

Wednesday, 11 September 2024

14:00 - 18:00 **Severe Aplastic Anaemia Working Party & Haemoglobinopathies Working Party Scientific Business Meetings**
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 Patient and family counseling for HCT and GT (peds and adults) + Q&A

10:30 - 11:00 **Coffee Break**

11:00 - 11:45 **Industry Symposium**

11:45 - 13:05 **Session II: Update on diagnosis, pathophysiology, natural history, and treatment of bone marrow failure syndromes: listening to the dinosaurs**

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

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

12:45 - 13:05 Round table: treatment decision in SAA + Q&A

13:05 - 13:45 **Lunch Break**

13:45 - 14:30 **Industry Symposium**

14:30 - 15:40 **Session III: Transplantation for hemoglobinopathies and bone marrow failure syndromes**
Chair: André Tichelli (CH)

14:30 - 14:50 Transplantation for TDT: where we are now + Q&A

Sarita Jaiswal (IN)

14:50 - 15:10 Transplantation for SCD: where we are now + Q&A

Adetola Kassim (USA)

15:10 - 15:20 Transplantation for acquired SAA + Q&A

Beatrice Drexler (CH)

15:20 - 15:40 Transplantation in inherited BMF (role of alternate donors, first line MUD in young adults, refractory to IST- is there an age limit?) + Q&A

15:40 - 16:10 **Coffee Break**

16:10 – 18:10	Session IV: Debate non-transplant treatments for AA and PNH Chair: Régis Peffault de Latour (FR) & Alicia Rovó (CH)	
16:10 – 16:30	Overview	Régis Peffault de Latour (FR)
16:30 – 16:50	Triple therapy for AA: updated results (EPAG in children)	
16:50 – 17:10	Treatment of AA in Elderly + Q&A	Constantijn Halkes (NL)
17:10 – 17:30	Treatment for moderate AA: when, why, and how (definition and treatment options) + Q&A	
17:30 – 17:50	Treatment of PNH: an update (anti-C + HSCT) + Q&A	Camilla Frieri (IT)
17:50 – 18:10	Eltrombopag (and Cyclosporin) 'dependence' (definition of relapse) + Q&A	Alicia Rovó (CH)

Friday, 13 September 2024

08:00 – 09:20	Session V: Innovative cell therapies: Gene therapy, induced pluripotent stem cells, and CAR-T Chair: Fabio Ciceri (IT)	
08:00 – 08:20	Gene therapy status of art in Haemoglobinopathies + Q&A	
08:20 – 08:40	Innovation in Gene therapy + Q&A	
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	Franco Locatelli (IT)
09:00 – 09:20	Round table on gene therapy today: availability, costs, and priority criteria	
09:20 – 09:50	<i>Coffee Break</i>	
09:50 – 10:35	Industry Symposium	
10:35 – 12:35	Session VI: Hot topics: the unknowns	
10:35 – 10:55	Overview	
10:55 – 11:15	Mutations in AA- Transplant or not? Is it MDS or AA? How often do you monitor? Frequency of marrow assessment? + Q&A	
11:15 – 11:35	Clonal dynamics of hematopoiesis after HSCT & Gene Therapy + Q&A	
11:35 – 11:55	Role of Upfront Haplo- Is this the 'new' standard in AA? + Q&A	
11:55 – 12:15	How to manage and monitor chimerism in TDT/SCD? + Q&A	Selim Corbacioglu (DE)
12:15 – 12:35	How to manage and monitor chimerism in AA? Role of mixed chimerism (including poor graft function and graft failure) + Q&A	
12:35 – 13:15	<i>Lunch Break</i>	

13:15 - 15:35	Session VII: Status of art in emerging geographic areas, and special topics in cell therapies for non-malignant hematology Chair: <i>Per Ljungman (SE) & John Snowden (UK)</i>	
13:15 - 13:35	New understanding of iron-related toxicity & Iron toxicity and the hemopoietic stem cell (AA and hemoglobinopathies) + Q&A	Federica Pilo (IT)
13:35 - 13:55	Late effects after HSCT for TCD/SCD + Q&A	Erfan Nur (NL)
13:55 - 14:15	Biology of Fanconi anaemia + Q&A	
14:15 - 14:35	Update on telomeropathies + Q&A	
14:35 - 14:55	New entities in acquired BMF + Q&A	
14:55 - 15:15	Fertility in patients transplanted for AA and hemoglobinopathies + Q&A	
15:15 - 15:35	Luspatercept and ESA for hemoglobinopathies + Q&A	
15:35 - 15:55	Status of art in emerging geographic areas, and special topics in cell therapies for non-malignant haematology	Mahmoud Aljurf (SA)
15:55 - 16:00	Take home messages & conclusions	