Friday, 27 September 2024

13:00 – 13:45 Registration & Welcome Coffee
13:45 – 14:00 Welcome

14:00 – 15:35 Session I: Stem cell therapy in metabolic diseases (I)
14:00 – 14:15 Update on HSPC-GT for ML
14:15 – 14:30 Retrospective allo-HSCT data in ‘older’ Hurler patients
14:30 – 14:43 Update on HSPC-GT for MPSIH
14:43 – 14:56 Update on HSPC-GT for MPSII
14:56 – 15:09 Update on HSPC-GT for MPSIII A
15:09 – 15:19 Platform approach for LSD with skeletal involvement (MPSIVA, MPSIVB, alphaMAN)
15:19 – 15:35 Update on HSPC-GT for MLD

15:35 – 16:00 Coffee Break

16:00 – 17:30 Session I: Stem cell therapy in metabolic diseases (II)
16:00 – 16:15 Retrospective allo-HSCT data on Osteopetrosis
16:15 – 16:30 Lentiviral-based HSPC-GT for Osteopetrosis
16:30 – 17:30 To be decided

17:30 – 18:30 Session II: Pathophysiology of IEI and its role in cellular therapy
17:30 – 17:55 New molecular causes of SCID
17:55 – 18:15 STAT1 and STAT3 GOFs- JAKi, HSCT, or both?
18:15 – 18:30 In vitro assays for the assessment of thymic stromal cell defects

All participations include time for Q&A.
09:00 – 10:15 Session II: Infections (focus on viruses)

09:00 – 09:10 Letermovir PK, safety and efficacy data in children
09:10 – 09:20 Letermovir EBM study
09:20 – 09:35 Metagenomic NGS (London/Paris experience)
09:35 – 09:50 HPV infection in SCID and beyond
09:50 – 10:05 Enteric virus infection in the HSCT setting
10:05 – 10:15 To be decided

10:15 – 10:45 Coffee Break

10:45 – 11:15 Keynote Lecture
Gene therapy for IEI

11:15 – 12:45 Session IV: Gene therapy

11:15 – 11:35 Lentiviral gene therapy for XLA
11:35 – 11:45 Clinical trial of gene edited T cells for CD40L
11:45 – 11:55 Gene editing for RAG1-SCID
11:55 – 12:05 Lentiviral gene therapy for hypomorphic RAG
12:05 – 12:15 Clinical trial of T cell gene therapy for XLP
12:15 – 12:25 Update on clinical trial for p47-CGD
12:25 – 12:35 Artemis SCID ongoing trial update
12:35 – 12:45 To be decided

12:45 – 14:00 Lunch Break

14:00 – 14:45 Keynote Lecture
Genetics of Incomplete Penetrance

All participations include time for Q&A.

For further information, please contact education.events@ebmt.org
14:45 – 15:55 Session V: Challenging indications and novel approaches

14:45 – 14:55 Case presentation
14:55 – 15:15 Autoimmunity in patients with IEIs:
   - A challenge before, during, and post-HSCT
15:15 – 15:25 Case presentation
   - HSCT for SCID: a box of chocolate (or: you never know what you’re gonna get)
15:45 – 15:55 Case presentation

15:55 – 16:30 Coffee Break

16:30 – 17:30 Session VI: Characteristic diseases of the Finnish population

16:30 – 16:45 Clinical and immunological aspects of CHH
16:45 – 17:00 HSCT in CH
17:00 – 17:20 Mechanism-based therapeutic interventions for autoimmunity in APECED patients
17:20 – 17:30 To be decided

17:30 – 18:15 Session VII: IEWP Studies and proposals

17:30 – 17:40 CTLA4 T-cell gene therapy
17:40 – 17:55 Clinical practice guideline: Long-term follow-up in patients transplanted for IEI
17:55 – 18:00 TTC7A
18:00 – 18:15 haplo HSCT for CGD

All participations include time for Q&A.
09:00 – 10:10  Session VIII: HSCT for IEI in AYAs (focus on CVID)

09:00 – 09:10  Case presentation
09:10 – 09:30  Challenges with diagnosis of CVID and selection of patients for Allo SCT
09:30 – 09:50  GI & Liver disease in CVID and the role of liver transplantation
09:50 – 10:10  Recent CVID Allo SCT outcomes and proposed COCOA clinical study

10:10 – 11:20  Session IX – Haplo HSCT for IEI

10:10 – 10:35  PTCY or TCRαβ: takeaways from the IEWP study - a discussion
10:35 – 10:55  How to best use T-cell directed serotherapy in haplo HSCT
10:55 – 11:05  Real-time treosulfan PK
11:05 – 11:20  Adoptive cellular therapy with CD45RA depleted add-backs

11:20 – 11:50  Coffee Break

11:50 – 12:25  Session X: Bridging strategies to HSCT

11:50 – 12:00  CTLA4 fusion proteins as bridge to HSCT in LRBA deficiency
12:00 – 12:10  CTLA4 fusion proteins as bridge to HSCT in CTLA4 deficiency
12:10 – 12:25  HSCT in STAT1GOF with/without previous JAKi treatment
12:25 – 12:35  Leniolisib in APDS1/2 patients under named patient program in Europe
12:35 – 12:45  Emapalumab as a bridge to HSCT

12:45 – 13:00  Closing remarks

All participations include time for Q&A.