Seventh International
Bone Marrow Failure Disease
Scientific Symposium

Collaborating to Transform Treatment and Improve Outcomes

July 15 – 17, 2020

New Virtual Platform

AA·MDS INTERNATIONAL FOUNDATION

Dear Colleagues,

Welcome to the Seventh International Bone Marrow Failure Disease Scientific Symposium!

Our biennial Symposium is unique in its dedication to this group of diseases, the combination of the most recent basic and clinical work in the field, and our aim of translating research to benefit patients. This year's program is particularly notable as the first to be presented in a virtual format due to the global COVID-19 pandemic. Although we shall miss the in-person interaction and camaraderie, we believe that the higher international participation this virtual format enables will result in a broader range of perspectives and ultimately more inclusive collaboration for future research.

We especially welcome and encourage young investigators who have developing interests in bone marrow failure – many of our colleagues who first attended one of the previous symposia as new researchers are now leading innovators in the field. Please take the time to visit the virtual Poster Hall and the Resource Center to learn about exciting research taking place at the National Institutes of Health and around the world.

The primary goal of our symposium remains constant – to develop meaningful insights from molecular, cellular, and genomic experiments and observations that will guide clinical research as well as improve our understanding of aplastic anemia, MDS, PNH, AML and related bone marrow failure diseases.

The Symposium has been organized and sponsored by the leading nonprofit in the area – the Aplastic Anemia and MDS International Foundation, in collaboration with government, academic and industry partners.

Thank you for joining us and for your continued commitment to our rare disease community.

Sincerely,

Richard M. Stone, MD
Co-Chair

Neal Young, MD
Co-Chair

AAMDSIF thanks these supporters whose generous contributions help fund this educational program

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Wednesday, July 15

9:30 – 9:35 am	**Welcome by Symposium Co-Chairs** Richard Stone, MD, Dana-Farber Cancer Institute Neal Young, MD, National Heart, Lung, and Blood Institute
	Genetics and Genomics of Bone Marrow Failure Session Co-Chairs: Jaoslaw Maciejewski, MD, PhD, Cleveland Clinic Austin Kulasekararaj, MBBS, MD, MRCP, FRCPath, King's College Hospital
9:35 am – 10:00 am	**Acquired abnormalities in BMF – focus on HLA mutations** Speaker: Daria Babushok, MD, PhD, University of Pennsylvania
10:00 am – 10:30 am	**Novel syndromes predisposing to BMF/MDS** Speaker: Marcin Wlodarski, MD, PhD, St. Jude Children's Research Hospital
10:30 am – 11:00 am	**Germline predisposition in late onset BMF/MDS** Speaker: Jaroslaw Maciejewski, MD, PhD, Cleveland Clinic
11:00 am – 11:30 am	**Mechanisms of somatic transformation in MDS patients** Speaker: R. Coleman Lindsley, MD, PhD, Dana-Farber Cancer Institute
11:30 am – 12:00 pm	**Session Discussion**

Thursday, July 16

	Transplantation for Bone Marrow Failure Session Co-chairs: H. Joachim Deeg, MD, Fred Hutchinson Cancer Center Antonio Risitano, MD, PhD, University of Naples
9:35 am – 10:00 am	**Conventional and haplo-identical transplants** Speaker: Amy E. DeZern, MD, MHS, Johns Hopkins University
10:00 am – 10:30 am	**Transplantation for inherited bone marrow failure disorders and franconi anemia** Speaker: Carmem Bonfim, MD, PhD, University of Parana

Thursday, July 16 CONTINUED

	Non-transplant Therapies for Marrow Failure Session Co-Chairs: Phillip Scheinberg, MD, University of Sao Paulo Neal Young, MD, National Heart, Lung, and Blood Institute
10:30 am – 10:45 am	**RACE study update** Speaker: Antonio Risitano, MD, PhD, University of Naples
10:45 am – 11:15 am	**NIH long term follow-up** Speaker: Bhavisha Patel, MD, National Heart, Lung, and Blood Institute
11:15 am – 11:45 am	**Future directions in non-transplant therapies** Speakers: Phillip Scheinberg, MD, University of Sao Jose Neal Young, MD, National Heart, Lung, and Blood Institute
11:45 am – 12:00 pm	**Session Discussion**
12:00 pm – 12:30 pm	**Apellis Pipeline Report**

Friday, July 17

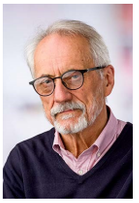
	Treatments for MDS and secondary AML Session Co-Chairs: Olatoyosi Odenike, MD, University of Chicago David Sallman, MD, Moffitt Cancer Center
9:30 am – 10:00 am	**Novel strategies for TP53 mutated AML/MDS** Speaker: David Sallman, MD, Moffitt Cancer Center
10:00 am – 10:30 am	**TGF beta inhibition in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

PROGRAM FACULTY

** Session Co-Chair,*
+ AAMDSIF Medical Advisory Board Member

Daria Babushok, MD, PhD
Assistant Professor of Medicine and Pediatrics
University of Pennsylvania
Philadelphia, PA

Carmem Bonfim, MD, PhD
Associate Professor of Pediactrics
Head of Blood and Marrow Transplantation Program Hospital de Clinicas,
Federal University of Parana
Curitiba, Brazil



H. Joachim Deeg, MD*+
Professor, Clinical Research Division, Fred Hutchinson Cancer Research Center
Professor,
Division of Medical Oncology,
University of Washington
Seattle, WA

Amy DeZern, MD, MHS+
Assistant Professor of Oncology and Medicine,
Johns Hopkins University
Baltimore, MD

Tim Graubert, MD+
Professorof Medicine ,
Harvard Medical School
Director, Hematologic Malignancies Program,
Massachusetts General Hospital
Boston, MA

Austin Kulasekararaj, MBBS, MD, MRCP*
Consultant Heamatologist
King's College Hospital
London, England

R. Coleman Lindsley, MD, PhD
Assistant Professor of Medicine,
Harvard Medical School;
Assistant Professor of Medical Oncology,
Dana-Farber Cancer Center
Boston, MA

Jaroslaw Maciejewski, MD, PhD*+
Professor of Medicine and Pathology,
School of Medicine,
Case Comprehensive Cancer Center ;
Chairman, Department of Translational Hematology and Oncology Research,
Taussig Cancer Institute, Cleveland Clinic
Cleveland, OH

PROGRAM FACULTY

** Session Co-Chair,*
+ AAMDSIF Medical Advisory Board Member

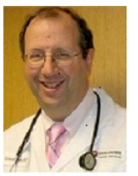
Olatoyosi Odenike, MD*+
Professor of Medicine;
Director, Leukemia Program
University of Chicago
Chicago, IL

Bhavisha Patel, MD
Staff Clinician
National Institutes of Health
Bethesda, MD

Antonio Risitano, MD, PhD*
Director, Bone Marrow
Transplant Program,
University of Naples Federico II
Naples, Italy

David Sallman, MD*
Assistant Member
Dept of Malignant Hematology
Moffitt Cancer Center
Tampa, FL

Phillip Scheinberg, MD*+
Chief of Clinical Hematology
Hospital Sao Jose
Sao Paulo, Brazil



Richard Stone, MD +
Professor of Medicine
Harvard Medical School;
Clinical Director, Adult Leukemia Program
Dana-Farber Cancer Institute
Boston, MA

Amit Verma, MD
Director, Division of Hematologic
Malignancies;
Professor, Medicine, Oncology,
Developmental and Molecular Biology,
Albert Einstein College of Medicine,
New York, New York

Marcin Wlodarski, MD, PhD
Assistant Member
St. Jude Children's Research Hospital
Memphis, TN

Neal Young, MD*+
Director,
Center for Human Immunology
National Heart, Lung, and Blood Institute
Bethesda, MD

Purpose of the Symposium

This 7th International Bone Marrow Failure Disease Scientific Symposium brings together physicians treating bone marrow failure diseases and laboratory researchers studying the immunology and cell biology of bone marrow failure to discuss areas of controversy, share recent research results, and propose innovative directions for future basic and clinical research.

Aplastic anemia, myelodysplastic syndromes (MDS), and paroxysmal nocturnal hemoglobinuria (PNH) are rare diseases that all result in bone marrow failure. Once considered distinct, these diseases are now believed to be linked by similar pathophysiologies.

When AAMDSIF convened the inaugural International Bone Marrow Failure Disease Scientific Symposium in October 2005, it was the first such gathering of clinical and basic scientists studying these diseases. In the subsequent symposia held in 2010, 2012, 2014, 2016 and 2018 many new working relationships and collaborations were established to advance the field of bone marrow failure research.

Exploration of current research issues in bone marrow failure will greatly benefit from collaboration among basic and clinical scientists studying these diseases. Increased understanding of the molecular events driving these diseases and of the response to treatment are needed to define at-risk populations and improve current therapies.

Symposium Evaluation

Feedback from symposium participants is extremely helpful for future planning and required for the grant reporting process. A symposium evaluation will be sent electronically to all participants immediately following the program and we appreciate your prompt response. This information will be used to assess the symposium content and format and to develop future programs and other methods of fostering collaborative bone marrow failure research.

AAMDSIF Leadership in Science Award

Recipients, nominated by the Foundation's Medical Advisory Board, are selected for their unique contributions to bone marrow failure disease treatment or research. We are pleased to present this award to Pamela Becker, MD, PhD at this Symposium.

Past Awardees:
Neal Young, MD
H. Joachim Deeg, MD
Richard Stone, MD
Jaroslaw Maciejewski, MD, PhD
Mikkael Sekeres, MD, MS
Alan List, MD
David Steensma, MD
Valeria Santini, MD

About AAMDSIF

Aplastic Anemia and MDS International Foundation

AAMDSIF is the world's leading nonprofit health organization committed to supporting patients and families living with aplastic anemia, MDS, PNH and related bone marrow failure diseases.

For 36 years, the Foundation has been the resource of choice for patients and families trying to cope with these rare disorders. In addition to offering an evolving range of educational tools, programs and services, we provide a strong, compassionate community of peer and professional support for patients and caregivers.

What We Do

- Provide patients and families with education and support through regional conferences, online webinars, digital and print publications, support groups and peer support network
- Fund medical research through our grants program to further the development of better treatments and to discover the cures for bone marrow failure diseases
- Provide educational opportunities for medical professionals to help increase their understanding of bone marrow failure, improve their diagnostic ability and update them on the latest treatments
- Promote public awareness of bone marrow failure disease through regional and local events, online publications and social media outreach

AAMDSIF has awarded over $5 million in research grants over 30 years

1989-2019 RESEARCH GRANTEES

2019
Christin DeStefano, MD
Elissa Furutani, MD

2018
Tushar Bhagat, PhD
Yoshimi Akihide, MD, PhD
Alina Dulau-Florea, MD
Sergei Vatolin, PhD
Simona Pagliuca, MD

2017
Joseph Oved, MD
Coleman Lindsley, MD
Sergei Vatolin, MD

2016
Tushar Bhagat, PhD
Yoshimi Akihide, MD, PhD
Alina Dulau-Florea, MD
Kate MacNamara, PhD
Simona Pagliuca, MD

2015
Jing Fang, MD, PhD
Anastastios Karadimitris, MD, PhD
Katherine King, PhD
Shahram Kordasti, MD, PhD
Sicheng Wen, MD, PhD
Britta Will, PhD

2014
Daria Babushok, MD, PhD
Luis Batista, PhD
Rosannah Cameron, PhD
Youmna Kfoury, PhD
Patrizia Ricci, PhD
Chao-Yie Yang, MD, PhD

2013
Andrew Dancis, MD
Hideki Makishima, MD, PhD
Rosario Notaro, MD
Eirini Papapetrou. MD, PhD
Akiko Shimamura, MD, PhD

2012
David Araten, MD
Lisa Minter, PhD
Jeffrey Pu, PhD
Matthew J. Walter, MD
Zhe Yang, PhD

2011
Kim-Hien T. Dao, DO,PhD
Keith R. McCrea, MD
Parinda Mehta, MD
Mridul Mukherji, PhD

2010
Gregory A. Abel,MD,MPH
Christian Bellodi,PhD
Muneyoshi Futami,MD
Ramon Tiu,MD

2009
Kazuhiko Ikeda, MD, PhD
Regis Peff ult de Latour, MD, PhD
Archibald Perkins, MD, PhD

2008
Benjamin Braun, MD, PhD
Jaroslaw Maciejewski, MD, PhD
Lisa Minter, PhD
Antonio Maria Risitano, MD, PhD

2007
Hiromi Gunshin, MD, PhD
Kay Macleod, PhD
Lubomire Sokol, MD, PhD

2006
Lukasz Gondek, MD, PhD
Hinh Ly, PhD
Lisa Minter, PhD
Christine O'Keefe, PhD

2005
Seth Joel Corey, MD, MPH
Eva Guinan, MD
Catriona H.M. Jamieson, MD, PhD
Jane L. Liesveld, MD
Gabrielle Meyers, MD
Elena Solomou, MD, PhD
Matthew Walter, MD

2004
Monica Bessler, MD,PhD
Jaroslaw Maciejewski,MD,PhD

2003
Jaroslaw Maciejewski, MD, PhD
Archibald Perkins, MD, PhD
Russell Ware, MD, PhD

2002
Michael S. Boosalis, PhD

2001
Marianne Greene, MD

2000
Jen Chin Wang, MD

1999
Sherilyn Gross, LN, PhD, CCRP
Sujit S. Sheth, MD

1998
Richard Carter, MD
Tatiana Zorina, MD, PhD

1996
David Araten, MD

1995
Chaker Nadim Adra, PhD
Hagop Youssoufian, MSc, MD

1994
Surapol Issaragrisil, MD
Ronald L. Paquette, MD

1992
Leslie G. Biesecker, MD

1991
Jeffrey P. Novack, MD

1990
Hildegard Greinix, MD
Stephen R. Paul, MD

1989
Winald Gerritsen, MD, PhD

Poster Title	Author(s)
Poster Presentations	
The Clonal and Mutational Diversity of Myeloid Neoplasia with SF3B1 Mutations	Hassan Awada[1], Cassandra M. Kerr[1], Vera Adema[1], Carmelo Gurnari[1], Simona Pagliuca[1], Jibran Durrani[1], Sunisa Kongkiatkamon[1], Teodora Kuzmanovic[1], Jack Khouri[2], Heesun J. Rogers[3], Manja Meggendorfer[4], Torsten Haferlach[4], Hetty Carraway[5], Mikkael A. Sekeres[5], Jaroslaw P. Maciejewski[1], Valeria Visconte[1] *1Department of Translational Hematology and Oncology Research, Taussig Cancer Institute, Cleveland Clinic, Cleveland, OH, USA, 2Department of Hematology and Medical Oncology, Taussig Cancer Institute, Cleveland Clinic, Cleveland, OH, USA, 3Department of Laboratory Medicine, Cleveland Clinic, Cleveland, OH, USA, 4MLL Munich Leukemia Laboratory, Munich, Germany, 5Leukemia Program, Department of Hematology and Medical Oncology, Taussig Cancer Institute, Cleveland Clinic, Cleveland, OH, USA.*
Downregulated Staufen1 compromises hematopoietic stem cell activity in vivo and elicits expression signatures characteristic of clinical anemias	Derek Chan[1,2], Lina Liu[3], Damian Tran[3], Ana Vujovic[3], Ruilin Wu[3], Laura de Rooij[4], Joshua Xu[3,5], Yu Lu[3], Kristin Hope[6,7] *1Department of Pediatrics, Faculty of Medicine, University of British Columbia, Vancouver, British Columbia,Canada; 2BC Children's Hospital, Vancouver, British Columbia, Canada; 3Stem Cell and Cancer Research Institute,Department of Biochemistry and Biomedical Sciences, Faculty of Health Sciences, McMaster University, Hamilton,Ontario, Canada; 4Laboratory of Angiogenesis and Vascular Metabolism, Department of Oncology, Biomedical Sciences Group, KU Leuven, Leuven, Flanders, Belgium; 5Michael G. DeGroote School of Medicine, Faculty of Health Sciences, McMaster University, Hamilton, Ontario, Canada; 6Princess Margaret Cancer Centre, University Health Network, Toronto, Ontario, Canada; 7Department of Medical Biophysics, University of Toronto, Toronto,Ontario, Canada*
Large differences in PNH clone size between neutrophils and monocytes	Alina Dulau-Florea[1], Irina Maric[1], Emma M. Groarke[2], Bhavisha A. Patel[2], Nisha Patel[1], Katherine R. Calvo[1], Neal S. Young[2], Raul C. Braylan[1] *1Hematology Section, Department of Laboratory Medicine, Clinical Center, National Institutes of Health, Bethesda. 2Hematology Branch, National Heart Lung and Blood Institute, National Institutes of Health, Bethesda*
Cyclosporine enhances the sensitivity to lenalidomide in Myelodysplastic Syndromes	Xiaofei He[1], Aixia Dou[1], Saran Feng[1]#, Ashley Roman-Rivera[1], Caleb Hawkins[1], Lauren Lawley[1], Jiajia Zhang[1], Mark Wunderlich[3], Benjamin Mizukawa[3,4], Stephanie Halene[5], Amisha Patel[5], Jing Fang[1] *1Department of Drug Discovery and Biomedical Sciences, University of South Carolina College of Pharmacy, Columbia, SC, USA; 2Department of Epidemiology and Biostatistics, Arnold School of Public Health, University of South Carolina, Columbia SC, USA; 3Cancer and Blood Diseases Institute, Cincinnati Children's Hospital Medical Center, Cincinnati, OH, USA; 4University of Cincinnati College of Medicine, Cincinnati, OH, USA; 5Section of Hematology/Department of Internal Medicine and Yale Cancer Center, Yale University School of Medicine, New Haven, CT, USA.*
IFNγ promotes hematopoietic stem cell homing and niche relocalization	Marcus A. Florez[1,6‡], Katie A. Matatall[2‡], Youngjae Jeong[3], Laura Ortinau[3], Paul W. Shafer[4,6], Anne M. Lynch[5], Roman Jaksik[7], Marek Kimmel[7], Dongsu Park[3,6*], and Katherine Y. King[1,4,5,6*] *1Medical Scientist Training Program and Program in Translational Biology and Molecular Medicine; 2Section of Infectious Disease, Department of Pediatrics; 3Department of Human and Molecular Genetics; 4Program in Immunology; 5Program in Developmental Biology; 6Dan L. Duncan Cancer Center and Center for Cell and Gene Therapy, Baylor College of Medicine, Houston, Texas USA 77030; 7Department of Systems Biology and Engineering, Silesian University of Technology, Gliwice, Poland and Department of Statistics, Rice University, Houston, Texas USA 77005‡-Equal contribution*

POSTER PRESENTATIONS

Poster Title	Author(s)
Poster Presentations	
Immunogenomics of Paroxysmal Nocturnal Hemoglobinuria: a model of immune escape	Carmelo Gurnari[1,2]*, Simona Pagliuca[1,3]*, Cassandra M. Kerr[1], Hassan Awada[1], Sunisa Kongkiatkamon[1], Valeria Visconte[1] and Jaroslaw P. Maciejewski[1] *1Translational Hematology and Oncology Research Department of Cleveland Clinic; 2Department of Biomedicine and Prevention, University of Rome Tor Vergata, Rome, Italy; 3Université de Paris, Paris, France*
Molecular Characterization of the Histone Acetyltransferase p300 (EP300) Gene in Myeloid Neoplasia	Sunisa Kongkiatkamon[1], Vera Adema[1], Simona Pagliuca[1], Wencke Walter[3], Cassandra M. Kerr[1], Yasunobu Nagata[1], Hassan Awada[1], Stephan Hutter[3], Carmelo Gurnari[1], Hetty E. Carraway[1,2], Manja Meggendorfer[3], Mikkael A.Sekeres[1,2], Torsten Haferlach[3], Valeria Visconte[1], Jaroslaw P. Maciejewski[1,2] *1Department of Translational Hematology and Oncology Research, Lerner Research Institute, Cleveland Clinic, Cleveland, OH, USA; 2Leukemia Program, Department of Hematology and Medical Oncology, Taussig Cancer Institute, Cleveland Clinic, Cleveland, OH, USA; 3MLL Munich Leukemia Laboratory, Munich, Germany*
Batf2 drives depletion of hemetopoietic stem cells during chronic infection	Duy T. Le[1,2,3], and Katherine Y. King[1,2,3] *1Section of Infectious Diseases, Department of Pediatrics, Baylor College of Medicine (BCM), Houston, TX; 2Program in Immunology, Graduate School of Biomedical Sciences, BCM, Houston, TX; 3Center for Cell and Gene Therapy, BCM, Houston, TX*
Diacylglycerol kinase ζ limits IL-15 mediated cytotoxicity in the bone marrow	Martin-Salgado[1], M; Andrada, E[1,2]; Liébana, R[1]; López-Santalla, M[3]; Mérida I[1] *1Department of Immunology and Oncology, National Centre for Biotechnology. Spanish Research Council (CNB-CSIC). 2Department of Immunology and Oncology, National Centre for Biotechnology, Campus UAM, Cantoblanco, Madrid. 3Division of Hematopoietic Innovative Therapies, Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas (CIEMAT), Madrid, Spain*
Evolutionary divergence of class I and II HLA genes as predictor of disease severity and response to immune-suppression in patients diagnosed with aplastic anemia and paroxysmal nocturnal hemoglobinuria	Simona Pagliuca[1,2]*, Carmelo Gurnari[1,3]*, Hassan Awada[1], Cassandra M. Kerr[1], Kongiatkamon Sunisa[1], Valeria Visconte[1] and Jaroslaw P. Maciejewski[1] *1Translational Hematology and Oncology Research Department of Cleveland Clinic; 2Université de Paris, Paris, France; 3Department of Biomedicine and Prevention, University of Rome Tor Vergata, Rome, Italy*
Mutations in RAS pathway genes correlate with Type of Failure to Azacitidine: at Randomization onto the INSPIRE Trial	Koichi Takahashi, MD[1], Anna Jonasova, MD[2], PhD, Selina M. Luger, MD, FRCPC[3], Aref Al-Kali,MD[4], David Valcárcel, MD[5], Erica D. Warlick, MD[6], Wieslaw W. Jedrzejczak, MD, PhD[7], María Díez-Campelo, MD, PhD[8], Patrick S. Zbyszewski, MBA[9], Christopher Cavanaugh[9], Richard C. Woodman, MD[9], Steven M. Fruchtman, MD[9], Guillermo Garcia-Manero, MD[1] *1University of Texas MD Anderson Cancer Center, Department of Leukemia, Houston, TX; 21st Medical Department - Hematology, General Hospital, Prague, Czech Republic; 3Abramson Cancer Center, University of Pennsylvania, Philadelphia, PA; 4Division of Hematology, Mayo Clinic, Rochester, MN; 5Planta Baixa, Hospital Universitari Vall d'Hebron, Barcelona, Spain; 6Division of Hematology, Oncology and Transplantation, University of Minnesota, Minneapolis, MN; 7MTZ Clinical Research, Medical University of Warsaw, Warsaw, Poland; 8Hematology Department, Institute of Biomedical Research of Salamanca, University Hospital of Salamanca, Salamanca, Spain; 9Onconova Therapeutics, Inc., Newtown, PA; 1Department of Leukemia, The University of Texas MD Anderson Cancer Center, Houston, TX*