ESH EUROPEAN SCHOOL OF HAEMATOLOGY

ENDORSED BY: EBMT

2nd ESH-EBMT Translational Research Conference
BONE MARROW FAILURE SYNDROMES:
From the cell to the cure of the disease

Paris, France - November 18-20, 2022

Chairpersons: Tim Brümmendorf (Aachen), Carlo Dufour (Genova), Régis Peffault de Latour (Paris), Antonio Risitano (Avellino)

Friday, November 18, 2022

09:00–09:10	**Welcome**	Conference chairs

SESSION I – Stem cell biology: from stress to failure
Chairs: Tim Brümmendorf (Aachen) and Markus Manz (Zürich)

09:10-09:15	Introduction	Tim Brümmendorf (Aachen) Markus Manz (Zürich)
09:15-09:30	Inflamm-ageing of the hematopoietic system	Markus Manz (Zürich)
09:30-09:35	*Discussion*	
09:35-09:50	Clonal hematopoiesis in inherited BMFD	Coleman Lindsley (Boston)
09:50-09:55	*Discussion*	
09:55-10:10	Clonal tracking of hematopoietic stem cells	Peter Campbell (Cambrigde)
10:10-10:15	*Discussion*	
10:15-10:22	**Selected brief oral communication** *Ap2a2 deleted mice link fetal liver haematopoiesis exhaustion to loss of LT-HSC quiescence, perturbed asymmetrical cell division and upregulation of iron and lipids, critical elements of ferroptosis.*	Stephen B. Ting (Melboume)
10:22-10:25	*Discussion*	
10:25-10:55	*Panel discussion*	
10:55-11:25	***Coffee Break***	

SESSION II – Constitutional marrow failure 1: fanconi anaemia (FA)
Chairs: Carlo Dufour (Genova) and Inderjeet Dokal (London)

11:25-11:30	Introduction	Carlo Dufour (Genova) Inderjeet Dokal (London)
11:30-11:45	Fanconi anaemia : diagnostic and natural history in the bone marrow	Jean Soulier (Paris)

11:45-11:50	*Discussion*	
11:50-12:05	Classical and new treatment of Fanconi anaemia	Carlo Dufour (Genova)
12:05-12:10	*Discussion*	
12:10-12:25	Gene therapy for Fanconi anaemia and other iBMF	Juan Bueren (Madrid)
12:25-12:30	*Discussion*	
12:30-12:37	**Selected brief oral communication** *Germline variants in MDM4 cause a disorder of p53 dysregulation and Insufficient Telomere Maintenance*	Robert Meyer (Aachen)
12:37-12:40	*Discussion*	
12:40-13:10	*Panel discussion*	
13:10-14:50	***Lunch***	

14:10-14:40 **SATELLITE SYMPOSIUM**

Unmet Medical Needs in Acquired Aplastic Anemia (AA) Immuno-suppressive Therapy

Chair: Tim Henrik Brümmendorf (Aachen)

Speakers: Austin Kulasekararaj (London), Phillip Scheinberg (São Paulo)

This symposium is organized and sponsored by Pfizer

SESSION III – Constitutional marrow failure 2: ribosome diseases
Chairs: Carlo Dufour (Genova) and Marc H.G.P. Raaijmakers (Rotterdam)

14:50-14:55	Introduction	Carlo Dufour (Genova) Marc H.G.P. Raaijmakers (Rotterdam)
14:55-15:10	Ribosomes and hematopoiesis	Alan Warren (Cambridge)
15:10-15:15	*Discussion*	
15:15-15:30	Management of SDS	Akiko Shimamura (Boston)
15:30-15:35	*Discussion*	
15:35-15:50	Management of Diamond-Blackfan anaemia (DBA)	Deena Iskander (London)
15:50-15:55	*Discussion*	
15:55-16:02	**Selected brief oral communication** *Results of prospective hematologic surveillance with correlative single cell DNA sequencing in children with SAMD9/SAMD9L related bone marrow failure and Monosomy 7*	Nathan Gray (Memphis)
16:02-16:05	*Discussion*	
16:05-16:35	*Panel discussion*	
16:35-17:05	***Coffee Break***	

SESSION IV – Constitutional marrow failure 3: telomere biology diseases and other…
Chairs: Inderjeet Dokal (London) and Tim Brümmendorf (Aachen)

17:05-17:10	Introduction	Inderjeet Dokal (London) Tim Brümmendorf (Aachen)
17:10-17:25	Telomeres biology in hematopoietic stem cell disorders	Tim Brümmendorf (Aachen)
17:25-17:30	*Discussion*	
17:30-17:45	Disease progression and clinical outcomes in TBD	Sharon Savage (Bethesda)
17:45-17:50	*Discussion*	
17:50-18:05	New IBMFs	Thierry Leblanc (Paris)
18:05-18:10	*Discussion*	
18:10-18:25	Clinical and biological aspects of SAMD9 & SAMD9L syndromes	Marcin Wlodarski (Memphis)
18:25-18:30	*Discussion*	
18:30-19:00	*Panel discussion*	

19:00-20:10 **Mentored Poster walk – Welcome Get-Together Buffet for All**
Poster Walk Leaders: Beatrice Drexler (Basel), Emma Groarke (Bethesda)

Saturday, November 19, 2022

8:20-09:20 **Simultaneous Meet the Expert sessions**

1.	Role of Next Generation Sequencing in the diagnosis and follow-up of patients with BMF	Austin Kulasekararaj (London) Jean Soulier (Paris)
2.	Management of challenging situations in PNH	Jens Panse (Aachen) Morag Griffin (Leeds)
3.	How to properly diagnose and treat telomere biology disorders in children and adults	Tim Brümmendorf (Aachen) Inderjeet Dokal (London)

09:30-10:30 **SATELLITE SYMPOSIUM**
What we have learnt in the last 20 years managing patients with PNH
Chair & Speaker: Régis Peffault de Latour (Paris)
Speaker: Shreyans Gandhi (London)

This symposium is organized and sponsored by by Alexion, AstraZeneca Rare Disease

10:15-10:40 ***Coffee Break***

SESSION V – Immune aplastic anaemia and paroxysmal nocturnal haemoglobinuria (PNH)
1. pathophysiology and diagnosis
Chairs: Philip Scheinberg (São Paulo) and Jens Panse (Aachen)

10:40-10:45	Introduction	Philip Scheinberg (São Paulo) Jens Panse (Aachen)

10:45-11:15	**Keynote Lecture:** New clues in AA pathophysiology	Neal Young (Bethesda)
11:15-11:25	*Discussion*	
11:25-11:40	Human leucocyte antigen in aplastic anemia: cause or consequence?	Simona Pagliuca (Nancy)
11:40-11:45	*Discussion*	
11:45-12:00	PNH: a model of adaptation	Lucio Luzzatto (Florence)
12:00-12:05	*Discussion*	
12:05-12:20	Advancing therapeutic complement inhibition from PNH to Cold Agglutinin Disease (CAD)	Alexander Röth (Essen)
12:20-12:25	*Discussion*	
12:25-12:32	**Selected brief oral communication** *High-dimensional mass cytometric analysis of the bone marrow compartment identifies a disease-specific immune cell network in aplastic anemia*	Emma S. Pool (Leiden)
12:32-12:35	*Discussion*	
12:35-13:05	*Panel discussion*	
13:05-14:45	***Lunch***	

13:35-14:35 **SATELLITE SYMPOSIUM**

Switching gear: Redefining treatment goals in PNH

Chair: Véronique Fremeaux-Bacchi (Paris)

Speakers: Austin Kulasekararaj (London), Jeff Szer (Melbourne)

This symposium is organized and sponsored by Sobi

SESSION VI – Idiopathic aplastic anaemia and paroxysmal nocturnal haemoglobinuria (PNH)
2. differential diagnosis and clonal evolution
Chairs: Regis Peffault de Latour (Paris) and Jaroslaw P. Maciejewski (Cleveland)

14:45-14:50	Introduction	Regis Peffault de Latour (Paris) Jaroslaw P. Maciejewski (Cleveland)
14:50-15:05	Innate immunity and clonal evolution in bone marrow failure	Jaroslaw P. Maciejewski (Cleveland)
15:05-15:10	*Discussion*	
15:10-15:25	Somatic mutations in AA: what we learned from RACE	Austin Kulasekararaj (London)
15:25-15:30	*Discussion*	
15:30-15:45	Malignant clonal evolutions in AA: any predictors	Philip Scheinberg (São Paulo)
15:45-15:50	*Discussion*	
15:50-15:57	**Selected brief oral communication** *Immune cell landscape of aplastic anemia reveals resemblance of innate to adaptive immunity and epitope-specific T cell signatures*	Sofie Lundgren (Helsinki)
15:57-16:00	*Discussion*	

16:00-16:30 *Panel discussion*

16:30-17:00 Coffee Break

SESSION VII – Treatment of immune aplastic anaemia (AA)
Chairs: Antonio Risitano (Avellino) and Beatrice Drexler (Basel)

17:00-17:05	Introduction	Antonio Risitano (Avellino) Beatrice Drexler (Basel)
17:05-17:20	The revolution of TPO agonists	Régis Peffault de Latour (Paris)
17:20-17:25	*Discussion*	
17:25-17:40	Transplantation for acquired SAA: when and how	Andrea Bacigalupo (Rome)
17:40-17:45	*Discussion*	
17:45-18:00	SAA in pediatric patients: transplant or IST?	Sujith Samarasinghe (London)
18:00-18:05	*Discussion*	
18:05-18:12	**Selected brief oral communication** *The transcription factor ETS-1 correlates with the increased age associated B cells in patients with acquired aplastic anemia*	Elena Solomou (Patras)
18:12-18:15	*Discussion*	
18:15-18:45	*Panel discussion*	

Sunday, November 20, 2022

07:50-08:50 Simultaneous Meet the Expert sessions

1.	Source and sequence of BMT in acquired aplastic anaemia	Andrea Bacigalupo (Rome) Hubert Schrezenmeier (Ulm)
2.	Management of pediatric BMFS both inherited and aquired	Marcin Wlodarski (Memphis) Carlo Dufour (Genova)
3.	Surveillance of leukaemia predisposition in bone marrow failure: How, and when to act?	Lucy Godley (Chicago) Emma Groarke (Bethesda)

09:00-10:00 **SATELLITE SYMPOSIUM**

Solving the puzzle of cold agglutinin disease (CAD): Clues, conundrums and a clinical presentation

Speakers: Antonio Risitano (Avellino), Alexander Röth (Essen)

This symposium is organized and sponsored by Sanofi

SESSION VIII – Treatment of paroxysmal nocturnal haemoglobinuria (PNH)
Chairs: Hubert Schrezenmeier (Ulm)

10:10-10:15	Introduction	Hubert Schrezenmeier (Ulm)
10:15-10:30	Classical treatment of PNH: terminal inhibitors	Flore Sicre de Fontbrune (Paris)
10:30-10:35	*Discussion*	
10:35-10:50	Challenging situations	Morag Griffin (Leeds)
10:50-10:55	*Discussion*	
10:55-11:10	Proximal complement inhibitors	Antonio Risitano (Avellino)
11:10-11:15	*Discussion*	
11:15-11:22	**Selected brief oral communication** *Evaluation of pegcetacoplan in paroxysmal nocturnal haemoglobinuria patients with aplastic anaemia*	Regis Peffault de Latour(Paris)
11:22-11:25	*Discussion*	
11:25-11:55	*Panel discussion*	
11:55-12:25	***Coffee Break***	

SESSION IX – Expanding Areas in BMFS
Chairs: Marc H.G.P. Raaijmakers (Rotterdam) and Lucy Godley (Chicago)

12:25-12:30	Introduction	Marc H.G.P. Raaijmakers (Rotterdam) Lucy Godley (Chicago)
12:30-12:45	Inflammation: Linking tissue failure to clonal evolution	Marc H.G.P. Raaijmakers (Rotterdam)
12:45-12:50	*Discussion*	
12:50-13:05	Immunederangement in AA	Shahram Kordasti (London)
13:05-13:10	*Discussion*	
13:10-13:25	Role of Complement in COVID-19	Rodrigo Calado (São Paulo)
13:25-13:30	*Discussion*	
13:30-13:37	**Selected brief oral communication** *Clinical, humanistic, and economic burden in patients with paroxysmal nocturnal haemoglobinuria across the United Kingdom, Germany, and France: insights from the commodore burden of illness study*	Louis Terriou (Lille)
13:37-13:40	*Discussion*	
13:40-14:10	*Panel discussion*	
14:10-14:20	***Closing Remarks***	Conference chairs

MEETING CLOSURE