

Tenth International
**BONE MARROW FAILURE DISEASE
SCIENTIFIC SYMPOSIUM**

MARCH 19-20, 2026



Note: Session times are approximate and subject to adjustment

Thursday, March 19

Time (U.S. EDT)	
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8:00 – 8:10 am **Welcome Remarks**

8:10 – 9:30 am **Genetics and Genomics of Bone Marrow Failure**
Session Co-Chairs: Torsten Haferlach and Christopher Reilly

Topic	Confirmed Speaker
BMF/germline predisposition in general population	Lisa McReynolds
Germline ERG Variants	Jiarna Zerella
Genetics of Schwachman Diamond Syndrome and other ribosomopathies	Allan Warren
Functional and clinical effects of germline TERT variants	Christopher Reilly
Functional characterization of germline DDX41 variants	Tim Chlon
Advanced Genomic Technologies in Bone Marrow Failure	Hetty Carraway

9:30 – 10:00 am **Sponsor Reports**

10:00 – 10:15 am **Break**

10:15 – 11:45 am Pathways of Clonal Progression Arising from Germline Predisposition
 Session Co-Chairs: Marcin Wlodarski and R. Coleman Lindsley

Topic	Confirmed Speaker
Acquired vs. hereditary CH patterns	Daria Babushok
<u>Polyclonal to Monoclonal</u> Trajectories of clonal hematopoiesis in TBD	Fernanda Gutiérrez-Rodrigues
Mechanisms of genetic rescue in SAMD9/9L and GATA2	Marcin Wlodarski
Clonal evolution in RUNX1	David Young
<u>How to Optimize Surveillance?</u> Surveillance of CH in children with BMF / leukemia predisposition syndromes	Enrico Attardi
Optimal tissue source for surveillance in BMF	Felicia Andresen

11:45 – 12:15 pm Sponsor Reports

12:15 – 1:00 pm Lunch

1:00 – 2:15 pm Clonality in Acquired Bone Marrow Failure, PNH, and VEXAS
 Session Co-Chairs: Emma Groarke and Yoshitaka Zaimoku

Topic	Confirmed Speaker
Insights of PNH using single-cell analysis	Hiroki Mizumaki
VEXAS – How clonality affects environment	Martina Fiumara
VEXAS – How therapies affect clonality	Thibault Comont
Clonality in AA after IST <ul style="list-style-type: none"> • HLA clonality • RACE study data • NIH study data • Case Discussion 	Yoshitaka Zaimoku Austin Kulasekararaj Bhavisha Patel Emma Groarke

2:15 – 3:45 pm

Advances in Paroxysmal Nocturnal Hemoglobinuria

Session Co-Chairs: Austin Kulasekararaj and Pedro Prata

Topic	Confirmed Speaker
PNH Updates for 2026	Regis Peffault de LaTour
Recognition and management of breakthrough hemolysis	Bruno Fattizzo
Effectively switching patients between complement inhibitors	Morag Griffin
PNH Treatment in Asia – changing landscape	Jun Ho Jang
Global PNH Patient Registry	David Araten

3:45 – 4:00 pm

Sponsor Report

4:00 – 4:15 pm

Break

4:15 – 5:45 pm

Non-transplant Therapies for Marrow Failure

Session Co-Chairs: Carlos DeCastro and Phil Scheinberg

Topic	Confirmed Speaker
Androgens, alemtuzumab, anti-cytokine therapies (anti-IL2R, anti-IFN gamma), daratumumab, and ruxolitinib in aplastic anemia.	Regis Peffault de LaTour
How can we avoid missing patients with SAA who have an underlying, unidentified, and unsuspected genetic defect?	Fernanda Gutierrez-Rodrigues
Encouraging non-transplant therapies for inherited marrow failure syndromes (Fanconi anemia)	Julian Sevilla
Encouraging non-transplant therapies for inherited marrow failure syndromes. (Dyskeratosis congenita)	Kasiani C Myers
Rewriting the Clock: Nucleoside Therapy to Restore Telomeres in Inherited Bone Marrow Failure	Suneet Agarwal
Discussion	

5:45 – 7:15 pm

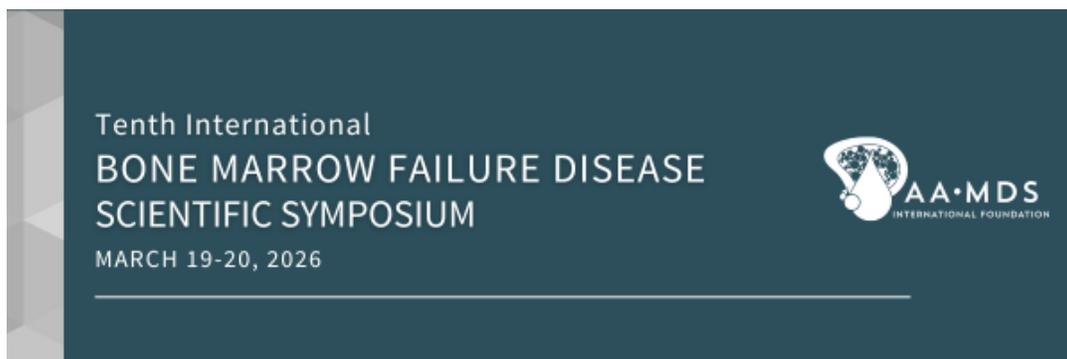
Transplantation for Bone Marrow Failure

Session Co-Chairs: Rick Childs and Amy DeZern

Topic	Confirmed Speaker
Two options for patients are better than one: <ul style="list-style-type: none">- Upfront alternative transplant for SAA- IST	Amy DeZern Neal Young
Optimal timing of transplant for MDS	Nicolaus Kroger
Recent advances in allogeneic BMT that have changed the transplant landscape <ul style="list-style-type: none">- Progress in conditioning regimens- Viral prophylaxis and treatment strategies- New therapies for managing acute and chronic GVHD	Rick Childs

7:15 – 8:30 pm

Reception and Poster Session



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Friday, March 20

Time (U.S. EDT)	
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8:00 – 8:05 am **Welcome to Day 2**

8:05 - 8:35 am **Sponsor Reports**

8:35 – 10:15 am **MDS and Secondary AML Pathophysiology**
 Session Co-Chairs: Jaroslaw Maciejewski and Maria (Ken) Figueroa

Topic	Confirmed Speaker
Objective pathogenetically driven molecular classification of MDS and Secondary AML	Valeria Visconte
Clonal architecture and invariant patterns of progression of CCUS and MDS	Lukasz Gondek
When monocytosis or MDS becomes CMML	Guillermo Montalban-Bravo
Immunome of MDS and Secondary AML	Carlos Bravo Perez
Somatic gene rescue as a unifying concept in MDS	Jarek Maciejewski
Single cell genomics and Epigenetics: new clues into the pathogenesis of MDS	Aly Karson
Discussion	

10:15 – 10:45 am Break

10:45 – 12:15 pm MDS Classification and Prognosis

Session Chairs: Elsa Bernard and Mateo Della Porta

Topic	Confirmed Speaker
Unifying classification schemes for MDS	TBD
State of the Art for prognostication in MDS (not including AI)	Maria Creignou
State of the Art for AI prognostication in MDS	Matteo Della Porta
Dynamic assessment of molecular prognostication	Luca Lanino
Can inclusion of immunological parameters refine MDS prognostication	Carmelo Gurnari
Discussion	

12:15 – 1:00 pm Lunch**1:00 – 2:00 pm Health-Related Quality of Life**

Session Co-Chairs: Namrata Chandhok and Thomas LeBlanc

Topic	Confirmed Speaker
How can we measure quality of life in MDS, to inform interventions?	Fabio Efficace
Interventions to improve psychological well-being in myeloid malignancy and transplant	Alison Kavanaugh
Transfusions and quality of life in MDS/AML - how to balance palliation and burden near the end of life?	Pam Egan
How can integrated specialist palliative care improve the patient experience of intensive chemotherapy?	Wil Santivasi
Discussion	

2:00 – 3:00 pm

Treatments for MDS and Secondary AML

- **Low-Risk MDS and CCUS**

Session Co-Chairs: Rena Buckstein and Valeria Santini

Topic	Confirmed Speaker
Reframing MDS and CHIP	Neal Young
Supportive Care in MDS – Optimizing Hgb targets, transfusion thresholds and iron chelation	Rena Buckstein
Sequencing Therapies for Lower-Risk MDS	Valeria Santini
When to Treat Lower Risk MDS with Higher Risk Therapies	Guillermo Garcia-Manero
Discussion	

3:00 – 4:00 pm

Treatments for MDS and Secondary AML

- **High-Risk MDS and Secondary AML**

Session Co-Chairs: Hetty Carraway and Amer Zeidan

Topic	Confirmed Speaker
Key Concepts in Clinical Trial Design For HR-MDS: Lessons Learned from Phase 3 Death Row	Mikkael Sekeres
How to We Best Incorporate Both HR-MDS and AML Together in Future Studies? (Addressing Inclusion Criteria and Response Assessment)	Andrew Brunner
Is it Time to Revisit the Decision Analysis of Allo-BMT in MDS for LR-MDS and Delayed BMT?	Reshma Ramlal
Tp53m Myeloid Neoplasms: Top Ten Priorities in Getting to a Win	Max Stahl
Potential innovations in Cellular Therapies for Myeloid Malignancies	TBD
Where To Go from Here? Strategic Approach in Advancing the Treatment of HR-MDS	Richard Stone

4:00 – 4:10 pm

Concluding Remarks