



Cellular Therapies in Non-Malignant Haematology

Genova, Italy
11-13 September 2024

WELCOME

On behalf of the EBMT, we are very pleased to invite you to a Joint Educational Meeting on Cellular Therapies in Non-Malignant Haematology organized by the Severe Aplastic Anaemia and Haemoglobinopathies Working Parties. The programme has been designed to cover the main aspects of pathophysiology, diagnosis and mostly treatment of bone marrow failure syndromes and haemoglobinopathies. These topics will include the management of these diseases, with special attention to haematopoietic stem cell transplantation, gene therapy, and other innovative non-transplant treatment strategies (such as immunosuppressive treatment for aplastic anaemia and complement inhibition for PNH).

The meeting includes two and half educational days: the first half day will be dedicated to Scientific Business Meetings of the WPs, to provide an update on relevant ongoing studies and new projects. The Scientific Business Meetings are open to any participant who wishes to be informed about and to participate actively in the Working Parties. The other two days will be devoted to educational lectures, with 7 sessions dealing with the most relevant topics of the field, In keeping with the scientific scope of the WPs and with the Educational purpose of the course, the meeting will allow participants to discuss and interact with all these experts during the sessions and the social events.

This stimulating Scientific programme will bring together the world's leading experts in the field, who are actively contributing to improving our knowledge in the field of bone marrow failure syndromes and haemoglobinopathies.

We look forward to seeing you in Genova for this unique and exciting course from the EBMT Severe Aplastic Anaemia and Haemoglobinopathies Working Parties.

Emanuele Angelucci, *Haemoglobinopathies Working Party Chair*
Josu de la Fuente, *Haemoglobinopathies Working Party Secretary*
Sandrine Bremathas, *Haemoglobinopathies Working Party Nurse*
Antonio Risitano, *Severe Aplastic Anaemia Working Party Chair*
Austin Kulasekararaj, *Severe Aplastic Anaemia Working Party Secretary*
Kelly Hennessy, *Severe Aplastic Anaemia Working Party Nurse*

Wednesday, 11 September 2024

- 14:00 – 18:00** **Severe Aplastic Anaemia Working Party Scientific Business Meeting**
Open for everybody
- 16:00 – 18:00** **Haemoglobinopathies Working Party 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 Josu de la Fuente (UK)
- 11:10 – 11:30** Practical considerations for patients with haemoglobinopathies on the transplant journey Mattia Algeri (IT)
- 11:30 – 11:40** Panel discussion
- 11:40 – 11:45** Summary and close

- 11:45 – 13:00** **Session II: Update on diagnosis, pathophysiology, natural history, and treatment of bone marrow failure syndromes: listening to the dinosaurs**
Chairs: *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 Régis Peffault de Latour (FR)
- 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**
Chairs: *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 Chairs: Antonio M. Risitano (IT) & 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)	Flore Sicre de Fontbrune (FR)
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* <i>This lecture is kindly supported by Novartis</i>	Camilla Frieri (IT)
17:25 – 17:45	Eltrombopag (and Cyclosporin) 'dependence' (definition of relapse) + Q&A	Alicia Rovó (CH)

* Novartis has had no involvement in the selection, development, content or organization of this lecture.

Friday, 13 September 2024

08:00 – 09:15	Session V: Innovative cell therapies: Gene therapy, induced pluripotent stem cells, and CAR-T Chairs: 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	Franco Locatelli (IT)
09:00 – 09:15	Round table on gene therapy today: availability, costs, and priority criteria	
09:15 – 09:45	Coffee Break	
09:45 – 11:45	Session VI: Hot topics: the unknowns Chair: Carlo Dufour (IT) & Austin Kulasekeraraj (UK)	
09:45 – 10:05	Overview	Austin Kulasekeraraj (UK)
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	M. Teresa Lupo-Stanghellini (IT)
10:25 – 10:45	Clonal dynamics of hematopoiesis after HSCT & Gene Therapy + Q&A	Michael Spencer Chapman (UK)
10:45 – 11:05	Role of Upfront Haplo- Is this the 'new' standard in AA? + Q&A	Pedro Prata (FR)
11:05 – 11:25	Haplo transplant for hemoglobinopathies + 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	Victoria Potter (UK)
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 Chairs: 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	Paula Rio (SP)
13:25 – 13:45	Update on telomeropathies + Q&A	Fabian Beier (DE)
13:45 – 14:05	How to manage and monitor chimerism in TDT and SCD + Q&A	Selim Corbacioglu (DE)
14:05 – 14:25	Luspatercept and ESA for hemoglobinopathies + Q&A	Maria Domenica Capellini (IT)
14:25 – 14:45	Status of art in emerging geographic areas, and special topics in cell therapies for non-malignant haematology	Mahmoud Aljurf (SA)
14:45 – 15:00	Take home messages & conclusions	



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Nurses' Track

Friday, 13 September in Genova, Italy
Starhotels President
Corte dei Lambruschini, 4, 16129 Genova

**An application for Italian CME credits has been made as follows:
Provider Studio ER Congressi SRL: ID 828- 427242
No. 5 CME credits for nurses and pediatric nurses**

Educational objective: Integrazione interprofessionale e multiprofessionale, interistituzionale

08:00 – 09:15	Session I: Bone Marrow Failure Syndromes (part I) Chairs: Kelly Hennessy (UK) & Sandrine Bremathas (UK)	
08:00 - 08:05	Introductions	
08:05 - 08:25	PNH: what is it? How do we treat it?	Kelly Hennessy (UK)
08:25 - 08:45	Understanding clonal evolution	Sandrine Bremathas (UK)
08:45 - 09:05	Diamond Blackfan Anaemia: diagnosis, pathophysiology & treatment	Joanna Large (UK)
09:05 - 09:15	Discussions and Q&A	Austin Kulasekararaj (UK)
		Yvonne Harrington (UK)
09:15 – 09:45	Coffee Break	
09:45 – 11:00	Session I: Bone Marrow Failure Syndromes (part II) Chairs: Kelly Hennessy (UK) & Sandrine Bremathas (UK)	
09:45 - 10:05	Supportive care in bone marrow failure syndromes	
10:05 - 10:25	Infections risks and management in aplastic anaemia	Marco Deiana (IT)
10:25 - 10:45	Aplastic anaemia: when to transplant?	Simona Calza (IT)
10:45 - 11:00	Discussions and Q&A	Malgorzata Mikulska (IT)
		Antonio Risitano (IT)
11:00 – 11:45	Session II: Case Presentations & Discussion Chairs: Marco Deiana (IT) & Simona Calza (IT)	
11:00 - 11:05	Introductions	
11:05 - 11:25	Fertility: What are the options?	Marco Deiana (IT)
11:25 - 11:45	Life after transplant	Simona Calza (IT)
		Nicholas Alexander (UK)
		Emilia Lerosé (IT)
11:45 – 12:30	Lunch Break	
12:30 – 15:10	Session III: Haemoglobinopathies Chairs: Kelly Hennessy (UK) & Sandrine Bremathas (UK)	
12:30 - 12:35	Introductions	
12:35 - 12:55	Supportive Care in haemoglobinopathies: SCD & TDT	Kelly Hennessy (UK)
12:55 - 13:15	Indication for HSCT: SCD & TDT	Sandrine Bremathas (UK)
13:15 - 13:35	Gene therapy: is this the way forward?	Charlotte van Tuijn (NL)
13:35 - 13:55	Preparation for HSCT & gene therapy	Hilda Mekelenkamp (NL)
13:55 - 14:10	Mobilisation and stem cell collections	Marjola Gjergji (IT)
14:10 - 14:40	Roundtable discussion: the nurse's perspective	Donatella Baronciani (IT)
14:40 - 14:45	Closing remarks	Kelly Hennessy (UK)

Scientific Committee:

Emanuele Angelucci, *Haemoglobinopathies Working Party Chair*
Josu de la Fuente, *Haemoglobinopathies Working Party Secretary*
Sandrine Bremathas, *Haemoglobinopathies Working Party Nurse*
Antonio Risitano, *Severe Aplastic Anaemia Working Party Chair*
Austin Kulasekararaj, *Severe Aplastic Anaemia Working Party Secretary*
Kelly Hennessy, *Severe Aplastic Anaemia Working Party Nurse*

**All registered participants and speakers will be given a badge which they must wear to be admitted to the scientific sessions.
A certificate will be given to all participants at the end of the meeting.**



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