Jefferson

Modalities of Genome Editing

CO-CHAIRS: John F. Tisdale, MD and Thomas Wechsler, PhD

SPEAKERS

1:30 PM – 2:00 PM
Thomas Wechsler, PhD. Grace Science, LLC, San Francisco, CA
DNA Nucleases in Gene Editing - "No need for a break?"

2:00 PM – 2:30 PM
Akihiko Kondo, PhD. RIKEN Center for Sustainable Resource Science, Kanagawa, Japan
Base Editing Therapeutics -"Targeted Nucleotide Editing using Hybrid Prokaryotic and Vertebrate Adaptive Immune Systems

2:30 PM – 3:00 PM
Lauren E. Woodard, PhD. Vanderbilt University/VA, Nashville, TN
***piggyBac* Transposons to Cut and Paste DNA**

Education Session 134

1:30 PM - 3:00 PM

Room: Georgetown

NK cells Versus iNKT cells

CO-CHAIRS: Leonid S. Metelitsa, MD, PhD and Katy Rezvani, MD, PhD

SPEAKERS

1:30 PM – 2:00 PM
Leonid S. Metelitsa, MD, PhD. Baylor College of Medicine, Houston, TX
Harnessing Naturaland Engineered Properties of iNKT cells for Adoptive Cancer Immunotherapy

2:00 PM – 2:30 PM
Gianpietro Dotti, MD. UNC Lineberger Cancer Center, Chapel Hill, NC
Redirecting Antigen Specificity of Natural Killer T cells

2:30 PM – 3:00 PM
Sarah Cooley, MD. Fate Therapeutics, San Diego, CA
Clincial Development of NK Cell Cancer Therapy

Education Session 135

1:30 PM - 3:00 PM

Room: Lincoln

Oncolytics

CO-CHAIRS: Tanja Meyer, PhD and Paola Grandi, PhD

SPEAKERS

1:30 PM – 2:00 PM
Howard Kaufman, MD. Replimune Inc., Woburn, MA
Next Generation Oncolytic Immunotherapy

2:00 PM – 2:30 PM
Kah-Whye Peng, PhD. Mayo Clinic, Rochester, MN
Theranostics-Guided Oncolytic Virotherapy

2:30 PM – 3:00 PM
John C. Bell, PhD. Ottawa Hospital Research Institute, Ottawa, ON, Canada
Going Viral with Cancer Immunotherapy

Oral Abstract Session 140

1:30 PM - 3:00 PM
Room: Heights Courtyard 2

Gene Editing for Musculo-Skeletal and Skin Diseases

CHAIR: Scott Harper, PhD

1:30 PM – 1:45 PM
85: DUX4 mRNA Silencing with CRISPR-Cas13 Gene Therapy as a Prospective Treatment for Facioscapulohumeral Muscular Dystrophy
Afrooz Rashnonejad, Research institute at Nationwide Children's Hospital, Columbus, OH

1:45 PM – 2:00 PM
86: Long-Term Evaluation of AAV-CRISPR Genome Editing for Duchenne Muscular Dystrophy
Christopher Nelson, Duke University, Durham, NC

2:00 PM – 2:15 PM
87: CRISPR/Cas9-Base Editing Mediated Correction for Recessive Dystrophic Epidermolysis Bullosa
Mark Osborn, University of Minnesota, Minneapolis, MN

2:15 PM – 2:30 PM
88: Excise-and-Replace Strategy Using AAV-Mediated Delivery of CRISPR/Cas9 and HITI Donor Restores Full-Length Dystrophin Expression in Human Cardiomyocyte Model of Duchenne Muscular Dystrophy
Sabrina Sun, University of California, Berkeley, Berkeley, CA

2:30 PM – 2:45 PM
89: DNA Base Editing to Modulate mRNA Splicing as a Therapeutic Strategy for Duchenne Muscular Dystrophy
Veronica Gough, Duke University, Durham, NC

2:45 PM – 3:00 PM
90: Allele-Specific Gene Correction Using CRISPR-Cas9 in Compound Heterozygote SGCAmutations
Simone Spuler, Charité Universitätsmedizin Berlin, Berlin, Germany

Oral Abstract Session 141

1:30 PM - 3:00 PM

Room: Heights Courtyard 1

Cardiovascular Gene Therapy

CHAIR: Eric Adler, MD

1:30 PM – 1:45 PM

91: AAV9.LAMP2B Reverses Metabolic and Physiologic Multiorgan Dysfunction in a Murine Model of Danon Disease
Ana Manso, UCSD, La Jolla, CA

1:45 PM – 2:00 PM

92: Ancestral AAV Delivery Catalyzes Rapid and Robust Myocardial Gene Expression within 72 Hours- A New Frontier for Early Stage Cardiac Disease
Anthony Fargnoli, Mount Sinai School of Medicine, New York, NY

2:00 PM – 2:15 PM

93: First in Human Phase I/Phase II Safety and Preliminary Efficacy Study Using Low Frequency Ultrasound in Addition to Adipose Derived Stem Cells in Patients with Moderate to Severe Lower Extremity Peripheral Arterial Disease
Wilson Wong, Arkansas Heart Hospital, Little Rock, AR

2:15 PM – 2:30 PM

94: MiR-125a-5p Regulates Cardiomyocyte Function and Prevents Pressure-Overload Induced Cardiac Dysfunction
Robin Verjans, Cardiovascular Research Institute Maastricht (CARIM), Maastricht, Netherlands

2:30 PM – 2:45 PM

96: Optimizing AAV Gene Therapy Strategies for Barth Syndrome
Suya Wang, Boston Children's Hospital, Boston, MA

Oral Abstract Session 142

1:30 PM - 3:00 PM

Room: Heights Courtyard 3

Directed Evolution of AAV Vectors II

CHAIR: Casey Maguire, PhD

1:30 PM – 1:45 PM

97: Machine-Guided Design of AAV Capsid Proteins with Experimentally Augmented Evolutionary Data
Sam Sinai, Harvard Medical School, Boston, MA

1:45 PM – 2:00 PM

98: Overcoming Transgene-Related Immunological Confounders in High-Throughput In Vivo AAV Capsid Screens Using Barcoded Non-Coding RNAs
Meiyu Xu, University of Massachusetts Medical School, Worcester, MA

2:00 PM – 2:15 PM

99: Multiplexed-CREATE Selection Yields AAV Vectors Targeting Different Cell Types of the Central Nervous System Following Systemic Delivery
Sripriya Ravindra Kumar, California Institute of Technology, Pasadena, CA

2:15 PM – 2:30 PM

100: Engineering A Humanized AAV8 Capsid through Iterative Structure-Guided Evolution

L Havlik, Duke University, Durham, NC

2:30 PM – 2:45 PM

101: Engineered AAVs for Enhanced Transduction of Submucosal Cells within the Lung Following Intravenous Delivery

Nick Goeden, California Institute of Technology, Pasadena, CA

2:45 PM – 3:00 PM

102: Engineered AAVS for CNS Transduction and Peripheral Organ De-Targeting across Species after Systemic Delivery

Nicholas Flytzanis, California Institute of Technology, Pasadena, CA

Exhibit Hall Coffee Social

3:00 PM - 3:30 PM

Room: Columbia Hall

Oral Abstract Session 150

3:30 PM - 5:15 PM

Room: Jefferson

New Advances in Chemical and Physical Gene Delivery

CO-CHAIRS: Richard Heller, PhD and Joseph Hacia, PhD

3:30 PM – 3:45 PM

103: Heat Shrinking DNA Nanoparticles for In Vivo Gene Delivery to the Liver

Nathan Delvaux, The University of Iowa, Iowa City, IA

3:45 PM – 4:00 PM

104: Engineered Amphiphilic Peptides Promote Delivery of Protein and CRISPR Cargo to Airway Epithelia In Vitro and In Vivo

Sateesh Krishnamurthy, Pappajohn Biomedical Institute, University of Iowa, Iowa City, IA

4:00 PM – 4:15 PM

105: S-Function Relationships of Branched Ester-Amine Quadpolymers for Non-Viral Retinal Gene Therapy

David Wilson, Johns Hopkins University, Baltimore, MD

4:15 PM – 4:30 PM

106: Transcutaneous Ultrasound Mediated Gene Delivery in Canine Liver

Alexander Novokhodko, Seattle Children's, Seattle, WA

4:30 PM – 4:45 PM

107: The Balancing Act between Required Stability and Sufficient Cargo Release: A Systematic Investigation of the Impact of Stabilizing Units within pDNA Lipo-Polyplexes

Simone Hager, LMU Munich, Munich, Germany

4:45 PM – 5:00 PM

108: Characterization of the DNA Nanoparticle Interactome and Potential Targets that Enhance Gene Delivery

Steven Rheiner, Cincinnati Children's Hospital Medical Center, Cincinnati, OH

5:00 PM – 5:15 PM

109: New Clinically Compatible Nanoplexes for Molecular-Genetic Imaging of Human Cancers

Il Minn, Johns Hopkins University, Baltimore, MD

Oral Abstract Session 151

3:30 PM - 5:15 PM

Room: Lincoln

Rational Engineering of AAV Vectors I

CHAIR: Chen Ling, PhD

3:30 PM – 3:45 PM

110: An RNAseq Directed Screening Method to Identify Genes Modulating Titer in rAAV Producing Cell Lines

Nicholas Richards, Ultragenyx Gene Therapy, Cambridge, MA

3:45 PM – 4:00 PM

111: AAV Gene Transfer with Tandem Promoter Design Prevents Anti-Transgene Immunity and Provides Persistent Efficacy in Neonate Disease Mouse Models

Pasqualina Colella, Genethon, UMR_S951 Inserm, Univ Evry, Université Paris Saclay, EPHE, Evry, France

4:00 PM – 4:15 PM

112: Novel AAV1 Mutants for Robust Transduction of the Central Nervous System Following Intravenous Injection

Kazuhiro Takahama, Oregon Health & Science University, Portland, OR

4:15 PM – 4:30 PM

113: Design Principles for AAV Mediated Circular RNA Expression in the Brain

Trevor Gonzalez, Duke University, Durham, NC

4:30 PM - 4:45 PM

114: Cryo-EM, Biophysical and In Vivo Tropism Analyses of a Novel Adeno-Associated Virus Capsid Isolated from Human Tissue

Hung-Lun Hsu, University of Massachusetts Medical School, Worcester, MA

4:45 PM – 5:00 PM

115: Development of AAV Vectors with the Potential to Dampen the Host Humoral Immune Response

Keyun Qing, University of Florida College of Medicine, Gainesville, FL

5:00 PM – 5:15 PM

116: The Potential Role of Surface-Exposed Tyrosine Residues on AAV Capsids in the Diminution of Vector Neutralization by Human Antibodies

Hua Yang, University of Florida College of Medicine, Gainesville, FL

Oral Abstract Session 152

3:30 PM - 5:15 PM

Room: Georgetown

Cancer Adoptive Immunotherapy

CHAIR: Chiara Bonini, MD

3:30 PM – 3:45 PM

117: Tunable IFNa-Based Gene Therapy Inhibits Glioblastoma Multiforme Growth in a New Syngeneic Mouse Model
Filippo Birocchi, SR-TIGET: San Raffaele Telethon Institute for Gene Therapy, Milano, Italy

3:45 PM – 4:00 PM

118: High Avidity T Cell Receptors Redirect Natural Killer T Cell Specificity While Outcompeting the Endogenous Invariant Chain T Cell Receptor
Elisa Landoni, University Of North Carolina, Chapel Hill, NC

4:00 PM – 4:15 PM

119: LAG-3, but Not Tim-3, Disruption in TCR Gene Edited Human Memory Stem T Cells Enhance the Anti Tumor Activity Against Multiple Myeloma
Beatrice Cianciotti, San Raffaele Scientific Institute, Milan, Italy

4:15 PM – 4:30 PM

120: Targeting the Tumour Vasculature with CAR T-Cells for Treatment of Solid Tumours
Mustafa Munye, Cell and Gene Therapy Catapult, London, United Kingdom

4:30 PM – 4:45 PM

121: Rejection-Resistant Off-The-Shelf T Cells for Adoptive Cell Therapy
Feiyan Mo, Baylor College of Medicine, Houston, TX

4:45 PM – 5:00 PM

122: A Novel Approach to Targeting NKG2D Ligands Using Engineered T cells Expressing Dimerizing Agent Regulated Immunoreceptor Complexes (DARIC)
Wai-Hang Leung, bluebird bio, Seattle, WA

5:00 PM – 5:15 PM

123: Exploiting Clonal Tracking of WT1-Specific T Cells to Generate a Library of Tumor-Specific T Cell Receptors (TCR), for TCR Gene Editing of Acute Leukemia
Eliana Ruggiero, San Raffaele Scientific Institute, Milan, Italy

Oral Abstract Session 153

3:30 PM - 5:15 PM

Room: Monroe

Nonclinical Studies and Assay Development

CHAIR: Kathleen Meyer, MPH, PhD, DABT

3:30 PM – 3:45 PM

124: A Novel Non-Integrating DNA Vector for the Persistent Genetic Modification of Embryonic and Hematopoietic Stem Cells
Alicia Roig-Merino, DKFZ, Heidelberg, Germany

3:45 PM – 4:00 PM

125: Failure to Mobilize Peripheral Blood Hematopoietic Stem Cells Upon Readministration of AMD3100 in Nonhuman Primates

Clare Samuelson, Fred Hutchinson Cancer Research Center, Seattle, WA

4:00 PM – 4:15 PM

126: DNA In Situ Hybridization Protocol for the Detection of Integrated Lentiviral Vector in Preclinical and Clinical Samples

James Rottman, Bluebird Bio, Cambridge, MA

4:15 PM – 4:30 PM

127: Biodistribution and Tolerability of HMI-102, a Novel AAVHSC15 Encoding Human Phenylalanine Hydroxylase, in Cynomolgus Monkeys

Teresa Wright, Homology Medicines, Bedford, MA

4:30 PM – 4:45 PM

128: Development of an Objective Image-Based Flow Cytometry Sickling Assay for Quantification of Amelioration of Sickle Cell Disease Phenotype in Erythroid Differentiated CD34+ Cells

Gretchen Lewis, Bluebird Bio, Cambridge, MA

4:45 PM – 5:00 PM

129: The Pi3k/Akt Pathway Impacts Early RAAV Transduction Events and is Activated by Insulin Receptor Signaling

Sean Carrig, St John's University, Queens, NY

5:00 PM – 5:15 PM

130: Comparison of ddPCR and qPCR in Quantification of Advanced Therapy Products in Biological Samples

Shingo Jogasaki, Shin Nippon Biomedical Laboratories, Kagoshima, Japan

Oral Abstract Session 154

3:30 PM - 5:15 PM

Room: International Ballroom East

Measuring and Mitigating Genotoxicity of Genome Editing

CO-CHAIRS: Angela Whatley, PhD and Matthew Porteus, MD, PhD

3:30 PM – 3:45 PM

131: Self Cleaving Guide RNAs for Selective Expansion of Precisely Gene Edited Hepatocytes In Vivo

Amita Tiyaboonchai, Oregon Health and Science University, Portland, OR

3:45 PM – 4:00 PM

132: A Novel Preclinical Genotoxicity Assay, CAST-Seq, Enables New Insights in DNA Repair Dynamics and Chromosomal Aberrations in CRISPR-Cas Edited Human Hematopoietic Stem Cells

Toni Cathomen, Medical Center - University of Freiburg, Freiburg, Germany

4:00 PM – 4:15 PM

133: Optimizing Nuclease Specificity via Catalytic Domain Engineering Enables Complete Gene Modification with No Detectable Off-Targets

Edward Rebar, Sangamo Therapeutics, Richmond, CA

4:15 PM – 4:30 PM

134: Evaluation of Homology-Independent CRISPR-Cas9 Off-Target Assessment Methods

Hemangi Chaudhari, CRISPR Therapeutics, Inc., Cambridge, MA

4:30 PM – 4:45 PM

135: Engineering Potent, Small, Chimeric, Synthetic, RNA-Guided Nucleases (sRGN) from Four Uncharacterized Cas9 Genes

Ashish Gupta, Bayer AG, Cologne, Germany

4:45 PM – 5:00 PM

136: In Vivo Validation and Tracking of CRISPR-Cas9 Off-Targets Predicted In Vitro by CircleSeq in Rhesus Macaques

Aisha AlJanahi, NIH, Bethesda, MD

Oral Abstract Session 155

3:30 PM - 5:15 PM

Room: International Ballroom West

CAR T Cell Therapies for Cancer

CHAIR: Dmitry Shayakhmetov, PhD

3:30 PM – 3:45 PM

138: Control of Natural Killer Cell Expansion, Anti-Tumor Activity and Safety with Small Molecule-Regulated Molecular Switches and Chimeric Antigen Receptors

Henri Bayle, Bellicum Pharmaceuticals, Houston, TX

3:45 PM – 4:00 PM

139: UCARTCS1A: Allogeneic CAR T-Cells Targeting CS1 as Treatment for Multiple Myeloma

Roman Galetto, Cellectis SA, Paris, France

4:00 PM – 4:15 PM

140: Base Editors Generate Allogeneic CAR-T Cells with No Detectable Genomic Rearrangements and Reduced Genotoxicity

Aaron Edwards, Beam Therapeutics, Cambridge, MA

4:15 PM – 4:30 PM

141: Point-of-Care Production of CD19 CAR-T Cells in an Automated Closed-System: Report on First Clinical Experience

Michael Maschan, Dmitriy Rogachev National Medical Center of Pediatric Hematology, Oncology and Immunology, Moscow, Russian Federation

4:30 PM – 4:45 PM

142: Design and Characterization of Persistent CAR-T Cells Targeting Multiple Tumor Antigens Simultaneously

Paul Rennert, Aleta Biotherapeutics, Natick, MA

4:45 PM – 5:00 PM

143: Enhancing Chimeric Antigen Receptor-Modified Gamma Delta T-Cell Killing of T-Cell Leukemia by Modulating Antigen Expression in the Effector Cells

Lauren Fleischer, Emory University, Atlanta, GA

5:00 PM – 5:15 PM

144: T-cells Expressing an Anti-Signaling Lymphocytic Activation Molecule F7 (SLAMF7) CAR and a Suicide Gene Exhibit Anti-Tumor Activity and Can be Eliminated On-Demand
Christina Amatya, National Cancer Institute/NIH, Rockville, MD

Oral Abstract Session 156

3:30 PM - 5:15 PM
Room: Heights Courtyard 2

Use of Immune Modulation in Gene Therapy

CHAIR: Alessio Cantore, PhD

3:30 PM – 3:45 PM

145: Direct Type I Interferon Signaling in Conventional Dendritic Cells and T Help are Required for Cross-Priming AAV Capsid-Specific CD8+ T Cells
Jamie Shirley, University of Florida, Gainesville, FL

3:45 PM – 4:00 PM

146: Tolerogenic ImmTOR™ Nanoparticles Enhance Vector Transduction, mRNA Synthesis and Transgene Expression after Initial and Repeated Administrations of AAV-Based Gene Therapy Vectors through Immunological and Non-Immunological Mechanisms
Petr Ilyinskii, Selecta Biosciences, Watertown, MA

4:00 PM – 4:15 PM

147: Combination Therapy Reduces Existing Anti-AAV Antibody by Logs and Allows for Safe and Efficacious Redosing
Julie Crudele, University of Washington, Seattle, WA

4:15 PM – 4:30 PM

148: Toll-Like Receptor Agonists Have Distinct Effects on Activation of Dendritic Cell Subsets and Antibody Formation in Muscle AAV Gene Transfer
John Butterfield, University of Florida, Gainesville, FL

4:30 PM – 4:45 PM

149: LV.Insulinb9-23/Anti-CD3 Combined Therapy Inhibits Recurrence of Autoimmunity in NOD Mice after Allogeneic Pancreatic Islets Transplant
Andrea Annoni, San Raffaele Telethon Institute for Gene Therapy, IRCCS San Raffaele Scientific Institute, Milan, Italy

4:45 PM – 5:00 PM

150: Transient Immunosuppression and Adeno-Associated Virus (AAV) Vector Gene Transfer of Programmed Death Ligand-1 (PD-L1) Prevents the Development of Chronic Cardiac Transplant Rejection
Susan Faust, ImmunoCurex LLC, Washington, DC

5:00 PM – 5:15 PM

151: In Vivo Chemoselection of Antibody-Secreting Hematopoietic Cells
Anne-Sophie Kuhlmann, Fred Hutchinson Cancer Research Center, Seattle, WA

Oral Abstract Session 157

3:30 PM - 5:15 PM

Room: Heights Courtyard 1

Regenerative Medicine I

CHAIR: Evan Y. Snyder, MD, PhD., FAAP

3:30 PM – 3:45 PM

152: Novel Commercially Available Autologous Homologous Skin Construct Yields Neo-Generation of Full-Thickness Skin Following Burn, Acute Traumatic, and Chronic Cutaneous Wounds That Can Be Genetically Modified
Nikolai Sopko, PolarityTE, Salt Lake City, UT

3:45 PM – 4:00 PM

153: Morphological and Functional Rescue of Retina in a Laser Induced RPE Injury Swine Model Using iPSC-Derived RPE Patch
Aaron Rising, NIH/NEI, Bethesda, MD

4:00 PM – 4:15 PM

154: Platelet-Based Cell Therapy Ameliorates CNS Inflammation in Mice with Neuropathic Gaucher Disease by Reversing Leukocyte Recruitment
Yi Lin, Cincinnati Children's Hospital Medical Center, Cincinnati, OH

4:15 PM – 4:30 PM

155: Genome Edited Airway Stem Cells as a Durable Cell-Based Therapy to Treat Cystic Fibrosis
Sriram Vaidyanathan, Stanford University, Stanford, CA

4:30 PM – 4:45 PM

156: Messenger RNA-Correction of MAGT1-Deficient T- and NK Cells from Patients with XMEN Disease
Julie Brault, National Institutes of Health, Bethesda, MD

4:45 PM – 5:00 PM

157: Rescue of Autophagic Flux in a Mouse Model of Danon Disease by Hematopoietic Stem and Progenitor Cell Transplantation
Sherin Hashem, University of California San Diego, La Jolla, CA

5:00 PM – 5:15 PM

158: Cdx2 Cells for Cardiac Regeneration
Sangeetha Vadakke-Madathil, Icahn School of Medicine at Mount Sinai, New York, NY

Oral Abstract Session 158

3:30 PM - 5:15 PM

Room: Heights Courtyard 3

Cellular Engineering; Novel Cell Types and Approaches

CHAIR: Kimberly Noonan, PhD

3:30 PM – 3:45 PM

159: Fully-Closed System Cell Sorting of CD90+ Results in More Efficient Hematopoietic Stem/Progenitor Cell Transduction
Stefan Radtke, Fred Hutchinson Cancer Research Center, Seattle, WA

3:45 PM – 4:00 PM

160: Analysis of T-Cell Vector Integration Sites for a Murine Gamma-Retroviral Vector Encoding the Anti-CD19 Chimeric Antigen Receptor Used in the Production of Axicabtagene Ciloleucel

Edmund Chang, Kite, A Gilead Company, Santa Monica, CA

4:00 PM – 4:15 PM

161: Ultra-Short Manufacturing of Quiescent Chimeric Antigen Receptor T Cells for Adoptive Immunotherapy

Saba Ghassemi, Upenn, Philadelphia, PA

4:15 PM – 4:30 PM

162: Intraocular Cell Implant Technology to Treat Diseases of the Eye

Konrad Kauper, Neurotech Pharmaceuticals, Cumberland, RI

4:30 PM – 4:45 PM

163: CAR-T Cell Manufacturing with CliniMACS Prodigy

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

4:45 PM – 5:00 PM

164: Highly Efficient and Selective CAR-Gene Transfer Using CD4- and CD8-Targeted Lentiviral Vectors

Laura Kapitza, Paul-Ehrlich-Institut, Langen, Germany

5:00 PM – 5:15 PM

165: An Automated Manufacturing Solution for Patient Specific CAR-T Cell Therapies

Joseph O'Connor, Lonza, Rockville, MD

Tools and Technologies Forum 2

4:00 PM - 6:00 PM

Room: Cardozo

CO-CHAIRS: Michael C. Holmes, PhD and Bartholomew J. Tortella, MD, MBA, FACS, FCCM

SPEAKERS

4:00 PM – 4:15 PM

Karen Doucette, MBA. Absorption Systems, Exton, PA

In Vitro Relative Potency Assays for Commercialization of Gene Therapy Products

4:15 PM – 4:30 PM

Jonathan Mitchell, PhD. Imanis Life Sciences, Rochester, MN

Peeking Behind the Curtain: Non-invasive Imaging Solutions for Tracking Gene Therapies in vivo

4:30 PM – 4:45 PM

Juan Carlos Ramirez, PhD. VIVEbiotech, Donostia-San Sebastián, Spain

Continuous Improvement on Lentiviral Vector Manufacturing

4:45 PM – 5:00 PM

Shawn Zhou, PhD. GenScript USA Inc., Piscataway, NJ

CRISPR KO/KI Cell Line Generation

5:00 PM – 5:15 PM
James Brown, PhD. Aldevron, Fargo, ND
Standardized Plasmids for Viral Vector Production

5:15 PM – 5:30 PM
Alfred Luitjens. Batavia Biosciences, Leiden, Netherlands
SIDUS® Technology for Process Development and Manufacturing of Viral Vectors

5:30 PM – 5:45 PM
Sandra Merino. Alfa Wassermann BV, Ijsselstein, Netherlands
Iodixanol Gradient Scale Up Using Large Scale Ultracentrifugation

5:45 PM – 6:00 PM
Alaina C. Schlinker, PhD. Fresenius Kabi USA LLC, Lake Zurich, IL
Introducing a New Small Volume Cell Processing System: Applications in Cryopreservation Preparation and Fill/Finish

Exhibit Hall Welcome Reception & Poster Session I

5:00 PM - 6:00 PM
Room: Columbia Hall
Reception sponsored by usp®

Industry Symposium 160

6:00 PM – 7:30 PM
Room: Jefferson
Scalable Viral Vector Manufacturing - Future Demand and Promise

Sponsored by GE

6:00 PM – 6:30 PM
Viral Vector Manufacturing: Key Strategies for Overcoming Viral Vector Manufacturing Challenges
Presenter: Spencer Hoover, PhD. Director Process and Analytical Development, CCRM

6:30 PM – 7:00 PM
Panel Discussion: How Do We Meet Future Demands of Viral Vector Manufacturing?
MODERATOR: Phil Vanek. GM Cell and Gene Therapy Strategy, GEHC
PANELISTS: Spencer Hoover, PhD. Director Process and Analytical Development, CCRM
Anandita Seth, PhD. Head of Research & Technology, Viral Therapy, Lonza
Richard Snyder, PhD. Chief Scientific Officer at Brammer Bio, LLC

7:00 PM – 7:20 PM
Networking Session: The Promise of Viral Vectors

Reconnection & Mentoring Event

6:00 PM - 8:00 PM
Room: Tenleytown
Sponsored by rocket pharma

Please join ASGCT in congratulating our newly elected Directors and Officers

Vice President
Bev Davidson, PhD

Secretary
Terry Flotte, MD

Directors
Philip Gregory, DPhil
Maritza McIntyre, PhD
Matthew Wilson, MD, PhD

ASGCT021919-17

Trainee Lounge

7:00 am – 5:00 pm

Room: Fairchild

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 200

8:00 AM - 10:00 AM

Room: Heights Courtyard 2

Beyond Cell Therapy: Extracellular vs. Multicellular Cardiovascular Regenerative Approaches - Organized by the Cardiovascular Gene & Cell Therapy Committee

CO-CHAIRS: Manfred Boehm, MD and Costanza Emanueli, PhD, FAHA

SPEAKERS

8:00 AM – 8:30 AM
Hélène M. Puccio, PhD. Institut de Génétique et de Biologie Moleculaire et Cellulaire (IGBMC), Illkirch, France
Gene Therapy for Cardiac Symptoms in Friedreich's Ataxia

8:30 AM – 9:00 AM
Susmita Sahoo, PhD. Icahn School of Medicine at Mount Sinai, New York, NY
Exosomes and Angiogenesis in Cardiac Regeneration

9:00 AM – 9:30 AM
Wolfram Zimmermann, MD. University Medical Center Göttingen, Göttingen, Germany
Engineered Human Myocardium for Heart Failure Repair

9:30 AM – 10:00 AM
Mark Mercola, PhD. Stanford University, Stanford, CA
Re-introducing the Patient Context into Cardiac Drug Discovery Using iPSCs

Scientific Symposium 201

8:00 AM - 10:00 AM

Room: Lincoln

Evaluating Gene & Cell Therapy Product Candidates - Organized by the Translational Science and Product Development Committee

CO-CHAIRS: Eduard Ayuso, DVM, PhD and Isabelle Riviere, PhD

SPEAKERS

8:00 AM - 8:30 AM
Rebecca Gardner, MD. University of Washington School of Medicine and Seattle Children's Research Institute, Seattle, WA
T cell Product Attributes that Correlate with Clinical Efficacy in B-ALL

8:30 AM – 9:00 AM
J. Fraser Wright, PhD. Wright Biologics Consulting, Princeton, NJ
AAV Vector Manufacturing and Analytics

9:00 AM – 9:30 AM
Boro Dropulic, PhD. Lentigen, a Miltenyi Biotec Company, Gaithersburg, MD
Automated and Decentralized CAR-T cell Manufacturing using Lentiviral vectors: Bench to Commercialization

9:30 AM – 10:00 AM
Fouad Atouf, PhD. United States Pharmacopeia, Rockville, MD
Overview of Standards Setting for Cell/Gene Therapies-- Case Studies

Scientific Symposium 202

8:00 AM - 10:00 AM
Room: International Ballroom West

Gene Editing: From Platform Engineering to Assessing Impact of Innate and Adaptive Responses - Organized by the Genome Editing Committee

CO-CHAIRS: Morgan L. Maeder, PhD and Angela S. Whatley, PhD

SPEAKERS

8:00 AM – 8:30 AM
J. Keith Joung, MD, PhD. Massachusetts General Hospital/Harvard Medical School, Charlestown, MA
Improving the Precision and Specificities of Gene-editing Nucleases and Base Editors

8:30 AM – 9:00 AM
Matthew H. Porteus, MD, PhD. Stanford University School of Medicine, Stanford, CA
The Prevalence of Adaptive Immunity to Cas9 Proteins

9:00 AM – 9:30 AM
Leonela Amoasii, PhD. Exonics Therapeutics, Boston, MA
CRISPR/Cas9-mediated Genome Editing Restores Dystrophin Expression in a Canine Model of Duchenne Muscular Dystrophy

9:30 AM – 10:00 AM
Samira Kiani, MD. Arizona State University, Tempe, AZ
Engineering CRISPR System for Safe and Controllable Gene Therapies

Scientific Symposium 203

8:00 AM - 10:00 AM
Room: International Ballroom East

Towards the Holy Grail of Cancer Gene Therapies: Universal Cells, Targeted Vectors and Solid Tumor CART Efficacy - Organized by the Cancer Gene & Cell Therapy Committee

CO-CHAIRS: Giedre Krenciute, PhD and Robert E. Sobol, MD

SPEAKERS

8:00 AM – 8:30 AM
Julianne Smith, PhD. Cellectis, New York, NY
Allogeneic Gene-Edited CAR T cells: From Preclinic to Clinical Proof of Concept

8:30 AM – 9:00 AM
Paola Grandi, PhD. Cold Genesys, Santa Ana, CA
Oncolytic Vectors: Understanding their Potential to Treat Cancer

9:00 AM – 9:30 AM
Keisuke Watanabe, MD, PhD. University of Pennsylvania, Philadelphia, PA
Combination Oncolytic Viral and Cellular Therapies to Improve Solid Tumor CART Efficacy: Treatment of Pancreatic Cancer with Combined Mesothelin-redirected Chimeric Antigen Receptor T cells and Cytokine-armed Oncolytic Adenoviruses

9:30 AM – 10:00 AM
Masataka Suzuki, PhD. Baylor College of Medicine, Houston, TX
Combination Local Oncolytic Adenoimmunotherapy and Systemic CAR T-cell Therapies for Advanced Solid Tumor Treatment

Scientific Symposium 204

8:00 AM - 10:00 AM
Room: Jefferson

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

CO-CHAIRS: Coy Heldermon, MD, PhD and Ko Mitani, PhD

SPEAKERS

8:00 AM – 8:30 AM
David Davidson, MD. bluebird bio, Cambridge, MA
Gene Therapy Using Lentiviral Vector Mediated Gene Addition - An Update from the Clinic

8:30 AM – 9:00 AM
Steven J. Howe, PhD. GlaxoSmithKline R & D, Stevenage, United Kingdom
Stable Cell Lines for Lentiviral Vector Production: The Corporate Perspective

9:00 AM – 9:30 AM
Junghae Suh, PhD. Rice University, Houston, TX
AAV Engineering

9:30 AM – 10:00 AM
Anandita Seth, PhD. Lonza, Houston, TX
Manufacturing Platforms for AAV Production - Taking the Next Steps

Education Session 205

8:00 AM - 10:00 AM
Room: Georgetown

"How-to" - Design, Delivery, and Application of RNA Therapeutics

CO-CHAIRS: Jonathan Finn, PhD and Millicent O. Sullivan, PhD

SPEAKERS

8:00 AM – 8:30 AM
Christopher A. Alabi, PhD. Cornell University, Ithaca, NY
Design, Synthesis, and Application of Antibody Nucleic Acid Analogs as Novel Biopharmaceuticals

8:30 AM – 9:00 AM
Kathryn A. Whitehead, PhD. Carnegie Mellon University, Pittsburgh, PA
Lipid Nanoparticle Delivery Technology for siRNA and mRNA Therapeutics

9:00 AM – 9:30 AM
David Rodman, MD. ProQR Therapeutics, Cambridge, MA
Small RNA Molecules for RNA Editing Therapy in LCA10 Patients

9:30 AM – 10:00 AM
Anton McCaffrey, PhD. Trilink Biotechnologies, San Diego, CA
mRNA Manufacturing

Oral Abstract Session 210

8:00 AM - 10:00 AM
Room: Monroe

Cancer Targeted Gene Cell Therapy

CHAIR: Masato Yamamoto, MD, PhD

8:00 AM – 8:15 AM
350: Toward a Hematopoietic Stem Cell Gene Therapy Approach for Cancer Prevention
Chang Li, University of Washington, Seattle, WA

8:15 AM – 8:30 AM
351: Tumoricidal Stem Cell Therapy Enables Killing in Novel Hybrid Models of Heterogeneous Glioblastoma
Andrew Satterlee, University of North Carolina at Chapel Hill, Chapel Hill, NC

8:30 AM – 8:45 AM
352: Gene Therapy for Chronic Eosinophilic Leukemia
Odelya Pagovich, Weill Cornell Medical College, New York, NY

8:45 AM – 9:00 AM
353: In Vivo Activity of CD33/CD3-Directed Bispecific Antibody and Protection of Normal Hematopoiesis by CRISPR/Cas9-Mediated Gene Editing
Olivier Humbert, Fred Hutchinson Cancer Research Center, Seattle, WA

9:00 AM – 9:15 AM
354: Neural Stem Cells Secreting Bispecific T Cell Engagers Improve Survival in a Preclinical Model of Glioblastoma
Katarzyna Pituch, Northwestern University, Chicago, IL

9:15 AM – 9:30 AM

355: Feasibility of Gene Replacement Therapy in Neurofibromatosis Type 1 (NF-1)-Related Tumors

Renyuan Bai, Johns Hopkins University, Baltimore, MD

9:30 AM – 9:45 AM

356: Novel Strategy to Enhance In Vivo Expression of Adeno-Associated Virus Vectors in Hepatocellular Carcinoma

Nadja Meumann*, Hannover Medical School, Hannover, Germany

9:45 AM – 10:00 AM

357: Therapeutic Effect of Extracellular Matrix Degradation Enzyme Delivered by Combination Engineered Bacteria with Anticancer Drugs

Seung-Hwan Park, Korea Research Institute of Bioscience and Biotechnology, Jeongeup-si, Korea, Republic of

Oral Abstract Session 211

8:00 AM - 10:00 AM

Room: Heights Courtyard 1

CAR T Cell Therapy

CHAIR: Barbara Savoldo, MD, PhD

8:00 AM – 8:15 AM

358: Long-Term Remission of CLL Sustained by Oligoclonal CD19-Specific Chimeric Antigen Receptor T Cell Clones

J. Melenhorst, University of Pennsylvania, Philadelphia, PA

8:15 AM – 8:30 AM

359: Genomic and Epigenetic Analysis of Patient-Derived Pediatric B Cell Acute Lymphoblastic Leukemia (B-ALL) to Define New Mechanisms of Resistance to CD19 CAR-T Cell Therapy

Rimas Orentas, Seattle Children's Research Institute, Seattle, WA

8:30 AM – 8:45 AM

360: Rational Combinatorial CAR Designs for Effective Immunotherapy

Mohamad Hamieh, MSKCC, New York, NY

8:45 AM – 9:00 AM

361: Dual-Switch CAR-T Cells: Inducible Cell Activation and Elimination to Manage Persistence and Toxicity

MyLinh Duong, Bellicum Pharmaceuticals, Houston, TX

9:00 AM – 9:15 AM

362: Toward Durable Multiple Myeloma Regressions with Anti-BCMA CAR T Cells with PI3K Inhibition and Reduced Time of Culture

Olivia Finney, bluebird bio, Cambridge, MA

9:15 AM – 9:30 AM

363: Developing Ligand-Based Chimeric Antigen Receptors to Target Leukemic and Bone Marrow Stem Cells

Jaquelyn Zoine, Emory University, Atlanta, GA

9:30 AM – 9:45 AM

364: Nonhuman Primate Stem Cell-Derived CAR T-Cells Traffic to Lymph Node Follicles and Engraft Following Reduced-Intensity Conditioning

Christopher Peterson, Fred Hutchinson Cancer Research Center, Seattle, WA

9:45 AM – 10:00 AM

365: Pharmacological Control of In Vivo Tumor Regression by T Cells Engineered with CD19-Car Regulated with PDE5 Derived Destabilizing Domains

Grace Olinger, Obsidian Therapeutics, Cambridge, MA

Oral Abstract Session 212

8:00 AM - 10:00 AM

Room: Heights Courtyard 3

Immunotherapy I

CHAIR: Hans-Peter Kiem, MD, PhD

8:00 AM – 8:15 AM

366: Modeling BCMA-Specific Regulated CAR T Cells to Target Long-Lived Plasma Cells or Multiple Myeloma

Yuchi Honaker, Seattle Children's Research Institute, Seattle, WA

8:15 AM – 8:30 AM

367: NKT Cells Co-Expressing a GD2-Specific Chimeric Antigen Receptor and IL-15 Show Enhancedin Vivopersistence and Antitumor Activity Against Neuroblastoma

Andras Heczey, Baylor College of Medicine, Houston, TX

8:30 AM – 8:45 AM

368: CIS Checkpoint Deletion in Cord Blood Derived iC9/CAR19/IL-15 NK Cells for the Treatment of B-Cell Hematologic Malignancies

May Daher, MD Anderson Cancer Center, Houston, TX

8:45 AM – 9:00 AM

369: Cytomegalovirus-Specific T Cells Expressing Anti-HIV CAR and CMV Vaccine Boost as Immunotherapy for HIV/AIDS: Pre-Clinical Data

Laura Lim, City of Hope, Duarte, CA

9:00 AM – 9:15 AM

370: Cord Blood Derived CAR-T Cells Targeting gp350 Containing CD28/CD3ζ or 4-1BB/CD3ζ Signaling Domains Directly React Against Cells Infected with EBV Bypassing the Need of HLA-Matched Memory T Cells

Maja Kalbarczyk, Hannover Medical School, Hannover, Germany

9:15 AM – 9:30 AM

371: 4-1BB CAR Costimulation Drives Non-Canonical NF-kB Signaling, Thereby Enhancing CAR T Cell Survival and Suppressing Expression of Pro-Apoptotic Protein, Bim

Benjamin Philipson, University of Pennsylvania, Philadelphia, PA

9:30 AM – 9:45 AM

372: First-In-Human CAR T for Solid Tumors Targets the MUC1 Transmembrane Cleavage Product

Cynthia Bamdad, Minerva Biotechnologies, Waltham, MA

9:45 AM – 10:00 AM

373: Pre-Clinical Investigations of CAR T Cells Directed Against the Tumour Antigen 5T4 (OXB-302) in Solid Tumor Models

Michelle Kelleher, Oxford BioMedica (UK) Limited, Oxford, United Kingdom

Exhibit Hall Coffee Social

10:00 AM - 10:45 AM

Room: Columbia Hall

Exhibit Hall Open

10:00 AM - 6:00 PM

Room: Columbia Hall

Plenary Session 220

10:45 AM - 12:00 PM

Room: International Ballroom

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

Sponsored by SANOFI GENZYME

CHAIR: Michele P. Calos, PhD

SPEAKERS

10:45 AM – 10:50 AM

Hans-Peter Kiem, MD, PhD. Fred Hutchinson Cancer Research Center, Seattle, WA

Remembering George Stamatoyannopoulos (1934-2018)

10:50 AM – 11:45 AM

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

The Makings of a Living Drug

Break

12:00 PM - 1:30 PM

Lunch Break *(On Own - Not Provided)*

Industry Symposium 230

12:00 PM – 1:30 PM

Room: Georgetown

Advances in T- and NK-cell Engineering: Bolstering Therapeutic Efficacy and Speed to Clinic of Sophisticated Cell Therapies

Sponsored by MaxCyte®

PRESENTER: Rama Shivakumar, Scientist, *MaxCyte, Inc.*

Industry Symposium 231

12:00 PM – 1:30 PM

Room: Monroe

The Latest Advances in Cell Therapy Research

Sponsored by MACS Miltenyi Biotec

12:10 PM – 12:35 PM

AIM ACT, a Novel Nanoparticle-based Technology that Generates Therapeutic Amounts of Functional Multi-Antigen Tumor Specific CD8+ T cells with T Stem cell like, Central and Effector Phenotype in 14 days

Mathias Oelke, PhD, VP, Head of Pre-Clinical and Platform Development
NexImmune, Inc.

12:35 PM – 1:00 PM

Closing and automating an autologous cell therapy process using Miltenyi CliniMACS Prodigy.

Jonathan Rubin, PhD, Senior Scientist, Janssen R&D

1:00 PM – 1:25 PM

Automated Gene Modified Cell Production: Concept to Process Verification

Azadeh Golipour, PhD, Senior Director, Manufacturing Operations, AVROBIO

Industry Symposium 232

12:00 PM – 1:30 PM

Room: Jefferson

Off-Target Screening of Biotherapeutics and Cell Therapies

Sponsored by RETROGENIX® SPOTTING YOUR TARGET

12:05 PM

Chair's opening remarks

Jim Freeth, PhD, Co-managing Director, Retrogenix Limited

12:10 PM

Understanding and predicting CAR T cell off-target activity

Thomas Long, PhD, Senior Scientist, Juno Therapeutics, a Celgene Company

12.35 PM

Monoclonality does not equal monospecificity - polyspecificity is a critical development risk for antibody-based therapeutics

William Finlay, PhD, CEO, UltraHuman Limited

1:00 PM

Early cross reactivity screening of various biologic modalities

Tim MacLachan PhD, Executive Director, Preclinical Safety, Novartis

1:25 PM

Chair's closing remarks

Industry Symposium 233

12:00 PM – 1:30 PM

Room: Lincoln

Accelerated Regulatory Approval's Impact on Manufacturing Process Decisions

Sponsored by TERUMOBCT Unlocking the Potential of Blood

SPEAKER

Lori Noffsinger, VP Technology Development, Cognate Bioservices Inc.

Plenary Session 240

1:30 PM - 3:00 PM

Room: International Ballroom

Outstanding New Investigator Symposium

Sponsored by BURROUGHS WELLCOME FUND

CHAIR: Guangping Gao, PhD

SPEAKERS

1:30 PM – 2:10 PM

Steven J. Gray, PhD. University of Texas Southwestern Medical Center, Dallas, TX
On the Brink of a Treatment Revolution for Inherited Pediatric Neurological Diseases

2:10 PM – 2:50 PM

Anna Kajaste-Rudnitski, PhD. San Raffaele Telethon Institute for Gene Therapy, Milano, Italy
Dissecting and Overcoming Innate Immune Barriers for Therapeutically Efficient Hematopoietic Stem Cell Gene Engineering

Exhibit Hall Coffee Social

3:00 PM - 3:30 PM

Room: Columbia Hall

Oral Abstract Session 250

3:30 PM - 5:15 PM

Room: Jefferson

Gene Therapy for Musculo-Skeletal Diseases

CO-CHAIRS: Chunping Qiao, PhD and Bing Wang, MD, PhD

3:30 PM – 3:45 PM

374: Optimizing Dystrophin Gene Therapy in Severe DMD Murine Model
Biao Kuang, The University of Pittsburgh, Pittsburgh, PA

3:45 PM – 4:00 PM

375: rAAV9- Mediated NDUFS3 Replacement Reverts the Myopathy Phenotype in Pre- And Post-Symptomatic Muscle Ndufs3 KO Mice
Carlos Moraes, University of Miami, Miami, FL

4:00 PM – 4:15 PM

376: Multiparameter In Vivo Screen of AncAAV Libraries in Muscle for Potency, Specificity and Impact of Disease State

Jennifer Green, Solid Biosciences, Cambridge, MA

4:15 PM – 4:30 PM

377: Gene Therapy for Mitochondrial Diseases

Sandra Bacman, University of Miami, Miami, FL

4:30 PM – 4:45 PM

378: Pre-Clinical Efficacy of Human Acid Sphingomyelinase as a Treatment for Limb Girdle Muscular Dystrophy 2B

Daniel Bittel, Childrens National Medical Center, Washington DC, DC

4:45 PM – 5:00 PM

379: Global Metabolic Profiling of Skeletal Muscle Reveals Profound Correction in FKRP-Deficient Mice after FKRP Gene Therapy

Charles Vannoy, Atrium Health, Charlotte, NC

5:00 PM – 5:15 PM

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

Adrian Pickar-Oliver, Duke University, Durham, NC

Oral Abstract Session 251

3:30 PM - 5:15 PM

Room: Lincoln

Preclinical Studies of Nonviral Gene Therapy and the Progress towards Translation

CO-CHAIRS: Kevin Rice, PhD and Carol Miao, PhD

3:30 PM – 3:45 PM

381: Development of a Reporter Mouse Model For In Vivo Evaluation of Genome Editing

Masato Ohtsuka, Tokai University, Isehara, Japan

3:45 PM – 4:00 PM

382: Immunomodulation of Factor FVIII Inhibitors in Hemophilia A Mice Using Messenger RNA Lipid Nanoparticles

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

4:00 PM – 4:15 PM

383: Efficient mRNA Therapy for Treating Ornithine Transcarbamylase Deficiency in Two Mouse Models

Lili Wang, University of Pennsylvania, Philadelphia, PA

4:15 PM – 4:30 PM

384: Monopolar Electrotransfer Enhances Gene Delivery to a Beating Heart

Anna Bulysheva, Old Dominion University, Norfolk, VA

4:30 PM – 4:45 PM

385: IR-Laser Assisted Gene Electrotransfer to the Skin for Non-Invasive DNA Vaccination

Chelsea Edelblute, Old Dominion University, Norfolk, VA

4:45 PM – 5:00 PM

386: Novel DNA Vaccination Utilizing a Newly Developed Pyro-Drive Jet Injector (PJI) Induced Serological and Cellular Immune Responses Leading to In Vivo Suppression of Tumor Growth in a Rat Model

Yoshihiro Miyahara, Mie University Graduate School of Medicine, Tsu, Japan

5:00 PM – 5:15 PM

387: Polymers for Transfection of mRNA: Polymer End Groups Dramatically Affect Efficiency and Biodistribution

Yuhang Jiang, Yale University, New Haven, CT

Oral Abstract Session 252

3:30 PM - 5:15 PM

Room: Georgetown

Rational Engineering of AAV Vectors II

CHAIR: Antonette Bennett, PhD

3:30 PM – 3:45 PM

388: Engineering AAV Vectors for Antibody-Mediated Retargeting to Specific Cell Types In Vitro and In Vivo

Leah Sabin, Regeneron Pharmaceuticals, Tarrytown, NY

3:45 PM – 4:00 PM

389: Development and Characterization of Caspase-Activatable Adeno-Associated Virus Vectors for Targeted Gene Delivery Following Systemic Injection

Mitchell Brun, Rice University, Houston, TX

4:00 PM – 4:15 PM

390: Optimization of MMP Activatable Provector for Targeting of Pancreatic Cancer

Susan Butler, Rice Uinversity, Houston, TX

4:15 PM – 4:30 PM

391: Customized Blood-Brain Barrier Shuttle Peptide to Increase AAV9 Vector Crossing the BBB and Enhance Transduction in the Brain

Xintao Zhang, UNC-Chapel Hill, Chapel hill, NC

4:30 PM – 4:45 PM

392: A Barcode System for Evaluating Adeno-Associated Virus Capsids with Low Immunogenicity in Nonhuman Primates

Qiang Wang, University of Pennsylvania, Philadelphia, PA

4:45 PM – 5:00 PM

393: Human Polyclonal Anti-AAV Neutralizing Antibody Epitope Mapping by NGS Identifies Common Epitopes and Enables the Design of Stealth Mutants

Helen Baggett, Oregon Health & Science University, Portland, OR

5:00 PM – 5:15 PM

394: Evaluation of Tropism and Transduction Rfficiency of AAV Variants in the CNS of NHP Using DNA/RNA Barcode-Seq Technology

Hongxing Wang, Voyager Therapeutics, Cambridge, MA

Oral Abstract Session 253

3:30 PM - 5:15 PM

Room: Monroe

Gene Replacement for Neurologic Diseases

CHAIR: Jodi McBride, PhD

3:30 PM – 3:45 PM

222: AXO-Lenti-PD: A Second-Generation Lentiviral Gene Therapy for the Treatment of Parkinson's Disease
Gavin Corcoran, Axovant Sciences, New York, NY

3:45 PM – 4:00 PM

396: Efficacy and Safety in Mice and Non-Human Primates of CSF Delivered AVXS-201 for the Treatment of Rett Syndrome
Kevin Foust, AveXis Research and Development, San Diego, CA

4:00 PM – 4:15 PM

397: CNS-Directed AAV9 Gene Therapy for the Treatment of Canine Globoid Cell Leukodystrophy (Krabbe Disease)
Allison Bradbury, University of Pennsylvania, Philadelphia, PA

4:15 PM – 4:30 PM

398: Intrathecal Administration of AAV9-SOD1 for Amyotrophic Lateral Sclerosis: Survival Extension and SOD1 Reduction in Mice and Nonhuman Primates
Gretchen Thomsen, AveXis Research and Development, San Diego, CA

4:30 PM – 4:45 PM

399: Review of Safety and Interim Analysis of Efficacy of a First-in-Human Intrathecal Gene Transfer Study for Giant Axonal Neuropathy
Dimah Saade, NINDS, NIH, Bethesda, MD

4:45 PM – 5:00 PM

400: GDNF Gene Therapy for Advanced Parkinson's Disease
Krystof Bankiewicz, University of California San Francisco, San Francisco, CA

5:00 PM – 5:15 PM

401: Intravenous Delivery of AAV Gene Therapy in GM1 Gangliosidosis
Amanda Gross, Auburn University, Auburn University, AL

Oral Abstract Session 254

3:30 PM - 5:15 PM

Room: International Ballroom

Oncolytic Viruses II

CHAIR: Paola Grandi, PhD

3:30 PM – 3:45 PM

402: Nectin-Elicited Cytoplasm Transfer: A New Process Extending the Tropism of an Oncolytic Virus
Roberto Cattaneo, Mayo Clinic, Rochester, MN

3:45 PM – 4:00 PM

403: A Novel Chimeric Poxvirus Encoding an Anti-PD-L1 Protein Shows Safety and Anti-Tumor Activity in Breast Cancer Model

Shyambabu Chaurasiya, City of Hope National Medical Center, Duarte, CA

4:00 PM – 4:15 PM

404: Cargocyte Biofactories: A Novel Platform for Delivering Oncolytic Viruses to Treat Metastatic Cancer

Huawei Wang, University of California, San Diego, La Jolla, CA

4:15 PM – 4:30 PM

405: Viral Derived Innate Immune Antagonists Sensitize Interferon Responsive Cancer Cells to Oncolytic Mengo and Vesicular Stomatitis Viral Vectors

Justin Maroun, Mayo Clinic, Rochester, MN

4:30 PM – 4:45 PM

406: Novel Approach for Treatment of Pediatric High-Grade Gliomas through the Combination of Oncolytic Adenoviruses and Gene Therapy Encoding a BiTE Directed to the EphA2 Tumor Antigen

Claudia Arnone, Bambino Gesù Children's Hospital, Rome, Italy

4:45 PM – 5:00 PM

407: Combination Treatment of VSV-mIFN-NIS with Ruxolitinib and PDL1 in 5TGM1 Mouse Model

Lianwen Zhang, Mayo Clinic, Rochester, MN

5:00 PM – 5:15 PM

408: MicroRNA-Detargeted CVA21 Infectious RNA; Delivery of an Oncolytic Virus as Infectious Nucleic Acid

Autumn Schulze, Mayo Clinic, Rochester, MN

Oral Abstract Session 255

3:30 PM - 5:15 PM

Room: Holmead

Adenovirus Vectors and Other DNA Virus Vectors

CHAIR: Ko Mitani, PhD

3:30 PM – 3:45 PM

409: SB100x-Transposase Mediates Integration of a 32.4kb Transposon and High-Level Gamma-Globin Expression after In Vivo HSC Transduction in Mice

Hongjie Wang, University of Washington, Seattle, WA

3:45 PM – 4:00 PM

410: Single Adenoviral Vectors Armed with HPV Oncogene Specific CRISPR/Cas9 as Efficient Tumor Gene Therapy Tools for the Treatment of HPV Related Cancers

Eric Ehrke-Schulz, Witten/Herdecke University, Witten, Germany

4:00 PM – 4:15 PM

411: Serotype-Specific Binding of Prothrombin to Adenovirus Vectors

Andrew Byrnes, Food and Drug Administration, Silver Spring, MD

4:15 PM – 4:30 PM

412: CRISPR-cas9 Gene Editing for Cystic Fibrosis
Emily Xia, PGCRL, Toronto, ON, Canada

4:30 PM – 4:45 PM

413: Biodistribution Analysis of Transgene Expression from a Non-Cytotoxic Herpes Simplex Virus Based Vector
Yoshitaka Miyagawa, Nippon medical school, Tokyo, Japan

4:45 PM – 5:00 PM

414: Enhanced Tropism of Species B1 Adenoviral-Based Vectors for Primary Human Airway Epithelial Cells
Ashley Cooney, University of Iowa, Iowa City, IA

5:00 PM – 5:15 PM

415: AdMG-MYOGp(S)-mMEF2-5/3F, Myogenin Promoter-Controlled Oncolytic Adenovirus, Selectively Kills PAX3-FOXO1+Rhabdomyosarcoma Cells
Hideki Yoshida, University of Minnesota, Minneapolis, MN

Oral Abstract Session 256

3:30 PM - 5:15 PM

Room: Heights Courtyard 2

Nonclinical Studies and Assay Development

CHAIR: Jennifer Marlowe, PhD

3:30 PM – 3:45 PM

416: Preclinical Validation of 64Copper as a Translational Tool for Evaluating the Pharmacodynamics of VTX-801 Gene Therapy in Wilson's Disease
Bernard Benichou, Vivet Therapeutics, Paris, France

3:45 PM – 4:00 PM

417: Early Versus Late Gestation Approaches in Fetal Gene Therapy
Jerry Chan, KK Women's and Children's Hospital / Duke NUS Medical School, Singapore, Singapore

4:00 PM – 4:15 PM

418: Advantages of DNA Barcoding versus Integration Site Analysis for In Vivo Clone Tracking after Transplantation
Mark Enstrom, Fred Hutch, Seattle, WA

4:15 PM – 4:30 PM

419: Computational Correction of Index Switching among Multiplexed Samples in Integration Site Analysis
Adriano De Marino, SR-TIGET, Milano, Italy

4:30 PM – 4:45 PM

420: Toxicology Studies for Hematopoietic Stem & Progenitor Cell Gene Therapy for Cystinosis Revealed an Unexpected Cytotoxicity of Polybrene to the Ctns-/- Sca1+Cells
Jay Sharma, University of California, San Diego, La Jolla, CA

4:45 PM – 5:00 PM

421: Preclinical CAR-T Cell Target Safety, Biodistribution, and Tumor Infiltration Analysis Using In Situ Hybridization Technology

Christopher Bunker, Advanced Cell Diagnostics, Inc, Newark, CA

5:00 PM – 5:15 PM

422: In Vitro Evaluation of Potential PRAME TCR-Mediated Off-Target Toxicity Using iPSC-Derived Neuronal Cells

Maja Buerdek, Medigene Immunotherapies GmbH, Martinsried, Germany

Oral Abstract Session 257

3:30 PM - 5:15 PM

Room: Heights Courtyard 1

Gene Therapy for Metabolic Disorders: Proof of Concept and Beyond

CHAIR: Nicola Brunetti-Pierri, MD

3:30 PM – 3:45 PM

423: Systemic AAV GGene Therapy Rescues Murine Models of Propionic Acidemia Caused Mutations in Pccb from Neonatal Lethality

Joshua Moise-Silverman, National Institutes of Health, Bethesda, MD

3:45 PM – 4:00 PM

424: NGS-Based Multiplexed AAV Library Screening across Species and Preclinical Liver Models

Carmen Unzu, Grousbeck Gene Therapy Center, Mass Eye and Ear, Harvard Medical School, Boston, MA

4:00 PM – 4:15 PM

425: The Combination Therapy of ImmTOR™ with AAV Anc80 is Therapeutic, Safe, and Repeatable in Mice with Methylmalonic Acidemia, and Compatible with the Low Seroprevalence of Anc80 Nabs in the Patient Population

Lina Li, NIH, Bethesda, MD

4:15 PM – 4:30 PM

426: AAV Expressing MDR3 (VTX-803) Mediates the Correction of Progressive Familial Intrahepatic Cholestasis Type 3 (PFIC3) in a Clinically Relevant Mouse Model

Nicholas Weber, Vivet Therapeutics, Pamplona, Spain

4:30 PM – 4:45 PM

427: Systemic AAV Gene Therapy for cblC Type Combined Methylmalonic Acidemia and Homocysteinemia Rescues Neonatal Lethality and Provides Lasting Phenotypic and Metabolic Correction Comparable to Chronic OHCbl Injections

Kelsey Murphy, NIH, Bethesda, MD

4:45 PM – 5:00 PM

428: Therapeutic Levels of FVIII Generated by CRISPR/Cas9-Mediated In Vivo Genome Editing in Hemophilia A Mice

Alan Brooks, Casebia Therapeutics LLC, Cambridge, MA

5:00 PM – 5:15 PM

429: In Utero Liver-Directed Lentiviral Gene Therapy Cures a Pig Model of Hereditary Tyrosinemia Type I

Clara Nicolas, Mayo Clinic, Rochester, MN

Oral Abstract Session 258

3:30 PM - 5:15 PM

Room: Heights Courtyard 3

Next Generation Gene Vector Engineering and Analytics

CHAIR: H. Trent Spencer, PhD

3:30 PM – 3:45 PM

430: Optimizing In Vivo Targeting of Lentiviral Vectors Pseudotyped with Tupaia Paramyxovirus Glycoproteins Bearing Cell-Specific Ligands

Takele Argaw, FDA, Silver Spring, MD

3:45 PM – 4:00 PM

431: Profiling the Heterogeneity of Inverted Terminal Repeat Sequences of Encapsidated Genomes in AAV Preparations by AAV-Genome Population Sequencing

Phillip Tai#, University of Massachusetts, Medical School, Worcester, MA

4:00 PM – 4:15 PM

432: A Novel, Non-Integrative DNA Nanovector System for the Modification and Manufacture of CAR-T Cells

Matthias Bozza, DKFZ Heidelberg, Heidelberg, Germany

4:15 PM – 4:30 PM

433: Integration Site Analysis of Ad5 and SV40 Large T Antigen in HEK293T Cells

Matteo Franco, GeneWerk GmbH, Heidelberg, Germany

4:30 PM – 4:45 PM

434: Detection and Characterization of Micro-RNAs Found as Residual Contaminants in AAV Vector Batches

Magalie Penaud-Budloo, INSERM, University of Nantes, CHU de Nantes, Nantes, France

4:45 PM – 5:00 PM

435: Improved Performance of Lenti-Viral Vectors by the Incorporation of Novel, Human Chromatin Insulators Displaying Both Enhancer-Blocking and Barrier Activity

Penelope Georgia Papayanni, Gene and Cell Therapy Center, Thessaloniki, Greece

5:00 PM – 5:15 PM

436: CombiAAV Enables Rapid Generation of Combinatorially Diverse Barcoded AAV Capsid Libraries

Eric Zinn, Department of Systems Biology, Harvard Medical School, Cambridge, MA

Tools and Technologies Forum 3

4:00 PM - 6:00 PM

Room: Cardozo

CO-CHAIRS: Kye Ehart and Joseph Lee, PhD

SPEAKERS

4:00 PM – 4:15 PM
Marco Schmeer, PhD. PlasmidFactory GmbH & Co. KG, Bielefeld, Germany
Minicircle DNA - New Tool for Cell Therapy

4:15 PM – 4:30 PM
Christian Thirion, PhD. SIRION Biotech, Munich, Germany
Improving Transduction for Gene and Cell Therapy Trials

4:30 PM – 4:45 PM
Katharina Hammer, PhD. Progen Biotechnik GmbH, Heidelberg, Germany
AAV Titration ELISAs: A Robust & Reliable Tool for AAV Quantification

4:45 PM – 5:00 PM
Wei Wang, PhD. GeneWerk GmbH, Heidelberg, Germany
Vector Safety in Gene and Immune Gene Therapies

5:00 PM – 5:15 PM
Stephen D. Rodriguez. VGXI Inc., The Woodlands, TX
Plasmid DNA Manufacturing for Gene Therapy Vectors

5:15 PM – 5:30 PM
Ken Hoffman. Cygnus Technologies, LLC, Southport, NC
Viral Vector Manufacturing - A look at Analytical Tools for Process-Related Impurities

5:30 PM – 5:45 PM
Henry George. MilliporeSigma, Saint Louis, MO
Development of a Scalable, Transient Lentivirus Production Platform

5:45 PM – 6:00 PM
Kamran Anwar, PhD. MilliporeSigma, St. Louis, MO
Determination of AAV Purity by AUC Sedimentation Velocity for Viral Vectors Used in Cell and Gene Therapy

Exhibit Hall Networking Reception & Poster Session II

5:00 PM - 6:00 PM
Room: Columbia Hall

New Member Reception

6:00 PM - 7:00 PM
Room: Tenleytown

Sponsored by avexis

Industry Symposium 260

6:00 PM – 7:30 PM
Room: Jefferson

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

Sponsored by PALL Biotech

6:00 PM – 6:05 PM
Clive Glover, PhD. Pall Biotech, Portsmouth, United Kingdom
Introduction

6:05 PM – 6:30 PM
Giuliana Vallanti, PhD. MolMed S.p.A., Milan, Italy
Implementation of iCELLis® Technology for the Manufacture of Lentivirus

6:30 PM – 6:55 PM
Markus Hörer, PhD. Freeline Therapeutics GmbH, Planegg, Germany
Implementation of iCELLis® Technology for the Manufacture of Adeno-associated Virus

6:55 PM – 7:15 PM
Daniel Smith, PhD. Cobra Biologics, Keele, United Kingdom
Continuous Chromatography for Viral Vector Manufacturing

7:15 PM – 7:30 PM
Panel Discussion

Trainee Lounge

7:00 am – 5:00 pm

Room: Fairchild

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 300

8:00 AM - 10:00 AM

Room: International Ballroom East

Basic and Translational Aspects of Immunology and Gene and Cell Therapy - Organized by the Immune Responses to Gene & Cell Therapy Committee

CO-CHAIRS: Irina V. Balyasnikova, PhD and Maria G. Castro, PhD

SPEAKERS

8:00 AM – 8:30 AM
Maria-Grazia Roncarolo, MD. Stanford University School of Medicine, Stanford, CA
Cell and Gene Therapy in Tregopathies

8:30 AM – 9:00 AM
Megan Sykes, MD. Columbia University College of Physicians and Surgeons, New York, NY
Mechanisms of Tolerance in Human Allograft Recipients

9:00 AM – 9:30 AM
Samuel D. Rabkin, PhD. Massachusetts General Hospital and Harvard Medical School, Boston, MA
Oncolytic Viruses Combined with Checkpoint Inhibitors

9:30 AM – 10:00 AM
Maria G. Castro, PhD. University of Michigan School of Medicine, Ann Arbor, MI
Epigenetic Reprogramming Within the Tumor Microenvironment Impacts the Efficacy of Immune-mediated Gene Therapy

Scientific Symposium 301

8:00 AM - 10:00 AM

Room: Monroe

Genetic Modification of Stem Cells - Organized by the Stem Cell Committee

CO-CHAIRS: M. Graça D. Almeida-Porada, MD, PhD and Linzhao Cheng, PhD

SPEAKERS

8:00 AM – 8:30 AM
Tippi C. MacKenzie, MD. University of California San Francisco, San Francisco, CA
In Utero Gene Therapy

8:30 AM – 9:00 AM
Eirini P. Papapetrou, MD, PhD. Icahn School of Medicine at Mount Sinai, New York, NY
Engineered iPSCs to Model Human Leukemia

9:00 AM – 9:30 AM
Dan Kaufman, MD, PhD. University of California - San Diego, La Jolla, CA
Engineered iPSC-derived Lymphocytes for Improved Cancer Therapy

9:30 AM – 10:00 AM
Edward J. Rebar, PhD. Sangamo Biosciences, Richmond, CA
Gene Modification of Hematopoietic Stem Cells for Research and Treatment of Hemoglobinopathies

Scientific Symposium 302

8:00 AM - 10:00 AM
Room: International Ballroom West

Genome Editing in the Retina and CNS - Organized by the Neurologic & Ophthalmic Gene & Cell Therapy Committee

CO-CHAIRS: Caroline E. Bass, PhD and David J. Segal, PhD

SPEAKERS

8:00 AM – 8:30 AM
Beverly L. Davidson, PhD. Children's Hospital of Philadelphia, Philadelphia, PA, University of Pennsylvania, Philadelphia, PA
Gene Editing for Huntington's Disease

8:30 AM – 9:00 AM
Jeremy Day, PhD. University of Alabama at Birmingham, Birmingham, AL
CRISPR Tools for Gene Editing in the Brain

9:00 AM – 9:30 AM
Vania Broccoli, PhD. San Raffaele Scientific Institute, Milan, Italy, CNR-Institute of Neuroscience, Milan, Italy
Gene Editing and Targeted Gene Expression Manipulation for Treating Neurological Disorders

9:30 AM – 10:00 AM
Charlie Albright, PhD. Editas Medicine, Cambridge, MA
Development of EDIT-101, A Gene-editing Approach to Restore Vision Loss in Leber Congenital Amaurosis Type 10

Scientific Symposium 303

8:00 AM - 10:00 AM
Room: Heights Courtyard 2

Prophylactic and Therapeutic Antibodies against Infectious Diseases - Organized by the Infectious Diseases and Vaccines Committee

CO-CHAIRS: Claire Evans, PhD and Matti Sallberg, PhD, DDS

SPEAKERS

8:00 AM – 8:30 AM
Claire Evans, PhD. Ichor Medical Systems, San Diego, CA
Electroporation-Mediated DNA Administration as an Antibody Delivery Platform for Passive Immunoprophylaxis

8:30 AM – 9:00 AM
Ulrike Protzer, MD. Technical University of Munich / Helmholtz Zentrum München, München, Germany
Redirection of T cells against HBV-infected cells

9:00 AM – 9:30 AM
Alejandro B. Balazs, PhD. The Ragon Institute of MGH, MIT and Harvard, Cambridge, MA
Vectored ImmunoTherapy for HIV Reveals 'Escapability' as a Key Feature of Broadly Neutralizing Antibody Therapeutic Efficacy

9:30 AM – 10:00 AM
Drew Weissman, MD, PhD. University of Pennsylvania, Philadelphia, PA
RNA-based Drugs Including Delivery of Antibodies and Vaccines Using mRNA

Scientific Symposium 304

8:00 AM - 10:00 AM
Room: Jefferson

Right to Try or Wrong to Try? - Organized by the Ethics Committee

CO-CHAIRS: Pilar N. Ossorio, PhD, JD and Bruce E. Torbett, PhD, MSPH

SPEAKERS

8:00 AM – 8:20 AM
Alison Bateman-House, PhD, MPH. NYU Langone Health, New York, NY
Impacts of the Right to Try Laws

8:20 AM – 8:40 AM
Peter Marks, MD, PhD. Food & Drug Administration, Silver Spring, MD
FDA's Programs to Promote Patient Access to Investigational Therapies

8:40 AM – 9:00 AM
Nadia Bodkin. Rare Advocacy Movement (RAM), Plainsboro, NJ
The Varying Perspectives of the Rare Disease Patient Advocacy Community on RTT

9:00 AM – 9:20 AM
Jerry R. Mendell, MD. The Research Institute at Nationwide Children's Hospital, Columbus, OH
Pathways Facilitating Access to Investigational Products: Past to Present (RTT)

9:20 AM – 10:00 AM
Panel Discussion

Scientific Symposium 305

8:00 AM - 10:00 AM
Room: Lincoln

Translating non-viral Gene Delivery to Human Therapeutics - Organized by the Physical Delivery, Therapeutics & Vector Development Committee

CO-CHAIRS: Dexi Liu, PhD and Lauren E. Woodard, PhD

SPEAKERS

8:00 AM – 8:30 AM
Carol H. Miao, PhD. Seattle Children's Research Institute, Seattle, WA, University of Washington, Seattle, WA
Ultrasound Mediated Gene Delivery in Large Animal Models

8:30 AM – 9:00 AM
David Liu, PhD. Harvard University and HHMI, Cambridge, MA, Broad Institute, Cambridge, MA
Base Editing: Chemistry on a Target Nucleotide in the Genome of Living Cells

9:00 AM – 9:30 AM
Scott White, MD. Inovio Pharmaceuticals, Inc., Plymouth Meeting, PA
DNA Electroporation for HIV and other Infectious Diseases

9:30 AM – 10:00 AM
Yoichi Negishi, PhD. Tokyo University of Pharmacy and Life Sciences, Hachioji, Japan
Nucleic Acid and Gene Delivery Systems by the Combination of Nanobubbles and Ultrasound

Oral Abstract Session 310

8:00 AM - 10:00 AM
Room: Georgetown

Non-Nuclease Mediated Genome Editing

CO-CHAIRS: Mark Osborn, PhD and Charles Gersbach, PhD

8:00 AM – 8:15 AM
628: Transcriptional and Position Effect Contributions to rAAV-Mediated Gene Targeting
Laura Spector, Stanford University, Stanford, CA

8:15 AM – 8:30 AM
629: Novel CRISPR Cytosine Base Editors with Minimized Off-Target Effects and Improved Editing Properties
Julian Grünewald, Harvard Medical School, Boston, MA

8:30 AM – 8:45 AM
630: Targeted Transcriptional Modulation with Type I CRISPR-Cas Systems in Human Cells
Adrian Pickar-Oliver, Duke University, Durham, NC

8:45 AM – 9:00 AM
631: Mechanisms of Non-Enzymatic PNA-Mediated Gene Editing
Nicholas Economos, Yale University, New Haven, CT

9:00 AM – 9:15 AM
632: A Novel and Highly Sensitive In Vitro Platform for Detection of Gene Editing Nuclease and Base Editor Off-Target Sites
Karl Petri, Massachusetts General Hospital, Charlestown, MA

9:15 AM – 9:30 AM

633: Cell-Specific CRISPR-Cas9 Activation by MicroRNA-Dependent Expression of Anti-CRISPR Proteins

Mareike Hoffmann, German Cancer Research Center (DKFZ), Heidelberg, Germany

9:30 AM – 9:45 AM

634: Engineered Anti-CRISPR Proteins for Precision Control of CRISPR-Cas9

Dominik Niopek, Synthetic Biology Group, Institute for Pharmacy and Biotechnology (IPMB) and BioQuant Center, University of Heidelberg, Heidelberg, Germany

9:45 AM – 10:00 AM

635: ProxyBE: Improving Efficiency of Single Base Editors by Proximal Cas9 Targeting

Michael Gapinske, University of Illinois at Urbana-Champaign, Urbana, IL

Oral Abstract Session 311

8:00 AM - 10:00 AM

Room: Heights Courtyard 1

Immune Response Mechanisms in Gene and Cell Therapy

CHAIR: Denise Sabatino, PhD

8:00 AM - 8:15 AM

636: Reducing AAV-Mediated Immune Responses and Pathology in a Subretinal Pig Model by Engineering the Vector Genome

Ying Kai Chan, Harvard University, Boston, MA

8:15 AM – 8:30 AM

637: Immune Analysis Following Intrathecal Gene Transfer: 3-Year Data from Clinical Intrathecal Gene Transfer Trial for Patients with Giant Axonal Neuropathy

Diana Bharucha-Goebel, NIH & Children's National Health System, Bethesda, MD

8:30 AM – 8:45 AM

638: Adaptive Immune Responses Directed Against Residual VSVG on Transduced Hematopoietic Stem Cells

Blake Rust, Fred Hutchinson Cancer Research Center, Seattle, WA

8:45 AM – 9:00 AM

639: Immunoprophylaxis of Influenza Using AAV Vector Delivery of Recombinant Cross-Neutralizing Nanobodies

Joanne Marie Del Rosario, University College London, London, United Kingdom

9:00 AM – 9:15 AM

640: Single Cell Transcriptome Analysis of Responses of Mouse Liver Immune Microenvironment Following Intravenous Administration of AAV Vectors

Detu Zhu, Weill Cornell Medical College, New York, NY

9:15 AM – 9:30 AM

641: Assessment of Anti-Cocaine Vaccine dAdGNE in Clinical Cohort 1 of Cocaine Addicts

Stephen Kaminsky, Weill Cornell Medical College, New York, NY

9:30 AM – 9:45 AM

642: Engineering AAV Vectors to Reduce CD8+ T Cell Responses Following Intramuscular Injection in Mice

Alexander Letizia, Harvard Medical School, Boston, MA

9:45 AM – 10:00 AM

643: Monitoring T Cell Responses Toward the *Streptococcus Pyogenes* Cas9 Nuclease

Dimitrios Wagner, Charité Universitätsmedizin, Berlin, Germany

Oral Abstract Session 312

8:00 AM - 10:00 AM

Room: Heights Courtyard 3

Oligonucleotide Therapeutics II

CHAIR: Dirk Grimm, PhD

8:00 AM – 8:15 AM

644: Amino-Acylated LeuCAG3'tsRNA Mediates Translational Elongation of Ribosomal Protein S28 mRNA and is a Key Regulatory Step in Ribosome Biogenesis

Hak Kyun Kim, Stanford Univ, Stanford, CA

8:15 AM – 8:30 AM

645: Splice-Switching Antisense Oligonucleotides for the Treatment of CLN3 Batten Disease

Jessica Centa, Rosalind Franklin University of Medicine and Science, North Chicago, IL

8:30 AM – 8:45 AM

646: Chemically Modified hCFTR mRNAs Recuperate Lung Function in a Mouse Model of Cystic Fibrosis

Michael Kormann, University Children's Hospital, Tübingen, Germany

8:45 AM – 9:00 AM

647: Small Hairpin RNAs Compete with Heart MicroRNAs and Lead to Cardiomyopathy

Meredith Course, University of Washington, Seattle, WA

9:00 AM – 9:15 AM

648: A Common Deep Intronic Mutation Causing Collagen VI-Related Muscular Dystrophy: Validation of Splice-Modulating Approaches *In Vitro* and Development of a Mouse Model

Veronique Bolduc, NINDS/NIH, Bethesda, MD

9:15 AM – 9:30 AM

649: Antisense-Mediated Increase of *SCN1A* Expression Using TANGO Technology for the Treatment of Dravet Syndrome

Isabel Aznarez, Stoke Therapeutics, Bedford, MA

9:30 AM – 9:45 AM

650: Artificial microRNA Silences C9ORF72 Variants *In Vivo* and Decreases the Most Abundant Toxic Dipeptides in BAC Transgenic Mouse

Gabriela Toro Cabrera, UMASS Med, Worcester, MA

9:45 AM – 10:00 AM
651: In Vivo MicroRNA Delivery by Innovative Inorganic Carrier
Xin Wu, Graduate School of Medicine, Osaka University, Suita, Japan

Exhibit Hall Coffee Social

10:00 AM - 10:45 AM
Room: Columbia Hall

Exhibit Hall Open

10:00 AM - 6:00 PM
Room: Columbia Hall

Plenary Session 320

10:45 AM - 11:45 AM
Room: International Ballroom

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

Sponsored by AUDENTES

CHAIR: Stephen J. Russell, MD, PhD

SPEAKER

10:55 AM – 11:45 AM
John J. Rossi, PhD. Beckman Research Institute City of Hope, Duarte, CA
My 40-year Journey to Develop RNA Based Therapies

Break

11:45 AM - 1:15 PM
Lunch Break *(On Own - Not Provided)*

Industry Symposium 330

11:45 AM - 1:15 PM
Room: Jefferson

Considerations for Neuromuscular and CNS-Targeted Gene Therapies

Sponsored by SAREPTA THERAPEUTICS

11:45 AM – 12:30 PM
Neuromuscular Targeted Therapies
Louise Rodino-Klapac, PhD, Sarepta Therapeutics

12:30 PM – 1:15 PM
CNS-Targeted Therapies
Barry Byrne, MD, PhD, University of Florida, College of Medicine
Ronald G. Crystal, MD, Weill Cornell Medical College

Industry Symposium 331

11:45 AM - 1:15 PM

Room: Lincoln

Essential Legal Strategies to Protect Healthcare Professionals License & Increase Their Tax Savings

Sponsored by LEGALLY MINE

SPEAKER

Daniel J. McNeff, Legally Mine, Orem, UT

Presidential Symposium 340

1:15 PM - 3:15 PM

Room: International Ballroom

Presidential Symposium & Presentation of the Top Abstracts

Sponsored by OxfordBioMedica

CHAIR: Michele P. Calos, PhD

SPEAKERS

1:20 PM – 2:15 PM

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

Synthetic Viruses & Tissue Interactions

Presentation of Top Abstracts

2:15 PM – 2:30 PM

652: ASPIRO Phase 1/2 Gene Therapy Trial In X-Linked Myotubular Myopathy (XLMTM): Update on Preliminary Safety and Efficacy Findings

Perry Shieh, UCLA Medical Center, Los Angeles, CA, United States

2:30 PM – 2:45 PM

653: Liver-Targeted Lentiviral Gene Therapy Achieves 100% of Normal Circulating FVIII Levels in Non-Human Primates

Tongyao Liu, Sanofi, Waltham, MA, United States

2:45 PM – 3:00 PM

654: Gene Therapy for the Treatment of AADC Deficiency in Children using MRI-Guided Convection-Enhanced Delivery to the Midbrain

Krystof Bankiewicz, University of California San Francisco, San Francisco, CA, United States

3:00 PM – 3:15 PM

655: Combinational T Cell Genome Modifications through Vector Coupled and Uncoupled Chemical Deamination

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

Exhibit Hall Coffee Social

3:15 PM - 3:45 PM

Room: Columbia Hall

Oral Abstract Session 350

3:45 PM - 5:30 PM

Room: Jefferson

Gene Therapies Directed at the Immune System

CO-CHAIRS: Cynthia Dunbar, MD and Luigi Notarangelo, MD

3:45 PM – 4:00 PM

656: Optimized CRISPR/Cas9 Editing Strategy for the Correction of CD40LG Gene in T Cells and Hematopoietic Stem Cells

Valentina Vavassori, SR-TIGET, Milan, Italy

4:00 PM – 4:15 PM

657: Gene Editing Models and Remedies Severe Congenital Neutropenia in Healthy Donor and Patient CD34+ HSPCs

Shuquan Rao, Boston Children's Hospital, Dana-Farber Cancer Institute, Harvard Stem Cell Institute, Broad Institute, Harvard Medical School, Boston, MA

4:15 PM – 4:30 PM

658: A Potential CRISPR/Cas9-Based Gene Therapy for Severe Congenital Neutropenia

Ngoc Tung Tran, Max Delbrück Center for Molecular Medicine in the Helmholtz Association, Berlin, Germany

4:30 PM – 4:45 PM

659: *In-Vivo* Gene Therapy for Canine X1-SCID via Delivery of Cocal Enveloped Lentiviral Vector Expressing γ-Chain

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

4:45 PM – 5:00 PM

660: Gene Editing to Enforce FOXP3 Expression and a Rapamycin-Inducible IL-2 Signaling Complex in Human Primary T Cells Allows Selective Expansion of Immunosuppressive Treg-Like Cells

Sam West, Seattle Children's Research Institute, Seattle, WA

5:00 PM – 5:15 PM

661: Immunoregulatory Cell Therapy with Lentiviral-Mediated FOXP3 Converted CD4+ T Cells into Treg Cells: Towards the Proof-of-Concept Application in IPEX Syndrome

Yohei Sato, Stanford University, Stanford, CA

5:15 PM – 5:30 PM

662: Engineering B Cells as an Evolving Drug to Fight HIV

Alessio Nahmad, Tel Aviv University, Tel Aviv, Israel

Oral Abstract Session 351

3:45 PM - 5:30 PM

Room: Lincoln

AAV Vector Biology II

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

3:45 PM – 4:00 PM

663: Characterization of Assembly-Activating Protein (AAP) Variants among Natural AAV Isolates with High-Packaging Efficiencies

Hung-Lun Hsu, University of Massachusetts Medical School, Worcester, MA

4:00 PM – 4:15 PM

664: LY6A (SCA-1) Drives AAV-PHP.B Transport Across the Mouse Blood-Brain Barrier

Yuan Yuan, University of Pennsylvania, Philadelphia, PA

4:15 PM – 4:30 PM

665: Functional Consequences of Adeno-Associated Virus Capsid Deamidation

Joshua Sims, University of Pennsylvania, Philadelphia, PA

4:30 PM – 4:45 PM

666: Unbiased Proteomic Approach Identifies Novel Cellular Factors Involved in Aadeno-Associated Vector Production

Anna Maurer, Grousbeck Gene Therapy Center, Mass Eye and Ear, Harvard Medical School, Boston, MA

4:45 PM – 5:00 PM

667: Genome-Wide CRISPR/Cas9 Screening Identifies GPR108 as a Highly Conserved AAV Entry Factor

Amanda Dudek, Grousbeck Gene Therapy Center, Mass Eye and Ear, Harvard Medical School, Boston, MA

5:00 PM – 5:15 PM

668: Identification and Validation of Novel Host Factors for AAV Infection

Hans Meisen, Amgen, South San Francisco, CA

5:15 PM – 5:30 PM

669: Discovery of a Novel Frameshifted ORF within the AAV Capsid Gene

Eric Kelsic, Dyno Therapeutics, Cambridge, MA

Oral Abstract Session 352

3:45 PM - 5:30 PM

Room: Georgetown

Neurological and Neurosensory Gene Therapy

CHAIR: Megan Keiser, PhD

3:45 PM – 4:00 PM

670: Optimizing In Vivo NGLY1 Gene Delivery Towards Gene Therapy for NGLY1 Deficiency

Lingzhi Ren, University of Massachusetts Medical School, Worcester, MA

4:00 PM – 4:15 PM

671: Therapeutic Rescue of Spinal Muscular Atrophy Mouse Models with AAV9-Exon Specific U1 snRNA

Franco Pagani, ICGEB, Trieste, Italy

4:15 PM – 4:30 PM

672: Sustained Mutant Huntingtin Lowering in the Brain and Cerebrospinal Fluid of Huntington Disease Minipigs Mediated by AAV5-miHTT Gene Therapy

Astrid Vallès, uniQure, Amsterdam, Netherlands

4:30 PM – 4:45 PM

673: Investigating Dual AAV-Based Treatments for MYO7A Usher Syndrome in Myo7a-/- Mice and Macaque

Kaitlyn Calabro, University of Florida, Gainesville, FL

4:45 PM – 5:00 PM

674: tIPE - a GMP-Grade Non-Viral Gene Therapy Medicinal Product (GTMP) to Treat Neovascular Age-Related Macular Degeneration (nvAMD

Gabriele Thumann, University of Geneva, Geneva, Switzerland

5:00 PM – 5:15 PM

675: CRISPR/Cas Based Evaluation of the Therapeutic Potential for USH2A Associated Diseases

Nachi Pendse, Harvard Medical School, Boston, MA

5:15 PM – 5:30 PM

676: Optimized Surgical Approach Leads to Highly Efficient AAV Gene Transfer to Inner Hair Cells in Rhesus Macaque

Eva Andres-Mateos, Grousbeck Gene Therapy Center, Mass Eye and Ear, Harvard Medical School, Boston, MA

Oral Abstract Session 353

3:45 PM - 5:30 PM

Room: Monroe

Cancer Gene Therapy

CHAIR: Maria Castro, PhD

3:45 PM – 4:00 PM

677: First in Human Phase I Trial of the Combination of Two Adenoviral Vectors Expressing Either HSV1-TK or FLT3L for the Treatment of Newly Diagnosed Resectable Malignant Glioma at First Diagnosis: Preliminary Results

Pedro Lowenstein, University of Michigan Medical School, Ann Arbor, MI

4:00 PM – 4:15 PM

678: CRISPR/Cas9-Mediated Ablation of the SHP-1 Gene Enhances the Therapeutic Efficacy of IL-13Ra2 CAR T Cells in Glioma Xenograft Models

Christopher Petersen, St. Jude Children's Research Hospital, Memphis, TN

4:15 PM – 4:30 PM

679: A Phase I/IIa Dose Escalation Study Evaluating the Safety and Efficacy of Autologous CD34+ Enriched Hematopoietic Progenitor Cells Genetically Modified for Human Interferon-Alpha2 in Patients with Glioblastoma Multiforme and an Unmethylated MGMT Promoter (TEM-GBM-001)

Gaetano Finocchiaro, Istituto Neurologico Carlo Besta, Milan, Italy

4:30 PM – 4:45 PM

680: Mullerian Inhibiting Substance Type 2 Receptor (MISIIR)-Specific CAR T Cells for the Treatment of Ovarian Cancer and Other Gynecologic Malignancies

Alba Rodriguez-Garcia, University of Pennsylvania, Philadelphia, PA

4:45 PM – 5:00 PM

681: γδ CAR-T Cells Show Dual CAR- and TCR-Mediated Mechanisms of Tumor Cell Recognition against Bone Metastatic Castrate Resistant Prostate Cancer
Emiliano Roselli, Moffitt Cancer Center, Tampa, FL

5:00 PM – 5:15 PM

682: Development of an Allogeneic Universally Tolerated NKT Cell Platform for Of-the-Shelf Cancer Immunotherapy
Leonid S. Metelitsa, Baylor College of Medicine, Houston, TX

5:15 PM – 5:30 PM

683: CAR T Persistence and Anti-Leukemic Efficacy In Vivo are Dependent Upon Lentiviral Vector Internal Promoter: MSCV vs EF-1 Alpha
Dina Schneider, Lentigen Technology, a Miltenyi Biotec Company, Gaithersburg, MD

Oral Abstract Session 354

3:45 PM - 5:30 PM
Room: International Ballroom

RNA Virus Vectors for Therapeutic Applications

CO-CHAIRS: Jennifer Adair, PhD and Axel Schambach, MD, PhD

3:45 PM – 4:00 PM

684: β-Globin Locus Control Region Core Sequences Driving Expression of Anti-Sickling Globin Ameliorates Disease Phenotype in a Mouse Model of Sickle Cell Disease
Richard Morgan, University of California, Los Angeles, CA

4:00 PM – 4:15 PM

685: Liver-Directed Gene Therapy with Lentiviral Vectors in Newborn Mice and Dog Puppies
Alessio Cantore*, San Raffaele Telethon Institute for Gene Therapy, Milan, Italy

4:15 PM – 4:30 PM

686: Evaluation of the Efficacy and Safety of Intraosseous Delivery of Platelet-Specific Factor VIII-Lentiviral Vectors as an In Vivo Gene Therapy for Hemophilia in Humanized NSG Mice
Julia Joo, Seattle Children's Research Institute, Seattle, WA

4:30 PM – 4:45 PM

687: Vectorised 'Mini' hU6 Pol III Promoter Exhibits Nucleosome Redundancy and Supports Multiplexed CRISPR Guide Expression
Roland Preece, GOS Institute of Child Health, University College London, London, United Kingdom

4:45 PM – 5:00 PM

688: Systemically Delivered Zinc Finger Protein Directed Silencing of HIV
Surya Shrivastava, City of Hope, Beckman Research Institute, Duarte, CA

5:00 PM – 5:15 PM

689: High-Efficiency Lentiviral Transduction of Human CD34+ Cells in High-Density Cell Culture with Poloxamer and Prostaglandin E2 Supplementation
Naoya Uchida, National Institutes of Health, Bethesda, MD

5:15 PM – 5:30 PM

690: Gene Delivery to the Corneal Limbal Stem Cells Using AAV and Lentiviral Vectors

Liujiang Song, University of North Carolina, Chapel Hill, NC

Oral Abstract Session 355

3:45 PM - 5:30 PM

Room: Holmead

Cell Therapy for Metabolic Disorders

CHAIR: Stephanie Cherqui, PhD

3:45 PM – 4:00 PM

691: Selective Expansion of Gene-Targeted Hepatocytes Using Acetaminophen Leads to Reproducible Long-Term Liver Repopulation

Anne Vonada, Oregon Health and Science University, Portland, OR

4:00 PM – 4:15 PM

692: Correction of a Metabolic Liver Disease after *Ex Vivo* Gene Editing of Human Hepatocytes

Mihaela Zabulica, Karolinska Institutet, Stockholm, Sweden

4:15 PM – 4:30 PM

693: Characterization of Hematopoietic System Reconstitution In Vivo in Metachromatic Leukodystrophy Gene Therapy Patients

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

4:30 PM – 4:45 PM

694: *Ex Vivo* and *In Vivo* Hematopoietic Stem Cell Gene Therapy of Hemophilia A in Mice

Hongjie Wang, University of Washington, Seattle, WA

4:45 PM – 5:00 PM

695: HSPC Gene Therapy for Cystinosis Remains Effective in Patients Carrying the Large Deletion abolishing*SHPK* expression

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

5:00 PM – 5:15 PM

696: Hepatocyte Transplantation into Lymph Nodes is Curative in the Pig Model of Tyrosinemia

Caitlin VanLith, Mayo Clinic, Rochester, MN

5:15 PM – 5:30 PM

697: Genome Edited Human Hematopoietic Stem Cells Correct Lysosomal Storage Disorders: Proof-of-Concept and Safety Studies for Mucopolysaccharidosis Type I and Gaucher Disease

Natalia Gomez-Ospina, Stanford, Stanford, CA

Oral Abstract Session 356

3:45 PM - 5:30 PM

Room: Heights Courtyard 2

Advances in Cell Product and Gene Vector Manufacturing

CHAIR: Johannes van der Loo, PhD

3:45 PM – 4:00 PM

698: A Robust Commercial rAAV-Vector Platform Process Using the iCELLis® 500 Fixed-Bed Bioreactor

Christian De Carli, Freeline®, Stevenage, United Kingdom

4:00 PM – 4:15 PM

699: Utility of Diatomaceous Earth in Lysate Clarification Simplifies AAV Production

Ru Xiao, Grousbeck Gene Therapy Center, Mass Eye and Ear, Harvard Medical School, Boston, MA

4:15 PM – 4:30 PM

700: Development of an NGS-Based Assay and Bioinformatics Workflow for CMC Characterization of Contaminating DNA in AAV Products

Christina Chaivorapol, Audentes Therapeutics, South San Francisco, CA

4:30 PM – 4:45 PM

701: Lentiviral/Retroviral Vector Large Scale Manufacturing

Margherita Neri, MolMed, Milano, Italy

4:45 PM – 5:00 PM

702: Optimization of Transfection and Culture Conditions to Maximize AAV Production in Suspension 293-Based Viral Production Cells

Bryan Piras, St. Jude Children's Research Hospital, Memphis, TN

5:00 PM – 5:15 PM

703: A Scalable Microfluidic Platform to Enhance Transduction Efficiency and Reduce Viral Vector Usage

Reginald Tran, Aflac Cancer and Blood Disorders Center, Emory University School of Medicine, Atlanta, GA

5:15 PM – 5:30 PM

704: High-Throughput CRISPR/Cas9 Genome-Wide Screens to Enhance AAV Manufacturing

Christopher Barnes, UC Berkeley, Berkeley, CA

Oral Abstract Session 357

3:45 PM - 5:30 PM

Room: Heights Courtyard 1

Oligonucleotide Therapeutics I

CHAIR: Bruce Sullenger, PhD

3:45 PM – 4:00 PM

705: In Vivo Immunoprophylaxis in Non-Human Primates Following Administration of Synthetic DNA-Monoclonal Antibodies (dMAbs)

Ami Patel, The Wistar Institute, Philadelphia, PA

4:00 PM – 4:15 PM

706: Drug Candidate MTL-CEBPA Sensitises Solid Tumours to Standard of Care Therapies

Nagy Habib, Imperial College London, London, United Kingdom

4:15 PM – 4:30 PM

707: Anti-Inflammatory Activity of MTL-CEBPA, a Small Activating RNA Drug, in LPS-Stimulated Monocytes and Humanized Mice

Jiehua Zhou, Beckman Research Institute of City of Hope, Monrovia, CA

4:30 PM – 4:45 PM

708: Inhalation of an RNA Aptamer Targeting Extracellular Histones Protects from Acute Lung Injury

Beilei Lei, Duke Univeristy, Durham, NC

4:45 PM – 5:00 PM

709: mRNA Therapy Improves Metabolic and Behavioral Abnormalities in a Murine Model of Citrin Deficiency

Jingsong Cao, Moderna Inc, Cambridge, MA

5:00 PM – 5:15 PM

710: The Delivery of miR-708 Impairs Breast Cancer Metastasis

Seung Koo Lee, Molecular Imaging Innovations Institute, Weill Cornell Medicine, New York, NY

5:15 PM – 5:30 PM

711: In Vivo SELEX of an Inhibitory NSCLC-Specific RNA Aptamer (RA16) from PEGylated RNA Library & Synthesized RA16 for Targeting & Inhibition

Hanlu Wang, Peking Union Medical College, Suzhou, China

Oral Abstract Session 358

3:45 PM - 5:30 PM

Room: Heights Courtyard 3

Regenerative Medicine II

CHAIR: Jose Cancelas, MD, PhD

3:45 PM – 4:00 PM

712: Impaired Therapeutic Efficacy of Bone Marrow Cells from Post-Myocardial Infarction Patients in the TIME and LateTIME Clinical Trials

Matthew Springer, University of California, San Francisco, San Francisco, CA

4:00 PM – 4:15 PM

713: Targeted Repair of p47-CGD Restores the Ability of iPSC-Derived Macrophages to Kill Bacteria

Denise Klatt, Hannover Medical School, Hannover, Germany

4:15 PM – 4:30 PM

714: Correcting Bleeding Disorders Using Blood Clotting Factors Produced by Shielded Engineered Allogenic Cells

Guillaume Carmona, Sigilon Therapeutics, Cambridge, MA

4:30 PM – 4:45 PM

715: Engineered B Cells as a Universal Platform for the Treatment of Enzymopathies

Branden Moriarity, University of Minnesota, Minneapolis, MN

4:45 PM – 5:00 PM

716: Induced Neural Stem Cell Therapy to Treat Primary Triple Negative Breast Cancer

Wulin Jiang, The University of North Carolina at Chapel Hill, Chapel Hill, NC

5:00 PM – 5:15 PM

717: Intracranial Pancreatic Islet Transplantation Induces Long-Term Cognitive Improvement in Rats with Sporadic Alzheimer's-Like Disease Dementia

Konstantin Bloch, Tel Aviv university, Tel Aviv, Israel

5:15 PM – 5:30 PM

718: Human Mesenchymal Stromal Cells Engineered to Express Collagen VII Can Restore Anchoring Fibrils in Recessive Dystrophic Epidermolysis Bullosa Skin Graft Chimeras

Anastasia Petrova, UCL Great Ormond Street Institute of Child Health, London, United Kingdom

Tools and Technologies Forum 4

4:00 PM - 6:00 PM

Room: Cardozo

CHAIRS: Larry A. Couture, PhD and Timothy J. Miller, PhD

SPEAKERS

4:00 PM – 4:15 PM

Jason Spacek. Alcami, Durham, NC

Meeting the Challenges of Raw Material Testing for Cell & Gene Therapy Manufacturing

4:00 PM – 4:15 PM

Kevin Ryan. Abeona Therapeutics, Cleveland, OH

Meeting the Challenges of Raw Material Testing for Cell & Gene Therapy Manufacturing

4:15 PM – 4:30 PM

Daniel L. Haus, PhD. Biological Industries USA, Cromwell, CT

Advancements in Cell-based Therapies Through Flexible Culture Systems and the Evolution of Custom Media Manufacturing

4:30 PM – 4:45 PM

Greg Crescenzi, MBA. GE Healthcare, Marlborough, MA

GE and G-CON: Solutions that Enable Efficiencies in cGMP Manufacturing

4:45 PM – 5:00 PM

Yan Zhi, PhD. FUJIFILM Diosynth Biotechnologies, Morrisville, NC

Advanced Therapies Capabilities

5:00 PM – 5:15 PM

Richard Hildreth, PhD. STEMCELL Technologies, Inc., Vancouver, BC, Canada

Tools and Reagents for Your Cell Therapy Research

5:15 PM – 5:30 PM
Samira Kiani, MD. Arizona State University, Tempe, AZ
Code of the Wild: AI Inspired Collaborative Story Telling Meets Genetic Engineering

5:30 PM – 5:45 PM
Nick Trotta, PhD. Cell Microsystems, Durham, NC
Automated CRISPR/Cas9 Screening, Sorting and Cloning using the CellRaft AIR™ System

5:45 PM – 6:00 PM
Xiulian Sun, MD, PhD. Yiming Cell, Jinan, China
Construction of a Positive Control for QPCR Quantification of Gene Integration in Gene Therapy Using Lentivirus

Exhibit Hall Networking Reception & Poster Session III

5:00 PM - 6:00 PM
Room: Columbia Hall

Industry Symposium 360

6:00 PM - 7:30 PM
Room: Jefferson

Achieving New Milestones in Neuromuscular Disease: AAV Gene Therapy for X-Linked Myotubular Myopathy

Sponsored by AUDENTES

6:00 PM – 6:05 PM
Introduction to AAV Gene Therapy for Neuromuscular Disorders
Alan Boyd BSc, MB, ChB, FRSB, PFPM. Chief Executive Officer, Boyds Consulting, Cheshire, UK

6:05 PM – 6:15 PM
Designing an AAV Gene Therapy Program for Children with X-Linked Myotubular Myopathy (XLMTM)
Suyash Prasad, MBBS, MRCP, MRCPCH, FFPM. Chief Medical Officer and Senior Vice President, Audentes Therapeutics, San Francisco, CA, USA

6:15 PM – 6:35 PM
The ASPIRO Study: Improvements in XLMTM Muscle Pathology and Biomarkers
Michael W Lawlor MD, PhD. Associate Professor, Pediatric Pathology Clinical Neuromuscular Laboratory and Congenital Muscle Disease Tissue Repository, Medical College of Wisconsin, Milwaukee, WI, USA

6:35 PM – 6:55 PM
ASPIRO: Achieving Ventilator Independence with AT132 in XLMTM
Geovanny Perez MD MSc. Assistant Professor of Pediatrics & Pulmonary Medicine, George Washington University School of Medicine and Health Sciences, Washington, DC, USA

6:55 PM – 7:15 PM
ASPIRO: Attaining Motor Developmental Milestones in Children with XLMTM
Perry Shieh MD, PhD. Associate Professor of Neurology, University of California, Los Angeles, CA, USA

7:15 PM – 7:20 PM
Summary
Alan Boyd
7:20 PM – 7:30 PM
Panel Discussion
Speakers and Audience, Moderated by Alan Boyd

Closing Night Reception

8:00 PM - 11:00 PM
Smithsonian National Portrait Gallery

Sponsored by University of Massachusetts Medical School

Shuttle Bus Transportation
ASGCT will provide free shuttle transportation for the reception at the Smithsonian National Portrait Gallery during the 22nd Annual Meeting on Wednesday, May 1, 2019.

Shuttle buses will leave from the T-Street Entrance (Terrace Level) of the Washington Hilton Hotel.

Meeting Times
Wednesday, May 1, 2019 – 7:30 pm Departure (Running continuously until 11:00 pm)

Business Meeting

7:30 AM – 8:00 AM
Room: Monroe

Coffee Break

7:30 AM - 8:00 AM

Scientific Symposium 400

8:00 AM - 10:00 AM
Room: International Ballroom East

Applications of Gene and Cell Therapy Technologies for Metabolic Diseases - Organized by the Gene & Cell Therapy of Genetic and Metabolic Diseases Committee

CO-CHAIRS: Randy J. Chandler, PhD, MB and Denise Sabatino, PhD

SPEAKERS

8:00 AM – 8:30 AM
Stephanie Cherqui, PhD. University of California, San Diego, La Jolla, CA
Hematopoietic Stem Cell Gene Therapy and Lysosomal Cross Correction for Cystinosis

8:30 AM – 9:00 AM
Alessandra Biffi, MD. DFCI/BCH Cancer and Blood Disorders Center, Boston, MA, University of Padua, Padua, Italy
Optimization of HSC Gene Therapy Approaches for the Treatment of Neurometabolic and Neurodegenerative Conditions

9:00 AM – 9:30 AM
Randy J. Chandler, PhD, MB. National Institutes of Health, Bethesda, MD
Nuclease-free Genome Editing Confers a Hepatocellular Growth Advantage for Corrected Cells in Methylmalonic Acidemia (MMA)

9:30 AM – 10:00 AM
Markus Grompe, MD. Oregon Health & Science University, Portland, OR
Making New Beta Cells by Reprogramming

Scientific Symposium 401

8:00 AM - 10:00 AM
Room: Monroe

Clinical Status of Oligonucleotide-based Drugs - Organized by the Oligonucleotide and RNAi Therapeutics Committee

CO-CHAIRS: Michelle L. Hastings, PhD and Janaiah Kota, PhD

SPEAKERS

8:00 AM – 8:30 AM
Michael A. Panzara, MD, MPH. Wave Life Sciences, Cambridge, MA
Selective Targeting of Mutant Huntingtin Using Stereopure Oligonucleotides as a Potential Therapeutic Approach for Huntington's Disease

8:30 AM – 9:00 AM
Claudia A. Chiriboga, MD, MPH. Columbia University Medical Center, New York, NY
ASO and SMA: Results of Clinical Trials

9:00 AM – 9:30 AM
Muthiah Manoharan, PhD. Alnylam Pharmaceuticals, Cambridge, MA
The First FDA Approved RNAi Therapeutic ONPATTRO and Beyond

9:30 AM – 10:00 AM
Brett P. Monia, PhD. Ionis Pharmaceuticals, Inc., Carlsbad, CA
Antisense Strategies Against Non-Liver Targets

Scientific Symposium 402

8:00 AM - 10:00 AM
Room: Lincoln

Commercializing Gene and Cell Therapies – Planning for Post-Approval Success and Pitfalls to Avoid - Organized by the Commercialization Committee

CO-CHAIRS: Sarah Pitluck, MSc and Katy Spink, PhD

SPEAKERS

8:00 AM – 8:10 AM
Mark W. Skinner, JD. Institute for Policy Advancement Ltd, Washington, DC, McMaster University, Hamilton, Ontario, Canada
Overview

8:10 AM – 8:30 AM
Ron Philip. Spark Therapeutics, Philadelphia, PA
Getting to Commercialization Including the Challenges, Guidelines, Lessons Learned etc.

8:30 AM – 8:50 AM
Chuck Bucklar. BioMarin Pharmaceutical, Inc, Novato, CA
Moving Towards Commercialization of a Potentially Curative Gene Therapy

8:50 AM – 9:10 AM
Jason Meyenburg. Orchard Therapeutics, Boston, MA
Getting to Commercialization including the Challenges, Guidelines, Lessons Learned

9:10 AM – 9:30 AM
Jon Garen. uniQure, Amsterdam, Netherlands
Emerging Business Models for Commercializing Gene Therapy Treatment for Hemophilia

9:30 AM – 10:00 AM
Panel Discussion

Scientific Symposium 403

8:00 AM - 10:00 AM

Room: International Ballroom West

Fitness by Design: Engineering Homing, Persistence and Potency in Cell Therapies - Organized by the Hematologic and Immunologic Gene and Cell Therapy Committee

CO-CHAIRS: Jennifer E. Adair, PhD and Jennifer L. Gori, PhD

SPEAKERS

8:00 AM – 8:30 AM
Jan Joseph. Melenhorst, PhD. University of Pennsylvania, Philadelphia, PA
CAR T Immunotherapies - Engineering Cell Fitness: TET2- disrupted CAR T cells

8:30 AM – 9:00 AM
Hal E. Broxmeyer, PhD. Indiana University School of Medicine, Indianapolis, IN
HSPC Therapies - Engineering Cell Fitness: Enhancing HSPC Homing and Engraftment

9:00 AM – 9:30 AM
Katy Rezvani, MD, PhD. MD Anderson Cancer Center, Houston, TX
NK immunotherapies - Engineering Cell Fitness: Use of IL15

9:30 AM – 10:00 AM
Aude Chapuis, MD. Fred Hutchinson Cancer Research Center, Seattle, WA
Unveiling Mechanisms of Response and Resistance to Instruct Next Generation Cancer Immunotherapy

Scientific Symposium 404

8:00 AM - 10:00 AM

Room: Georgetown

Gene Editing and Gene Modulation for Musculoskeletal Disease - Organized by the Musculo-Skeletal Gene & Cell Therapy Committee

CO-CHAIRS: Niclas E. Bengtsson, PhD and Joel R. Chamberlain, PhD

SPEAKERS

8:00 AM – 8:30 AM
Rhonda Bassel-Duby, PhD. UT Southwestern, Dallas, TX
Gene Editing for DMD

8:30 AM – 9:00 AM
Charles A. Gersbach, PhD. Duke University, Durham, NC
Gene Editing for Duchenne Muscular Dystrophy

9:00 AM – 9:30 AM
Ana Buj Bello, MD, PhD. Genethon, Evry, France, INSERM, Evry, France
CRISPR/Cas9 for Myotonic Dystrophy Type 1

9:30 AM – 10:00 AM
Steven Ghivizzani, PhD. University of Florida, Gainesville, FL
Local scAAV.IL-1Ra Delivery for Osteoarthritis in an Equine Model: Long Term Safety and Efficacy

Scientific Symposium 405

8:00 AM - 10:00 AM

Room: Jefferson

Getting to the Finish Line: Market Success - Organized by the Clinical Trials and Regulatory Affairs Committee

CHAIRS: Richard Morgan, PhD and Adora Ndu, PharmD, JD

SPEAKERS

8:00 AM – 8:30 AM
Lawrence S. Lamb, Jr., MN, PhD. Incysus Therapeutics, Inc., New York, NY
Getting to a Phase I Trial

8:30 AM – 9:00 AM
Sagar A. Vaidya, MD, PhD. Sangamo Therapeutics, Richmond, CA
IRB and Patient Safety 5 Things You Must Know to Start a Human Gene Therapy Trial

9:00 AM – 9:30 AM
Anne-Virginie L. Eggimann, MS. bluebird bio, Cambridge, MA
Industry Perspective on Development and Approval Process to Support Post-Approval Commercialization of Gene Therapy Products

9:30 AM – 10:00 AM
Kimberly Schultz, PhD. FDA, Silver Spring, MD
Potential Challenges Throughout the Development Process

Oral Abstract Session 410

8:00 AM - 10:00 AM

Room: Heights Courtyard 2

Next Generation RNA Virus Vector Technologies

CO-CHAIRS: Christian Brendel, PhD and Bruce Torbett, PhD, MSPH

8:00 AM – 8:15 AM
905: The Impact of Vector Integration on Chromatin Architecture
Monica Volpin, San Raffaele Telethon Institute for Gene Therapy (SR-TIGET), Milan, Italy

8:15 AM – 8:30 AM
906: Identifying Novel Erythroid Specific Enhancers to Optimize Transgene Expression in Beta Globin Gene Therapy
Nikoleta Psatha, Altius Institute for Biomedical Sciences, Seattle, WA

8:30 AM – 8:45 AM
907: High-Resolution Functional Dissection of Enhancers within the B-Globin Locus Control Region using a Lentiviral Vector-Based Massively Parallel Reporter Assay
Mildred Unti, UCLA, Los Angeles, CA

8:45 AM – 9:00 AM
908: Developing Strategies to Improve the Titers and Gene Transfer of Complex Lentiviral Vectors
Jiaying Han, UCLA, Los Angeles, CA

9:00 AM – 9:15 AM

909: Use of Syncytins for the Transduction of Murine and Human B Cells
Youna Coquin, Genethon, Evry, France

9:15 AM – 9:30 AM

910: Analysis of Human iPSCs Generated by a Non-Integrating Measles Virus Vector
Jiyuan Liao, Medical Science, University of Tokyo, Tokyo, Japan

9:30 AM – 9:45 AM

911: Assessing Functionality and Potential of the Next Generation BET-Independent Integrase-CBX MLV Vector for Safer Gene Therapy
Dominique Van Looveren, KU Leuven, Leuven, Belgium

9:45 AM – 10:00 AM

912: Towards Optimising Lentiviral Vectors Through Structure Informed Genome Modification
Eirini Vamva, University of Cambridge, Cambridge, United Kingdom

Oral Abstract Session 411

8:00 AM - 10:00 AM

Room: Heights Courtyard 1

Neurological Diseases

CHAIR: Guangping Gao, PhD

8:00 AM – 8:15 AM

913: Normalization of *Fmr1*RNA and Protein Expression in the Brains of Mice that Model Fragile X Associated Tremor/Ataxia Syndrome (FXTAS) Using CRISPR-Cas9
Carolyn Yrigollen, Children's Hospital of Philadelphia, Philadelphia, PA

8:15 AM – 8:30 AM

914: CRISPR/Cas9 Specific Editing of the Mutant HTT Allele
Alex Mas Monteys, University of Pennsylvania, Philadelphia, PA

8:30 AM – 8:45 AM

915: A GABA-Selective AAV Vector-Based Approach to Up-Regulate Endogenous Scn1a Expression Reverses Key Phenotypes in a Mouse Model of Dravet Syndrome
Stephanie Tagliatela, Encoded Therapeutics, South San Francisco, CA

8:45 AM – 9:00 AM

916: Reversal of Chronic Neuropathic Pain by Functional Neurotransmitter Rewiring of Spinal Nociceptive Neurons after Spinal Segment-Targeted Subpial Dual Gene (GAD65 and VGAT) Delivery
Takahiro Tadokoro, University of California, San Diego, LA JOLLA, CA

9:00 AM – 9:15 AM

917: Intrathecal scAAV9/SUMF1 Gene Therapy for Multiple Sulfatase Deficiency
Rachel Bailey, University of Texas Southwestern Medical Center, Dallas, TX

9:15 AM – 9:30 AM

918: Respiratory Directed Gene Therapy to Silence SOD1 Prolongs Survival in the SOD1 ALS Mouse

Allison Keeler, University of Massachusetts Medical School, Worcester, MA

9:30 AM – 9:45 AM

919: A NeuroD1-Based Gene Therapy for Brain Repair

Gong Chen, Penn State Univ, University Park, PA

9:45 AM – 10:00 AM

920: AAV Gene Transfer Halts Disease Progression in Sheep with CLN5 Batten Disease after Pre- Or Post-Symptomatic Administration

Nadia Mitchell, University of Otago, Christchurch, New Zealand

Oral Abstract Session 412

8:00 AM - 10:00 AM

Room: Heights Courtyard 3

AAV Vector Biology I

CHAIR: Eric Kelsic, PhD

8:00 AM – 8:15 AM

921: Identification of VP-Interacting Cellular Proteins in AAV Capsid Assembly by Proximity Proteomics

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

8:15 AM – 8:30 AM

922: A Single Amino Acid Residue in the VP1 Unique Region Plays an Essential Role in AAV Capsid Formation

Kei Adachi, Oregon Health & Science University, Portland, OR

8:30 AM – 8:45 AM

923: A CRISPR Screen Identifies Apical Polarity Determinant Crumbs 3 as an AAV Host Restriction Factor

Victoria Madigan, Duke University, Durham, NC

8:45 AM – 9:00 AM

924: Comparative Analysis of the Capsid Structures of AAVrh.10 and AAVrh.39 and Their Antigenic Interactions and AAVrh.39 and Their Antigenic Interactions

Mario Mietzsch, University of Florida, Gainesville, FL

9:00 AM – 9:15 AM

925: Mass Spectrometric Identification of Host Factors that Restrict AAV Vector Genome Transcription

Victoria Madigan, Duke University, Durham, NC

9:15 AM – 9:30 AM

926: AAVR Independent AAV Serotypes Require Neuraminidase 1 and Cathepsin A for Cellular Entry

Amanda Dudek, Grousbeck Gene Therapy Center, Mass Eye and Ear, Harvard Medical School, Boston, MA

9:30 AM – 9:45 AM

927: The Role of miRNA 17-92 Cluster in AAV2 Vector-Mediated Transgene Expression

Baozheng Li, University of Florida College of Medicine, Gainesville, FL

9:45 AM – 10:00 AM

928: Assembly Activating Protein Affects the Association between Ubiquitin and Adeno-Associated Virus Viral Proteins

Ana Cepeda Diaz, Grousbeck Gene Therapy Center, Schepens Eye Research Institute, Mass Eye and Ear, Boston, MA

Break

10:00 AM - 10:15 AM

Oral Abstract Session 420

10:15 AM - 12:15 PM

Room: Jefferson

Neurosensory Diseases

CHAIR: Alberto Auricchio, MD

10:15 AM – 10:30 AM

929: Therapeutic Efficacy of ARCUS Meganuclease Gene Editing for Autosomal Dominant Retinitis Pigmentosa

Robert Brown, Precision BioSciences, Durham, NC

10:45 AM – 11:00 AM

931: Ocular Safety of Long-Term Suppression of VEGF by Intravitreally-Administered Gene Therapy, ADVM-022, in Non-Human Primates

Claire Gelfman, Adverum Biotechnologies, Inc., Menlo Park, CA

11:00 AM – 11:15 AM

932: Restoration of Hearing in Tmc1-Deficient Mice Depends on Early and Well-Regulated Gene Delivery

Adam Palermo, Decibel Therapeutics, Boston, MA

11:15 AM – 11:30 AM

936: Novel AAV Capsids Display Expansive Transduction and Enhanced Potency in Subretinally Injected Mice

Sean Crosson, University of Florida, Gainesville, FL

11:30 AM – 11:45 AM

934: AAV-CRISPR/Cas9-Mediated Gene Knock-In Therapy to Rescue Photoreceptor Degeneration in the P23H Rhodopsin Mutant Mice

Duc Hoang, City University of Hong Kong, Hong Kong, Hong Kong

11:45 AM – 12:00 PM

935: Long-Term Rescue of Retinal Degeneration in *Rho*-P23H Knockin Mice *via* Dual AAV-Medicated Allele-Specific CRISPR-Cas9 Gene Editing

Andrea D'Amico, Ocular Genomics Institute, Mass Eye and Ear, Harvard Medical School, Boston, MA

12:00 PM – 12:15 PM

933: An AAV-CRISPR/Cas9 Gene Editing Approach for GUCY2D-Associated Cone Rod Dystrophy (CORD6)

R Mellen, University of Florida, Gainesville, FL

Oral Abstract Session 421

10:15 AM - 12:15 PM

Room: Lincoln

CAR T Cell Therapy II

CHAIR: Sarwish Rafiq, PhD

10:15 AM – 10:30 AM

937: Chimeric Antigen Receptors with a MyD88 and CD40 Endodomain Endow T Cells with Superior Antitumor Activity

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

10:30 AM – 10:45 AM

938: Library Assembly and Selection of 4-1BB:zeta CAR Activation-Dependent Synthetic Promoters in Primary Human T Cells

Jia Wei, Seattle Children's Research Institute, Seattle, WA

10:45 AM – 11:00 AM

939: Hampering Tumor Glycosylation Improves the Therapeutic Index of CAR-T Cells Against Solid Malignancies

Beatrice Greco, Vita-Salute San Raffaele University, Milan, Italy

11:00 AM – 11:15 AM

940: Augmenting CAR T Cell Mediated Anti-Tumor Efficacy through Genetic Modification to Secrete a Novel Cytokine IL-36

Xinghuo Li, Weill Cornell Graduate School of Medical Sciences, New York, NY

11:15 AM – 11:30 AM

941: Novel Genomic Safe Harbors for Effective CAR T Cell Engineering

Ashlesha Odak, MSKCC, New York, NY

11:30 AM – 11:45 AM

942: Uni-Vect: Antigen-Inducible Immunomodulatory Molecule Expression from a Single Lentiviral Vector Platform to Enhance CAR T Cell Functions

Anze Smole, Perelman School of Medicine at the University of Pennsylvania, Philadelphia, PA

11:45 AM – 12:00 PM

943: Improving CAR T Cell Therapy for Solid Tumors by Adopting a Dual-Targeting Binary Strategy

Pradip Bajgain, Baylor College of Medicine, Houston, TX

12:00 PM – 12:15 PM

944: Aborted Activation of CAR T Cells by Chronic Lymphocytic Leukemia Cells

McKensie Collins, University of Pennsylvania, Philadelphia, PA

Oral Abstract Session 422

10:15 AM - 12:15 PM

Room: Monroe

AAV Vectors and Disease Targets II

CHAIR: Nicole Paulk, PhD

10:15 AM – 10:30 AM

953: Pre-Clinical Gene Therapy in a Mouse Model for Charcot-Marie-Tooth Type 4J

Maximiliano Presa, The Jackson Laboratory, Bar Harbor, ME

10:30 AM – 10:45 AM

954: Novel AAVHSCs Demonstrate Efficient Crossing of the Blood-Brain-Barrier and Potential in Gene Therapy for Metachromatic Leukodystrophy (MLD)

Jacinthe Gingras, Homology Medicines Inc, Bedford, MA

10:45 AM – 11:00 AM

955: Whole Brain Delivery of an Instable MeCP2 Transgene Fully Protects from the Behavioral and Molecular Pathological Defects in Mouse Models of Rett Syndrome

Mirko Luoni, San Raffaele Scientific Institute, Milan, Italy

11:00 AM – 11:15 AM

956: Systemic Delivery of AAV9.LAMP2B for the Treatment of Danon Disease: Toxicology Studies in Mice and Cynomolgus Monkeys

Annahita Keravala, Rocket Pharmaceuticals, Inc, New York, NY

11:15 AM – 11:30 AM

957: Assaying Patterns of rAAV Integration in Humanized Mice: Non-Random Integration Targets Genes, Especially Exons

Stephanie Smith, National Institutes of Health, Bethesda, MD

11:30 AM – 11:45 AM

958: AAV Gene Therapy in a Canine Model of MPS1 Prevents and Reverses Corneal Blindness

Telmo Llanga, UNC Chapel Hill, Chapel Hill, NC

11:45 AM – 12:00 PM

959: Towards AAV5-Mediated Gene Therapy for Hemophilia A with a Factor IX Variant that Functions Independently of FVIII

Ying Poi Liu, uniQure Biopharma B.V., Amsterdam, Netherlands

12:00 PM – 12:15 PM

960: Development of an AAV5-Based Gene Therapy for Fabry Disease

Ying Poi Liu, uniQure Biopharma B.V., Amsterdam, Netherlands

Oral Abstract Session 423

10:15 AM - 12:15 PM

Room: Georgetown

AAV Vectors and Disease Targets III

CHAIR: Dan Wang, PhD

10:15 AM – 10:30 AM

945: Intra-Articular AAV9 α-Iduronidase Gene Therapy in the Canine Model of Mucopolysaccharidosis Type I Results in Rapid Synovial and Cartilage Iduronidase Expression, Clearance of Heparan Sulfate, and High Serum α-Iduronidase Levels

Shih-hsin Kan, CHOC Children's Hospital, Orange, CA

10:30 AM – 10:45 AM

946: Characterization of AAV5-FVIII-SQ Vector DNA in Human Blood by Real-Time and Droplet Digital™ PCR

Chris Russell, BioMarin Pharmaceutical Inc., Novato, CA

10:45 AM – 11:00 AM

947: Safety and Efficacy of an Artificial miRNA Targeting Huntingtin in *Cynomolgus Macaques*

Chris Mueller, UMass Medical School, Worcester, MA

11:00 AM – 11:15 AM

948: Evaluation of a Dual Function AAV3b Vector as a Therapy for Alpha-1 Antitrypsin Deficiency in Non-Human Primates

Gwladys Gernoux, UMass Medical School, Worcester, MA

11:15 AM – 11:30 AM

949: Efficient Corneal Gene Delivery Following Subconjunctival Administrations of AAV Vectors

Liujiang Song, University of North Carolina, Chapel Hill, NC

11:30 AM – 11:45 AM

950: Integration of rAAV in Human Hepatocytes

Dhwanil Dalwadi, OHSU, Portland, OR

11:45 AM – 12:00 PM

951: AAV-Mediated Expression of Monoclonal Antibodies for the Prevention of Marburg Virus Infection

Amira Rghei, University of Guelph, Guelph, ON, Canada

12:00 PM – 12:15 PM

952: Increased Volume Dramatically Enhances AAV Genome Persistence, Transduction Efficiency, and Spread Following Corneal Intrastromal Injection

Matt Hirsch, University of North Carolina, Chapel Hill, NC

Oral Abstract Session 424

10:15 AM - 12:15 PM

Room: International Ballroom East

Nuclease Mediated Genome Editing

CO-CHAIRS: Shondra Pruett-Miller, PhD and Scot Wolfe, PhD

10:15 AM – 10:30 AM

961: Precise Gene Editing Preserves Hematopoietic Stem Cell Function Following Transient p53-Mediated DNA Damage Response

Giulia Schiroli, SR-Tiget, Milan, Italy

10:30 AM – 10:45 AM

966: Genome Editing by Clade F AAV Homology Donors in the Presence and Absence of Site-Specific DNA Breaks

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

10:45 AM – 11:00 AM

963: CRISPR/Cas9 Fusion to Dominant Negative 53BP1 Confers High Efficiency HDR-Based Genome Editing and Inhibits NHEJ Only at Cas9 Target Sites

Devin Pillis, Cincinnati Children's Hospital Medical Center, Cincinnati, OH

11:00 AM – 11:15 AM

964: Site Specific Knock-In Genome Editing in Human HSCs Using Baboon Envelope gp Pseudotypedviral Derived "Nanoblades" Loaded with Cas9/sgRNA Combined with Donor Encoding AAV-6

Els Verhoeyen, CIRI, INSERM U1111, ENS de Lyon, Lyon, France

11:15 AM – 11:30 AM

965: CRISPR/Cas9-Engineered Transgenesis of Hematopoietic Stem Cells via NHEJ-Mediated Targeted Integration Retains Engraftment Potential

Gene Uenishi, Casebia Therapeutics LLC, Cambridge, MA

11:30 AM – 11:45 AM

962: *Ex Vivo* Editing of Hematopoietic Stem Cells for Erythroid-Specific Expression of Therapeutic Proteins

Giulia Pavani, Genethon, Evry, France

11:45 AM – 12:00 PM

967: HDR-CRISPR: A Novel System to Promote Cas9-Mediated Homology Directed DNA Repair

Antonio Carusillo, Universitätsklinikum Freiburg, Freiburg Im Breisgau, Germany

12:00 PM – 12:15 PM

968: Advances in Genome Editing Using Adeno-Associated Virus Delivery of a Compact, Hyper-Accurate Cas9 with a Dinucleotide PAM

Raed Ibraheim, University of Massachusetts Medical School, Worcester, MA

Oral Abstract Session 425

10:15 AM - 12:15 PM

Room: International Ballroom West

Gene Editing for Red Blood Cell Disorders

CO-CHAIRS: Jennifer Gori, PhD and Annarita Miccio, PhD

10:15 AM – 10:30 AM

969: Editing Aberrant Splice Sites with Cas9 and Cas12a Efficiently Restores β-Globin Expression in β-Thalassemia

Kevin Luk, University of Massachusetts Medical school, Worcester, MA

10:30 AM – 10:45 AM

970: Towards The Clinical Translation of Gene Correction in Hematopoietic Stem Cells for Sickle Cell Disease Treatment

Annalisa Lattanzi, Stanford University, Stanford, CA

10:45 AM – 11:00 AM

971: Correction of B-Thalassemia Phenotype by CRISPR/CAS9 Editing of the Human A-Globin Locus

Giulia Pavani, Genethon, Evry, France

11:00 AM – 11:15 AM

972: Zinc Finger Nuclease-Mediated Disruption of the BCL11A Erythroid Enhancer in Human Hematopoietic Stem and Progenitor Cells Results in Enriched Bialleleic Editing with Highly Replicable and Precise On-Target Small Indels and Allele-Additive Increases in Fetal Hemoglobin

Hui Ling, Rare Blood Disorders, Sanofi Genzyme, Waltham, MA

11:15 AM – 11:30 AM

973: Lentivirus-Mediated Expression of the RNA-Binding Protein IGF2BP1 Reverses Fetal-To-Adult Hemoglobin Production in Culture-Differentiated Erythroid Cells from Patients with Severe Hemoglobin Disorders

Andrew Wilber, SIU School of Medicine, Springfield, IL

11:30 AM – 11:45AM

974: Stable and Therapeutically Relevant Long-Term Engraftment of CRISPR/Cas9-Edited HSCs for HbF Reactivation in NHPs

Olivier Humbert, Fred Hutchinson Cancer Research Center, Seattle, WA

11:45 AM – 12:00 PM

975: Efficient Genome Editing of the PKLR Locus in Human Long-Term Hematopoietic Stem Cells Using Specific CRISPR/CAS9 RNP and AAV6-Delivery of Donor Templates to Treat Pyruvate Kinase Deficiency

Sara Fañanas-Baquero, Centro de Investigaciones Energéticas Medioambientales y Tecnológicas/Centro de Investigación, Madrid, Spain

12:00 PM – 12:15 PM

976: Gene Correction of Beta-Thalassemia Ex Vivo and In Vivo Mediated by PNA Nanoparticles

Alexandra Piotrowski-Daspit, Yale University, New Haven, CT

Oral Abstract Session 426

10:15 AM - 12:15 PM

Room: Heights Courtyard 2

Engineered Cell Therapies

CHAIR: Jane Lebkowski, PhD

10:15 AM – 10:30 AM

977: Mesenchymal Stem Cell-Mediated Delivery of Artificial Transcription Factors for Neurologic Disease

Peter Deng, UC Davis, Davis, CA

10:30 AM – 10:45 AM

978: Reprogramming CAR-T Cells to Metabolize Lactate as a Fuel in Oxygen-Deprived and Nutrient-Deficient Environments

Roddy O'Connor, University of Pennsylvania, Philadelphia, PA

10:45 AM – 11:00 AM

979: A Novel Aryl Hydrocarbon Receptor Antagonist Expands Adult Human Hematopoietic Stem Cells from Mobilized Peripheral Blood and Bone Marrow and Increases the Dose of CRISPR/Cas9 Gene-Edited NSG-Repopulating Cells
Megan Hoban, Magenta Therapeutics, Cambridge, MA

11:00 AM – 11:15 AM

980: Can't Live without "U": Genetic Engineering of *UMPS* to Create Auxotrophy in Human Cells
Volker Wiebking, Stanford University, Stanford, CA

11:15 AM – 11:30 AM

981: Directing Skeletal Myogenic Progenitor Cell Lineage Specification with CRISPR/Cas9-Based Transcriptional Activators
Jennifer Kwon, Duke University, Durham, NC

11:30 AM – 11:45 AM

982: Sustained Engraftment and Protein Secretion using Gene-Edited Human B Cells in Humanized Mouse Models
Richard James, Seattle Children's Research Institute, Seattle, WA

11:45 AM – 12:00 PM

983: UM171 Efficiently Expands Genetically Modified Haematopoietic Stem Cells from Mobilized Blood and Transduces Preferentially the Erythroid Lineage
Ioanna Vallianou, George Papanikolaou Hospital, Thessaloniki, Greece

12:00 PM – 12:15 PM

984: Generation of High-Purity Human iPS Cell-Derived Hepatocyte like Cells Using CRISPR-Cas9 System
Kazuo Takayama, Osaka University, Suita, Japan

Oral Abstract Session 427

10:15 AM - 12:15 PM

Room: Heights Courtyard 1

Immunotherapy II

CHAIR: Christopher Peterson, PhD

10:15 AM – 10:30 AM

985: Depletion of CD45RA-Positive Cells Removes an Inhibitory Component that Potentiates the Reactivation of EBV-Specific T-Cells from Lymphoma Patients
Sandhya Sharma, Baylor College of Medicine, Houston, TX

10:30 AM – 10:45 AM

986: Targeting Autophagy Enhances the Adoptive Immunotherapy of Glioblastoma with Multi-Functional Genetically-Engineered NK Cells
Jiao Wang, Purdue University, West Lafayette, IN

10:45 AM – 11:00 AM

987: Next Generation T-Cell Therapy for CMV Infection Post Allogeneic SCT
Manar Shafat, UCL Cancer Institute, London, United Kingdom

11:00 AM – 11:15 AM

988: Engineered Type-1 Regulatory T Cells for Treatment of Graft-versus-Host Disease in Allogeneic Hematopoietic Stem Cell Transplant Recipients

Jeffrey Liu, Stanford University, Stanford, CA

11:15 AM – 11:30 AM

989: Potentiating Engineered T Cells with Durable Epigenetic Repression of Immune Checkpoint Genes

Shon Green, Altius Institute for Biomedical Sciences, Seattle, WA

11:30 AM – 11:45 AM

990: Differentiating T-Cells from hiPSCs to Create Off-The-Shelf SPEAR T-Cell Therapies

Joanna Brewer, Adaptimmune, Abingdon, United Kingdom

11:45 AM – 12:00 PM

991: Nascent Transcripts of Target-Activated CD19-CAR-T Cells Reveal Early Activation of the C-REL and NFκB Pathway

Matti Korhonen, Finnish Red Cross Blood Service, Helsinki, Finland

12:00 PM – 12:15 PM

992: AdoptCell®-NK: A New Class NK Cells Manufactured in Accordance with GMP/GCTP that Can Eliminate the Solid Tumors

Yui Harada, Kyushu University, Fukuoka, Japan

Oral Abstract Session 428

10:15 AM - 12:15 PM

Room: Heights Courtyard 3

Use of New Technologies for Hepatic Therapy

CHAIR: Charles Venditti, MD, PhD

10:15 AM – 10:30 AM

993: Efficient and Long-Term Correction of Liver Metabolic Diseases by Coupling AAV-Mediated Promoterless Gene Targeting to SaCas9 Nuclease

Alessia De Caneva, ICGEB, Trieste, Italy

10:30 AM – 10:45 AM

994: Gene Editing Approach to Disrupt Hydroxyacid Oxidase 1 for the Treatment of Primary Hyperoxaluria Type 1

Jenny Greig, University of Pennsylvania, Philadelphia, PA

10:45 AM – 11:00 AM

995: Nuclease-Free and Promoter-Less Aavhsc-Mediated Genome Editing *In Vivo* Corrects the Disease Phenotype in a Mouse Model of Phenylketonuria

Jason Wright, Homology Medicines, Inc., Bedford, MA

11:00 AM – 11:15 AM

996: Reduction of Transthyretin Expression by AAV Gene Delivery of a Novel Endonuclease in Mice

Jenny Greig, University of Pennsylvania, Philadelphia, PA

11:15 AM – 11:30 AM

997: Exploiting the Regenerative Capacity of Liver for Nuclease-Free Genome Editing

Francesco Puzzo, Stanford University, Stanford, CA

11:30 AM – 11:45 AM

998: Genome Editing of the Liver for Treatment of Alpha-1 Antitrypsin Deficiency Using Homology-Independent Targeted Integration (HITI)

Joost van Haasteren, University of Oxford, Oxford, United Kingdom

11:45 AM – 12:00 PM

999: Hemophilia a Cured in Mice by Crispr-Based In Vivo Genome Editing of Human Factor FVIII

Ruby Chen-Tsai, Applied Stemcell Inc., Milpitas, CA

12:00 PM – 12:15 PM

1000: CRISPR/Cas9-Mediated Gene Knockout to Address Primary Hyperoxaluria

Anette Huebner, Intellia Therapeutics, Cambridge, MA

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Food and Drug Administration Center for Biologics Evaluation and Research

10903 New Hampshire Avenue
Silver Spring, MD 20993
Phone: (240) 402-8181
E-mail: industry.biologics@fda.hhs.gov
Website: www.fda.gov

BOOTH NUMBER: 422

Food and Drug Administration's Center for Biologics Evaluation and Research is a U.S. government agency. Knowledgeable staff can provide information on biological products including vaccines, blood and blood products, allergenics and cells, tissues, and gene therapy products.

Fresenius Kabi USA, LLC

3 Corporate Drive
Lake Zurich, IL 60047
Phone: (847) 550-2625
E-mail: ben.nelson@fresenius-kabi.com
Website: www.chooselovo.com

BOOTH NUMBER: 517 & 519

Fresenius Kabi is a leading global health care company that focuses on pharmaceuticals and medical devices used to care for critically and chronically ill patients. Our Lovo device is the only cell processing system that washes and concentrates white blood cells using filtration technology to serve the needs of the cellular therapies community.

FUJIFILM Diosynth Biotechnologies

101 J Morris Commons Lane
Morrisville, NC 27526
Phone: (919) 325-6972
E-mail: liza.rivera@fujifilm.com
Website: www.fujifilmdiosynth.com

BOOTH NUMBER: 232

As a CDMO our focus is to combine technical leadership in cell culture, microbial fermentation and viral vectors with world class cGMP manufacturing facilities to advance tomorrow's medicines. For over 25 years our focus has been on supporting our customers with the development and manufacture of recombinant proteins, viral vaccines and gene therapies.

GE Healthcare

100 Results Way
Marlborough, MA 01752
Phone: (978) 760-8186
E-mail: seth.cooper@ge.com
Website: http://www.gelifesciences.com/celltherapy

BOOTH NUMBER: 227 & 229

The emergence of cell therapy is rapidly changing the healthcare landscape. We are investing in the field for the long-term, and partnering with pharma, biotech and clinical researchers. GE brings expertise in quality, global distribution, R&D, and regulatory that will transform cell therapy to a globally-accessible personalized medicine.

Gene Therapy Center Austria (GTCA) | Takeda

Industriestraße 67
Vienna 1220
Austria
Phone: 43 12 0100 247
E-mail: GTCA@takeda.com
Website: www.GTCAustria.com

BOOTH NUMBER: 231

Gene Therapy Center Austria (GTCA), now part of Takeda, is your innovative partner for high-quality AAV solutions bringing your gene therapy product into phase III and to the market. GTCA is a one-stop-shop covering all steps from vector to product. Our well-established, suspension-based Gene Therapy platform allows vector optimization, process development and GMP manufacturing of your product. Our proprietary platform process is applicable to all AAVs and commercial-ready.

Gene Tools, LLC

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

BOOTH NUMBER: 213

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
E-mail: mrosenfeld@genesisbps.com
Website: www.genesisbps.com

BOOTH NUMBER: 530

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 427900
E-mail: contact@genewerk.com
Website: www.genewerk.com

BOOTH NUMBER: 331

GeneWerk specializes in vector safety and integration site analysis for gene therapy and offers a wide variety of other custom-tailored services around safety and efficacy such as analyses for gene-editing on/off-targets, TCR/BCR immune repertoire or AAV impurities. Our goal is to assist our clients in moving towards safer therapies.

Genezen Laboratories, Inc.

1075 Foster Road
Iowa City, IA 52245
Phone: (317) 822-8330
E-mail: BVincent@GenezenLabs.com
Website: www.GenezenLabs.com

BOOTH NUMBER: 208

Genezen Laboratories works with leading academic centers to make available their Viral Vector Manufacturing and Testing capabilities to commercial clients. We are also able to support our clients clinical trial efforts through cell transduction and expansion to support patient treatment.

GenoSafe

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Evry 91000
France
Phone: 33169471157
E-mail: ahuguenot@genosafe.com
Website: www.genosafe.com

BOOTH NUMBER: 518

GenoSafe is a CRO providing analytical testing services under GLP-GMP-GCP compliance. We help our clients through their drug development process by caring out the laboratory assays of their innovative products (AAV, Lentivirus, Retrovirus, CAR-T cell, Stem Cells). GenoSafe has been involved in immunogenicity studies, biodistribution-biodissemination studies and manufactured batches characterization.

GenScript USA Inc.

860 Centennial Ave.
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E-mail: tracy.yin@genscript.com
Website: www.genscript.com

BOOTH NUMBER: 324

GenScript encompasses extensive services in gene synthesis and molecular biology, peptide synthesis, protein expression and engineering, custom antibody development and engineering as well as variety of catalog products. With the goal of "Making Research Easy", GenScript has striven to remain a reliable research partner for scientists across the globe.

Imanis Life Sciences

221 1st Ave. SW, Suite 102
Rochester, MN 55902
Phone: (507) 218-2559
E-mail: gonzalez.brina@imanislife.com
Website: www.imanislife.com

BOOTH NUMBER: 420

Imanis Life Sciences founded by Mayo Clinic scientists with a mission to improve the accuracy, cost, and speed of noninvasive in vivo imaging technologies. We serve some of the world's leading academic and commercial therapy organizations with our NIS technology, and an encompassing line of reporter gene cell lines, lentiviral vectors, and oncolytic viruses.

Integrated DNA Technologies

1710 Commercial Park
Coralville, IA 52241
Phone: (800) 328-2661
E-mail: custcare@idtdna.com
Website: https://www.idtdna.com/pages

BOOTH NUMBER: 432

Integrated DNA Technologies (IDT) is the world leader in delivering custom nucleic acid products for life sciences and medical research, serving academic, clinical, biotechnology, pharmaceutical development, and agricultural research communities. IDT product applications include qPCR, gene construction, CRISPR genome editing, next generation sequencing, and functional genomics.

IsoPlexis

35 NE Industrial Rd.
Branford, CT 06405
Phone: (203) 208-4111
E-mail: events@isoplexis.com
Website: www.isoplexis.com

BOOTH NUMBER: 235

IsoPlexis has developed a novel single cell proteomics platform at the forefront of the revolution in immuno-oncology. Our platform allows researchers from Yale, Caltech, UCLA, and Memorial Sloan Kettering Cancer Center to identify and predict patient responses from the single-cell level, advancing our understandings of personalized cancer therapy.

JPT Peptide Technologies GmbH

Volmerstr. 5
Berlin 12489
Germany
Phone: 49-30-6392-7878
E-mail: cortes@jpt.com
Website: www.jpt.com

BOOTH NUMBER: 528

JPT provides peptide based products and services for all the development phases of new generation immunotherapeutics.

KBI Biopharma Inc.

1101 Hamlin Rd.
Durham, NC 27704
Phone: (919) 479-9898
E-mail: bnewsom@kbibiopharma.com
Website: https://www.kbibiopharma.com/

BOOTH NUMBER: 410

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.

LakePharma Inc.

201 Industrial Rd.
San Carlos, CA 94070
Phone: (888) 406-5658
E-mail: inquiries@lakepharma.com
Website: www.lakepharma.com

BOOTH NUMBER: 323

LakePharma is the leading US-based biologics CRDMO. The company has developed a range of technologies and capabilities in antibody discovery, protein engineering, cell line and process development, and GMP manufacturing. With comprehensive technologies and integrated platforms, LakePharma enables and supports the discovery and development of the biologics of tomorrow.

Logos Biosystems

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E-mail: info@logosbio.com
Website: www.logosbio.com

BOOTH NUMBER: 425

Logos Biosystems specializes in advanced imaging tools and technologies for diverse applications including basic research, quality control, and drug discovery. High content image acquisition and analysis is simplified with the CELENA X High Content Imaging System. Automated cell counters provide cell concentration and viability data with speed, accuracy, and reliability.

Lonza Pharma & Biotech

14905 Kirby Drive
Houston, TX 77047
Phone: (713) 568-6190
E-mail: pharma@lonza.com
Website: http://pharma.lonza.com/

BOOTH NUMBER: 523 & 525

We provide CDM services that enable pharma and biotech companies to bring medicines to patients in need. From the building blocks of life to the final drug product, our solutions are created to simplify your outsourcing experience and provide a reliable outcome, at the time you expect it.

Lovelace Biomedical

2425 Ridgecrest Dr. SE
Albuquerque, NM 87108
Phone: (202) 819-5108
E-mail: mmcmullen@lovelacebiomedical.org
Website: https://lovelacebiomedical.org/

BOOTH NUMBER: 316

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

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Westborough, MA 01581
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E-mail: michele.giordano@malvern.com
Website: www.malvernpanalytical.com

BOOTH NUMBER: 309

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.malvern.com

Mary Ann Liebert, Inc.

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Phone: (914) 740-2100
E-mail: jgatti@liebertpub.com
Website: www.liebertpub.com/hgt

BOOTH NUMBER: 317

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 gene therapy field! Visit us at our booth to get free access.

MaxCyte

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E-mail: marketing@maxcyte.com
Website: www.maxcyte.com

BOOTH NUMBER: 311 & 313

MaxCyte is a global cell, gene and protein-based life sciences company helping to advance unmet medical needs. Nine of the top 10 global biopharmaceutical companies are developing next-generation therapeutics utilizing MaxCyte's Flow Electroporation® Technology. MaxCyte's more than 55 partnered program licenses includes in excess of 25 licensed for clinical use.

MilliporeSigma

400 Summit Drive
Burlington, MA 01803
Phone: (978) 762-5100
E-mail: duncan.liew@milliporesigma.com
Website: http://www.emdmillipore.com

BOOTH NUMBER: 216

MilliporeSigma, a leader in life science, is a business of Merck KGaA, Darmstadt, Germany. Our purpose is to solve the toughest problems in life science by collaborating with the global scientific community. We provide scientists and engineers with best-in-class lab materials, technologies and services.

Miltenyi Biotec Inc.

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

BOOTH NUMBER: 407 & 409

At Miltenyi Biotec, we are committed to enabling the delivery of new cell and gene therapy (CGT) treatment options to patients with unmet medical needs. We develop tailored cell processing protocols for our GMP-compliant cell processing platform, the CliniMACS Prodigy®, to enable fully closed and automated manufacturing of complex CGT products.

National Gene Vector Biorepository (NGVB)

980 West Walnut Street, Bldg. R3 C650
Indianapolis, IN 47460
Phone: (317) 274-4519
E-mail: lrubin@iu.edu
Website: https://www.ngvbcc.org

BOOTH NUMBER: 318

The National Gene Vector Biorepository (NGVB) is a NIH/NHLBI funded resource 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 318 for more information.

Nature Technology Corporation

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E-mail: hodgson@natx.com
Website: www.natx.com

BOOTH NUMBER: 234

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 Nanoplasmids. NTC provides custom design, synthesis, manufacturing and tech transfer, resulting in rapid development of APIs, ready for preclinical testing.

NIH - NINDS
6001 Executive Boulevard
Rockville, MD 20852
Phone: (301) 496-1779
E-mail: timothy.lyden@nih.gov
Website: https://www.ninds.nih.gov/Current-Research/Research-Funded-NINDS/Translational-Research
BOOTH NUMBER: 116

Novasep
23 Creek Circle
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Phone: (610) 494-0447
E-mail: novasep@novasep.com
Website: www.novasep.com
BOOTH NUMBER: 522
Novasep is a CDMO specialized in viruses & viral vectors production for tox, clinical and commercial phases. Novasep offers global and integrated services from process development to fill and finish, including investment in new commercial assets (addressing both drug substance and drug product manufacturing processes).

NxGEN VECTOR SOLUTIONS, LLC
455 Massachusetts Ave. N.W., Suite 369
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Phone: (202) 765-7475
E-mail: media@nxgenvectorsolutions.com
Website: www.nxgenvectorsolutions.com
BOOTH NUMBER: 332
NxGEN VECTOR SOLUTIONS, LLC, has discovered how to build gene therapy vectors that are stealthy, coming in under the body's immune radar. By reducing or eliminating the body's immune reaction, AAV vectors built using NxGEN technology can deliver their healing power without activating the body's immune system.

Orchard Therapeutics
2 Seaport Lane, 8th Floor
Boston, MA 02210
Phone: (404) 545-9910
E-mail: egrice@cambridgebmg.com
Website: https://www.orchard-tx.com/
BOOTH NUMBER: 130 & 132
Orchard Therapeutics is a leading global fully integrated commercial-stage company dedicated to transforming the lives of patients with rare diseases through innovative gene therapies.

OriGen Biomedical
7000 Burleson Rd., Bldg. D
Austin, TX 78744
Phone: (512) 474-7278
E-mail: r.patino@origenbio.com
Website: https://www.origen.com/
BOOTH NUMBER: 221
At OriGen Biomedical we manufacture a full range of cryopreservation products including bags for cell and tissue storage. We have sterile DMSO solutions available in syringe and vial configurations and OriGen has FEP cell culture bags in many standard sizes. Stop by the OriGen booth or visit OriGen.com.

Oxford BioMedica
Windrush Court, Transport Way
Oxford OX4 6LT
United Kingdom
Phone: 44 1865 783 300
E-mail: j.slingsby@oxb.com
Website: www.oxb.com
BOOTH NUMBER: 524
Using our unique LentiVector® delivery platform, we have a portfolio of gene and cell therapy product candidates in the areas of oncology, ophthalmology and CNS disorders. We have partnerships with Novartis, Bioverativ, Immune Design, Orchard Therapeutics & other companies providing access to our intellectual property & state-of-the-art production facilities.

Oxford Genetics
Medawar Centre, Robert Robinson Avenue
Oxford OX4 4HG
United Kingdom
Phone: 44 1865 415107
E-mail: award@oxgene.com
Website: www.oxfordgenetics.com

BOOTH NUMBER: 531

Oxford Genetics is a leading synthetic biology company focused on developing novel technologies to overcome the challenges associated with the discovery, development and production of biologics, gene therapies, cell therapies and vaccines.

Paragon Bioservices, Inc.
801 W Baltimore St, Suite 302
Baltimore, MD 21201
Phone: (410) 975-4050
E-mail: eearll@paragonbioservices.com
Website: www.paragonbioservices.com

BOOTH NUMBER: 416 & 418

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, Room 2018
Philadelphia, PA 19104
Phone: (215) 573-1076
E-mail: aksandhu@upenn.edu
Website: https://gtp.med.upenn.edu/core-laboratories-public/vector-core

BOOTH NUMBER: 128

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
Phone: (609) 423-0007
E-mail: hklemens@peprotech.com
Website: www.peprotech.com

BOOTH NUMBER: 310

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.

PerkinElmer
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Waltham, MA 02451
Phone: (781) 663-6900
E-mail: customercareus@perkinelmer.com
Website: http://www.perkinelmer.com/

BOOTH NUMBER: 219

PerkinElmer, Inc. offers automated solutions which improve the efficiency of genomic and proteomics workflows. With our nucleic acid isolation technology, liquid handlers, library preparation kits, automated nucleic acid and protein analysis systems, and solutions for single cell genetic analysis, PerkinElmer is eliminating the challenges associated with genomic and proteomic analysis.

Phacilitate
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United Kingdom
Phone: 4420 7384 2858
E-mail: sarah.rosenberg@phacilitate.co.uk
Website: www.phacilitate.co.uk

BOOTH NUMBER: 233

We bring people together through our award-winning events, which focus on the business and the science of advanced therapies. Phacilitate Leaders World, Phacilitate Leaders Asia and Phacilitate Leaders Europe are the pillars of our calendar and bring together the entire advanced therapies ecosystem.

PhoenixBio

65 Broadway, Suite 605
New York, NY 10006
Phone: (212) 379-6411
E-mail: rpbd2.pxbusa@phoenixbiousa.com
Website: http://phoenixbio.co.jp/en/

BOOTH NUMBER: 520

PhoenixBio - supplier of the PXB-Mouse® and PXB-cells®, provides comprehensive drug development study services including those tailored for gene therapy involving liver-specific genes. The humanized liver PXB-Mouse® features up to 95% mouse-to-human hepatocyte replacement, ensuring accurate and predictable translatability in pre-clinical development studies resulting in accelerated discovery pipelines.

PlasmidFactory GmbH & Co. KG

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E-mail: info@plasmidfactory.com
Website: www.PlasmidFactory.com

BOOTH NUMBER: 336

PlasmidFactory is a globally active biopharmaceutical company, founded in 2000. As a leading CMO for plasmid and minicircle DNA, it has a strong customer base in the fields of gene and cell therapy and vaccination. PlasmidFactory produces plasmids and minicircles according to customer's requirements in modern laboratories with high quality standards.

Polyplus-transfection

850 Boulevard sebastien-Brant, Bioparc
Illkirch Graffenstaden 67400
France
Phone: 33390406180
E-mail: info@polyplus-transfection.com
Website: www.polyplus-transfection.com

BOOTH NUMBER: 312

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: pgtc.ufl.edu

BOOTH NUMBER: 210

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.

PROGEN Biotechnik GmbH

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Phone: 49622182780
E-mail: info@progen.com
Website: www.progen.com

BOOTH NUMBER: 333

PROGEN is an established manufacturer & supplier of AAV Titration ELISA tests and antibodies for gene therapy research and development. In the past 20 years, the DIN EN ISO 13485 certified company has established a unique portfolio of AAV tools for basic and clinical research as well as pharmaceutical applications.

Puresyn

87 Great Valley Parkway
Malvern, PA 19355
Phone: (610) 640-0800
E-mail: leslie@puresyn.com
Website: www.puresyn.com

BOOTH NUMBER: 303

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 o TransfectionReady o ResearchReady o INDReady™

RegMedNet
Unitec House, 2 Albert Place, n3 1qb
London N3 1QB
United Kingdom
Phone: 7925720314
E-mail: d.courtney@future-science-group.com
Website: https://www.regmednet.com
BOOTH NUMBER: 122

RegMedNet is a community site that unites the diverse regenerative medicine community. Through partnership with our sister journal, Regenerative Medicine, we seek to educate and inspire to help this exciting field move forward at an even faster rate.

Retrogenix Limited
Crown House, Bingswood Estate,
Whaley Bridge, High Peak SK23 7LY
United Kingdom
Phone: 44 (0)7788 716027
E-mail: alex.kelly@retrogenix.com
Website: www.retrogenix.com
BOOTH NUMBER: 529

Retrogenix provides a fast and accurate solution for primary receptor identification and specificity (off-target) screening of antibodies, proteins and cell therapies (including CAR T), amongst other ligands. Widely used by major pharmaceutical companies, the technology identifies physiologically-relevant interactions through screening against thousands of plasma membrane proteins expressed in human cells.

RoosterBio, Inc.
5295 Westview Drive, Suite 275
Frederick, MD 21703
Phone: (301) 200-5366
E-mail: tomc@roosterbio.com
Website: www.roosterbio.com
BOOTH NUMBER: 431

RoosterBio is a privately-held Maryland-based company focused on manufacturing and supplying MSC systems to accelerate the regenerative medicine industry. The company's products are high-volume, efficient and well-characterized adult hMSCs paired with bioprocess media - built for rapid manufacturing scale-up. This approach revolutionizes how cell therapies are developed, clinically-translated, and commercialized.

Sangamo Therapeutics, Inc.
501 Canal Blvd.
Richmond, CA 94804
Phone: (510) 970-6000
E-mail: info@sangamo.com
Website: www.sangamo.com
BOOTH NUMBER: 314

Sangamo Therapeutics is focused on translating ground-breaking science into genomic therapies that transform patients' lives using our platform technologies in genome editing, gene therapy, gene regulation and cell therapy. Our pipeline includes Phase 1/2 clinical trials for Hemophilia A and Hemophilia B, MPS I and MPS II, and beta thalassemia.

Sarepta Therapeutics
215 First Street
Cambridge, MA 02142
Phone: (617) 274-4000
E-mail: info@sarepta.com
Website: https://www.sarepta.com/
BOOTH NUMBER: 320 & 322

Sarepta is at the forefront of precision genetic medicine, having built an impressive and competitive position in Duchenne muscular dystrophy (DMD) and more recently in Limb-girdle muscular dystrophy (LGMD), Charcot-Marie-Tooth (CMT) and CNS-related disorders, totaling over 20 therapies in various stages of development. Sarepta is proud to support the 22nd Annual Meeting of the ASGCT.

SCIEX
1201 Radio Road
Redwood City, CA 94065
Phone: (877) 740-2129
E-mail: customercare@sciex.com
Website: www.sciex.com
BOOTH NUMBER: 430

We pioneer extraordinary solutions. But we don't just develop products. It's what we do together that sets us apart. With our customers and partners, we bring the power of life-changing answers to the questions you have today, and those that you have yet to ask.

SGS Vitrology Limited

Clydebank Business Park
Glasgow G812LG
United Kingdom
Phone: 1419520022
E-mail: biosafety@sgs.com
Website: www.sgs.com/biosafety

BOOTH NUMBER: 212

SGS are a testing partner for purity, stability, and identity testing of your cell and gene therapy products. Part of the world's largest testing company with a 40-year history in life science. We add value to your business through scientific expertise, reliability, and pro-active communication with a refreshing customer focus.

SIRION Biotech GmbH

Am Klopferspitz 19, SIRION Biotech
Martinsried/Planegg 82152
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Phone: 49 89 700 961 9918
E-mail: christel@sirion-biotech.com
Website: www.sirion-biotech.com

BOOTH NUMBER: 334

SIRION Biotech provides transduction technologies to improve clinical gene therapy and immune-oncology trials, offers custom engineering and manufacturing of viral vectors (AAV, Lentivirus, Adenovirus) and performs R&D collaborations to develop new AAV serotypes for clinical use. Clinical applications of SIRION technology licenses have grown from two in 2017 to currently five, and growing.

STEMCELL Technologies

1618 Station Street
Vancouver, British Columbia V6A 1B6
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Phone: (604) 675-7877
E-mail: conferences@stemcell.com
Website: https://www.stemcell.com

BOOTH NUMBER: 214

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.

STEMSOFT Software

401-570 West 7th Avenue
Vancouver, British Columbia V5Z 1B3
Canada
Phone: (800) 671-3234
E-mail: info@stemsoft.com
Website: http://www.stemsoft.com/

BOOTH NUMBER: 215

STEMSOFT, the leading cellular therapy informatics provider, offers a configurable, commercial-off-the-shelf electronic batch process record that supports barcoding, instrument integration and in-process calculation. Visit STEMSOFT to learn how we can save you time, maximize quality and keep you in compliance. STEMSOFT Software Track | Share | Grow www.stemsoft.com

Synbio Technologies

1 Deer Park Drive, Suite L-1
Monmouth Junction, NJ 08852
Phone: (732) 230-3003
E-mail: shumei.chen@synbio-tech.com
Website: https://www.synbio-tech.com/

BOOTH NUMBER: 225

Synbio Technologies is founded by professionals with extensive experience in scientific research and industrial management. Through our distinctive Syno® platforms, we can satisfy customers' needs, including construction of a humanized antibody library, optimization of industrial enzymes, chromosome/genome synthesis, development of genetic engineering vaccines and DNA informatics storage technology.

Terumo BCT

10811 W Collins Ave
Lakewood, CO 80215
Phone: (303) 231-4357
E-mail: celltherapy.orders@terumobct.com
Website: https://www.terumobct.com/cell-therapy-technologies

BOOTH NUMBER: 534 & 536

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

5823 Newton Drive
Carlsbad, CA 92008
Phone: (800) 678-5599
E-mail: bp@thermofisher.com
Website: thermofisher.com/celltherapy

BOOTH NUMBER: 121

Thermo Fisher Scientific provides the quality products, services, and support you need to translate your gene and cell therapy from drug discovery through large-scale commercial production. We're working alongside the scientific community to accelerate the pace of gene and cell therapy development and enabling scientists like you to transform our approach to health care.

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: 435

TriLink BioTechnologies specializes in the synthesis and production of complex and highly-modified nucleic acids for research, diagnostics, pre-clinical therapeutic and pharmaceutical applications. Since 1996, TriLink has been developing and manufacturing custom oligonucleotides, mRNA transcripts, nucleotides, PCR & RT-PCR reagents, NGS library preparation kits, bioconjugation, custom chemistry, and other small molecules.

Vaccine Stabilization Institute

820 Calle Plano
Camarillo, CA 93012
Phone: (805) 445-8422
E-mail: kijeong.lee@integritybio.com
Website: www.integritybio.com

BOOTH NUMBER: 421

Vaccine Stabilization Institute (VSI) is focusing on stabilization of viruses and vaccines. The company has developed a technology that is stabilizing an enveloped virus retaining 90% of infectivity after lyophilization. The company can also provide virus/viral vector's formulation and stability testing with additional analytical technologies.

VGXI, Inc.

2700 Research Forest Drive, Suite 180
The Woodlands, TX 77381
Phone: (281) 296-7300
E-mail: cfranco@vgxii.com
Website: www.vgxii.com

BOOTH NUMBER: 411

VGXI is a leading CMO for DNA pharmaceuticals with over 15 years' experience providing cGMP products to clinical trials worldwide. The company's production services include high purity plasmid DNA for pre-clinical research, Highly Documented (HD) plasmid for GMP virus production and toxicology studies, and cGMP DNA for clinical through commercial supply.

Vigene Biosciences

9430 Key West Ave., Suite 105
Rockville, MD 20850
Phone: (301) 251-6638
E-mail: orders@vigenebio.com
Website: https://www.vigenebio.com/

BOOTH NUMBER: 535

We strive to be the leading supplier of viral vector-based gene delivery technologies to advance biomedical research and to improve human health.

Virovek Incorporation

22429 Hesperian Blvd.
Hayward, CA 94541
Phone: (510) 887-7121
E-mail: info@virovek.com
Website: www.virovek.com

BOOTH NUMBER: 224

Virovek's mission is to design, produce, and purify the highest quality AAV vectors to support basic research and clinical-stage drug development. Since 2006, Virovek has produced over 1,300 AAV vectors of at least 1E+13vg scale each to support research projects in over 100 university labs, non-profit institutions, and companies worldwide.

VIVEbiotech

Paseo Mikeletegi 81, Parque Científico y Tecnológico de Gipuzkoa
San Sebastián 20009
Spain
Phone: 34943308568
E-mail: ntejados@vivebiotech.com
Website: www.vivebiotech.com

BOOTH NUMBER: 114

Fully specialized in lentiviral vectors. Expertise: 1. GMP CDMO: manufacture from early stages to GMP. Working with European and US-based companies. 2. Innovation. Development of: a. Cost-effective processes. b. Enhanced safety profile of viral vectors. Improved producer cell line development, new pseudo-typing strategies and own worldwide licensed technology: a non-integrative episomal stable lentiviral vector (LENTISOMA).

Waisman Biomanufacturing

1500 Highland Ave.
Madison, WI 53705
Phone: (608) 262-9547
E-mail: waisbio@gmail.com
Website: https://gmpbio.org/

BOOTH NUMBER: 307

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 Corporation

33 5th Ave. NW, Suite 800
New Brighton, MN 55112
Phone: (651) 628-9259
E-mail: josh.ludwig@wilsonwolf.com
Website: www.wilsonwolf.com

BOOTH NUMBER: 436

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.

WORLDSymposium

6001 Broken Sound Pkwy, Suite 340
Boca Raton, FL 33487
Phone: (561) 609-0438
E-mail: eli@gmimeetings.com
Website: www.WORLDSymposia.org

BOOTH NUMBER: 217

WORLDSymposium is an annual research conference dedicated to lysosomal diseases. W.O.R.L.D. is an acronym that stands for "We're Organizing Research on Lysosomal Diseases". Since the inception in 2002, WORLDSymposium has grown to an international scientific meeting attracting 1800+ researchers and clinicians from over 50 different countries. WORLDSymposium 2020 will be the 16th Annual Meeting.

Yecuris

15055 SW Sequoia Pkwy, #130
Tigard, OR 97224
Phone: (503) 352-4663
E-mail: johnbial@yecuris.com
Website: www.yecuris.com

BOOTH NUMBER: 228

The Yecuris FRG KO liver humanized platform uses primary human hepatocytes along with the existing mouse tissue scaffold to reconstitute the native liver architecture into a functional chimeric organ.

Our in vitro & in vivo products offer a complete system to drive your liver-targeted Gene Editing & Therapy program.

Yposkesi

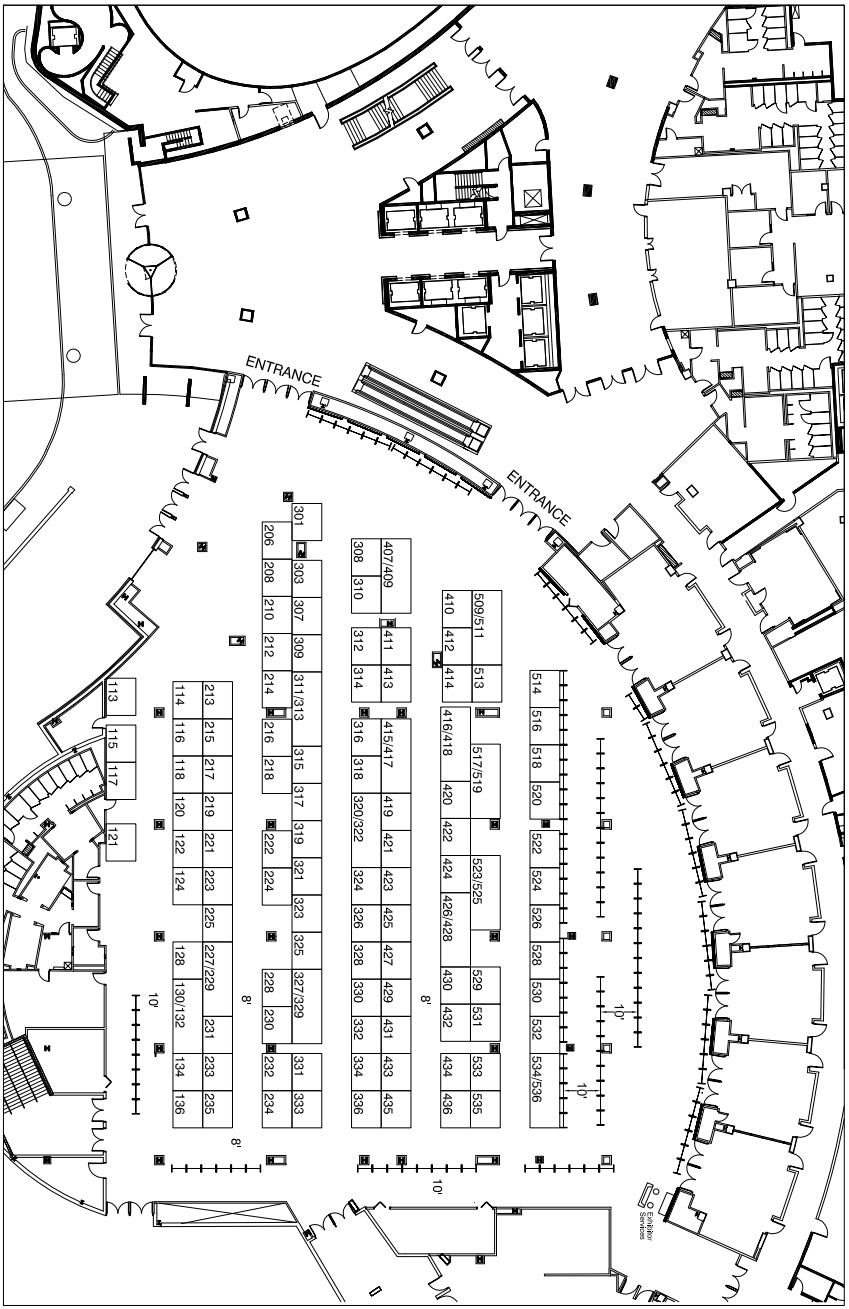
26 rue Henri Auguste Desbrueres
Corbeil Essonnes 91100
France
Phone: 33 760 113 938
E-mail: pcollet@yposkesi.com
Website: www.yposkesi.com

BOOTH NUMBER: 526

Yposkesi is one of the largest CDMO in Europe for viral vector production. Its current 50,000 sq ft facility houses four independent manufacturing suites for drug substance and two Fill&Finish suites. By 2021, it is doubling this footprint to 100,000 sq ft for clinical and future commercial clients' needs.

EXHIBIT HALL FLOOR PLAN

ASGCT 22ND ANNUAL MEETING
April 29 - May 2, 2019 • Washington Hilton - Columbia Hall



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SAREPTA THERAPEUTICS

Invites you to join our symposium taking place
during the 22nd ASGCT Annual Meeting

CONSIDERATIONS FOR NEUROMUSCULAR AND CNS-TARGETED

GENE THERAPIES

WEDNESDAY	TIME	CONCOURSE LEVEL
MAY 1, 2019	**11:45 AM** to **1:15 PM**	**JEFFERSON**

Washington Hilton 1919 Connecticut Ave NW Washington, DC 20009

NEUROMUSCULAR TARGETED THERAPIES

LOUISE RODINO-KLAPAC, PhD
Sarepta Therapeutics
COLUMBUS, OH

CNS-TARGETED THERAPIES

BARRY BYRNE, MD, PhD
University of Florida, College of Medicine
GAINESVILLE, FL

RONALD G. CRYSTAL, MD
Weill Cornell Medical College
NEW YORK, NY

VISIT US AT BOOTHS # 320 & 322

SAVE THE DATE

AMERICAN SOCIETY OF GENE & CELL THERAPY

23RD ANNUAL MEETING

MAY 12-15, 2020

ASGCT
23rd ANNUAL MEETING
BOSTON
MAY 12-15 ▪ 2020

AMERICAN SOCIETY of GENE & CELL THERAPY