ESH-EBMT-EHA-IPIG 3rd Translational Research Conference
BONE MARROW FAILURE AND LEUKAEMIA PREDISPOSITION SYNDROMES
Paris, France - November 15-17, 2024
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 and the EHA SWG on Granulocyte and Bone Marrow Failures and the International PNH Interest Group

Friday, November 15, 2024

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

SESSION I – Stem cell biology
Chairs: Michael Milsom (Heidelberg), Satu Mustjoki (Helsinki)

08:40-09:00	Inflammation and stem cell reserve	Michael Milsom (Heidelberg)
09:00-09:20	Stem cell ageing	Maria Carolina Florian (Barcelona)
09:20-09:40	Role of CHIP in allogeneic SCT/bone marrow failure	Coleman Lindsley (Boston)
09:40-10:10	*Panel discussion*	
10:10-10:40	***Coffee Break***	

SESSION II – Inherited predisposition to leukaemia
Chairs: Simone Feurstein (Heidelberg), Jean Soulier (Paris)

10:40-11:00	Germeline predisposition to leukaemia: challenges in variant interpretation	Simone Feurstein (Heidelberg)
11:00-11:20	Genome-first approaches to uncovering leukaemia etiology	Lisa McReynolds (Bethesda)
11:20-11:40	Mechanisms of familial predisposition in MPN	Steffen Koschmieder (Aachen)
11:40-12:00	Secondary leukemia in inherited BMF with stressed hematopoiesis	Jean Soulier (Paris)
12:00-12:30	*Panel discussion*	
12:30-14:30	***Lunch***	

13:20-14:20 **SATELLITE SYMPOSIUM**

Adding new dimensions to proximal inhibition: *Transforming paroxysmal nocturnal haemoglobinuria (PNH) management*

Chair & Speaker: Regis Peffault de Latour (Paris), Richard Kelly (Leeds)

This symposium is organised and supported by Sobi

SESSION III – Constitutional marrow failure 2
Chairs: Marena Niewisch (Hannover), Marcin Wlodarski (Memphis)

14:30-14:50	What is new in GATA2?	Miriam Erlacher (Freiburg)
14:50-15:10	The molecular interplay of clonal evolution in ribosomal disorders	Alan Warren (Cambridge)
15:10-15:30	New approaches to Diamond-Blackfan Anemia	Deena Iskander (London)
15:30-15:50	Non transplant strategies for inherited BMFS	Simona Pagliuca (Nancy)
15:50-16:30	*Panel discussion: Does everyone need a genetic diagnosis?*	Marena Niewisch (Hannover)
16:30-17:00	***Coffee break***	

SESSION IV – Constitutional marrow failure 3: telomere biology diseases and other...
Chairs: Sharon Savage (Bethesda), Tim Brümmendorf (Aachen)

17:00-17:20	How telomere shortening in the gut causes systemic aging	Miguel Godinho Ferreira (Nice)
17:20-17:40	Clonal signatures of telomere biology disorders	Fernanda Rodrigues (Bethesda)
17:40-18:00	Diagnostics algorithms and therapy of TBDs in adults	Fabian Beier (Aachen)
18:00-18:20	Non-hematopoietic complications of telomere biology disorders in adolescents and young adults	Sharon Savage (Bethesda)
18:20-18:50	*Panel discussion*	

19:00-20:30 **Mentored poster walk - Welcome get-together cocktail for all**
Biology Leaders: Thierry Leblanc (Paris), Satu Mustjoki (Helsinki)
Clinical Leaders: Beatrice Drexler (Basel), Morag Griffin (Leeds)

Saturday, November 16, 2024

08:00-09:00 **3 simultaneous Meet the Experts sessions**

1. Management of hemolytic anemias — Morag Griffin (Leeds), Hubert Schrezenmeier (Ulm)
2. Choosing therapies: differences in pediatric and adult AA patients — Beatrice Drexler (Basel), Carlo Dufour (Genova)

3. How molecular findings may inform therapeutical decisions — Gérard Socié (Paris), Jaroslaw Maciejewski (Cleveland)

09:10-09:40 **SATELLITE SYMPOSIUM**

Real-world insights and implications for managing elderly patients with aplastic anemia

Chair: Beatrice Drexler (Basel)

Speaker: Morag Griffin (Leeds)

This symposium is organised and supported by Pfizer

SESSION V – Top scoring abstracts
Chairs: Carmem Bonfim (Durham), Tim Brümmendorf (Aachen)

09:50-10:00	Selected brief oral communication Pilot prospective study of reduced intensity conditioning allogeneic hematopoietic stem cell transplantation for patients with VEXAS syndrome	Abhishek Mangaonkar (Rochester)
10:00-10:03	*Discussion*	
10:03-10:13	Selected brief oral communication Lentiviral-mediated gene therapy (RP-L102) for fanconi anemia [Group A] is associated with polyclonal integration patterns in the absence of conditioning	Julian Sevilla (Madrid)
10:13-10:16	*Discussion*	
10:16-10:26	Selected brief oral communication Understanding the role of the R525H DDX41 somatic variant in inducing leukaemia in individuals with DDX41 mutant germline predisposition syndrome	Ludovica Marando (Rochester)
10:26-10:29	*Discussion*	
10:29-10:39	Selected brief oral communication Interferon-gamma activity in impaired haematopoietic stem cell (HSC) proliferation and graft-versus-host disease	Régis Peffault De Latour (Paris)
10:39-10:42	*Discussion*	
10:42-10:52	Selected brief oral communication Natural history and predictors of survival in adult patients with telomere biology disorders	Abhishek Mangaonkar (Rochester)
10:52-10:55	*Discussion*	
10:55-11:05	Selected brief oral communication Somatic *TP53* mutations rescue the hematopoietic stem and progenitor cell defect at the expense of increased risk for Myeloid Malignancies in ERCC6L2-associated inherited Bone Marrow Failure Syndrome	Roman Schimmer (Zurich)
11:05-11:08	*Discussion*	

11:08-11:35 ***Coffee break***

SESSION VI – AA & PNH: 1. pathophysiology and diagnosis
Chairs: Austin Kulasekararaj (London), Regis Peffault de Latour (Paris)

11:35-11:55	New vs old pathophysiologic concepts in AA	Jaroslaw Maciejewski (Cleveland)
11:55-12:15	Deciphering the bone marrow microenvironment and TCR signature in AA	Satu Mustjoki (Helsinki)
12:15-12:35	Recognizing Inherited marrow failure disorders in adult age	Carlo Dufour (Genova)
12:35-13:05	*Panel discussion*	
13:05-15:05	***Lunch***	
13:55-14:55	**SATELLITE SYMPOSIUM** **Advances in the management of patients with PNH** **Speakers**: Regis Peffault de Latour (Paris), Austin Kulasekararaj (London) *This symposium is organised and supported by Alexion*	

SESSION VII – AA & PNH: 2. CHIP and clonal evolution in BMFS
Chairs: Jaroslaw Maciejewski (Cleveland), Gérard Socié (Paris)

15:05-15:25	Clonal dynamics of hematopoietic stem cell compartment in aplastic anemia	Valeria Visconte (Cleveland)
15:25-15:45	The meaning of somatic mutations in acquired AA	Austin Kulasekararaj (London)
15:45-16:05	HSCT in clonal evolution after SAA	Pedro Henrique De Lima Prata (Limoges)
16:05-16:35	*Panel discussion*	
16:35-17:05	***Coffee break***	

SESSION VIII – Treatment of aplastic anemia
Chairs: Beatrice Drexler (Basel), Neal Young (Bethesda)

17:05-17:25	Triple therapy and beyond	Emma Groarke (Bethesda)
17:25-17:45	Treatment of moderate aplastic anemia	Britta Höchsmann (Ulm)
17:45-18:05	Alternative BMT - unrelated	Andrea Bacigalupo (Rome)
18:05-18:25	Alternative HCT - Haplo	Carmem Bonfim (Durham)
18:25-18:55	*Panel discussion*	

Sunday, November 17, 2024

08:00-09:00 Roundtable: Hot treatment decisions in aplastic anaemia
Chairs: Antonio Risitano (Avellino), Carmem Bonfim (Durham)
Speakers: Beatrice Drexler (Basel), Gérard Socié (Paris), Regis Peffault de Latour (Paris), Neal Young (Bethesda), Andrea Bacigalupo (Rome), Carlo Dufour (Genova), Britta Höchsmann (Ulm)

SESSION IX – Treatment of PNH
Chairs: Morag Griffin (Leeds), Hubert Schrezenmeier (Ulm)

09:05-09:25	Inherited complement deficiencies: any lesson for pharmacological complement inhibition?	Ronald Taylor (Charlottesville)
09:25-09:45	Bringing complement inhibitors in the clinic: a 30-year journey	John Lambris (Philadelphia)
09:45-10:05	Novel treatments for PNH: has a new era started?	Regis Peffault de Latour (Paris)
10:05-10:25	Residual hemolysis on complement inhibitors: climbing our learning curve	Antonio Risitano (Avellino)
10:25-10:55	*Panel discussion*	
10:55-11:25	***Coffee break***	

SESSION X – Expanding areas in BMFS
Chairs: Carlo Dufour (Genova), Fabian Beier (Aachen)

11:25-11:45	BMF and immune deficiencies	Maurizio Miano (Genova)
11:45-12:05	Cold agglutinin disease – current status and future prospects	Sigbjørn Berentsen (Haugesund)
12:05-12:25	VEXAS	Bhavisha Patel (Bethesda)
12:25-12:45	Branching trajectories and diversification of clonal escape in aplastic anemia revealed by single-cell genomics	Marcin Wlodarski (Memphis)
12:45-13:15	*Panel discussion*	
13:15-13:25	***Closing remarks***	Conference chairs

MEETING CLOSURE