

Wednesday, 11 September 2024

14:00 – 18:00 **Severe Aplastic Anaemia & Haemoglobinopathies WP Scientific Business Meeting**
Open for everybody

Thursday, 12 September 2024

09:00 – 10:30 **Session I: Update on diagnosis, pathophysiology, and clinical presentation of hemoglobinopathies**

Chairs: Annalisa Ruggeri (IT) & Emanuele Angelucci (IT)

09:00 – 09:10 Welcome and Introduction

Emanuele Angelucci (IT)

09:10 – 09:30 TDT medical care: today's outcome in different parts of the world and unmet medical need + Q&A

Gian Luca Forni (IT)

09:30 – 09:50 SCD medical care: today's outcome in different parts of the world and unmet medical need + Q&A

09:50 – 10:10 Work-up for HCT and gene therapy in hemoglobinopathies + Q&A

Donatella Baronciani (IT)

10:10 – 10:30 Discussion

10:30 – 11:00 **Coffee Break**

11:00 – 11:45 **Industry Symposium "Practical considerations for patients with haemoglobinopathies on the haematopoietic stem cell transplant (HSCT) journey" by Vertex**

11:00 – 11:10 Welcome and introduction

11:10 – 11:30 Practical considerations for patients with haemoglobinopathies on the transplant journey

11:30 – 11:40 Panel discussion

11:40 – 11:45 Summary and close

Further information will be available soon

11:45 – 13:00 **Session II: Update on diagnosis, pathophysiology, natural history, and treatment of bone marrow failure syndromes: listening to the dinosaurs**

Chair: André Tichelli (CH) & Antonio M. Risitano (IT)

11:45 – 12:05 50 years of AA: how treatment outcomes have evolved + Q&A

Andrea Bacigalupo (IT)

12:05 – 12:25 Changing 'face' of BMF in Children + Q&A

Carlo Dufour (IT)

12:25 – 12:45 Long-term outcome of AA patients + Q&A

12:45 – 13:00 Round table: treatment decision in SAA + Q&A

13:00 – 14:00 **Lunch Break**

14:00 – 15:20 **Session III: Transplantation for hemoglobinopathies and bone marrow failure syndromes**

Chair: André Tichelli (CH) & Josu de la Fuente (UK)

14:00 – 14:20 Transplantation for TDT: where we are now + Q&A

Sarita Jaiswal (IN)

14:20 – 14:40 Transplantation for SCD: where we are now + Q&A

Adetola Kassim (USA)

14:40 – 14:55 Transplantation for acquired SAA + Q&A

Beatrice Drexler (CH)

14:55 – 15:15 Transplantation in inherited BMF (role of alternate donors, first line MUD in young adults, refractory to IST- is there an age limit?) + Q&A

Maurizio Miano (IT)

15:15 – 15:45 **Coffee Break**

15:45 – 17:45	Session IV: Debate non-transplant treatments for AA and PNH Chair: Régis Peffault de Latour (FR) & Alicia Rovó (CH)	
15:45 – 16:05	Overview	Régis Peffault de Latour (FR)
16:05 – 16:25	Triple therapy for AA: updated results (EPAG in children)	
16:25 – 16:45	Treatment of AA in Elderly + Q&A	Constantijn Halkes (NL)
16:45 – 17:05	Treatment for moderate AA: when, why, and how (definition and treatment options) + Q&A	Britta Höchsmann (DE)
17:05 – 17:25	Treatment of PNH: an update (anti-C + HSCT) + Q&A	Camilla Frieri (IT)
17:25 – 17:45	Eltrombopag (and Cyclosporin) 'dependence' (definition of relapse) + Q&A	Alicia Rovó (CH)

Friday, 13 September 2024

08:00 – 09:15	Session V: Innovative cell therapies: Gene therapy, induced pluripotent stem cells, and CAR-T Chair: Fabio Ciceri (IT) & Mattia Algeri (IT)	
08:00 – 08:20	Gene therapy status of art in Haemoglobinopathies + Q&A	Josu de la Fuente (UK)
08:20 – 08:40	Innovation in Gene therapy + Q&A	Alice Bertaina (IT)
08:40 – 09:00	Gene editing therapy for hemoglobinopathies: lessons learned from the clinical trials that can be translated to future real-world use + Q&A	
09:00 – 09:15	Round table on gene therapy today: availability, costs, and priority criteria	Franco Locatelli (IT)
09:15 – 09:45	Coffee Break	
09:45 – 11:45	Session VI: Hot topics: the unknowns Chair: Simona Pagliuca (FR)	
09:45 – 10:05	Overview	
10:05 – 10:25	Mutations in AA- Transplant or not? Is it MDS or AA? How often do you monitor? Frequency of marrow assessment? + Q&A	
10:25 – 10:45	Clonal dynamics of hematopoiesis after HSCT & Gene Therapy + Q&A	Michael S. Chapman (UK)
10:45 – 11:05	Role of Upfront Haplo- Is this the 'new' standard in AA? + Q&A	
11:05 – 11:25	How to manage and monitor chimerism in TDT/SCD? + Q&A	Selim Corbacioglu (DE)
11:25 – 11:45	How to manage and monitor chimerism in AA? Role of mixed chimerism (including poor graft function and graft failure) + Q&A	
11:45 – 12:00	Late effects after HSCT for TCD/SCD + Q&A	Erfan Nur (NL)
12:00 – 12:45	Lunch Break	
12:45 – 14:45	Session VII: Status of art in emerging geographic areas, and special topics in cell therapies for non-malignant hematology Chair: Per Ljungman (SE) & John Snowden (UK)	
12:45 – 13:05	New understanding of iron-related toxicity & Iron toxicity and the hemopoietic stem cell (AA and hemoglobinopathies) + Q&A	Federica Pilo (IT)
13:05 – 13:25	Biology of Fanconi anaemia + Q&A	
13:25 – 13:45	Update on telomeropathies + Q&A	Fabian Beier (DE)
13:45 – 14:05	New entities in acquired BMF + Q&A	
14:05 – 14:25	Fertility in patients transplanted for AA and hemoglobinopathies + Q&A	
14:25 – 14:45	Luspatercept and ESA for hemoglobinopathies + Q&A	
14:05 – 15:05	Status of art in emerging geographic areas, and special topics in cell therapies for non-malignant haematology	Mahmoud Aljurf (SA)
15:05 – 15:15	Take home messages & conclusions	