Cellicon Valley '23: The Future of Cell and Gene Therapies

Wednesday-Friday, June 21-23, 2023 – Hybrid Symposium

Irvine Auditorium and Perelman Quadrangle, 3401 Spruce Street, Philadelphia, PA 19104 presented by Penn's Abramson Cancer Center and Children's Hospital of Philadelphia

Overview

As home to the first FDA-approved cell and gene therapies, Penn Medicine and Children's Hospital of Philadelphia are international leaders who have helped propel Philadelphia into Cellicon Valley, with the University of Pennsylvania ranking 1st among global universities for CAR-T cell patents. 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 approvals of chimeric antigen receptor (CAR) T cells offers even patients with highly chemo-refractory hematologic malignancies additional treatment options. World experts in CAR T-cellular therapy, hematologic malignancies, and hematopoietic stem cell transplantation (HSCT) have been assembled to discuss the development and implementation of this therapy and weigh in on current applications, best practices, novel strategies and future developments for CAR T cells.

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 understanding new strategies to optimize and improve CAR T 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. Research participants will leave with 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, hematologists, immunologists, immunologists, 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:

- Identify current application of CAR T-cell therapy including: disease indication, accurate patient selection, manufacturing and treatment administration for adult and pediatric patients
- Describe the latest for CAR T-Cell Therapy for T Cell ALL, multiple myeloma and CLL
- List challenges and opportunities for equitable patient access
- Explain the risk of malignancies and curative therapies for hemoglobinopathies
- Identify the current status of globalization of cell and gene therapies
- Describe the latest targets and technologies for CARs
- Define toxicity and its management
- Identify mechanisms of resistance and how to overcome it
- List recent advances in cell therapy for solid tumors
- Describe chimeric antigen receptor T Cells for T Cell malignancies
- Identify the latest evidence based standards in nursing care resulting from the recent advances in cell therapy and transplant:
 - o Identify advances in re-treatment
 - List the latest updates in fertility preservation
 - Describe the challenges with navigating and coordination of BMT/cell gene therapies and CAR-T
 - Explain how bispecific antibodies work
 - List standards of care and practice related to cell therapy infusion day
 - Repeat the rationale for nursing to remain current with new and upcoming therapies

AGENDA

Wednesday, June 21, 2023

4:00 pm In-Person: Check-In and Visit Exhibits

Virtual: Log-In and Visit Virtual Exhibit Hall

5:00 pm WELCOME REMARKS

J. Larry Jameson, MD, PhD | University of Pennsylvania

5:05 pm SPECIAL GUEST SPEAKER

Siddhartha Mukherjee, MD, DPhil | Columbia University | VOR Biopharma | Immuneel

6:00 pm NETWORKING RECEPTION AND BOOK SIGNING

7:00 pm Adjourn

Thursday, June 22, 2023

7:00 am In-Person: Check-In, Visit Exhibits and Breakfast

Virtual: Log-In and Visit Virtual Exhibit Hall

8:00 am Welcome Remarks

<u>Carl H. June, MD</u> | Penn Medicine | Symposium Co-Chair

John L. Jackson Jr., PhD | University of Pennsylvania

<u>Joseph W. St Geme, MD</u> | Children's Hospital of Philadelphia

8:15 am KEYNOTE ADDRESS

Nucleoside-Modified mRNA-LNP Therapies

<u>Drew Weissman, MD, PhD</u> | Penn Medicine

8:45 am Discussion

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

PLENARY SESSION 1: Driving Clinical CAR's

Moderator: <u>David L. Porter, MD</u> | Penn Medicine | Symposium Co-Chair

9:00 am 3 Second Line DLBCL Studies, What Happened and Why

Stephen J. Schuster, MD | Penn Medicine

9:20 am CAR T-Cell Therapy for T Cell ALL

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

9:40 am Optimizing Road Conditions to Get Everyone to the Finish Line

Krina K. Patel, MD, MSc | MD Anderson Cancer Center

10:00 am Discussion

	PLENARY SESSION 2: Gene Genies - Engineering Immune Effector Cells Moderator: Bruce L. Levine, PhD Penn Medicine Symposium Co-Chair				
10:10 am	Base Editing and Prime Editing: Correcting Mutations that Cause Genetic Disease in Cells, Animals, and Patients David R. Liu, PhD Broad Institute, Harvard University, and HHMI				
10:30 am	Programming Cellular Machines to Sense and Treat Disease Wendell Lim, PhD University of California San Francisco				
10:50 am	TBA <u>Fyodor D. Urnov, PhD</u> University of California, Berkeley				
11:10 am	Discussion				
11:20 am - 2:45 pm	CONCURRENT SESSIONS See 2023 Concurrent Sessions pages 6-7				
12:30 pm	LUNCH BREAK VISIT EXHIBITS				
1:30 pm	CONCURRENT SESSIONS continue				
2:40 pm	BREAK VISIT EXHIBITS				
	PLENARY SESSION 3: Covering the Cost of Cell Therapy: Financing and Advocacy Moderator: David L. Porter, MD Penn Medicine Symposium Co-Chair				
3:00 pm	TBA <u>Kevin B. Mahoney, DBA</u> Penn Medicine				
3:20 pm	Provider Solutions - Where We Are and Where We Should Be? Mark Trusheim, MS NEWDIGS				
3:40 pm	Striving for Equitable Access – Challenges and Opportunities Louis J. DeGennaro, PhD Leukemia & Lymphoma Society				

PLENARY SESSION 4: Global Expansion of CAR T-Cell Therapy

Moderator: <u>Bruce L. Levine</u>, <u>PhD</u> | Penn Medicine | Symposium Co-Chair

4:05 pm Costa Rica

3:55 pm

Román Macaya Hayes, PhD | Costa Rican Social Security System

4:20 pm Australia

Simon Harrison, MBBS, MRCP (UK), PhD, FRCPath (UK), FRACP | Peter MacCallum Cancer

Centre

Discussion

4:35 pm Middle East

Shahrukh K. Hashmi, MD | Mayo Clinic

4:50 pm India

Arun Anand, MD | Immuneel

5:05 pm Discussion

5:15 pm SPECIAL ADDRESS

The Improbable History of CAR T Cells

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

5:50 pm Discussion

6:00 pm COCKTAIL RECEPTION

7:30 pm Adjourn

Friday, June 23, 2023

7:00 am In-Person: Check-In, Visit Exhibits and Breakfast

Virtual: Log-In and Visit Virtual Exhibit Hall

8:00 am WELCOME

Alison Wakoff Loren, MD, MSCE | Penn Medicine

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

PLENARY SESSION 1: The Next Frontier is Here - Hemoglobinopathies

Moderator: Alexis A. Thompson, MD, MPH | Children's Hospital of Philadelphia | Symposium

Co-Chair

8:15 am Genome Edited Cell Based Medicines

Matthew Porteus, MD, PhD | Stanford University

8:35 am Gene Editing

Mitchell J. Weiss MD, PhD | St. Jude Children's Research Hospital

8:55 am Risk of Malignancies and Curative Therapies for Hemoglobinopathies

John F. Tisdale, MD | National Heart, Lung and Blood Institute

9:15 am Discussion

PLENARY SESSION 2: The Peril and the Promise of Cell Therapy in Solid Tumors

Moderator: TBD

9:25 am Two-in-One CAR-T Cells for Solid Tumors

Marcela V. Maus, MD, PhD | Massachusetts General Hospital

9:45 am **CAR-T Cell Therapy for Advanced Prostate Cancer: Clinical trials and tribulations** Vivek K. Narayan, MD, MS | Penn Medicine 10:05 am **TBA** Crystal L. Mackall, MD | Stanford University 10:25 am Discussion **BREAK | VISIT EXHIBITS** 10:35 am PLENARY SESSION 3: CAR T Cells Can Cure Patients: What are we waiting for? Moderator: TBA 10:55 am Long Term Data in Lymphoma Frederick Locke, MD | Moffitt Cancer Center **Curative Potential CART for ALL** 11:15 am Stephan A Grupp, MD, PhD | Children's Hospital of Philadelphia | Symposium Co-Chair 11:25 am Discussion 11:35 am **BREAK | VISIT EXHIBITS** PLENARY SESSION 4: Cell and Gene Academia to Industry Translation Moderator: Lindsey A. George, MD | Children's Hospital of Philadelphia 11:45 am **Hemophilia Gene Therapy** <u>Lindsey A. George, MD</u> | Children's Hospital of Philadelphia 12:05 pm **Gene Therapy: A Case Study for Disruptive Technology** James M. Wilson, MD, PhD | University of Pennsylvania 12:25 pm Perspectives on CHOP, Spark Therapeutics and AskBio Katherine A. High, MD | Children's Hospital of Philadelphia 12:45 pm Discussion **CLOSING REMARKS** 12:55 pm Cellicon Valley Co-Chairs **ADJOURN** 1:00 pm Lunch to Go

11:20 am – 12:30 pm CONCURRENT SESSIONS (70 minutes)

	ACK 2 AM	TRACK 3 AM	TRACK 4 AM	TRACK 5 AM
Moderator: <u>Timothy S. Olson, MD, PhD</u> Children's Hospital of Philadelphia Ope Mode	nical Operations/Business erations/Investment lerator: Mark Duckworth dren's Hospital of Philadelphia	Nursing Keynotes Moderators: Patricia Mangan, MSN, CRNP Penn Medicine Symposium Co-Chair Trish Hankins, RN, CCRC Children's Hospital of Philadelphia Symposium Co-Chair	Present and Future Cell Therapies Moderator: Elizabeth O. Hexner, MD, MSTR Penn Medicine	COMING SOON!
University of Minnesota Or AT Proteomic Analysis of CRS Caroline Diorio, MD, FRCPC, FAAP Children's Hospital of Philadelphia Novel Approaches Combining Immunosuppression (Reverse CART) Bhavya S. Doshi, MD Children's Hospital of Philadelphia Discussion OPE Best Deve Thera Onboo with a TBA Childr Expan Kathle Penn I Emma Unive Lanca Nik Bu Penn I	e Miceli dren's Hospital of Philadelphia ene Wambach ersity of Pennsylvania ussion ERATIONS t practices (so far) of eloping Cell and Gene rapy Programs	SPECIAL WELCOME Lindsey Zinck, MSN, RN, OCN, NEA-BC Penn Medicine Ellen Tracy, RN, MSN, NEA-BC Children's Hospital of Philadelphia KEYNOTES Modifying CART: Advance in retreatment Jakub Svoboda, MD Penn Medicine Fertility Preservation Alison W. Loren MD, MS Jill P. Ginsberg, MD Discussion Add nurses Moderator: TBA	Cell Therapies of the Present Toxicity and Toxicity Management (CRS/Prophylactic and Neurotoxicity) Noelle V. Frey, MD, MSCE Penn Medicine CAR T for Multiple Myeloma Edward A. Stadtmauer, MD Penn Medicine CAR T Cells in CLL. Why or Why Not? David L. Porter, MD Penn Medicine Symposium Co-Chair Mechanisms of Resistance? Overcoming Resistance? David B. Miklos, MD, PhD Stanford University Discussion	

1:30 – 2:40 pm (70 minutes) CONCURRENT SESSIONS continue

Pediatric Nursing Care Moderator: TBA Clinical Operations/Business Operations/Investment Moderator: Robert (Robb) E. Richards, MS, MBA University of Pennsylvania All speakers from Children's Hospital of Philadelphia Navigation and Coordination of These Complex Therapeutts BMT7 Cell Gene Therapies Denthe Betcher CART Brooke Leibfreid Nould Case Reviews Vision Vision NBL CART TBA Children's Hospital of Philadelphia TBA Children's Hospital of Philadelphia TBA Children's Hospital of Philadelphia Discussion Clinical Operations/Business Operations/Investment Moderator: Moderator: Moderator: Moderator: TBA Adult Nursing Care Moderator: TBA Adult Nursing Care Moderator: TBA Adult Nursing Care Moderator: TBA Adult Nursing Care Moderator: TBA Adult Nursing Care Moderator: TBA Adult Nursing Care Moderator: TBA Cell Therapies of the Future Resenchynal Stem Cells and Their Exosomes for Gene Delivery Elzabeth J. Shpail MD Mo Andrestors Caner Center Reschel Lumberp, PAC Penn Medicine Case Reviews Case Reviews Case Reviews Case Reviews Children's Hospital of Philadelphia Discussion Discussion Case Reviews Case Reviews Children's Hospital of Philadelphia Discussion Discussion Clinical Operations/Investment Moderator: TBA Adult Nursing Care Moderator: TBA Cell Therapies of the Future Research Rescher Resche	TRACK 1 PM	TRACK 2 PM	TRACK 3 PM	TRACK 4 PM	TRACK 5 PM
Philadelphia How do you Organize/Develop Onboarding of Staff/Sites as Denise Belcher Expands? Ex	•	Operations/Investment Moderator: Robert (Robb) E. Richards, MS, MBA	Moderator: <u>Patricia Mangan, MSN, CRNP</u> Penn	Therapies	COMING SOON!
Discussion	Navigation and Coordination of These Complex Therapeutics BMT/ Cell Gene Therapies Denise Belcher CAR-T Brooke Leibfreid Case Reviews Vctl NBL CART TBD CART Barbara McGlynn, BSN, RN Samantha Barone, MSN, CPNP-AC, CPHON Diane Baniewicz, RN, MSN, CRNP Colleen Callahan, RN, MSN, CRNP Cell Therapy Infusion Day! TBA	How do you Organize/Develop Onboarding of Staff/Sites as Cell and Gene Therapy Expands? TBA Children's Hospital of Philadelphia TBA University of Pennsylvania Discussion QUALITY DEVELOPMENT TBA Children's Hospital of Philadelphia TBA University of Pennsylvania	Bispecifics Mary Elizabeth Sanchez, CRNP, MSN Penn Medicine Unmet Needs Rachel Lundberg, PA-C Penn Medicine Case Reviews Chronic Graft Versus Host Disease – Linda M. Perry, PA-C Lymphoma – Danielle Land, MSN, CRNP Challenging CART Coordination Case – Elizabeth A. Weber, RN, BSN	Mesenchymal Stem Cells and Their Exosomes for Gene Delivery Elizabeth J. Shpall, MD MD Anderson Cancer Center More Than Just T Cells: CAR macrophage or gene engineering of stem cells to generate CAR T resistance Saar I. Gill, MD, PhD Penn Medicine Chimeric Antigen Receptor T Cells for T Cell Malignancies Marco Ruella, MD Penn Medicine IMAGEining CARS Michael D. Farwell, MD Penn Medicine	