

Cellicon Valley '21: Future of Cell and Gene Therapies
 2 Day Live Virtual Educational Symposium - Thursday, May 6 and Friday, May 7, 2021
PennMedicine.org/cellicon21

THURSDAY, MAY 6, 2021

8:00 am EDT Log-in to Virtual Conference - Visit Virtual Exhibit Hall and Abstracts

9:00 am EDT CONCURRENT SESSIONS (Pre- Recorded) BEGIN

Concurrent Session Track 1 NURSING CNE-ACCREDITED*	Concurrent Session Track 2 PEDIATRIC CARE	Concurrent Session Track 3 PRE- CLINICAL/MANUFA CTURING	Concurrent Session Track 4 NEXT-GEN CLINICAL RESEARCH	Concurrent Session Track 5 REGULATORY AND COMMERCIAL DEVELOPMENT	Concurrent Session Track 6 SOLID TUMOR CARs	Concurrent Session Track 7 FINANCIAL IMPLICATIONS	Concurrent Session Track 8 FROM LOCAL TO GLOBAL: DEVELOPMENT OF ENGINEERED CELL AND GENE THERAPIES
<p><u>Joint Adult and Pediatric Sessions- Nursing and Patient Care</u></p> <p>State of the Union in Nursing in the era of Cell and Gene Therapies Welcome Regina Cunningham, RN, PhD Penn Medicine</p> <p>Ellen Tracy, RN, MSN, NEA-BC Children's Hospital of Philadelphia</p> <p>KEYNOTE SESSION The Use of CRISPR Technology in the Treatment of Malignant and Non-Malignant Diseases</p> <p>Making CAR T Cells "CRISPeR" Edward A. Stadtmauer, MD Penn Medicine Adult Q&A Moderator:</p>	<p><u>Future of Gene Therapy in Pediatrics</u> Moderator: Timothy S Olson, MD, PhD Children's Hospital of Philadelphia</p> <p>Clinical Overview/Impact Timothy S Olson, MD, PhD Children's Hospital of Philadelphia</p> <p>Gene Therapy Techniques Matthew Porteus, MD, PhD Stanford University</p> <p><u>Implication of Gene Therapy Treatment in Pediatrics</u></p>	<p><u>Target Discovery and Immunogenicity</u> Moderator: Robert H. Vonderheide, MD, DPhil Abramson Cancer Center</p> <p>Antibody Discovery for New CAR Constructs Donald L. Siegel, PhD, MD University of Pennsylvania</p> <p>Immune Responsiveness or Immune Ignorance? Lisa Butterfield, PhD Parker Institute for Cancer Immunotherapy</p> <p>Immune Health Robert H. Vonderheide, MD, DPhil Abramson Cancer Center</p> <p><u>Viral Vector Free Delivery</u></p>	<p><u>Newest Model CARs</u> Moderator: Joseph A. Fraietta, PhD University of Pennsylvania</p> <p>Engineering Light-Controllable CAR T Cells for Cancer Immunotherapy Yingxiao Wang, PhD University of California San Diego</p> <p>T cell Antigen Couplers Jonathan Bramson, PhD McMaster University, Canada</p> <p>in vivo CAR's Christian Buchholz, PhD Paul-Ehrlich-Institut, Germany</p> <p><u>CAR's on the Unpaved Road</u></p>	<p><u>Managing Toxicities</u> Moderator: Noelle Frey, MD, MS Penn Medicine</p> <p>Consistency in Grading and Reporting Stephan A Grupp, MD, PhD Children's Hospital of Philadelphia</p> <p>Pathophysiology of Neurotoxicity Saad J Kenderian, MB, ChB Mayo Clinic</p> <p>Title: CAR T-Cells: Subacute and Late Toxicities beyond CRS Nirali N Shah, MD, MHSc National Institute of Health</p> <p><u>Gene Therapy IND's and Regulatory Considerations Parent/Child, Master</u></p>	<p><u>Solid Tumor CARs - ONE</u> Moderator: Partow Kebriaei, MD MD Anderson</p> <p>CAR T for Solid Tumors: Unique considerations Partow Kebriaei, MD MD Anderson</p> <p>GBM Trials Donald O'Rourke, MD Penn Medicine</p> <p>Prostate Cancer Trials Naomi B. Haas, MD Penn Medicine</p> <p><u>Solid Tumor CARs – "TOO"</u> Moderator: Mark H O'Hara, MD Penn Medicine</p> <p>Muc1 Trials</p>	<p><u>Financial Implications</u> Moderator: Kevin B. Mahoney, DBA Penn Medicine</p> <p>Pricing Tolerance and the Evolution of Cell Therapy Costs of Care Richard T Maziarz, MD Oregon Health & Science University</p> <p>Cancer Moonshot: How do we pay for the fuel? Kevin B. Mahoney, DBA Penn Medicine</p> <p>TBA Ezekiel J Emanuel, MD, PhD University of Pennsylvania</p>	TBA

<p>Patricia Mangan, MSN, CRNP Penn Medicine</p> <p>Nursing Considerations/Implications for Patients with SCD in the Era of Gene Tx Claire M. White, MSN, RN Children's Hospital of Philadelphia</p> <p>Pediatric Q&A Moderator: Elizabeth A Worster, MSN, CRNP Children's Hospital of Philadelphia</p> <p>Pediatric Q&A Panel: Catherine Hamilton, RN, BSN Children's Hospital of Philadelphia Denise Gallagher, MS Children's Hospital of Philadelphia</p> <p><u>PEDIATRIC NURSING SESSION</u> Moderator: Laura Smith, RN, MSN, CRNP Children's Hospital of Philadelphia</p> <p>Immunotherapy in Pediatrics</p> <p>AML/CART 33: Nursing Considerations during COVID Pandemic Diane Baniewicz, RN, MSN, CRNP Children's Hospital of Philadelphia</p> <p>Nursing Considerations for U CART in Pediatrics Colleen Callahan, RN, MSN, CRNP Children's Hospital of Philadelphia</p> <p><u>ADULT NURSING SESSION</u> Moderator: Patricia Mangan, MSN, CRNP Penn Medicine</p> <p>Preparing for an Autologous Stem Cell Transplant Kelly Salam, RN, BSN Penn Medicine Keri McDevitt, RN, MSN Penn Medicine</p>	<p>Moderator: Timothy S Olson, MD, PhD Children's Hospital of Philadelphia</p> <p>Hemoglobinopathies Janet L Kwiatkowski, MD, MSCE Children's Hospital of Philadelphia</p> <p>Immune deficiencies Sung-Yun Pai, MD National Institute of Health</p> <p>Leukodystrophies Amy Waldman, MD Children's Hospital of Philadelphia</p>	<p>Moderator: Bruce L Levine, PhD Penn Medicine</p> <p>Overview/Survey of Tech Bruce L Levine, PhD Penn Medicine</p> <p>Electro-mechanical Transfection Cullen R Buie, PhD Massachusetts Institute of Technology</p> <p>Transient Cell Volume Exchange Todd Sulchek, PhD Georgia Institute of Technology</p> <p>Intracellular Delivery by Soluporation Michael Maguire, PhD, CEO Avectas</p> <p><u>Gene Delivery and Editing for Better CARs</u> Moderator: Fyodor Urnov, PhD University of California at Berkley</p> <p>CRISPR and Beyond Alexander Marson, MD, PhD Gladstone-UCSF Institute of Genomic Immunology</p> <p>Piggybac in Trials Eric Ostertag, MD, PhD Poseida</p> <p>Sleeping Beauty in Trials Laurence Cooper, MD, PhD Ziopharm Oncology</p>	<p>Moderator: James L Riley, PhD University of Pennsylvania</p> <p>CAR's in Fibrosis Jonathan A Epstein, MD University of Pennsylvania</p> <p>CAAR's PV by Cabaletta Michael C Milone, MD, PhD University of Pennsylvania</p> <p>CAR's in HIV James L Riley, PhD University of Pennsylvania</p> <p><u>Allogeneic and Donor Derived CAR T cells, NKs, iPSC's</u> Moderator: Melody Smith, MD, MS Memorial Sloan Kettering Cancer Center</p> <p>Allo CARs intro Melody Smith, MD, MS Memorial Sloan Kettering Cancer Center</p> <p>Allogene David Chang MD, PhD Allogene Therapeutics</p> <p>Fate Therapeutics Bob Valamehr, PhD, MBA Fate Therapeutics</p> <p>Donor-Derived Gene-Edited Allogeneic CAR-T Samarth Kulkarni, PhD CRISPR Therapeutics</p> <p><u>CAR-T Vaccine Booster</u> Moderator: Michael C Milone, MD, PhD University of Pennsylvania</p> <p>RNA Vaccine Boosting CAR T Cells Katharina Reinhard, PhD Immunoreceptor Therapy</p> <p>CD19 CAR T Booster Vaccine Colleen Elizabeth Annesley MD Seattle Children's</p> <p>CMV Vaccine Stephen J Forman, MD City of Hope</p>	<p><u>Protocol/Basket Trial Design Considerations</u> Moderator: Anne Chew, PhD Penn Medicine</p> <p>TBA Wilson W Bryan, MD U.S. Food and Drug Administration</p> <p>TBA Ke Liu, MD, PhD Sana Biotechnology</p> <p>Regulatory Considerations to Accelerate Gene Therapy Development Jiwen Zhang, PhD PassageBio</p> <p>Parent-Child Development Strategy: A novel clinical and regulatory framework to evaluate multiple generations of NY-ESO1 targetet cell Therapies in Rapid Sequence Aiman Shalabi PharmD, MBA GlaxoSmithKline</p> <p><u>Updates on Approved CAR T Cell Therapies</u> Moderator: David L Porter, MD Penn Medicine</p> <p>Mechanisms of Treatment Resistance and Toxicity Related Markers in Context of Axicabtagene Ciloleucel for Lymphoma Adrian Bot, MD, PhD Kite</p> <p>International Supply Chain Expansion and Approvals during COVID Amir Hefni, PhD Novartis</p> <p>Define CD4 CDA Doses in CAR T Cell Therapy TBA Bristol Myers Squibb</p>	<p>Mark H O'Hara, MD Penn Medicine</p> <p>MESO/Pancreatic Janos L Tanyi, MD, PhD Penn Medicine</p> <p><u>What's "Neo" in Solid Tumors</u> Moderator: Gerald P Linette, MD, PhD Penn Medicine</p> <p>TBA CRUK (Cancer Research UK)</p> <p>Immunotherapy for Clonal Driver Neoantigens Gerald P Linette, MD, PhD Penn Medicine</p>		
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<p>Preparing for an Allogeneic Transplant Rachel Golding, RN, BSN Penn Medicine Lila Colleluori, BSN, RN, OCN Penn Medicine</p> <p>COVID Management of Liquid Oncology Patients Abbey Walsh, MSN, RN, OCN Penn Medicine Angela Rubin, BSN, RN, OCN Penn Medicine</p> <p>Updates on Immunotherapies in Myeloma Patricia Mangan, MSN, CRNP Penn Medicine</p> <p>Late Effects and Survivorship Care Following Donor Stem Cell Transplant: What to Expect After the 1st Year? Linda M. Perry, MS PA-C Penn Medicine</p>							
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THURSDAY, MAY 6, 2021

- 1:00 pm EDT** **Welcome**
Amy Gutmann, PhD | University of Pennsylvania
- 1:15 pm EDT** **KEYNOTE ADDRESS**
Engineering Smarter T Cells
Stanley Riddell, MD | Fred Hutchinson Cancer Research Center
- 2:15 pm EDT** **Cell and Gene Startups and Innovation in Cellicon Valley**
Moderator: John S Swartley, PhD | University of Pennsylvania
- Aimee Payne, MD, PhD | Founder, **Cabaletta**
- Debora Barton, MD | CMO, **Carisma**
- Usman “Oz” Azam, MD | CEO, **Tmunity**
- Jeff Marazzo | CEO, **Spark**
- Audrey Greenberg, MBA, CPA | Executive Director, **Discovery Labs**

<p style="text-align: center;">Thursday May 6 - 3:00 pm EDT Live Q&A FOR CONCURRENT SESSIONS Featuring Presenters and Moderators from Pre-recorded concurrent sessions</p>							
<p>Concurrent Session Track 1 NURSING</p> <p>CNE-ACCREDITED</p>	<p>Concurrent Session Track 2 PEDIATRIC CARE</p>	<p>Concurrent Session Track 3 PRE-CLINICAL/MANUFACTURING</p>	<p>Concurrent Session Track 4 NEXT-GEN CLINICAL RESEARCH</p>	<p>Concurrent Session Track 5 REGULATORY AND COMMERCIAL DEVELOPMENT</p>	<p>Concurrent Session Track 6 SOLID TUMOR CARs</p>	<p>Concurrent Session Track 7 FINANCIAL IMPLICATIONS</p>	<p>Concurrent Session Track 8 FROM LOCAL TO GLOBAL: DEVELOPMENT OF ENGINEERED CELL AND GENE THERAPIES</p>
<p>3:00 pm EDT Q&A Joint ADULT AND PEDIATRIC SESSIONS- NURSING AND PATIENT CARE</p> <p>The Use of CRISPR Technology in the Treatment of Malignant and Non-Malignant Diseases:</p> <p>3:00 pm EDT Q&A Making CAR T Cells “CRISPeR”</p> <p>3:15 pm EDT Q&A Nursing Considerations/ Implications for Patients with SCD in the Era of Gene Tx with panel</p> <p>3:30 pm EDT Q&A PEDIATRIC NURSING SESSION</p> <p>Q&A Immunotherapy in Pediatrics</p> <p>AML/CART 33: Nursing Considerations during COVID Pandemic</p>	<p>3:00 pm EDT Q&A Future of Gene Therapy in Pediatrics</p> <p>Clinical Overview/Impact</p> <p>Gene Therapy Techniques</p> <p>3:15 pm EDT Q&A Implication of Gene Therapy Treatment in Pediatrics</p> <p>Hemoglobinopathies</p> <p>Immune deficiencies</p> <p>Leukodystrophies</p>	<p>3:00 pm EDT Q&A Target Discovery and Immunogenicity</p> <p>Antibody Discovery for New CAR Constructs</p> <p>Immune Responsiveness or Immune Ignorance?</p> <p>Immune Health</p> <p>3:15 pm EDT Q&A Viral Vector Free Delivery</p> <p>Overview/Survey of Tech</p> <p>Electro-mechanical Transfection</p> <p>Transient Cell Volume Exchange</p> <p>Intracellular Delivery by Soluporation</p>	<p>3:00 pm EDT Q&A Newest Model CARs</p> <p>Engineering Light-Controllable CAR T Cells for Cancer Immunotherapy</p> <p>T cell Antigen Couplers</p> <p>in vivo CAR's</p> <p>3:15 pm EDT Q&A CAR's on the Unpaved Road</p> <p>CAR's in Fibrosis</p> <p>CAAR's PV by Cabaletta</p> <p>CAR's in HIV</p> <p>3:30 pm EDT Q&A Allogeneic CAR-Ts, NKs, iPSC's</p> <p>Allo CARs intro</p> <p>Allogene</p>	<p>3:00 pm EDT Q&A Managing Toxicities</p> <p>Consistency in Grading and Reporting</p> <p>Pathophysiology of Neurotoxicity</p> <p>Long Term Toxicities, Hypogammaglobulinemia, Cytopenia, Infection Risk</p> <p>3:15 pm EDT Q&A Gene Therapy IND's and Regulatory Considerations</p> <p>Parent/Child, Master Protocol/Basket Trial Design Considerations</p> <p>Regulatory Considerations to Accelerate Gene Therapy Development</p> <p>3:30 pm EDT Q&A Updates on Approved CAR T Cell Therapies</p>	<p>3:00 pm EDT Q&A Solid Tumor CARs ONE</p> <p>CAR T for Solid Tumors: Unique considerations</p> <p>GBM Trials</p> <p>Prostate Cancer Trials</p> <p>3:15 pm EDT Q&A Solid Tumor CARs TOO</p> <p>Muc1 Trials</p> <p>MESO/Pancreatic</p> <p>3:30 pm EDT Q&A What's “Neo” in Solid Tumors</p> <p>Immunotherapy for Clonal Driver Neoantigens</p>	<p>3:00 pm EDT Q&A</p>	<p>3:00 pm EDT Q&A</p>

<p>Nursing Considerations for U CART in Pediatrics</p> <p>3:30 pm EDT Q&A ADULT NURSING SESSION</p> <p>Preparing for an Autologous Stem Cell Transplant</p> <p>Preparing for an Allogenic Transplant</p> <p>Impact of Telemedicine</p> <p>Updates on Immunotherapies in Myeloma</p> <p>Late Effects and Survivorship Care Following Donor Stem Cell Transplant: What to Expect After the 1st Year?</p>		<p>3:30 pm EDT Q&A Gene Delivery and Editing for Better CARs</p> <p>CRISPR and beyond</p> <p>Piggybac in trials</p> <p>Sleeping beauty in trials</p>	<p>Fate Therapeutics</p> <p>Donor-Derived Gene-Edited Allogeneic CAR-T</p> <p>3:45 pm EDT Q&A CAR-T Vaccine Booster</p> <p>RNA Vaccine Boosting CAR T Cells</p> <p>CD19 CAR T Booster Vaccine</p> <p>CMV Vaccine</p>	<p>Mechanisms of Treatment Resistance and Toxicity Related Markers in Context of Axicabtagene Ciloleucel for Lymphoma</p> <p>International Supply Chain Expansion and Approvals during COVID</p> <p>Define CD4 CDA Doses in CAR T Cell Therapy</p>			
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FRIDAY, May 7, 2021

PLENARY SESSIONS // VISIT EXHIBITS // MEET THE SPEAKERS // NETWORKING // VIEW ABSTRACTS

- 7:30 am EDT** **Log-in to Virtual Conference - Visit Virtual Exhibit Hall and Abstracts**
- 9:00 am EDT** **Welcome and Introductions**
 J Larry Jameson, MD, PhD | University of Pennsylvania
 Madeline Bell | Children's Hospital of Philadelphia
 Robert H. Vonderheide, MD, DPhil | Abramson Cancer Center
- 9:15 am EDT** **PLENARY SESSION 1: CARS of Today and Tomorrow**
 Moderator: Richard T Maziarz, MD | Oregon Health & Science University
- Lymphoma** David Maloney, MD, PhD | Fred Hutchinson Cancer
- Myeloma** Alfred L Garfall, MD, MTR | Penn Medicine
- ALL** Terry J. Fry, MD | Children's Hospital of Colorado

Q&A Discussion

10:15 am EDT

PLENARY SESSION 2: Overcoming Resistance to Monotherapy CAR's

Moderator: Shannon L Maude, MD, PhD | Children's Hospital of Philadelphia

CHOP/Penn 19+22 Trials

Noelle V Frey, MD | Penn Medicine

AUTO3 and Beyond - Experience with Co-Targeting of CD19 and CD22 in DLBCL and B-ALL

Martin Pule, MB BCh, MRCP | University College London

Bispecific CAR 19-22 and Sequential CAR22 Rescue- the Experience of a few Prodigies

David Miklos, MD, PhD | Stanford University

Q&A Discussion

11:15 am EDT

PLENARY SESSION 3: Cell Therapy during the Pandemic

Moderator: David L Porter, MD | Penn Medicine

Clinical Experience CHOP

Shannon L Maude, MD, PhD | Children's Hospital of Philadelphia

CAR T in NYC During the Height of the Disaster

Gunjan L. Shah, MD | Memorial Sloan Kettering Cancer Center

Challenges Providing Donor Hematopoietic Products Globally During COVID-19

Steve Devine, MD | National Marrow Donor Program/Be The Match

Development of SARS-CoV2 T Cell Products for Administration After BMT

Catherine Bollard, MD, MBChB | Children's National Hospital

Q&A and Discussion

12:30 pm EDT

Network / Visit Exhibits / View Abstracts / Catch up on Pre-Recorded Concurrent Sessions

3:00 pm EDT **PLENARY SESSION 4: Globalization of CAR T Cell Therapies**

India, Costa Rica and Brazil

Moderator: Bruce L Levine, PhD | Penn Medicine

Immuneel

Siddhartha Mukherjee, MD, DPhil | Vor Biopharma

General Director of National Children's Hospital, Costa Rica

Román Macaya Hayes | Costa Rican Social Security System

Center for Cell Based Therapy, Brazil

Renato Luiz Guerino Cunha | Hospital das Clínicas de Ribeirão Preto, Brazil

4:00 pm EDT **Future of Cell and Gene Therapy**

Carl H June, MD | Penn Medicine

4:30 pm EDT **Adjourn**

Not Final

Overview

As home to the first FDA-approved cell and gene therapies Penn and Children's Hospital of Philadelphia are international leaders who have helped propel Philadelphia into Cellicon Valley, with Penn ranking first among global universities for CAR-T cell patents according to *Nature*. CAR T represents a turning point in the history of human medicine, a genuine revolution in our approach to disease within the field of cellular therapy and transplant. The FDA approvals of chimeric antigen receptor (CAR) T cells offers even patients with highly chemo-refractory hematologic malignancies additional treatment options. World experts in cell and gene therapy and have been assembled to discuss the development and implementation of these therapies and to weigh in on current applications, best practices, novel strategies and future developments in this field. Activity format will provide significant opportunity for interaction with expert faculty and other attendees with the aim of implementing new standards of patient care and understand new strategies to optimize and improve gene and cell therapies. Participants will leave with up-to-date, practical information which will have immediate clinical application, and an understanding of critical research initiatives that are rapidly driving this field forward, as well as the technical and scientific background on the current state, and where the latest scientific advances may take the field.

Who should attend

These activities are intended for both adult and pediatric hematologist-oncologists, medical oncologists, hematologists, hematopathologists, immunologists, immunobiologists, transfusion medicine, researchers, pharmacists, fellows, nurses, nurse practitioners, physician assistants, industry professionals and other healthcare professionals interested in the latest advances in cell and gene therapy. Additionally, patients and their caregivers, family members, advocates and members of the public who may benefit from understanding current innovative approaches to gene and cell therapy are also invited.

Learning Objectives

Upon completion of this course, participants should be able to:

- Identify current application of CAR T-cell therapy including: disease indication, accurate patient selection, manufacture and treatment administration
- List approaches to overcome resistance to monotherapy CAR's
- Implement guidelines for patient management, and identify causes of toxicities of CAR T-cell therapies including cytokine release syndrome and neurologic toxicity.
- Describe gene delivery and editing for CARs
- Identify the latest targets and technologies for CARs
- Identify the current status of globalization of CAR T-cell therapies and identify principles in ethics of patient access
- Describe current approaches in treatment and supportive care for hematopoietic stem cell transplantation (HSCT) in pediatric patients with nonmalignant conditions
- Identify the latest evidence based standards in nursing care resulting from the recent advances in cell/gene therapy and transplant

*Accreditation

Penn Medicine is approved as a provider of nursing continuing professional development by the Pennsylvania State Nurses Association, an accredited approver by the American Nurses Credentialing Center's Commission on Accreditation.