

Preliminary Programme

Helsinki, Finland
27-29 September 2024

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 MPSIIIA

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.

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Saturday, 28 September 2024

09:00 - 10:15 Session III: Infections (focus on viruses)

09:00 - 09:10 Letermovir PK, safety and efficacy data in children

09:10 - 09:20 Letermovir EBMT 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.

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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 IELs:
A challenge before, during, and post-HSCT

15:15 - 15:25 Case presentation

15:25 - 15:45 Immunological reconstitution post-secondary
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.

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Sunday, 29 September 2024

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 TCR $\alpha\beta$: 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.