



# Inborn Errors Working Party Annual Conference

## Preliminary Programme

Helsinki, Finland  
27-29 September 2024

**Friday, 27 September 2024**

- 13:00 – 13:45**    **Registration & Welcome Coffee**
- 13:45 – 14:00**    **Welcome**  
*Chairs: Bénédicte Neven (FR) & Helja Lang (FI)*
- 14:00 – 16:00**    **Session I: Stem cell therapy in metabolic diseases**  
*Chairs: María Ester Bernardo (IT) & Rob Wynn (UK)*
- 14:00 – 14:15    Update on HSPC-GT for MLD
- 14:15 – 14:30    Retrospective allo-HSCT data in 'older' Hurler patients
- 14:30 – 14:45    Update on HSPC-GT for MPSIH
- 14:45 – 15:00    Update on HSPC-GT for MPSII
- 15:00 – 15:15    Update on HSPC-GT for MPSIIIA
- 15:15 – 15:30    Retrospective allo-HSCT data on Osteopetrosis
- 15:30 – 15:45    Lentiviral-based HSPC-GT for Osteopetrosis
- 15:45 – 16:00    Discussion, Q&A
- 16:00 – 16:30**    **Coffee Break**
- 16:30 – 17:45**    **Session II: Pathophysiology of IEI and its role in cellular therapy**  
*Chairs: Mikko Seppänen (FI) & Thomas Fox (UK)*
- 16:30 – 17:45    New molecular causes of SCID

**Saturday, 28 September 2024**

- 09:00 – 10:15**    **Session III: Infections (focus on viruses)**  
*Chairs: Olaf Neth (ES) & Krzysztof Kalwak (PL)*
- 09:00 – 09:10    Letermovir PK, safety and efficacy data in children - NN
- 09:10 – 09:20    Letermovir EBMT study
- 09:20 – 09:35    Metagenomic NGS
- 09:35 – 09:50    HPV infection in SCID and beyond
- 09:50 – 10:05    Enteric virus infection in the HSCT setting
- 10:05 – 10:20    Discussion, Q&A
- 10:20 – 10:50**    **Coffee Break**

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- 10:50 – 11:20**    **Keynote Lecture**  
Gene therapy for IEI
- 11:20 – 12:50**    **Session IV: Gene therapy**  
*Chairs: Claire Booth (UK) & Sung-Yun Pai (US)*
- 11:20 – 11:40    Lentiviral gene therapy for XLA
- 11:40-11:50    Clinical trial of gene edited T cells for CD40L
- 11:50-12:00    Gene editing for RAG1-SCID
- 12:00-12:10    Lentiviral gene therapy for hypomorphic RAG
- 12:10-12:20    Clinical trial of T cell gene therapy for XLP
- 12:20-12:30    Update on clinical trial for p47-CGD
- 12:40-12:50    Artemis SCID ongoing trial update
- 12:50-13:05    Discussion, Q&A
- 13:05-14:00**    **Lunch Break**
- 14:00 – 14:45**    **Keynote Lecture**  
Genetics of Incomplete Penetrance
- 14:45 – 16:00**    **Session V: Challenging indications and novel approaches**  
*Chairs: Despina Moshous (FR) & Manfred Hönig (DE)*  
Case presentations + discussion
- 16:00– 16:30**    **Coffee Break**
- 16:30 – 17:30**    **Session VI: Characteristic diseases of the Finnish population**  
*Chairs: Helja Lang (FI) & Samppa Ryhänen (FI)*
- Clinical and immunological aspects of CHH
- HSCT in CHH
- APECED
- 17:30 – 18:15**    **Session VIII: IEWP Studies and proposals**  
*Chairs: Bénédicte Neven (FR) & Michael Albert (DE)*
- CTLA4 T-cell gene therapy

**Sunday, 29 September 2024**

**09:00 – 10:00** **Session IX: HSCT for IEI in AYAs (focus on COVID)**  
*Chairs: Emma Morris (UK) & Klaus Warnatz (DE)*

**10:00 – 11:00** **Session X – Haplo HSCT for IEI**  
*Chairs: Andy Gennery (UK) & Arjan Lankester (NL)*

PTCY or TCRab: advantages and pitfalls- a discussion

PTCY single center

TCRab single center

Treosulfan PK

**11:00 – 11:30** **Coffee Break**

**11:30 – 12:50** **Session XI: Bridging strategies to HSCT**  
*Chairs: Reem Elfeky (UK) & Carsten Speckmann (DE)*

11:30 – 11:40 CTLA4 fusion proteins as bridge to HSCT in LRBA deficiency

11:40-11:50 CTLA4 fusion proteins as bridge to HSCT in CTLA4 deficiency

11:50-12:05 HSCT in STAT1GOF with/without previous JAKi treatment

12:05-12:15 Leniolisib in APDS1/2 patients under named patient program in Europe

12:15-12:35 Emapalumab as a bridge to HSCT

12:35-12:50 Discussion, Q&A

**12:50-13:05** **Final remarks**  
*Chairs: Bénédicte Neven (FR) & Michael Albert (DE) & Helja Lang (FI)*