ESH-EBMT-EHA-IPIG 4th Translational Research Conference
BONE MARROW FAILURE AND LEUKAEMIA PREDISPOSITION SYNDROMES
November 6-8, 2026 – Vienna, Austria
Chairs: Carmem Bonfim, Tim Brümmendorf, Antonio Risitano, Sharon Savage
Scientific Committee: Beatrice Drexler, Carlo Dufour, Morag Griffin, Régis Peffault de Latour
With the support of the Severe Aplastic Anaemia Working Party of the EBMT,
the EHA SWG on Granulocyte and Bone Marrow Failures and the International PNH Interest Group

Friday, November 6, 2026

08:30–08:40	**Welcome**	Conference chairs

SESSION I – Stem cell biology
Chairs: Tim Brümmendorf (Aachen), Markus Manz (Zurich)

08:40-09:00	Inflamm-Ageing of Haematopoietic Stem Cells	Markus Manz (Zurich)
09:00-09:20	Clonal hematopoiesis in beta hemoglobinopathies: implications for treatment	Coleman Lindsley (Boston)
09:20-09:40	Inherited resilience to clonal hematopoiesis by modifying stem cell RNA regulation	Gaurav Agarwal (Boston)
09:40-09:50	Selected Brief Oral Communication Epigenetic memory of the Fanconi Anemia environment persists in donor-derived hematopoietic cells after hematopoietic stem cell transplantation	Filomena Pierri (Genoa)
09:50-10:20	*Panel Discussion*	
10:20-10:50	***Coffee Break***	

SESSION II – Inherited predisposition to leukaemia
Chairs: Sharon Savage (Bethesda), Marcin Wlodarski (Memphis)

10:50-11:10	MDS predisposition Syndromes	Marcin Wlodarski (Memphis)
11:10-11:30	RUNX 1 – genomic landscape	Marc Raaijmakers (Rotterdam)
11:30-11:50	Stem cell dysfunction as a driver of leukemia predisposition	Jean Soulier (Paris)
11:50-12:00	Selected Brief Oral Communication Biallelic vs. Monoallelic Status Defines Distinct Phenotypes of Germline SH2B3 MPD Predisposition	Enrico Attardi (Rome)

12:00-12:10	Selected Brief Oral Communication Metabolic vulnerabilities in ERCC6L2-associated bone marrow failure and leukemia predisposition	Ilse Kaaja (Helsinki)
12:10-12:40	*Panel Discussion*	
12:40-14:25	***Lunch***	

13:25-14:25 **SATELLITE SYMPOSIUM**
Transforming PNH Care: Precision in Patient Management and Real-World Insights
Chair: Jens Panse (Aachen)
Speakers: Ana Boban (Zagreb), Lenka Mastikova (Prague)
This symposium is organised and supported by Sobi

SESSION III – Constitutional marrow failure
Chairs: Carmen Bonfim (Durham), Carlo Dufour (Genoa)

14:35-14:55	Cancer screening in Fanconi anaemia and other bone marrow failure disorders	Eunike Velleuer (Düsseldorf)
14:55-15:15	What we can learn from Registries - the FA Spanish Experience	Julian Sevilla (Madrid)
15:15-15:35	From multiomics to mechanism in Diamond-Blackfan Anaemia Syndrome	Deena Iskander (London)
15:35-15:45	Selected Brief Oral Communication Clonal hematopiesis in cancer predisposition syndromes: Data from the Italian Pediatric Consortium	Luca Arcuri (Genoa)
15:45-16:15	*Panel discussion*	
16:15-16:45	***Coffee break***	

SESSION IV – Constitutional marrow failure: telomere biology diseases and other...
Chairs: Fabian Beier (Aachen), Marena Niewisch (Ulm)

16:45-17:05	New molecular biomarkers in the Telomere Biology Disorders	Sharon Savage (Bethesda)
17:05-17:25	Immune disfunction in TBD	Rodrigo Calado (São Paulo)
17:25-17:45	Pulmonary complications of TBDs	Christine Kim Garcia (New York)
17:45-17:55	Selected Brief Oral Communication Age-related hematologic manifestations in Telomere biology disorders	Jane Koo (Cincinnati)
17:55-18:05	Selected Brief Oral Communication Spatial multiomic profiling reveals conserved endothelial depletion across fibrotic lung and liver in telomere biology disorders	Alfredo Rodriguez (Mexico City)

18:05-18:35 *Panel discussion*

18:40-20:30 Mentored poster walk - Welcome get-together for all

Saturday, November 7, 2026

07:30-08:30 3 simultaneous Meet the Expert sessions

1.	Differences between inherited and acquired BMF	Deena Iskander (London) Thierry Leblanc (Paris)
2.	Expanding donor options for patients with inherited bone marrow failures	Carmem Bonfim (Durham) Carlo Dufour (Genoa)
3.	Hemolysis under treatment with complement inhibitors	Jens Panse (Aachen) Antonio Risitano (Avellino)

08:40-09:40 SATELLITE SYMPOSIUM
PNH in Focus: Real-World Evidence on Disease Control, Residual Burden, and Treatment Transition
Chair: Regis Peffault de Latour (Paris)
Speakers: Austin Kulasekararaj (London), Morag Griffin (Leeds)
This symposium is organised and supported by Alexion

SESSION V – Clonal Haematopoiesis
Chairs: Austin Kulasekararaj (London), Thierry Leblanc (Paris)

09:45-10:05	CH vs. transformation in acquired AA	Austin Kulasekararaj (London)
10:05-10:25	CH in inherited BMFS	Mikko Myllymäki (Helsinki)
10:25-10:45	Somatic genetic rescue	Patrick Revy (Paris)
10:45-10:55	Selected Brief Oral Communication Specific clonal hematopoiesis patterns support pathogenicity of germline SAMD9 and SAMD9L variants	Nathan Gray (Memphis)
10:55-11:25	*Panel discussion*	

11:25-11:55 Coffee break

SESSION VI – Complement & PNH
Chairs: Morag Griffin (Leeds), Jens Panse (Aachen)

11:55-12:15	Complement and disease	Daniel Ricklin (Basel)
12:15-12:35	Challenges in PNH treatments	Antonio Risitano (Avellino)
12:35-12:55	Complement gene mutations/polymorphisms	Dimitrios Mastellos (Athens)

12:55-13:05	Selected Brief Oral Communication MASP-3 inhibition for upstream complement inhibition in paroxysmal nocturnal haemoglobinuria: integrated translational and clinical evidence with zaltenibart	Morag Griffin (Leeds)
13:05-13:35	*Panel Discussion*	
13:35-14:50	***Lunch***	

SESSION VII – Pathophysiology of aplastic anaemia
Chairs: Beatrice Drexler (Basel), Phillip Scheinberg (São Paulo)

14:50-15:10	Evolutionary biology, somatic mutations and clonal haematopoiesis	Neal Young (Bethesda)
15:10-15:30	Tracing the evolution of clonal T cell responses in LGL leukemia with single-cell whole genome sequencing	Satu Mustjoki (Helsinki)
15:30-15:50	Regulatory T Cells in Aplastic Anaemia: From Immune Dysregulation to Engineered Bone Marrow Homing and Survival	Shahram Kordasti (London)
15:50-16:20	*Panel discussion*	
16:20-16:50	***Coffee break***	

SESSION VIII – New therapies in haematology disorders
Chairs: Suneet Agarwal (Boston), Neal Young (Bethesda)

16:50-17:10	Gene therapy for IBMFS	Sentil Bhoopalan (Memphis)
17:10-17:30	Gene therapies in Fanconi Anaemia	Paula Rio (Madrid)
17:30-17:50	Telomere Diseases: Beyond BMT	Suneet Agarwal (Boston)
17:50-18:00	Selected Brief Oral Communication Androgen treatment for Telomere Biology Disorders: long-term results from a retrospective registry analysis	Nergis Meyer (Aachen)
18:00-18:30	*Panel discussion*	

Sunday, November 8, 2026

07:50-08:50	**3 simultaneous Meet the Expert sessions**	
	1. Clinical presentation of TBDs	Fabian Beier (Aachen) Marena Niewisch (Ulm)
	2. Management of moderate AA	Britta Höchsmann (Ulm) Phillip Scheinberg (São Paulo)

	3. Diagnostics for iBMF in 2026	Jean Soulier (Paris) Marcin Wlodarski (Memphis)

SESSION IX – Treatment of aplastic anaemia
Chairs: Austin Kulasekararaj (London), Regis Peffault de Latour (Paris)

08:55-09:15	Treatment of moderate AA	Britta Höchsmann (Ulm)
09:15-09:35	Treatment of v/sAA	Phillip Scheinberg (São Paulo)
09:35-09:55	HSCT in AA	Victoria Potter (London)
09:55-10:05	Selected Brief Oral Communication A randomized trial of sirolimus for relapse prevention in patients with severe aplsatic anemia responsive to immunosuppressive therapy	Nicholas Lee (Bethesda)
10:05-10:35	*Panel discussion*	
10:35-11:05	***Coffee break***	

SESSION X – Unmet medical needs in BMFS
Chairs: Emma Groarke (Dublin), Deena Iskander (London)

11:05-11:25	VEXAS	Emma Groarke (Dublin)
11:25-11:45	New Clinical and biological insights in DADA 2	Maurizio Miano (Genoa)
11:45-12:05	Treatment of Refractory/relapse SAA outside BMT in 2026	Regis Peffault de Latour (Paris)
12:05-12:15	Selected Brief Oral Communication Refining germline testing in myeloid neoplasms: clinical variables improve diagnostic yield in a real-world cohort	Guillermo Ramil (Barcelona)
12:15-12:45	*Panel discussion*	
12:45-12:55	***Closing remarks***	Conference chairs

MEETING CLOSURE