

### Friday, 27 September 2024

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

**13:45 – 14:00**      **Welcome**  
**Chairs:** *Bénédicte Neven (FR) & Heljä Lång (FI)*

**14:00 – 15:30**      **Session I: Stem cell therapy in metabolic diseases (I)**  
**Chairs:** *María Ester Bernardo (IT) & Robert Wynn (UK)*

12 min.+ 3 Q&A      Update on HSPC-GT for MLD  
10 min.+ 3 Q&A      Update on HSPC-GT for MPSIH  
10 min.+ 3 Q&A      Update on HSPC-GT for MPSII  
10 min.+ 3 Q&A      Update on HSPC-GT for MPSIIIA  
10 min.+ 3 Q&A      Platform approach for LSD with skeletal involvement (MPSIVA, MPSIVB, alphaMAN)  
5 min.+ 5 Q&A      *Case Presentation*

Valeria Calbi (IT)  
María Ester Bernardo (IT)  
Claire Horgan (UK)  
Robert Wynn (UK)  
Alessandro Aiuti (IT)  
Nathaniel Lucas (NZ)

**15:30 – 16:00**      **Coffee Break**

**16:00 – 16:30**      **Session I: Stem cell therapy in metabolic diseases (II)**  
**Chairs:** *María Ester Bernardo (IT) & Robert Wynn (UK)*

12 min.+ 3 Q&A      Retrospective allo-HSCT data on Osteopetrosis  
12 min.+ 3 Q&A      Lentiviral-based HSPC-GT for Osteopetrosis

Robert Chiesa (UK)  
Francesca Tucci (IT)

**16:30 – 17:30**      **Session II: Pathophysiology of IEI and its role in cellular therapy**  
**Chairs:** *Mikko Seppänen (FI) & Kate Orf (UK)*

20 min.+ 5 Q&A      New molecular causes of SCID  
15 min.+ 5 Q&A      STAT1 and STAT3 GOFs- JAKi, HSCT, or both?  
10 min.+ 5 Q&A      In vitro assays for the assessment of thymic stromal cell defects

Sophie Hambleton (UK)  
Carsten Speckmann (DE)  
Alexandra Kreins (UK)

### Saturday, 28 September 2024

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

**Chairs:** *Krzysztof Kalwak (PL) & Olaf Neth (SP)*  
7 min.+ 3 Q&A      Letermovir PK, safety and efficacy data in children  
5 min.+ 5 Q&A      Letermovir EBMT study  
12 min.+ 3 Q&A      Metagenomic NGS (London/Paris experience)  
12 min.+ 3 Q&A      HPV infection in SCID and beyond  
12 min.+ 3 Q&A      Enteric virus infection in the HSCT setting

Krzysztof Kalwak (PL)  
Krzysztof Kalwak (PL)  
Austen Worth (UK)  
Corentin Le Floch (FR)  
Bénédicte Neven (FR)

**10:15 – 10:45**      **Coffee Break**

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

Donald Kohn (USA)

*All participations include time for Q&A.*

11:15 – 12:45

### Session IV: Gene therapy

**Chairs:** *Sung-Yun Pai (US) & Claire Booth (UK)*

15 min.+ 5 Q&A  
7 min.+ 3 Q&A  
7 min.+ 3 Q&A  
7 min.+ 3 Q&A  
7 min.+ 3 Q&A  
7 min.+ 3 Q&A  
7 min.+ 3 Q&A

Lentiviral gene therapy for XLA  
Clinical trial of gene edited T cells for CD40L  
Gene editing for RAG1-SCID  
Gene therapy in RAG1 deficiency  
Clinical trial of T cell gene therapy for XLP  
Update on clinical trial for p47-CGD  
Artemis SCID ongoing trial update

David Rawlings (USA)  
Daniele Canarutto (IT)  
Anna Villa (IT)  
Arjan Lankester (NL)  
Claire Booth (UK)  
Claire Booth (UK)  
Marina Cavazzana (FR)

12:45 – 14:00

### Lunch Break

14:00 – 14:45

### Keynote Lecture

Genetics of Incomplete Penetrance

Dusan Bogunovic (US)

14:45 – 15:30

### Session V: Challenging indications and novel approaches

**Chairs:** *Despina Moshous (FR) & Manfred Höning (DE)*

15 min.+ 5 Q&A  
15 min.+ 5 Q&A

The role of somatic variants in IEI  
Immunological reconstitution post-secondary HSCT for SCID:  
a box of chocolate (*or: you never know what you're gonna get*)

Despina Moshous (FR)  
Manfred Höning (DE)

16:00 – 16:30

### Coffee Break

16:30 – 17:30

### Session VI: Characteristic diseases of the Finnish population

**Chairs:** *Samppa Ryhänen (FI) & Heljä Lång (FI)*

12 min.+ 3 Q&A  
12 min.+ 3 Q&A  
15 min.+ 5 Q&A

Clinical and immunological aspects of CHH  
HSCT in CH  
Mechanism-based therapeutic interventions for  
autoimmunity in APECED patients

Svetlana Vakkilainen (FI)  
Heljä Lång (FI)  
Saila Laakso (FI)

17:30 – 18:35

### Session VII: IEWP Studies and proposals

**Chairs:** *Bénédicte Neven (FR) & Michael Albert (DE)*

10 min.+ 5 Q&A  
10 min.+ 5 Q&A  
10 min.+ 5 Q&A  
5 min.+ 5 Q&A  
7 min.+ 3 Q&A

9/10 permissive HLA mismatch  
Clinical practice guideline: Long-term follow-up in patients transplanted for IEI  
TTC7A  
DADA2 proposal  
HSCT in STAT1GOF with/without previous JAKi treatment

Arjan Lankester (NL)  
Reem Elfeky (UK)  
Giulia Prunotto (IT)  
Etaï Adam (IL)  
Emmeline Buddingh (NL)

*All participations include time for Q&A.*

### Sunday, 29 September 2024

09:00 – 10:15

#### Session VIII: HSCT for IEI in AYAs (focus on CVID)

**Chair:** Emma Morris (UK) & Klaus Warnatz (DE)

5 min.+ 5 Q&A  
*Case presentation*

15 min.+ 5 Q&A  
Challenges with diagnosis of CVID and selection of patients for Allo SCT

15 min.+ 5 Q&A  
*GI & Liver disease in CVID and the role of liver transplantation*

5 min.+ 5 Q&A  
*Case presentation*

12 min.+ 3 Q&A  
Recent CVID Allo SCT outcomes and proposed COCOA clinical study

Raffaella Muratori (BR)

Klaus Warnatz (DE)

Neil Halliday (UK)

Muriel Schmutz (FR)

Emma Morris (UK)

10:15 – 11:15

#### Session IX – Haplo HSCT for IEI

**Chair:** Andrew Gennery (UK) & Arjan Lankester (NL)

10 min.+ 10 Q&A  
PTCY or TCR $\alpha\beta$ : takeaways from the IEWP study - a discussion

10 min.+ 5 Q&A  
How to best use T-cell directed serotherapy in haplo HSCT

7 min.+ 3 Q&A  
Real-time treosulfan PK

10 min.+ 5 Q&A  
Adoptive cellular therapy with CD45RO+ memory T-cell addback?

Michael Albert (DE)

Mary Slatter (UK)

Peter Shaw (AU)

Su Han Lum (UK)

11:15 – 11:45

#### Coffee Break

11:45 – 12:45

#### Session X: Bridging strategies to HSCT

**Chairs:** Reem Elfeky (UK) & Carsten Speckmann (DE)

7 min.+ 3 Q&A  
CTLA4 fusion proteins as bridge to HSCT in LRBA deficiency

7 min.+ 3 Q&A  
CTLA4 fusion proteins as bridge to HSCT in CTLA4 deficiency

7 min.+ 3 Q&A  
Leniolisib in APDS1/2 patients under named patient program in Europe

7 min.+ 3 Q&A  
Emapalumab as a bridge to HSCT

Safa Baris (TR)

Christo Tsilifis (UK)

Reem Elfeky (UK)

Pietro Merli (IT)

12:45 – 13:00

#### Closing remarks

Bénédicte Neven (FR), Michael Albert (DE) & Heljä Lång (FI)

All participations include time for Q&A.