CAR T Cell Therapies Workshop

May 15, 2018
Hilton Chicago
Continental B
Chicago
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CAR T Cell Therapies Workshop Supporters

The American Society of Gene & Cell Therapy is honored to acknowledge bluebird bio for their support of the CAR T Cell Therapies Workshop:

This activity was supported by an educational grant from Celgene Corporation

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Committee Listing

Co-Chairs
Helen Heslop, MD
Baylor College of Medicine
Houston, TX

Krishna Komanduri, MD
University of Miami Miller School of Medicine
Miami, FL

Members
Sergio A. Giralt, MD, FACP
Memorial Sloan-Kettering Cancer Center
New York, NY

Elizabeth J. Shpall, MD
University of Texas MD Anderson Cancer Center
Houston, TX

Sarah Nikiforow, MD, PhD
Dana-Farber Cancer Institute
Boston, MA

Kara Wacker, MBA
Foundation for the Accreditation of Cellular Therapy
Omaha, NE

Faculty Listing

Stephanie Farnia, MPH
American Society for Blood and Marrow Transplantation
Madison, WI

Sergio A. Giralt, MD, FACP
Memorial Sloan-Kettering Cancer Center
New York, NY

Helen Heslop, MD
Baylor College of Medicine
Houston, TX

Samer Khaled, MD
City of Hope
Duarte, CA

Krishna Komanduri, MD
University of Miami Miller School of Medicine
Miami, FL

C. Fred LeMaistre, MD
Sarah Cannon Market Operations and Physician-in-Chief of Blood Cancers

Elizabeth J. Shpall, MD
University of Texas MD Anderson Cancer Center
Houston, TX

Phyllis I. Warkentin, MD
University of Nebraska Medical Center
Omaha, NE

Frederick L. Locke, MD
Moffitt Cancer Center
Tampa, FL

Sarah Nikiforow, MD, PhD
Dana-Farber Cancer Institute
Boston, MA
Faculty Bios

Stephanie Farnia, MPH
Stephanie Farnia has served as director, health policy and strategic relations for the American Society for Blood and Marrow Transplantation since December 2016. Farnia leads initiatives focused on improving access to hematopoietic cell transplant, CAR-T and other cellular therapies through legislative, regulatory, and stakeholder activities, including collaborative partnerships between providers and national payers. Previously, Farnia served in roles as the director for payer policy at the National Marrow Donor Program/Be The Match and director, Blue Distinction development at the Blue Cross Blue Shield Association. Farnia holds a master’s degree in public health policy and administration from the University of Minnesota, where she was a Judd fellow.

Sergio A. Giralt, MD, FACP
Sergio A. Giralt is the chief of the adult bone marrow transplant service in the division of hematologic oncology at Memorial Sloan Kettering Cancer Center in New York. He is affiliated with Weill Cornell Medical College as a professor of medicine. He received his medical degree from Universidad Central de Venezuela in Caracas, Venezuela, and completed his postgraduate internship at the University Hospital of Caracas. He also completed an internal medicine residency at Good Samaritan Hospital in Cincinnati, Ohio and a postdoctoral fellowship in hematology and oncology at The University of Texas MD Anderson Cancer Center. Board certified in internal medicine and hematology, Giralt holds membership in several professional societies, including the American Society of Hematology, the American Society of Clinical Oncology, the North American Society of Blood and Bone Marrow Transplantation, and the International Society of Haematology. He holds key positions with several organizations, including the International Bone Marrow Transplant Registry Executive Committee, the Blood and Marrow Transplant Clinical Trials Network (BMTCTN) Steering Committee, the National Marrow Donor Program Board of Directors, and the Clinical Advisory Board of the Web site Managing Myeloma. He is the past President of the American Society for Blood and Marrow Transplantation as well as the past chair of the BMT-CTN and of the Center for International Blood and Marrow Transplant Research. Giralt’s clinical research career has focused in three areas: 1) Developing better-tolerated conditioning regimens for older or medically infirmed patients with hematological malignancies to allow them access to this procedure; 2) Developing novel HCT therapies (conditioning regimens plus post-transplant therapies) for autologous and allogeneic HCT for myeloma and 3) Pursuing strategies that will significantly reduce HCT symptom burden and toxicities. As chief of the adult BMT service, he has extensive experience designing, implementing, and performing HCT studies both as a principal investigator and as a collaborator. Giralt has published over 400 articles and abstracts in the peer-reviewed literature and written chapters for several books. Additionally, Giralt is a reviewer and editorial board member for several journals.

Helen Heslop, MD
Helen Heslop is professor of medicine and pediatrics at Baylor College of Medicine, and the director of the Center for Cell and Gene Therapy at Baylor College of Medicine, Houston Methodist Hospital and Texas Children’s Hospital. She is also associate director for clinical research at the Dan L. Duncan Cancer Center. Heslop is a physician scientist engaged in translational research focusing on adoptive immunotherapy with gene-modified effector cells to improve hematopoietic stem cell transplantation and cancer therapy. She has extensive experience in developing and conducting transplant studies and cell and gene therapy studies and currently holds over 20 INDs. She was a Doris Duke Distinguished Clinical Research Scientist and serves as principal investigator on several peer-reviewed research programs, including an NCI-funded program project grant (Enhancing T-Cell Therapy of Cancer), a Leukemia and Lymphoma Society Specialized Center of Research (SCOR) award (Immunotherapy of Lymphoma) and, a SPORE in lymphoma from the NCI. She is current president of the American Society of Gene and Cell Therapy and a past president of the American Society of Blood and Marrow Transplantation (ASBMT) and the Foundation for Accreditation of Cellular Therapy (FACT).

Samer Khaled, MD
Samer Khaled completed his residency and hematology/oncology fellowship training at The Brooklyn Hospital in New York, then moved to California where he completed an additional fellowship in hematopoietic stem cell transplantation at City of Hope. In 2010, Khaled joined the faculty at City of Hope National Medical Center as an assistant clinical professor. Khaled’s research focuses on acute leukemia and MDS, and he also has a particular interest in immunotherapy in leukemia. Khaled is a member of the Immunotherapy Center within the Hematological Malignancies and Stem Cell Transplantation Institute and is the principal investigator of the City of Hope CAR-T cell trial for ALL. He is chairing the CART cell patient care Integrated Steering Committee (ISC) and co-chairing the clinical immunotherapeutic cellular committee, both at City of hope.

Krishna Komanduri, MD
Krishna Komanduri holds the Kalish Family Chair in Stem Cell Transplantation and is professor of medicine, microbiology and immunology and is the director of the adult stem cell transplant program and associate director for clinical innovation at the Sylvester Comprehensive Cancer Center at the University Of Miami Miller School Of Medicine.

Dr. Komanduri received his undergraduate education at MIT (1987), his MD at the University of Minnesota Medical School (1991) and trained at UCLA (in internal medicine) and UCSF (in hematology/oncology). Prior to moving to Miami in 2008, he was a faculty member at UCSF and at the University of Texas MD Anderson Cancer Center. His laboratory research is focused on studies of cancer immunology and has been widely published and supported by the NIH and cancer-related foundations.

Komanduri is the immediate past-president of the American Society for Blood and Marrow Transplantation (ASBMT). He also serves as co-chair of the CIBMTR working committee on infections
Faculty Bios – continued

and immune reconstitution and as a member of the board of directors of the National Marrow Donor Program (BeTheMatch) and as a member of the MIT NEWDIGS “think and do” tank on financing of novel curative therapies in oncology. He has also served as chair of the immunology and host defense scientific committee for the American Society of Hematology. He has been the recipient of awards including election to the American Society for Clinical Investigation (in 2009).

C. Fred LeMaistre, MD
Senior Vice President, Sarah Cannon Market Operations and Physician-in-Chief of Blood Cancers

C. Fred LeMaistre is the senior vice president, Sarah Cannon market operations and physician-in-chief of blood cancers at Sarah Cannon. He joined Sarah Cannon in 2012 and is responsible for the clinical operations of the Sarah Cannon markets and leads the development of the Sarah Cannon Blood Cancer Network which provides a multidisciplinary approach to standardize blood cancer therapies including blood and marrow transplantation (BMT). Previously the medical director for Texas Institute of Medicine and Surgery, LeMaistre is board certified in internal medicine and medical oncology, and has published more than 200 manuscripts.

He is a founding member and former president of the Foundation for the Accreditation of Cellular Therapy (FACT) and he established the first community-based BMT program in 1993. LeMaistre previously served as the president of the American Society for Blood and Bone Marrow Transplant and is currently a member of the American Society of Hematology. He received his medical degree from Southwestern Medical School in Texas and performed a fellowship in medical oncology at the University of Texas Health Science Center with training in BMT at Fred Hutchinson Cancer Center in Seattle, Wash.

Frederick L. Locke, MD
Fred Locke is the vice chair and an associate member of the department of blood and marrow transplant and cellular immunotherapy, and co-leader of the Moffitt Cancer Center Immunology Research Program. Locke is an oncologist and translational investigator dedicated to the development of cellular therapies for lymphoma and multiple myeloma. He is a nationwide lead investigator for several multi-center studies treating aggressive lymphoma patients with anti-CD19 CART cells. Results from the ZUMA-1 trial were recently reported in the New England Journal of Medicine and supported FDA approval of Yescarta, the first FDA approved gene modified cellular therapy to treat lymphoma. Locke is the research director and clinical director for the Moffitt Immune Cell Therapy program and also serves as chair of both the Moffitt Cellular Therapy Advisory Committee and the Moffitt Immunotherapy Working Group.

Sarah Nikiforow, MD, PhD
Sarah Nikiforow is currently a clinical instructor with the Dana-Farber Cancer Institute Stem Cell Transplant Program, assistant medical director of the Cell Manipulation Core Facility (CMCF), and technical director of DFCI’s Immune Effector Cell Program. 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.

Nikiforow has embarked on 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 (FACT) and the Center for International Blood & Marrow Transplant Research aims to promote education and safe implementation within the broader and ever-growing cellular therapy field.

Elizabeth J. Shpall, MD
Elizabeth J. Shpall is the director of the cell therapy laboratory and the cord blood bank and deputy chair of the department of stem cell transplantation and cellular therapy at the University of Texas MD Anderson Cancer Center. Shpall has more than 30 years of experience performing stem cell transplants and translating stem cell graft manipulations from the laboratory to the clinic. She has had R01 funding from the National Cancer Institute for the past 21 years, lead a P01 on improving cord blood transplantation, projects focusing on the ex vivo expansion of cord blood in mesenchymal stem cell co-cultures, and enhancing the homing of cord blood to the bone marrow or sites of inflammation using exofucosylation of the relevant progenitors. She has received Cancer Prevention and Research in Texas (CPRIT) funding for cord expansion and homing and for the development of cord blood-derived Natural Killer (NK) cells targeting hematologic cancers. Shpall was the founding president of the Foundation for Accreditation of Cellular Therapy (FACT) and recently reappointed to the FACT Board, she was a past president of the American Society of Blood and Marrow Transplantation (ASBMT) and a past vice-president of Netcord. Shpall has served as a reviewer and a former member of the NCI Committee, reviewing clinical and translational program project grants.

Phyllis I. Warkentin, MD
Phyllis I. Warkentin is the chief medical officer of the Foundation for the Accreditation of Cellular Therapy (FACT), professor of pathology/microbiology and pediatrics at the University of Nebraska Medical Center College of Medicine, and medical director of the UNMC Biologics Production Facility. Warkentin is board certified in pediatric hematology/oncology and transfusion medicine. As a founding member of the FACT Board of Directors, Dr. Warkentin established the standards-setting and accrediting arm of FACT’s parent organizations: The American Society for Blood and Marrow Transplantation (ASBMT) and the International Society for Cellular Therapy (ISCT). She is a recipient of the ASBMT Public Service Award and serves on international committees for numerous cellular therapy professional societies and organizations.
## Disclosure of Relevant Financial Relationships

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<thead>
<tr>
<th>Name</th>
<th>Disclosure</th>
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</thead>
<tbody>
<tr>
<td>Sergio A. Giralt, MD, FACP</td>
<td>Honorarium, Novartis; Honorarium, Kite; Honorarium, Amgen</td>
</tr>
<tr>
<td>Helen Heslop, MD</td>
<td>Consulting Fee, Novartis; Research Support and Royalty, Cell Medica; Ownership Equity, Viracyte; Ownership Equity, Marker Therapeutics; Research Support, Tessa Therapeutics</td>
</tr>
<tr>
<td>Frederick L. Locke, MD</td>
<td>Consulting Fee, Cellular Biomedicine Group Inc.</td>
</tr>
<tr>
<td>Sarah Nikiforow, MD, PhD</td>
<td>Honoraria, Kite Pharma</td>
</tr>
<tr>
<td>Elizabeth J. Shpall, MD</td>
<td>Consulting Fee, Novartis</td>
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Program

1 PM – 1:10 PM

Welcome and Introductory Remarks
SPEAKERS: Helen Heslop, MD
           Krishna Komanduri, MD

1:10 PM – 2:20 PM

Setting up an Immune Effector Program
In this session there will be talks describing different models for setting up immune effector programs either as stand-alone programs or as programs integrated with a hemopoietic stem cell transplant program. The resources required for financial co-ordination, care co-ordination, clinical care and data management will be discussed in different models covering both adult and pediatric patients.
CHAIRS: Helen Heslop, MD and Sergio A. Giralt, MD, FACP
SPEAKERS: Helen Heslop, MD
           Sergio A. Giralt, MD, FACP
           EJ Shpall, MD
           Samer Khaled, MD

2:20 PM – 3 PM

Accreditation
In this session Drs. Nikiforow and Warkentin will describe the development of standards to promote quality practice in immune effector cell administration. They will also discuss the voluntary, peer-led FACT accreditation program.
CHAIR: Sarah Nikiforow, MD, PhD
SPEAKERS: Phyllis Warkentin, MD
           Sarah Nikiforow, MD, PhD

3 PM – 3:30 PM

Coffee Break

3:30 PM – 5 PM

Implementing Commercial Products
In this session we will discuss the challenges of implementing commercial adoptive immunotherapy including practical considerations and our current understanding of the financial systems and potential barriers faced by patients and centers.
CHAIR: Krishna Komanduri, MD
SPEAKERS: Stephanie Farnia, MPH
           Krishna Komanduri, MD
           C. Fred LeMaistre, MD
           Fred Locke, MD
Notes