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CAR T AND IMMUNE EFFECTOR CELL THERAPIES WORKSHOP SPONSORS

The American Society of Gene & Cell Therapy is honored to acknowledge the following organizations for their support of the CAR T and Immune Effector Cell Therapies Workshop:

[Logos of sponsors]
CAR T and Immune Effector Cell Therapies Workshop

PROGRAM
All times are listed in Eastern Daylight Time (EDT UTC -4).

8:00 AM – 12:00 PM
ROOM: BALLROOM B
CO-CHAIRS: Aude Chapuis, MD and Marco Davila, MD, PhD

Applications of CAR T-Cells as a Standard of Care

8:00 AM - 8:20 AM
Clinical Characteristics That Associate With Outcomes in Patients Treated With CD19 CAR T-Cell Therapy for Lymphoma
Michael D. Jain, MD, PhD

8:20 AM - 8:40 AM
CAR T-Cell Associated Toxicities: Mechanisms, Diagnosis, Grading, and Management
Matthew J. Frigault, MD

8:40 AM - 9:00 AM
Adoptive Cellular Therapy for Multiple Myeloma: BCMA CAR T-Cells and Beyond
Eric Smith, MD, PhD

9:00 AM – 9:20 AM
CAR T-Cells Enter the Real World for B-ALL: Early Results and Remaining Questions
Theodore Laetsch, MD

TCRs for Solid Tumors

9:20 AM - 9:40 AM
Advancing CAR T-Cells for Solid Tumors
Saul Priceman, PhD

9:40 AM – 10:00 AM
TCR-Based Cancer Immunotherapies to Novel pMHC Targets
Amir Alpert, PhD

10:00 AM – 10:20 AM
Break

Off the Shelf Advances

10:20 AM - 10:40 AM
New Tools for Non-viral CRISPR Gene Editing in Primary Human Immune Cells
David N. Nguyen, MD, PhD

10:40 AM – 11:00 AM
NK Cell Therapy: Individualized Products to Off-The-Shelf Strategies
Jeffrey Miller, MD
PROGRAM, CONTINUED

Challenges in T-Cell Therapy

11:00 AM - 11:20 AM

Can We Target AML With Gene-Engineered Cell Therapies?
Lihua E. Budde, MD, PhD

11:20 AM - 11:40 AM

Understanding Resistance to Develop Better CAR T Immunotherapies
Marco Ruella, MD

11:40 AM - 12:00 PM

Human CD83-targeted CAR T-Cells to Prevent GVHD and Eliminate AML
Brian C. Betts, MD
CO-CHAIRS

Aude G. Chapuis, MD  
Fred Hutchinson Cancer Research Center  
Seattle, WA

Marco L. Davila, MD, PhD  
H. Lee Moffitt Cancer Center and Research Institute  
Tampa, FL

Amir Alpert, PhD  
Immatics, Inc.  
Houston, TX

SPEAKERS

Brian C Betts, MD  
University of Minnesota  
Minneapolis, MN

Lihua E. Budde, MD, PhD  
City of Hope  
Duarte, CA

Matthew J Frigault, MD  
Massachusetts General Hospital  
Boston, MA

Michael D. Jain, MD, PhD  
Moffitt Cancer Center  
Tampa, FL

Theodore Laetsch, MD  
UT Southwestern  
Dallas, TX

Jeffrey Miller, MD  
University of Minnesota  
Minneapolis, MN

David N Nguyen, PhD, MD  
University of California, San Francisco  
San Francisco, CA

Saul Priceman, PhD  
City of Hope  
Duarte, CA

Marco Ruella, MD  
University of Pennsylvania  
Philadelphia, PA

Eric Smith, MD, PhD  
Memorial Sloan-Kettering Cancer Center  
New York, NY
Amir Alpert, PhD

Amir Alpert PhD is passionate about bridging the great divide separating bench side experiments and clinical responses in solid tumor adoptive cell therapy. Currently serving as the Associate Director of Immunology at Immatics US Inc, he is performing the molecular immunomonitoring for the multi-targeting T-Cell ACTolog® trial, where he aims to shed light on T-cell biopsy infiltration. In early stage drug development, he is passionate about evolving current immunological assays in effort to extract clinically relevant observations.

Brian Betts, MD

Allogeneic hematopoietic cell transplantation (allo-HCT) can cure high-risk leukemia, lymphoma, and marrow failure syndromes. However, graft-versus-host disease (GVHD) remains a life-threatening side effect of this procedure. The Betts lab is interested in developing selective pharmacologic and cellular immunotherapy strategies to separate GVHD from beneficial graft-versus-leukemia (GVL) effects of the allograft. This includes small molecules targeting T-cell costimulation and cytokine activation, dendritic cell ER stress response, and various pathways directing donor T-cell differentiation. The lab also uses xenotransplantation models to test engineered human immune cells for GVHD and solid organ rejection prophylaxis. Importantly, the lab is focused on translation and our work has directly led to innovative GVHD prevention trials. Lab PI, Brian C. Betts MD, was chief resident at the University of Minnesota (internal medicine) and chief fellow at Memorial Sloan Kettering Cancer Center (medical oncology and hematology). Dr. Betts is an Associate Professor of Medicine at the University of Minnesota, where he values making discoveries in the lab and caring for patients with hematologic malignancies in need of transplantation.

Lihua E. Budde, MD, PhD

Dr. Budde is an assistant professor in the Department of Hematology & Hematopoietic Cell Transplantation at City of Hope. Dr. Budde’s academic and clinical interests include investigating and designing novel means of immunotherapy for patients with lymphoma and leukemia, and she is part of the T Cell Therapeutics Research Laboratory, investigating the use of CAR T cells to treat blood cancers and other diseases. Board certified in medical oncology and internal medicine, Dr. Budde has received many honors and awards for her work. She was chosen as a Special Fellow in Clinical Research awardee by the Leukemia & Lymphoma Society, a recipient of the Edson Immunotherapy Pilot Award from the J. Orin Edson Foundation and an awardee of the Stop Cancer Foundation seed grant in 2016. She is also a recipient of the prestigious Damon Runyon Cancer Research Foundation Clinical Investigator Award (2014-2017). She has served as the principal investigator on several innovative translational research protocols and has published her work in many peer-reviewed journals, and given presentations at national and international meetings.

Matthew Frigault, MD

Dr. Frigault is a medical oncologist in the Hematologic Malignancy Program at the Massachusetts General Hospital Cancer Center, as well as Assistant Director of the Cellular Immunotherapy Program. In addition, he serves as an instructor at Harvard Medical School. Dr. Frigault completed his oncology fellowship at the combined MGH/Dana Farber Cancer Institute training program where he worked with Dr. Marcela Maus, head of the Cellular Immunotherapy Program at MGH. Dr. Frigault received his Bachelor of Arts degree in Biology and Biological Sciences from the College of the Holy Cross, and his master’s and MD degrees from the University of Pennsylvania. His research experience includes pre-clinical development and correlative studies relevant to T-cell immunotherapy in the lab of Dr. Carl June while in graduate school at Penn. During his postgraduate training at Johns Hopkins, he focused on cellular therapies utilizing marrow infiltrating lymphocytes and chimeric switch receptors in the lab of Dr. Ivan Borelo. Dr. Frigault’s current clinical activities include attending on the inpatient bone marrow transplant, managing cellular therapy patients and overseeing the cellular therapy service. His current research is focused on the translational aspects of cellular therapy with the goal of developing the next generation of cellular therapies through phase I clinical trials.
Dr. Michael Jain is an Assistant Member in the Dept. of Blood and Marrow Transplant and Cellular Immunotherapy at Moffitt Cancer Center in Tampa, Florida. His clinical and research interests involve the treatment of lymphoma with chimeric antigen receptor T-cell therapy.

Theodore Laetsch, MD
Theodore W. Laetsch, MD is an Associate Professor in the Department of Pediatrics, Member of the Harold C Simmons Comprehensive Cancer Center, Norma and Jim Smith Professor of Clinical Excellence, and Eugene P. Frenkel, MD Scholar in Clinical Medicine at the University of Texas Southwestern Medical Center in Dallas, TX. He leads the Experimental and Cellular Therapeutics Program (ECTP) in the Gill Center for Cancer and Blood Disorders at Children’s Health and serves as the Rare Tumors Disease Committee Chair for the Children’s Oncology Group. Dr. Laetsch received a BS in Agricultural and Biosystems Engineering from the University of Arizona and then a MD from the University of California, San Francisco. Dr. Laetsch completed his residency at the University of Colorado/Children’s Hospital Colorado, where he also served as chief resident. He completed his fellowship training and an instructorship at the Children’s Hospital of Philadelphia (CHOP) and joined the faculty at UT Southwestern in 2013. Dr. Laetsch conducts both early phase clinical and translational laboratory-based research testing new treatments for relapsed/refractory pediatric cancers with a goal of “bridging the gap” between laboratory research and early phase clinical trials. As the leader of the CAR T-cell program at UT Southwestern/Children’s, Dr. Laetsch has treated a large number of children with B-ALL with CAR T-cells and is actively involved in studying the outcomes of this group of patients, including the impact of therapy on quality of life and the safety and efficacy of CAR T-cells in the real world setting.

Jeffrey S. Miller, MD, is currently a Professor of Medicine at the University of Minnesota, Deputy Director of the Masonic Cancer Center, and is in the Division of Hematology, Oncology and Transplantation. Dr. Miller was the recipient of the National Cancer Institute Outstanding Investigator Award for 2015 and has more than 20 years of experience studying the biology of NK cells and other immune effector cells and their use in clinical immunotherapy with over 200 peer-reviewed publications. He is a member of numerous societies such as the American Society of Hematology, the American Association of Immunologists, and a member of the American Society of Clinical Investigation since 1999. He serves on the editorial board for Blood and is a reviewer for a number of journals and NIH grants.

David Nguyen MD PhD completed his PhD in Materials Science and Medical Engineering at MIT in the HST program, where he studied nanoparticle delivery of DNA and RNA to the immune system in the laboratory of Prof. Robert Langer with applications in DNA vaccines and immunostimulatory RNA vaccine adjuvants. His early work was published in PNAS, Molecular Therapy, Advanced Materials, Nature Materials, and Nature Biotechnology. He then went to Stanford University for medical school, and since 2012 has been at the UCSF Medical Center where he completed medical residency and a clinical fellowship in Infectious Diseases. He currently attends on the Infectious Disease service at UCSF and is a research fellow of the UCSF CIRM Alpha Stem Cell Clinic. Dr. Nguyen has clinical interests in treating infections in patients with compromised immune systems, and research interests in therapeutic gene editing to correct mutations that drive primary immunodeficiency (PID). He is conducting research in the laboratory of Prof. Alex Marson, who studies the use of CRISPR genome editing in primary human cells for both functional genetics and therapeutic utility in genome engineering and gene therapy. Dr. Nguyen has leveraged his background in nucleic acid delivery to create new tools for CRISPR Cas gene editing achieving some of the highest non-viral gene editing in primary human immune cells, work that has recently been published in Nature Biotechnology and upcoming in Nature Communications. He is applying these tools to discover and model mutations associated with PIDs with an eventual goal of developing novel non-viral gene therapy strategies in human HSCs and T-cells for treatment of PID.
Dr. Saul Priceman is a trained tumor immunologist with expertise in T-cell biology and cancer immunotherapy. Dr. Priceman received his PhD in 2010 from the University of California, Los Angeles, and subsequently moved to the City of Hope (COH) for his postdoctoral training. In 2018, he became Assistant Professor in the Department of Hematology and Hematopoietic Cell Transplantation, and Associate Director of Translational Sciences in the T Cell Therapeutics Program. Dr. Priceman’s research interests are centered on cellular immunotherapy, primarily utilizing chimeric antigen receptor (CAR) T-cell therapy. The Priceman lab focuses on translational research relevant to developing innovative cellular immunotherapy for the treatment of solid tumors including prostate cancer, ovarian cancer, and breast cancer. Dr. Priceman is the recipient of several prestigious awards, including a National Comprehensive Cancer Network (NCCN) Young Investigator award, a Prostate Cancer Foundation (PCF) Young Investigator award, and he is a principal investigator on several grants, including a PCF Challenge award, a Department of Defense (DOD) Idea Development award, and two recent California Institute for Regenerative Medicine (CIRM) awards. Dr. Priceman has authored or co-authored over 30 publications in the field of cancer immunotherapy. Work from the Priceman lab and his colleagues has also resulted in more than 6 patents (awarded or pending).

Marco Ruella, MD, is Assistant Professor of Medicine in the Division of Hematology/Oncology and the Center for Cellular Immunotherapies and Scientific Director of the Lymphoma Program at the Hospital of the University of Pennsylvania. His research focuses on the study of the mechanisms of relapse after chimeric antigen receptor T-cell (CART) immunotherapies with the goal of rationally designing combined innovative immunotherapies for relapsing/refractory leukemia and lymphoma. He is the author of numerous peer-reviewed publications on targeted immunotherapies for hematological cancers and is an inventor in several patents on CAR T therapy. His work has been recognized through numerous awards including the inaugural SITC EMD-Serono Cancer Immunotherapy Clinical Fellowship (2014), the AACR-BMS Oncology Fellowship in Clinical Cancer Research (2015), the ASH Scholar Award (2016), a NIH K99-R00 award (2017), the “Paola Campese” Award Leukemia Research (2017), the Cancer Support Community Award (2018), and most recently the 2018 American Society of Hematology Joanne Levy, MD, Memorial Award for Outstanding Achievement. Dr. Ruella obtained his medical degree with high honors and completed his specialization in clinical hematology at the University of Torino, Italy. After completing his fellowship, he was an attending physician in the Hematology and Cell Therapy Division of the Mauriziano Hospital and an Instructor at the Biotechnology School at the University of Torino. From 2012, he was a postdoctoral fellow, and then an instructor at the University of Pennsylvania in the Center for Cellular Immunotherapies where he worked with Drs. June and Gill until appointment to his current position in 2018.

Eric Smith, MD, PhD is the Director of Translational Science for the Cellular Therapeutics Center, an Assistant Member of the Center for Cellular Engineering, and an Assistant Attending Physician on the Myeloma Service at Memorial Sloan Kettering Cancer Center. His lab work attempts to advance the field of CAR T-cell therapy using multiple myeloma as model. Several CAR T-cell vectors stemming from his pre-clinical research are under clinical investigation.
# DISCLOSURE OF RELEVANT FINANCIAL RELATIONSHIPS

<table>
<thead>
<tr>
<th>Name</th>
<th>Disclosure</th>
</tr>
</thead>
<tbody>
<tr>
<td>Amir Alpert, PhD</td>
<td>Immatics US Inc., Salary, Employee</td>
</tr>
<tr>
<td>Brian C Betts, MD</td>
<td>Patent, CD83 CAR T, N/A, co-inventor; CTI BioPharma, Pacitinib for GVHD prevention trial, R01 PI</td>
</tr>
<tr>
<td>Matthew J Frigault, MD</td>
<td>Novartis, Consulting Fees/Honorarium, Consultant; Kite/Gilead, Honorarium, Internal Speaker; Juno/BMW, Consulting Fees, Consultant; Arcelix, Consulting Fees, Consultant</td>
</tr>
<tr>
<td>Michael D. Jain, MD, PhD</td>
<td>Kite/Gilead, Consulting fee, Advisory; Novartis, Consulting Fee, Advisory</td>
</tr>
<tr>
<td>Theodore Laetsch, MD</td>
<td>Loxo Oncology, Consulting; Eli Lilly, Consulting; Bayer, Consulting; Novartis, Consulting; Novartis, Research Funding; Pfizer, Research Funding; Bayer, Research Funding</td>
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<td>Saul Priceman, PhD</td>
<td>Mustang Therapeutics</td>
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<td>Marco Ruella, MD</td>
<td>AbClon, Advisory Board, Consultant; NanoString, Honorarium, Consultant; Novartis, Research Funding, Researcher</td>
</tr>
<tr>
<td>Eric Smith, MD, PhD</td>
<td>BMS, Patent/royalties, Inventor; BMS, Research Funding, Investigator; BMS, Honorarium, Advisory Board; Precision Biosciences, Honorarium, Advisory Board, Fate Therapeutics, Honorarium, Advisory Board</td>
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