



7th International Bone Marrow Failure Disease Scientific Symposium Virtual Agenda - July 15, 16 & 17, 2020

Wednesday, July 15, 2020 - 9:30 am to 12:00 noon EDT

9:30 – 9:35 am **Welcome & Opening Remarks**
*Symposium Co-Chairs Richard Stone, MD, Dana-Farber Cancer Institute
and Neal Young, MD, National Heart, Lung, and Blood Institute*

Genetics and Genomics of Bone Marrow Failure
*Co-Chairs: Jaroslaw Maciejewski, MD, PhD, Cleveland Clinic
Austin Kulasekararaj, MBBS MD MRCP FRCPath, Kings College Hospital*

9:35 – 10:00 am **Acquired abnormalities in BMF – focus on HLA mutations**
Daria Babushok, MD, PhD, University of Pennsylvania

10:00 – 10:30 am **Novel syndromes predisposing to BMF/MDS**
Marcin Wlodarski, MD, PhD, St. Jude Children’s Research Hospital

10:30 – 11:00 am **Germline predisposition in late onset BMF/MDS**
Jaroslaw Maciejewski, MD, PhD, Cleveland Clinic

11:00 – 11:30 am **Mechanisms of Somatic Transformation in SDS Patients**
R. Coleman Lindsley, MD, PhD, Dana-Farber Cancer Institute

11:45 – 12:00 pm **Session Discussion**

Thursday, July 16 – 9:30 am to 12:00 noon EDT

Transplantation for Bone Marrow Failure

***Co-Chairs: H. Joachim Deeg, MD, Fred Hutchinson Cancer Center
Antonio Risitano, MD, PhD, University of Naples***

9:30 – 10:00 am

Conventional and haplo-identical transplants

Amy E. DeZern, MD, MHS, Johns Hopkins

10:00 – 10:30 am

**Transplantation for inherited bone marrow failure disorders and
fanconi anemia**

Carmem Bonfim, MD, PhD, University of Parana

Non-transplant Therapies for Marrow Failure

***Co-Chairs: Phillip Scheinberg, MD, University of Sao Paulo and
Neal Young, MD, NHLBI***

10:30 – 10:45 am

RACE study update

Antonio Risitano, MD, PhD, University of Naples

10:45 – 11:15 am

NIH long term follow-up

Bhavisha Patel, MD, National Heart, Lung, and Blood Institute

11:15 – 11:45 am

Future directions in non-transplant therapies

Phillip Scheinberg, MD, University of Sao Paulo and
Neal Young, MD, NHLBI

11:45 – 12:00 pm

Session Discussion

12:00 – 12:30 pm

Apellis Pipeline Report

Friday, July 17 – 9:30 am to 12:00 noon EDT

Treatments for MDS and Secondary AML

***Co-Chairs: Olatoyosi Odenike, MD, University of Chicago and
David Sallman, MD, Moffitt Cancer Center***

- 9:30 – 10:00 am** **Novel strategies for TP53 mutated AML/MDS**
David Sallman, MD, Moffitt Cancer Center
- 10:00 – 10:30 am** **TGF beta inhibition in MDS**
Amit Verma, MD, Albert Einstein College of Medicine
- 10:30 – 11:00 am** **Spliceosome modulation as a target in MDS**
Timothy Graubert, MD, Massachusetts General Hospital
- 11:00 – 11:30 am** **Oral azanucleosides and novel combination strategies in
higher risk MDS**
Olatoyosi Odenike, MD, University of Chicago
- 11:30 – 12:00 pm** **Session Discussion**
- 12:00 – 12:15 pm** **AAMDSIF Leadership in Science Award Presentation**
Recipient; Pamela Becker, MD, PhD