14:00 - 18:00  Severe Aplastic Anaemia Working Party & Haemoglobinopathies Working Party Scientific Business Meetings
Open for everybody

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

09:00 - 09:10  Welcome and Introduction
09:10 - 09:30  TDT medical care: today's outcome in different parts of the world and unmet medical need + Q&A
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
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
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

14:30 - 14:50  Transplantation for TDT: where we are now + Q&A
14:50 - 15:10  Transplantation for SCD: where we are now + Q&A
15:10 - 15:20  Transplantation for acquired SAA + Q&A
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

For further information, please contact education.events@ebmt.org
### Session IV: Debate non-transplant treatments for AA and PNH

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#### Overview

- **16:10 - 16:30**
  - Overview

#### Triple therapy for AA: updated results (EPAG in children)

- **16:30 - 16:50**
  - Treatment of AA in Elderly + Q&A

#### Treatment for moderate AA: when, why, and how (definition and treatment options) + Q&A

- **17:10 - 17:30**
  - Treatment of PNH: an update (anti-C + HSCT) + Q&A

- **17:50 - 18:10**
  - Eltrombopag (and Cyclosporin) ‘dependence’ (definition of relapse) + Q&A

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### Session V: Innovative cell therapies: Gene therapy, induced pluripotent stem cells, and CAR-T

#### Chairs:

- **08:00 - 09:20**
  - Session V: Innovative cell therapies: Gene therapy, induced pluripotent stem cells, and CAR-T

#### Gene therapy status of art in Haemoglobinopathies + Q&A

- **08:00 - 08:20**
  - Gene therapy status of art in Haemoglobinopathies + Q&A

#### Innovation in Gene therapy + Q&A

- **08:20 - 08:40**
  - Innovation in Gene therapy + Q&A

#### Gene editing therapy for hemoglobinopathies: lessons learned from the clinical trials that can be translated to future real-world use + 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

#### Round table on gene therapy today: availability, costs, and priority criteria

- **09:00 - 09:20**
  - Round table on gene therapy today: availability, costs, and priority criteria

#### Coffee Break

- **09:20 - 09:50**
  - Coffee Break

#### Industry Symposium

- **09:50 - 10:35**
  - Industry Symposium

#### Session VI: Hot topics: the unknowns

- **10:35 - 12:35**
  - Session VI: Hot topics: the unknowns

#### Overview

- **10:35 - 10:55**
  - Overview

#### Mutations in AA- Transplant or not? Is it MDS or AA? How often do you monitor? Frequency of marrow assessment? + Q&A

- **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

#### Clonal dynamics of hematopoiesis after HSCT & Gene Therapy + Q&A

- **11:15 - 11:35**
  - Clonal dynamics of hematopoiesis after HSCT & Gene Therapy + Q&A

#### Role of Upfront Haplo- Is this the ‘new’ standard in AA? + Q&A

- **11:35 - 11:55**
  - Role of Upfront Haplo- Is this the ‘new’ standard in AA? + Q&A

#### How to manage and monitor chimerism in TDT/SCD? + Q&A

- **11:55 - 12:15**
  - How to manage and monitor chimerism in TDT/SCD? + Q&A

#### How to manage and monitor chimerism in AA? Role of mixed chimerism (including poor graft function and graft failure) + Q&A

- **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

#### Lunch Break

- **12:35 - 13:15**
  - Lunch Break

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13:15 - 15:35  Session VII: Status of art in emerging geographic areas, and special topics in cell therapies for non-malignant hematology

13:15 - 13:35  New understanding of iron-related toxicity & Iron toxicity and the hemopoietic stem cell (AA and hemoglobinopathies) + Q&A

13:35 - 13:55  Late effects after HSCT for TCD/SCD + Q&A

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  Transplantation outside the US and UE

15:55 - 16:00  Take home messages & conclusions