

Memorial Sloan Kettering Cancer Center presents

Transplant and Cellular Therapy Options for Non-Malignant Hematologic Disorders

Complimentary Live Virtual Course
October 15-16, 2021



Memorial Sloan Kettering
Cancer Center



Transplant and Cellular Therapy Options for Non-Malignant Hematologic Disorders

Therapies available for the treatment of non-malignant hematologic disorders have significantly improved survival rates, but many are unaware of new and innovative options available to their patients. The goal of this interactive live symposium is to review and discuss cutting edge treatments for pediatric and adult patients. Relevant clinical cases and treatment modalities to be covered include:

- Stem cell transplant and/or gene therapy options for non-malignant disorders in pediatric and adult patients including:
 - Hemoglobinopathies (Sickle cell disease)
 - Congenital and acquired immunodeficiencies (Primary immunodeficiencies, SCID, HIV)
 - Inherited and acquired bone marrow failure disorders (Fanconi anemia, aplastic anemia)
- Disease specific expert panels addressing integration of care, innovative therapies and recent changes in transplant and cellular therapy options
- Optimized transplant preparative regimens including individualized conditioning, chemotherapy and radiation sparing conditioning regimens
- Integration of care with referring physicians/centers for complex care of non-malignant disorders

This virtual program will include both didactic and interactive sessions.

The target audience for this course includes physicians and allied health professionals interested in the care and treatment of patients with non-malignant hematologic disorders.

COMPLIMENTARY REGISTRATION:

mskcc.org/nonmalignanthemedisorders

Learning Objectives

- Recall new and cutting edge treatment options for patients
- Identify institutions and opportunities for collaboration that will improve patient care and access
- Determine differences in potential treatment options
- Recommend various interprofessional teams and interdisciplinary services for patient care support.

Accreditation

Memorial Sloan Kettering Cancer Center is accredited by the Accreditation Council for Continuing Medical Education to provide continuing medical education for physicians.

AMA Credit Designation Statement

Memorial Sloan Kettering Cancer Center designates this live activity for a maximum of **9.25 AMA PRA Category 1 Credits™**. Physicians should claim only the credit commensurate with the extent of their participation in the activity.

ABIM and ABP MOC Recognition Statement

Successful completion of this CME activity, which includes participation in the evaluation component, enables the participant to earn up to:

- **9.25 Medical Knowledge MOC points** in the American Board of Internal Medicine's (ABIM) Maintenance of Certification (MOC) program; and
- **9.25 MOC points** in the American Board of Pediatrics' (ABP) Maintenance of Certification (MOC) program.

It is the CME activity provider's responsibility to submit participant completion information to ACCME for the purpose of granting ABIM or ABP MOC credit.



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MSK Course Directors



Maria Cancio, MD
Pediatric Hematologic Oncologist



Rekha Parameswaran, MD
Hematologic Oncologist

MSK Course Planning Committee



Jaap Jan Boelens, MD
Chief, Pediatric Stem Cell Transplantation and
Cellular Therapies Service
Richard J. O'Reilly Chair in Honor of Kevin Zoltan Merszei



Christina Cho, MD
Medical Oncologist



Sergio Giralt, MD
Deputy Division Head, Division of Hematologic Malignancies
Melvin Berlin Family Chair in Multiple Myeloma



Jodi V. Mones, MD
Hematologist



Miguel-Angel Perales, MD
Chief, Adult Bone Marrow Transplant Service



Roni Tamari, MD
Hematologic Oncologist

Invited Course Faculty

Alice Bertaina, MD, PhD

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Stanford, CA

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Francesca Fumagalli, MD, PhD

Neurologist
San Raffaele Telethon Institute For Gene Therapy
San Raffaele Hospital
Milan, Italy

R. Brad Jones, PhD

Viral Immunologist; Associate Professor
Division of Infectious Disease
Weill Cornell Medicine

Neena Kapoor, MD

Director, Blood and Marrow Transplant Laboratory
Attending Physician; Hematology, Oncology
and Blood and Marrow Transplantation
Investigator, Hematology-Oncology
Professor of Clinical Pediatrics,
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Giovanna Lucchini, MD

Locum Consultant Immunologist in Immunology
and Stem Cell Transplantation
Great Ormond Street Hospital for Children
London, England

Margaret MacMillan, MD

Professor, Department of Pediatrics
Division Director, Division of Blood and Marrow
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Minneapolis, MN

Eric Mallack, MD, MBE

Assistant Professor of Pediatrics and Neurology
New York-Presbyterian
New York, NY

Ewelina Mamcarz, MD

Assistant Member, St. Jude Faculty
St. Jude Children's Research Hospital
Memphis, TN

Deepa Manwani, MD

Professor, Department of Pediatrics,
Albert Einstein College of Medicine
Director of Hematology, Department of Pediatrics
The Children's Hospital at Montefiore
Bronx, NY

Parinda Mehta, MD

Professor, UC Department of Pediatrics
Cincinnati Children's
Cincinnati, OH

Invited Course Faculty

Sung-Yun Pai, MD

Chief, Immune Deficiency Cellular Therapy Program
National Institutes of Health
Bethesda, MD

Matthew Porteus, MD, PhD

Sutardja Chuk Professor of Definitive and Curative Medicine
Stanford University
Stanford, CA

Charles Quinn, MD, MS

Medical Director, Pediatric Sickle Cell Program
Director, Erythrocyte Diagnostic Laboratory
Professor, UC Department of Pediatrics
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Gerald Raymond, MD

Professor of Genetic Medicine
Professor of Neurology
Johns Hopkins Hospital
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Sharon Savage, MD

DCEG Clinical Director
Branch Chief
Senior Investigator
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Elisa Seeger

Founder
Aidan Jack Seeger Foundation/ALD Alliance
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Akiko Shimamura, MD, PhD

Director, Bone Marrow Failure and Myelodysplastic Syndrome Programs
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Annemarie Wensing, MD, PhD

Clinical Virologist
University Medical Center Utrecht
Utrecht, Netherlands

MSK Course Faculty

Deborah Goldfrank, MD, FACOG

Head, General Gynecology

Julia Kearney, MD

Child Psychiatrist

Jennifer Kennedy, MS, CGC

Genetic Counselor

Ross Levine, MD

Laurence Joseph Dineen Chair in Leukemia Research
Chief, Molecular Cancer Medicine Service, HOPP

Alina Markova, MD

Assistant Attending of Dermatology

Ariela Noy, MD

Medical Oncologist

Richard O'Reilly, MD

Claire L. Tow Chair in Pediatric Oncology Research

Susan Prockop, MD

Pediatric Oncologist

Bhuvanesh Singh, MD, PhD, FACS

Director, Laboratory of Epithelial Cancer Biology
Co-Director, Multidisciplinary Skin Cancer Management Program

Mike Walsh, MD

Geneticist & Pediatric Oncologist

FACULTY DISCLOSURE | It is the policy of MSK to make every effort to insure balance, independence, objectivity, and scientific rigor in all continuing medical education activities which it provides as an ACCME accredited provider. In accordance with ACCME guidelines and standards, all faculty participating in an activity provided by MSK are expected to disclose any significant financial interest or other relationship with the manufacturer(s) of any commercial product(s) and/or provider(s) of commercial services which are discussed by the faculty members in an educational presentation. As required by the ACCME, when an unlabeled use of a commercial product or an investigational use not yet approved for any purpose is discussed during an educational activity, MSK requires the speaker to disclose that the product is not labeled for the use under discussion or that the product is still investigational.

Friday, October 15

All times are Eastern Standard Time

8:45 AM		Virtual Course Check-in
9:00 AM		Welcome and Opening Remarks Maria Cancio, MD
9:10 AM	LECTURE	Introduction to Leukodystrophy Eric Mallack, MD, MBE
9:25 AM	PANEL	ALD/MLD <div> <div> MODERATORS Jaap Jan Boelens, MD Eric Mallack, MD, MBE </div> <div> FACULTY Gerald Raymond, MD Elisa Seeger Melissa Wasserstein, MD </div> </div>
10:05 AM		Q&A with Faculty
10:15 AM	PANEL	Future Vision of Gene Therapy for Leukodystrophies <div> <div> MODERATORS Jaap Jan Boelens, MD Eric Mallack, MD, MBE </div> <div> FACULTY Florian Eichler, MD Francesca Fumagalli, MD, PhD Caroline Lindemans, MD, PhD </div> </div>
10:50 AM		Q&A with Faculty
11:00 AM		Break
11:15 AM	DEBATE	Immunodeficiencies: Gene Therapy or HSCT <div> <div> MODERATOR Susan Prockop, MD </div> <div> FACULTY Neena Kapoor, MD Giovanna Lucchini, MD Ewelina Mamcarz, MD Sung-Yun Pai, MD </div> </div>
12:35 PM		Q&A with Faculty
12:45 PM	SPECIAL SESSION	Honoring Dr. Nancy Kernan INTRODUCTION: Richard O'Reilly, MD
1:00 PM		Lunch Break
1:30 PM	BREAKOUT SESSIONS	<p>Ask the Professor: Individualized Conditioning Regimens*</p> <p>INTRODUCTION: Maria Cancio, MD</p> <div> <div> Antibody Based Conditioning MODERATOR Susan Prockop, MD FACULTY Agnieszka Czechowicz, MD, PhD </div> <div> How to Lower Toxicity of Conditioning Regimens MODERATOR Christina Cho, MD FACULTY Neena Kapoor, MD </div> </div> <p>*We invite attendees to turn on their cameras during the interactive breakout sessions.</p>
2:15 PM		Break
2:30 PM	PANEL	Patient Cell Therapies, Transplant for HIV+ Patients <div> <div> MODERATOR Ariela Noy, MD </div> <div> FACULTY Catherine Bollard, MD, MBChB R. Brad Jones, PhD Miguel-Angel Perales, MD Annemarie Wensing, MD, PhD </div> </div>
3:10 PM		Q&A with Panel
3:15 PM	LECTURE	Clonal Hematopoiesis Ross Levine, MD
3:30 PM		Closing Remarks Miguel-Angel Perales, MD
3:35 PM		Meet the Faculty (Optional) Attendees are invited to turn on their cameras and join the course faculty for an optional meet and greet
4:00 PM		Adjourn

Saturday October 16

All times are Eastern Standard Time

8:45 AM		Virtual Course Check-in
9:00 AM		Welcome Rekha Parameswaran, MD
9:05 AM	SPECIAL SESSION	Introduction and Interview with Agata Smogorzewska INTERVIEWER: Jennifer Kennedy, MS, CGC
9:25 AM	PANEL	Fanconi Anemia: MSK Multidisciplinary Care MODERATOR Julia Kearney, MD FACULTY Deborah Goldfrank, MD, FACOG Alina Markova, MD Bhuvanesh Singh, MD, PhD, FACS Agata Smogorzewska, MD, PhD
9:55 AM		Q&A with Faculty
10:00 AM	DEBATE	T-Cell Depletion vs. Conventional Transplant for Patients with Fanconi Anemia MODERATOR Maria Cancio, MD FACULTY Alice Bertaina, MD, PhD Marc Bierings, MD, PhD Carmem Bonfim, MD, PhD Margaret MacMillan, MD Parinda Mehta, MD
10:30 AM		Q&A with Faculty
10:35 AM	SPECIAL SESSION	Introduction and History of Immunodeficiencies - Interview with Dr. Richard O'Reilly INTERVIEWER: Sergio Giralte, MD
11:05 AM		Break
11:20 AM	PANEL	Sickle Cell Disease - How Research is Impacting Clinical Care MODERATOR Monica Bhatia, MD FACULTY Selim Corbacioglu, MD Jeffrey Lebensburger, DO Deepa Manwani, MD Matthew Porteus, MD, PhD Charles Quinn, MD, MS
12:05 PM		Q&A with Faculty
12:10 PM	PANEL	Bone Marrow Failures MODERATOR Roni Tamari, MD FACULTY Amy DeZern, MD, MHS Sharon Savage, MD Akiko Shimamura, MD, PhD Mike Walsh, MD
12:55 PM		Q&A with Panel
1:00 PM		Closing Remarks Jaap Jan Boelens, MD
1:05 PM		Meet the Faculty (Optional) Attendees are invited to turn on their cameras and join the course faculty for an optional meet and greet
1:30 PM		Adjourn

Agenda and faculty subject to change. Please visit the course website for the most up-to-date brochure:
mskcc.org/nonmalignanthemedisorders

Registration for this course is
complimentary to all attendees.

Register online at:
mskcc.org/nonmalignanthemedisorders

Access details will be emailed to all registered attendees before the start of the course. If you need to cancel your registration, please notify cme@mskcc.org at least seven (7) days prior to the start of the course.

Online registration is available on the MSK CME Portal until 7:00 AM on October 15, 2021. If you wish to register after this date, please email cme@mskcc.org and we will ensure your registration is processed as well as access instructions are emailed to you prior to the start of the course.

Please note that MSK CME reserves the right to cancel or postpone any course due to unforeseen circumstances. In the unlikely event we must cancel or postpone this course, you will be notified via email from MSK CME cme@mskcc.org.



Join the conversation with **#MSKCME**
using the course hashtag:

#tct4nonmalignant2021

MSK Kids

Memorial Sloan Kettering's pediatric program, is dedicated to all children, young adults, and families facing childhood cancer. With specialized expertise and care focused on the individual child, we're proud to say that more families turn to us for cancer care than any other hospital in the United States.

And as pioneers in research, no one fights harder than our physicians and scientists to develop and deliver more effective strategies to prevent, treat, and ultimately cure childhood cancer — for our patients at MSK Kids, and for those everywhere.

For more information, visit: mskcc.org/kids

The largest of Memorial Sloan Kettering's clinical departments, our **Department of Medicine** encompasses experts in medical oncology, cardiology, dermatology, endocrinology, gastroenterology, hematology, infectious diseases, geriatrics, nephrology, pain and palliative care, and pulmonology, among other specialties. Offering a range of services both in New York City and at our suburban outpatient locations, Department of Medicine physicians oversee the care of the whole patient.

Our experts provide the complete spectrum of medical oncology treatments such as chemotherapy, hormone therapy, immunotherapy, and cellular therapy, leading the development and testing of new drug therapies in all areas. In addition, our specialists deliver healthcare that addresses the broader health considerations of our patients with cancer, including the side effects of cancer treatment and the comorbid illnesses that are often present but may hinder our ability to provide cancer treatment if they remain unaddressed.

For more information, visit: mskcc.org/departments/medicine



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