

Preliminary Programme

Genova, Italy
11-13 September 2024

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**

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**

16:10 – 18:10 Session IV: Debate non-transplant treatments for AA and PNH

16:10 – 16:30 Overview

16:30 – 16:50 Triple therapy for AA: updated results (EPAG in children)

16:50 – 17:10 Treatment of AA in Elderly + Q&A

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

17:50 – 18:10 Eltrombopag (and Cyclosporin) 'dependence' (definition of relapse) + Q&A

Friday, 13 September 2024

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

Chairs:

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

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

09:20 – 09:50 *Coffee Break*

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

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 *Lunch Break*

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**