



CELLICON VALLEY '25

THE FUTURE OF CELL AND GENE THERAPIES EDUCATIONAL SYMPOSIUM

THURSDAY 04/30

FRIDAY 05/01

PennMedicine.org/Cellicon25

CELLICON VALLEY '25

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CELLICON VALLEY '25

THE FUTURE OF CELL AND GENE THERAPIES **EDUCATIONAL SYMPOSIUM**

Presented by Penn's Abramson Cancer Center and Children's Hospital of Philadelphia

OVERVIEW

Penn Medicine and Children's Hospital of Philadelphia remain at the forefront of cell and gene therapy, solidifying Philadelphia's status as "Cellicon Valley." The University of Pennsylvania continues to lead in CAR T-cell research, reflecting its ongoing commitment to innovation in this field, particularly through the pioneering work of the Center for Cellular Immunotherapies.

While CAR T-cell therapy has already revolutionized cancer treatment, the field continues to evolve rapidly. This activity provides a unique opportunity to interact with world-renowned experts in CAR T-cell therapy for cancer, hematologic disorders and autoimmune diseases.

Clinicians will discover cutting-edge strategies to optimize patient care and overcome challenges in the field. Researchers will delve into the scientific and technical breakthroughs shaping the future of this revolutionary treatment. Business leaders will gain a comprehensive understanding of the CAR T-cell therapy landscape with a focus on commercial partnering, product development and entrepreneurship.

TARGET AUDIENCE

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 symposium, participants should be able to:

- Evaluate barriers to equitable CAR T-cell therapy access and evaluate how philanthropic initiatives can improve access.
- Summarize recent advances in CAR T-cell therapy, including applications for autoimmunity and immunoregulation.
- Discuss the challenges and future directions of translating stem cell research into clinical practice.
- Evaluate the therapeutic potential of stem cellbased interventions.
- Explain how gene editing technologies are used in stem cell engineering.
- Review how mRNA technology is transforming cell and gene therapies.
- Identify novel targets and strategies for overcoming treatment resistance in solid tumors.
- · Analyze the risks and benefits of cell and gene therapies, including clinical and long-term outcomes.
- Explore the career paths and influence of successful women leaders in STEM.
- Assess the cost-effectiveness of cell and gene therapies within the context of healthcare economics.
- Examine strategies for successful technology transfer and collaboration between academia and industry.
- Define the role of industry partnerships and regulatory pathways in advanced therapy development.
- Recognize effective leadership strategies in the cell and gene therapy field.
- Analyze the challenges and opportunities in commercializing cell and gene therapies.
- Outline the process of translating advanced therapies from research to clinical practice and commercialization.

AGENDA

WEDNESDAY, APRIL 30

Check-In Irvine Auditorium 4:00 pm Visit Exhibit Hall and View Posters Houston Hall **WELCOME REMARKS** 5:00 pm Irvine Auditorium Jonathan A. Epstein, MD | University of Pennsylvania **SPECIAL ADDRESS CAR T-Cell Therapy in 2025: Navigating Innovations and** 5:05 pm **Overcoming Challenges** Carl H. June, MD | Penn Medicine | Symposium Co-Chair **Discussion** 5:35 pm **MODERATOR:** Bruce L. Levine, PhD | Penn Medicine | Symposium Co-Chair SPECIAL GUEST SPEAKER 5:45 pm **Developing mRNA for Therapy** Katalin Karikó, PhD | Penn Medicine **Discussion** 6:15 pm **MODERATOR:** Bruce L. Levine, PhD | Penn Medicine | Symposium Co-Chair **NETWORKING RECEPTION AND BOOK SIGNING** 6:30 pm Houston Hall 8:00 pm **ADJOURN**

AGENDA

THURSDAY, MAY 1

7:00 am

Check-In Irvine Auditorium

Breakfast, Visit Exhibits and View Posters Houston Hall

8:00am

WELCOME REMARKS

Irvine Auditorium

Carl H. June, MD | Penn Medicine | Symposium Co-Chair J. Larry Jameson, MD, PhD | University of Pennsylvania

KEYNOTE PANEL

Closing the Gap: Philanthropy's Role in Equitable CAR-T Access

MODERATORS:

Carl H. June, MD | Penn Medicine | Symposium Co-Chair David L. Porter, MD | Penn Medicine | Symposium Co-Chair

8:15 am

PANEL:

Valerie A. Arkoosh, MD, MPH | Pennsylvania Secretary of Human Services Tricia A. Bruning | Penn Medicine Development Lee Greenberger, PhD | LMG Bio1 Consulting Stephen Kelly | Children's Hospital of Philadelphia Tom Whitehead | Emily Whitehead Foundation

PLENARY SESSION 1

Risk and Cost vs Reward and Benefit for Cell and Gene Therapies

MODERATOR

Stephan A. Grupp, MD, PhD | Children's Hospital of Philadelphia | Symposium Co-Chair

9:00 am

Balancing CAR T Promise and Rare Events: Precision, doubt, and assessing risk

Bruce L. Levine, PhD | Penn Medicine | Symposium Co-Chair

9:20 am

Ensuring Access: Policy Challenges and Opportunities in Cell and Gene Therapy

Scott McGoohan, JD | Vertex Pharmaceuticals



PLENARY SESSION 1

Risk and Cost vs Reward and Benefit for Cell and Gene Therapies (Continued)

9:40 am

Immune Reconstitution after CAR-T: Restoration, Renewal, or **Beyond Repair?**

Stephen J. Schuster, MD | Penn Medicine

10:00 am

Discussion

ORAL PRESENTATION WINNER

10:10 am

Gene Delivery and Editing for Better CARs; Enhanced Conversion of T-Cells into CAR T-Cells by Modulation of the MAPK/ERK Pathway

Elham Adabi, PhD | Paul-Ehrlich-Institut

PLENARY SESSION 2

Leadership in Cell and Gene Therapies: The XX Factor

MODERATOR: Amanda Finck, PhD | Penn Medicine

10:15 am

Enhancing CAR T-Cell Therapy for Pancreatic Cancer

M. Angela Aznar Gomez, PhD | Penn Medicine | The Muriel Joan Drew Hege, MD, Fund Oral Abstract Winner

10:20 am

Leading the Charge For In Vivo Cell Therapy for The Treatment of Autoimmune Disorders

Laura Shawver, PhD | Capstan Therapeutics

10:35 am

Harnessing the Tumor Microenvironment by Genome Editing of T Cells

Chiara Bonini, MD | Vita-Salute San Raffaele University

10:50 am

CAR-T Trailblazers: Grand Adventures and the Power of Women Leadership in Cell and Gene Therapy

Kristen M. Hege, MD | Adaptimmune, Evolvelmmune, Kelonia, KSQ, Mersana

11:05 am

11:20 am	CONCURRENT SESSION I See Page 10
12:30 pm	LUNCH BREAK VISIT EXHIBITS VIEW POSTERS Houston Hall
1:30 pm	CONCURRENT SESSIONS II See Page 12
2:40 pm	BREAK VISIT EXHIBITS VIEW POSTERS Houston Hall

PLENARY SESSION 3

Are In Vivo Cell Therapies the Key to the Future of Medicine?

Irvine Auditorium

MODERATOR:

Bruce L. Levine, PhD | Penn Medicine | Symposium Co-Chair

3:30 pm

Nucleoside-Modified mRNA-LNP Therapeutics
Drew Weissman, MD, PhD | Penn Medicine

Cell-Specific In Vivo Gene Delivery: A Pipe Dream No Longer
Philip Johnson, MD | Interius Bio Therapeutics

In Vivo CAR T Therapeutics for Treatment of B Cell
Lineage Malignancies
Andy Scharenberg, MD | Umoja

4:30 pm

Discussion

ADJOURN

AGENDA

FRIDAY, MAY 2

7:00 am

Check-In Irvine Auditorium

Breakfast, Visit Exhibit Hall and View Posters Houston Hall

8:00am

WELCOME REMARKS

Irvine Auditorium

Carl H. June, MD | Penn Medicine | Symposium Co-Chair Alison W. Loren, MD, MSCE | Penn Medicine Joseph W. St. Geme, III, MD | Children's Hospital of Philadelphia

PLENARY SESSION 4

The Next Frontier in CAR T **Autoimmune Disease**

MODERATOR

David L. Porter, MD | Penn Medicine | Symposium Co-Chair

8:15 am

Rationale for B Cell Directed Cell Therapy in Autoimmune Disease

Peter A. Merkel, MD, MPH | Penn Medicine

8:35 am

CAR T Cells in Autoimmune Diseases: Novel Insights from Growing Experience

Fabian Müller, MD | University Hospital of Erlangen

8:55 am

Suppress or Destroy: Two Approaches to Engineer T Cells for the **Treatment of Autoimmune Disease**

James L. Riley, PhD | Penn Medicine

9:15 am

PLENARY SESSION 5

Pioneering Novel Frontiers in Stem Cell Transplantation and Engineering

MODERATOR:

Alexis A. Thompson, MD, MPH | Children's Hospital of Philadelphia Symposium Co-Chair

9:25 am

Engineering Immunity: $\alpha\beta$ T-Cell Depleted and $\gamma\delta$ T-Cell Enhanced Haplo-HSCT for Transplantation and Immunotherapy

Alice Bertaina, MD, PhD | Stanford University

9:45 am

From Ex Vivo to In Vivo Gene Therapy

Stefano B. Rivella, PhD | Children's Hospital of Philadelphia

10:05 am

Genetically Engineered Hematopoietic Stem Cell Transplantation for Cancer

Saar I. Gill, MD, MBBS, PhD, FRACP | Penn Medicine

10:25 am

Discussion

10:35 am

BREAK | VISIT EXHIBITS | VIEW POSTERS

Houston Hall

PLENARY SESSION 6

Location, Location: CAR T in Solid Tumors

Irvine Auditorium

MODERATOR:

Carl H. June, MD | Penn Medicine | Symposium Co-Chair

10:55 am

Engineered NK Cells for Cancer Immunotherapy: From Bench to Bedside

Katy Rezvani, MD, PhD, FRCP, FRCPath | MD Anderson Cancer Center

11:10 am

GFRa4 as a CAR T-Cell Target for the Treatment of Medullary Thyroid Cancer

Donald L. Siegel, MD, PhD | Penn Medicine

PLENARY SESSION 6

Location, Location: **CAR T in Solid Tumors** (Continued)

11:25 am

The Geography of CAR T-Cell Therapy: Optimizing Expansion, Persistence, Trafficking and Efficacy

Joseph A. Fraietta, PhD | Penn Medicine

11:40 am

Discussion

PLENARY SESSION 7

Celling Advanced Therapies: Navigating the Translation from Academia to Industry

MODERATOR:

Bruce L. Levine, PhD | Penn Medicine | Symposium Co-Chair

11:50 am

Reprogramming CAR T-Cell Therapy for Lymphomas: Innovations in Targeting and Efficacy

Marco Ruella, MD | Penn Medicine

12:10 pm

Towards a Platform for In Vivo Corrective Editing of Ultra-Rare and N-of-1 Metabolic Disorders

Kiran Musunuru, MD, PhD, MPH, ML, MRA | Penn Medicine

12:30 pm

Vision vs. Execution: Is There a Secret Ingredient in Turning **Ideas into Pharmaceutical Formulations?**

Dora Mitchell, PhD | Interius Biotherapeutics

12:50 pm

Discussion

Session speakers and Carl H. June, MD | Penn Medicine | Symposium Co-Chair

1:00 pm

CLOSING REMARKS

Symposium Co-Chairs

1:05 pm

ADJOURN

Lunch to Go Houston Hall

ACKNOWLEDMENTS

We gratefully acknowledge the following organizations for providing:

CORPORATE EXHIBITS

AbbVie

ADC Therapeutics

Astellas Pharma

Autolus

BeiGene

BioLife Solutions

Bristol Myers Squibb

Gamida Cell

Genmab

GSK

Incyte

J&J Innovative Medicine

Jazz Pharmaceuticals

Johnson & Johnson

Kite, a Gilead Company

Legend Biotech

Lonza

MaxCyte

Novartis CAR T - Patient Access &

Support (2 tables)

Novartis Pharmaceuticals

OmniaBio

Pharmacyclics, An AbbVie Company

PharmaEssentia

ScaleReady

Servier Pharmaceutical

Vertex Pharmaceuticals

Wexford Science and Technology

IN-KIND SUPPORTERS

American Society for Transplantation and Cellular Therapy

Emily Whitehead Foundation

HEADstrong Foundation

Leukemia & Lymphoma Society

CONCURRENT SESSIONS I

11:20 am - 12:30 pm

TRACK 1

Pediatric Medical Care

Café 58, Irvine

All speakers from Children's Hospital of Philadelphia

MODERATOR:

Stephan Grupp, MD, PhD

Emerging Gene Therapies for Inherited Bone Marrow Failure Syndromes

Julia T. Warren, MD, PhD

Immune Related Adverse Events of CAR T: What Can We Learn from Pediatric B-ALL?

Caroline Diorio, MD, FRCPC, FAAP

Leveraging Cell Therapies to Treat Autoimmunity in Pediatric Patients

Caitlin Elgarten, MD, MSCE

Discussion

TRACK 2

Clinical & Business Operations

Amado Recital Hall, Irvine

MODERATORS:

Mark J. Duckworth Children's Hospital of Philadelphia

Robert (Robb) E. Richards, MS, MBA Penn Medicine

WELCOME

Legal Considerations for Cell and Gene Therapies

Denene M. Wambach, Esq. Penn Medicine

Keith Stroup, Esq. Children's Hospital of Philadelphia

Data Science in Cell Therapy: From Collection to Dissemination

Lindsey MacDonald, MSN, MBA, RN, OCN Penn Medicine

Chris Catania, MPH Penn Medicine

Discussion

Collaborating for Success:

A Nurse and Systems Analyst Perspective on Implementing the **Epic Cell Therapy Module at CHOP**

Roni Devassy, BS Children's Hospital of Philadelphia

Ryan Burke, BSN, RN, CPHON, BMTCN Children's Hospital of Philadelphia

Discussion

TRACK 3

Nursing Keynotes

Hall of Flags, Ground FI Houston Hall

MODERATORS:

Heather Difilippo, CRNP Penn Medicine

Anna Sparrow, MSN, MPH, RN Children's Hospital of Philadelphia

SPECIAL WELCOME REMARKS

KEYNOTES

Afami-cel for Synovial Sarcoma: The First Approved TCR Therapy

Daniel Lefler, MD Penn Medicine

Creating A Space for All: The Pursuit of Belonging

Anna D'Aprile, MSN, RN, CPHON, NEA-BC Children's Hospital of Philadelphia

Hope Heffner-Solimeo, MA, ATR-BC, LPC Children's Hospital of Philadelphia

Panel Discussion

Session speakers and

Katherine Ebby, MSN, RN, CPHON Children's Hospital of Philadelphia

Marissa Zangardi, BSN, RN, CPN Children's Hospital of Philadelphia

Eleni Hindle, BSN, RN, CPHON Children's Hospital of Philadelphia

Selina Plummersturgis, BSN, RN Children's Hospital of Philadelphia

TRACK 4

The Next Disruptions in Cell and Gene Therapy:Hot Topics Lightning Talks from Muriel Joan Drew Hege, MD, Fund Scholars

Irvine Auditorium

MODERATOR:

Kristen M. Hege, MD Adaptimmune, Evolvelmmune, Kelonia, KSQ, Mersana

All speakers are from The Center for Cellular Immunotherapies and The Muriel Joan Drew Hege, MD, Fund awardees

IL-9 Redirects CAR T-Cell Fate, Enhancing Efficacy Against Solid Tumors Sofia Castelli, PhD

Transient Overexpression of IKZF1 Enhances Memory Precursor Phenotype in CAR T-Cells

Sisi Chen, PhD

Modulation of the Gut Microbiota Engages Antigen Cross-Presentation to Enhance Antitumor effects of CAR T Cell

Francesca Costabile, MSc, PhD

Overcoming Resistance; Deciphering the Glycan and Protein Interactome of Siglec-15: Unraveling its Role in Tumor-Associated Immunosuppression

Nohelly Derosiers, BE

Bridge Production: The Role of Product Development Laboratories in Advancing Cellular Therapies from Research to GMP Manufacturing

Laura Garcia Gerique, PhD

Deciphering the Impact of Immunological Aging in CLL CAR T-Cell Therapy

Julia Han Noll, MS

p:Hishing for T-Cells: Using 'Chimeric Antigen-Presenting' (CAP) T Cells to Target and Eliminate Antigen-Specific T-Cell Populations

Kaitlin A. Read. PhD

Off-the-Shelf CAR-Invariant NKT Cells for Human and Canine Solid Tumors

Antonia Rotolo, MD, PhD

Novel CAR T-Cells for Prostate Cancer

Nour Shobaki, PharmD, PhD

Chimeric Autoantigen Receptor T-Cells for Treatment of Antineutrophil Cytoplasmic Antibody Vasculitis

Heather Sosnoski, BS

Discussion

TRACK 5

Commercial Partnering & Creating Products for Patients

Class of '49, 2nd FI Houston Hall

MODERATOR:

John S. Swartley, MBA, PhD Penn Medicine

Workshop on Protecting, Translating and Commercializing Cell and Gene Therapy Intellectual Property

PANEL:

Mark Engleka, PhD Penn Medicine

Tess Cameron, BA RA Capital Management

Mir Masud-Elias, JD, MBE Arnold & Porter

Discussion

TRACK 6

Expanding Access While Maintaining Quality: Going Where No CAR T-Cell Has Gone Before

Benjamin Franklin Room, 2nd Fl Houston Hall

MODERATOR

David L. Porter, MD Penn Medicine

Expanding Access to Cell Therapies

Steven Devine, MD
National Marrow Donor Program (NMDP) /
Be The Match

Discussion

Accelerating Global Access to Point of Care Cell Therapy

Stephan Kadauke, MD, PhD Children's Hospital of Philadelphia

Discussion

Quality Program Considerations for Cell Therapies Outside Heme Malignancies

PANEL:

Victoria Collier, RN, BSN Children's Hospital of Philadelphia

Jenna Fetters, MSHS, MLS (ASCP) Penn Medicine

Becca Pesce-Noga, MS, CSSBB Penn Medicine



CONCURRENT SESSIONS II

1:30 pm - 2:40 pm

TRACK 1

Pediatric Nursing Care

Café 58, Irvine

All speakers from Children's Hospital of Philadelphia

MODERATOR:

Diane Baniewicz, MSN, CPNP

Samantha Barone, MSN, CPNP-AC, **CPHON**

TOXICITY MANAGEMENT

Immune Effector Cell Associated Hemophagocytic Lymphohistiocytosis Syndrome with CAR 19/22

Colleen Callahan, RN, MSN, CRNP

Mackenzie Stewart, MSN, CRNP, CPNP-AC

Discussion

IMPROVING PATIENT PARTNERSHIP IN CARE

Implementation and Compliance with **ICE Scores in AYA Patients Post-CAR T**

Katie A. Smith, DNP, MPH, RN, A CCNS-P, CPN

Anna Sparrow, MSN, MPH,RN

All Smiles for Clabsi Prevention: Improving Oral Care Completion on the Inpatient Oncology and Blood and **Marrow Transplant Units**

Catherine Aronowitz, MSN, RN, CPN, **CPHON**

IMPROVING PROVIDER PRACTICE

Teamwork Makes the Marrow Work: Interprofessional Rounds on Inpatient Oncology and Blood and Marrow Transplant Units

Emily Roth, BSN, RN, CPN, CPHON, CPHQ

Sara Gunnoe, MPAS, PA-C

BMT Bootcamp: Education and training best practices for new BMT nurses

Ryan Burke, BSN, RN, CPHON, BMTCN

Megan Atkinson, BS, CHTC

Discussion

TRACK 2

Clinical & Business Operations

Amado Recital Hall, Irvine

MODERATORS:

Mark J. Duckworth

Children's Hospital of Philadelphia

Robert (Robb) E. Richards, MS, MBA Penn Medicine

From Coverage Policies to Prior **Authorization: Financial Clearance for Cell and Gene Therapies**

Claire White, MSN, RN

Children's Hospital of Philadelphia

Denise Belcher, MBA Children's Hospital of Philadelphia

Kimberly Sacco, MHA Children's Hospital of Philadelphia

Blueprint for Success: Constructing a Unified Approach for the Future of Cell & Gene Therapy

Kaitlyn Lyons, MBA Penn Medicine

Discussion

TRACK 3

Adult Nursing Care

Hall of Flags, Ground FI Houston Hall

All speakers from Penn Medicine

MODERATORS:

Heather Difilippo, CRNP

Patricia Mangan, RN, MSN, CRNP, APRN-BC

CART T Cell-Therapy and Bispecifics for Multiple Myeloma

Patricia Mangan, RN, MSN, CRNP, APRN-BC

Overcoming Challenges and Complexities for Cell Therapy Coordinators

Matthew P. Casazza, RN

Natalie K. Stefano, RN

Harnessing EPIC for Cell Therapy **Workflows and Data Management**

Lindsey MacDonald, MSN, MBA, RN, OCN

Elizabeth A. Weber, RN. BSN

TIL Therapy and Inpatient Process Management of Infusion and IL2 Toxicities

Lauren B. Hollander, CRNP

Rachel E. Hartman, PharmD, BCOP

TRACK 4

Hot Topics in Cell Therapies

Irvine Auditorium

MODERATOR:

Elizabeth O. Hexner, MD, MSTR Penn Medicine

Lessons from Spatial Biology on CAR HLH/MAS

Andrew J. Rech, MD, PhD Penn Medicine

Novel CAR Targets for Prostate Cancer and Potential Applications in Renal Cancer

Naomi B. Haas, MD Penn Medicine

Cytokine Biology Behind CAR T Toxicity and Patient Management

David T. Teachey, MD Children's Hospital of Philadelphia

Discussion

TRACK 5

Commercial Partnering & Creating Products for Patients

Class of '49, 2nd FI Houston Hall

MODERATOR:

Audrey E. Greenberg, MBA, CPA Discovery Labs Center for Breakthrough Medicines

Amplifying the Academia and Industry Ecosystem in Cellicon Valley

PANEL:

R. Carter Caldwell, MBA Penn Medicine

Jen Gilburg BS, MS Pennsylvania Department of Community and Economic Development

John Grady, MPA Wexford Science & Technology

Discussion

TRACK 6

Expanding Access While Maintaining Quality: Going Where No CAR T-Cell Has Gone Before

Benjamin Franklin Room, 2nd Fl Houston Hall

MODERATOR:

Zev Binder, MD, PhD Penn Medicine

LATE BREAKERS

Cell Therapies in Brain Cancer

Donald M. O'Rourke, MD Penn Medicine

Hideho Okada, MD, PhD University of California San Francisco



CONFERENCE CHAIRS



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SPECIAL GUEST SPEAKER



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KEYNOTE PANELISTS



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CONFERENCE SPEAKERS

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Philadelphia, PA, USA

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Assistant Professor, Department of Microbiology

Adjunct Assistant Professor, Department of Pathology and Laboratory Medicine

Director, Translational and Correlative Sciences

Richard 'Buzz' Cooper Scholar, Abramson Cancer Center

University of Pennsylvania

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