



Monday, May 10

3 - 7 p.m. ET

# Expanding the Success of Immune Effector Cell Therapies for Cancer

Pre-Meeting Workshop



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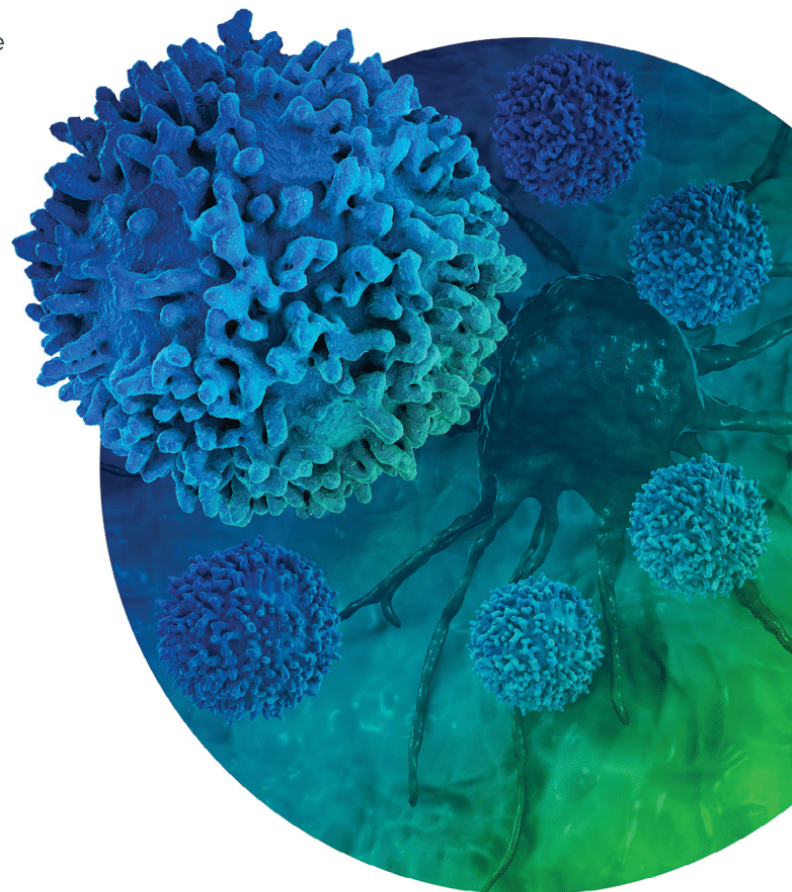
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## Expanding the Success of Immune Effector Cell Therapies for Cancer

Co-Chairs: Terry Fry, M.D., and Sarah Nikiforow, M.D., Ph.D.

**3 - 3:23 p.m.**

### **Rational Synthetic Receptor Design: Signaling**

Gianpietro Dotti, M.D., UNC Lineberger Cancer Center

**3:23 - 3:46 p.m.**

### **Engineering CAR T Cells to Overcome Therapeutic Resistance**

Robbie Majzner, M.D., Stanford University School of Medicine

**3:46 - 4:09 p.m.**

### **Challenges of Simultaneous Targeting CD19 and CD22 - Insights from Design and Clinical Experimental**

Martin Pule, M.D., University College London

**4:09 - 4:19 p.m.**

### **Panel Discussion**

- Gianpietro Dotti, M.D., UNC Lineberger Cancer Center
- Robbie Majzner, M.D., Stanford University School of Medicine
- Martin Pule, M.D., University College London

**4:19- 4:42 p.m.**

### **Challenges of Immune Effector Cell Therapies: Genomics and Transcriptomics of Leukemic Relapse**

Andrei Thomas-Tikhonenko, Ph.D., Children's Hospital of Philadelphia



## Expanding the Success of Immune Effector Cell Therapies for Cancer

**4:42 - 5:05 p.m.**

### **Challenges of Immune Effector Cell Therapies: Toxicity**

Bianca Santomasso, M.D., Ph.D., Memorial Sloan-Kettering Cancer Center

**5:05 - 5:28 p.m.**

### **Challenges in Adoptive Cell Therapies: Manufacturing & Access**

Isabelle Rivière, Ph.D., Memorial Sloan-Kettering Cancer Center

**5:28 - 5:38 p.m.**

### **Panel Discussion**

- Andrei Thomas-Tikhonenko, Ph.D., Children's Hospital of Philadelphia
- Bianca Santomasso, M.D., Ph.D., Memorial Sloan-Kettering Cancer Center
- Isabelle Rivière, Ph.D., Memorial Sloan-Kettering Cancer Center

**5:38 - 6:01 p.m.**

### **Challenges for Cell Therapies in Solid Tumors**

Lisa Butterfield, Ph.D., UCSF

**6:01 - 6:24 p.m.**

### **T-cell Gene Editing and Insertion Site Genotoxicity**

Frederic Bushman, Ph.D., University of Pennsylvania

**6:24 - 6:47 p.m.**

### **Global Analysis of Shared T cell Specificities in Human Non-Small Cell Lung Cancer**

Diane Tseng, M.D., Ph.D., University of Washington

## Expanding the Success of Immune Effector Cell Therapies for Cancer

6:47 - 6:57 p.m.

### Panel Discussion

- Lisa Butterfield, Ph.D., UCSF
- Frederic Bushman, Ph.D., University of Pennsylvania
- Diane Tseng, M.D., Ph.D., University of Washington



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## **Frederic Bushman, Ph.D.**

University of Pennsylvania

Frederic Bushman is the W. M. Measey Professor and Chair of Microbiology at the Perelman School of Medicine at the University of Pennsylvania. Dr. Bushman received his bachelor's degree in Biology and English at Amherst College, and his Ph.D. in Cellular and Developmental biology at Harvard University. His research centers on human genome modification, the human microbiome, and pathogenesis of HIV and Coronavirus. Dr. Bushman was named a Pioneer of Human Gene Therapy, and served as a Principal investigator for the Human Microbiome Project, and is a founding Principal Investigator of the PennCHOP Microbiome Program and the Penn Center for Research on Coronavirus and Other Emerging Pathogens. Dr. Bushman has published over 350 research papers (cited over 54,000 times; ISI h factor of 100) and two books.

## **Lisa Butterfield, Ph.D.**

UCSF

Lisa H. Butterfield, Ph.D. is the Vice President, PCI Research and Development at the Parker Institute for Cancer Immunotherapy, and an Adjunct Professor of Microbiology and Immunology, University California San Francisco. She is focused on cancer vaccines and cellular therapies for melanoma, hepatocellular cancer and other tumor types. Dr. Butterfield was most recently Professor of Medicine, Surgery, Immunology and Clinical and Translational Science at the University of Pittsburgh (2003-2018) and Director of the Hillman Cancer Center Immunologic Monitoring and Cellular Products Laboratory. She has a PhD in Biology from UCLA, followed by postdoctoral fellowships in Cellular Immunology and Cancer Gene Therapy also at UCLA. She was the President of the Society of Immunotherapy of Cancer (SITC, 2017-2018) and a member of the SITC Executive Committee (2015-2020). She led the Immunology Reference Lab for the ECOG-ACRIN NCI cooperative group (2006-2018) and collaborates on biomarker studies in many clinical trials. She investigates immunotherapy for hepatocellular cancer and melanoma, involving peptides, dendritic cells and adenoviruses, and effector responses to tumor antigens. She has published over 170 peer-reviewed manuscripts, reviews and book chapters, and mentored over 20 students and postdocs.

**Gianpietro Dotti, M.D.**

UNC Lineberger Cancer Center

Dr. Dotti studied immunotherapy strategies to treat patients with hematologic malignancies including lymphomas and leukemia and solid tumors such as neuroblastoma. In particular, he developed the program of the CD19-specific chimeric antigen receptor at Baylor College of Medicine and cloned a novel chimeric antigen receptor targeting the light chain of human immunoglobulins. This chimeric molecule engrafted in human T-lymphocytes allows a selective elimination of tumor cells expressing the kappa-light chain of human immunoglobulin while sparing the normal compartment of B-lymphocytes expressing the lambda-light chain. Dr. Dotti was also involved in developing CAR-based strategies to target solid tumors such as neuroblastoma in pediatric patients and triple negative breast cancer in adult patients. In collaboration with Dr. Brenner he also developed the clinical phase of a novel safety switch for T cells based on the human caspase-9. A significant focus of Dr. Dotti research is the development of strategies of T cell engineering aimed at overcoming immune suppressive mechanisms of the tumor microenvironment.

**Terry Fry, M.D.**

University of Colorado Denver

Dr. Fry is a Professor of Pediatrics, Hematology and Immunology and Co-Director of the Human Immunology and Immunotherapy Initiative at the University of Colorado School of Medicine and holds the Robert and Kathleen Clark Endowed Chair in Pediatric Cancer Therapeutics at the Children's Hospital Colorado. He arrived at Children's Hospital Colorado in 2018 after serving as Head of the Hematologic Malignancies Section in the Pediatric Oncology Branch at the NIH where he led efforts in Cellular Immunotherapy for pediatric leukemia. Prior to the NIH, Dr. Fry was Chief of Blood and Marrow Transplantation at Children's National Medical Center in Washington, DC. Dr. Fry's research focuses on the preclinical and clinical development of chimeric antigen receptor T cells for pediatric cancers. He serves on the Committee for Scientific Affairs for the American Society of Hematology, Vice Chair for Biology in the Cellular Therapy Committee of the Children's Oncology Group and was elected into the American Society for Clinical Investigation.



**Robbie Majzner, M.D.**

Stanford University School of Medicine

Robbie Majzner is an Assistant Professor of Pediatrics in the Division of Hematology and Oncology. After graduating with a BA from Columbia University, Dr. Majzner attended Harvard Medical School, where he developed an interest in pediatric oncology. He completed his residency training in pediatrics at New York Presbyterian-Columbia and fellowship training in pediatric hematology-oncology at Johns Hopkins and the National Cancer Institute. During his fellowship, he cared for some of the first pediatric patients to receive CD19 chimeric antigen receptor (CAR) T cells, children with B cell acute lymphoblastic leukemia (B-ALL) who often had no other therapeutic option. Witnessing the success of CAR T cells in these patients drove Dr. Majzner to the laboratory, where he focuses on extending the use of CAR T cells to solid tumors. He has generated and optimized novel receptors to recognize antigens over-expressed on pediatric solid tumors such as GD2 (Mount/Majzner et al., *Nature Medicine*, 2018), B7-H3 (Majzner et al., *Clinical Cancer Research*, 2019), and ALK (Walker/Majzner et al., *Molecular Therapy*, 2017). Several of these publications have led to Phase 1 clinical trials in children. Current work in the Majzner Laboratory focuses on understanding CAR T cell resistance and on optimizing CARs to enhance their efficacy when the amount of target (antigen density) is limiting (Majzner et al., *Cancer Discovery*, 2020). By drawing on state of the art bioengineering techniques, the Majzner Laboratory focuses on enhancing the potency and specificity of CAR T cells.

**Sarah Nikiforow, M.D., Ph.D.**

Dana-Farber Cancer Institute

Dr. Nikiforow is currently an Assistant Professor at Harvard Medical School with the Stem Cell Transplant Program at Dana-Farber Cancer Institute, Medical Director of the Cell Manipulation Core Facility (CMCF), and Technical Director of DFCI's Immune Effector Cell Program. Dr. Nikiforow earned her MD and a PhD in Immunobiology at Yale University working on the differential roles of CD4 and CD8 T cells in immune control over Epstein-Barr virus-induced B-cell transformation. Dr. Nikiforow has pursued a translational research career focusing on immune reconstitution after stem cell transplant and therapeutic use of adoptive cellular products. Through the CMCF and as Principal Investigator of Phase I and II clinical trials, she is working to bring cellular therapies such as chimeric antigen-receptor T cells, genetically-modified stem cells, and regulatory T-cell infusions into the clinic at Dana-Farber. Her work with the International Society of Cellular Therapy, the Foundation for the Accreditation of Cellular Therapy and the Center for International Blood & Marrow Transplant Research have promoted education and set standards for safe implementation of new approaches within the broader and ever-growing cellular therapy field.

**Martin Pule, M.D.**

University College London

Martin Pule is a senior lecturer in Haematology at University College London. He has been working on engineered T cells since 2001. He is director of the CAR T cell programme at UCL. He is also founder and Chief Scientific Officer of Autolus Ltd. Martin Pule's interests range from CAR and module design all the way through to vector and CAR T manufacture. He has a particular interest in CAR T cell therapy of B and T cell malignancies.

**Isabelle Rivière, Ph.D.**

Memorial Sloan-Kettering Cancer Center

Isabelle Rivière received her Ph.D. in Cellular and Molecular Biology from the University of Paris. She initiated her graduate studies at the Institut Curie in Paris and completed her thesis at the Whitehead Institute in Cambridge, MA. She developed retroviral vectors for in vivo long-term expression of transgenes in hematopoietic cells, which are widely used in clinical studies worldwide. After completing her postdoctoral work at NYU, she joined the faculty of Memorial Sloan-Kettering in 1999 where she focuses on developing novel strategies for cell therapies and immunotherapies. Her laboratory investigates genetic approaches to enhance various cell types including T lymphocytes and stem cells for the treatment of cancer and genetic blood disorders. Over the past 20 years, she has conceived and implemented multiple cell manufacturing processes for several Phase I/II clinical trials. Her lab currently supports multiple CAR T cell-based clinical trials for the treatment of hematological malignancies and solid tumors. She also investigates immunological functions of CAR T cells in clinical trials and animal models. She has served as a member of the Board of Directors of the American Society of Gene and Cell Therapy (ASGCT) and of the Alliance for Regenerative Medicine (ARM). She currently serves on the Advisory Board of the Center for Commercialization of Cancer Immunotherapy C3i (Canada) and the National Science Foundation (NSF) Engineering Research Center (ERC) for Cell Manufacturing Technologies



**Bianca Santomasso, M.D., Ph.D.**

Memorial Sloan-Kettering Cancer Center

Bianca Santomasso M.D., Ph.D., is an Assistant Attending Neuro-Oncologist and scientist at Memorial Sloan Kettering Cancer Center (MSK) with expertise in the diagnosis and management of neurologic complications of immunotherapies. She led the development of MSK protocols for neurologic monitoring of CAR T-cell therapies and participated in the development of the ASTCT consensus grading system for CRS and ICANS. She is a member of the ASCO, NCCN, and SITC expert clinical guidelines panels on management of immune-related adverse events and immune effector cell toxicities. Her research focuses on understanding acute and chronic neurotoxicity arising from CD19 CAR T-cell and immune checkpoint blockade treatments.

**Andrei Thomas-Tikhonenko, Ph.D.**

Children's Hospital of Philadelphia

My laboratory has a long-standing interest in pathobiology of solid and hematopoietic malignancies, in particular lymphomas and leukemias and other cancers driven by MYC overexpression. I trained in the MYC field in the early '90s as post-doctoral Research Associate and then Leukemia Society Special Fellow at the renowned Fred Hutchinson Cancer Research Center in Seattle. In 1997, I was recruited to the University of Pennsylvania, where I rose through the ranks to become tenured Professor of Pathology & Laboratory Medicine. For almost 25 years, I have been continuously funded by NCI. In addition, I have received grants from numerous private foundations including American Cancer Society, Swiss Cancer League, Leukemia & Lymphoma Society, Alex's Lemonade Stand Foundation, WW Smith Charitable Trust, The V Foundation, William Lawrence & Blanche Hughes Foundation, and St Baldrick's Foundation. Twelve years ago, I moved my lab across campus to The Children's Hospital of Philadelphia, where it became an integral part of the Center for Childhood Cancer Research. This integration allowed me to foster new collaborations with key physician-scientists and pursue several translational projects. Since 2013, I have been an investigator of the multi-institutional Stand Up to Cancer-St. Baldrick's Pediatric Cancer Dream Team and since 2018 - the Cancer Moonshot-funded Pediatric Immunotherapy Discovery and Development Network.

**Diane Tseng, M.D., Ph.D.**

University of Washington

Diane Tseng M.D. Ph.D., is a thoracic oncologist at the University of Washington with expertise in T cell immunology and immunotherapy. She completed internal medicine residency at Massachusetts General Hospital and medical oncology fellowship at Stanford University. During her postdoctoral training in the laboratories of Dr. Crystal Mackall and Dr. Mark Davis at Stanford, she developed an approach for identifying disease-relevant T cells and their antigens in human lung cancer. Her current research focuses on studying T cells in lung cancer and the relationship between anti-tumor and anti-viral immunity.



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### Join our symposium: *Latest Advances in Cell and Gene Therapy*

Wednesday, May 12, from 2:00 p.m. to 3:30 p.m. EDT

Welcome three key opinion leaders as they share their latest updates from the ever-evolving field of cell and gene therapy.

**Matthew Porteus, MD, PhD**, Stanford University School of Medicine  
Manufacturing genome edited hematopoietic stem cells: From now to the future

**Kenneth Chrobak, PhD**, Wugen  
Development of WU-NK-101, an Off-the-Shelf Memory NK Cell Therapy for the Treatment of AML

**Ruipeng Wang, PhD**, NexImmune, Inc.  
*In vivo* and *in vitro* characterization of MART-1 specific T cells generated using the AIM technology and Prodigy<sup>®</sup> system

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**F.D. Bushman**

Pseida; Honorarium; Speaker/consultant

Novartis; Royalties; Licensed patent

**L. Butterfield**

StemImmune/Calidi; Scientific and medical advisory board; Advisory board

Western Oncolytics; Scientific advisory board

Khloris; Scientific advisory board

Pyxis; Scientific advisory board

Cytomix; Scientific advisory board

RAPT; Scientific advisory board

EnaraBio; Scientific advisor

**T. Fry**

Sana Biotechnology; Salary; Employment

Immunotherapy patents owned by the NIH; Royalties; Inventor

**R.G. Majzner**

Syncopation Life Sciences; Equity, consulting fees; Co-founder

Lyell Immunopharma; Equity, consulting fees; Consultant

Zai Labs; Consulting fees; Consultant

Aptorum Group; Equity; Consultant

Illumina Radiopharmaceuticals; Equity, consulting fees; Consultant

Gamma Delta Therapeutics; Consulting fees; Consultant

**S. Nikiforow**

Kite/Gilead; Honoraria; Advisory board

Iovance; Honoraria; Advisory board

**M.A. Pule**

Autolus Ltd.; Salary contribution; Employment

Research funding; Research principle investigator

**I. Rivière**

Fate Therapeutics; Consulting

FloDesign Sonics; Honorarium, equity interests; Advisory board

Juno Therapeutics/BMS; Intellectual property rights; Spouse

Centre for Commercialization of Cancer Immunotherapy; Honorarium;  
Advisory board

Akron; Honorarium; Advisory board

Mnemo Therapeutics; Intellectual property rights, honorarium; Consulting

**B. Santomasso**

Janssen; Consulting fee; Consulting/advisory board

Celgene/BMS; Consulting fee; Consulting

In8bio; Consulting fee; Advisory board