ACDRS-NIH Workshop: Cell-Based Immunotherapy: From Bench to Bedside and Beyond

Masur Auditorium, National Institutes of Health Clinical Center, Bethesda, Maryland

January 22, 2019

Time	Topic
8:00 am	**Opening Remarks & Dedication of Workshop to Memory of ACDRS Director, Louis Cantilena** *Carl C. Peck, MD, ACDRS Interim Co-Director and Adjunct Professor, University of California, San Francisco*
8:05	**Keynote Address** *ADM Brett P. Giroir, MD, Assistant Secretary for Health, US Department of Health and Human Services*
8:30	**Session 1: Harnessing the Immune System to Fight Disease** *Chair: Nicholas P. Restifo, MD, Director, Center for Cell-Based Therapy and Head, Center of Excellence in Immunology, National Cancer Center (NCI), NIH*
	8:30 **T Cell Stemness: An Emerging Principle of Successful Adoptive Cell Therapy** *Nicholas P. Restifo*
	8:50 **Cancer Immunotherapy** *Stephan Grupp, MD PhD, Director of the Cancer Immunotherapy Program, Director of Translational Research, Center for Childhood Cancer Research and Medical Director, Stem Cell Laboratory, Children's Hospital of Philadelphia*
	9:10 Q & A
9:30	**Break**
10:20	**Session 2: Regulatory Realities** *Chair: Ellen G. Feigal, MD, Partner, NDA Partners LLC*
	10:20 **Regulatory Considerations for Cell-Based Immunotherapies** *Peter Marks, MD PhD, Director, Center for Biologics Evaluation and Research (CBER), FDA*
	10:40 **Preclinical Considerations for Cell-Based Immunotherapies** *Allen Wensky, PhD, Biologist, Office of Tissues and Advanced Therapies (OTAT), CBER, FDA*
	11:00 **Approval of Kymriah (tisagenlecleucel) & Yescarta (axicabtagene ciloleucel)** *Roger J. Kurlander, MD, Medical Officer, CBER, FDA*
	11:20 **Panel Discussion** *Speakers above*
11:50	**Lunch Break**

12:50 pm **Session 3: Challenges and Opportunities in Manufacturing of Cell-based Immunotherapies**
Chair: David Stroncek, MD, Chief, Cell Processing Section and Co-Director, HLA Laboratory, Department of Transfusion Medicine (DTM), NIH

Exploratory initial studies with autologous cell therapies –Collection issues; Can exceptions be made from strict GMP when novel reagents are needed? What are the issues in changing the process in manufacturing? When does the manufacturing need to be "locked down"?

12:50 **Challenges in Process and Analytical Development of Cell Therapies: An Academic Perspective**
David DiGiusto, PhD, Chief Technical Officer, Semma Therapeutics

1:10 **Early Stage CMC Considerations for Source Materials and Manufacturing in Cellular Therapy**
Jaikumar Duraiswamy, PhD, Biologist, Cell Therapies Branch, OTAT, CBER, US FDA

1:30 **Industry Perspective on Large Scale Manufacture of Autologous Cell-Based Immunotherapies**
Thomas Fellner, PhD MBA, Head of Commercial and Business Development, Cell and Gene Therapy, Lonza Pharma & Biotech

1:50 **Panel Discussion**
Speakers above

2:20 **Break**

2:40 **Session 4: Challenges and Opportunities in Clinical Studies**
Co-Chairs: Ellen G. Feigal, MD, Partner, NDA Partners LLC and Nirali N. Shah, MD MHSc, Associate Research Physician, Hematologic Malignancies Section, Pediatric Oncology Branch, NCI, NIH

2:40 **Issues in Clinical Trial Design in Cell Therapy**
Telba Irony, PhD, Deputy Director, Office of Biostatistics and Epidemiology, CBER, FDA

3:00 **Early Phase Trials of CAR T Cells: Lessons Learned**
Crystal Mackall, MD, Ernest and Amelia Gallo Family Professor of Pediatrics and Medicine, Founding Director Stanford Center for Cancer Cell Therapy, Stanford University

3:20 **CRISPR-Edited CAR T Cells**
Jorge A. Mansilla-Soto, PhD, Research Associate, Center for Cell Engineering and Immunology Program, Memorial Sloan Kettering Cancer Center

3:40 **New Cellular Immunotherapy for Solid Tumors**
Steven A. Rosenberg, MD PhD, Chief of Surgery Branch, Head of Tumor Immunology Section, NCI, NIH

4:00 **Panel Discussion**
Speakers above and Greg Campbell, PhD, Principal Statistician, GCStat Consulting

4:30 **Wrap Up and Summary**
Harvey Klein, MD, Chief, Department of Transfusion Medicine, NIH Clinical Center

4:40 **Adjourn**