

## Preliminary Programme

Helsinki, Finland  
27-29 September 2024

### Friday, 27 September 2024

13:00 – 13:45	<b>Registration &amp; Welcome Coffee</b>	
13:45 – 14:00	<b>Welcome</b> <b>Chairs:</b> <i>Bénédicte Neven (FR) &amp; Heljä Lång (FI)</i>	
14:00 – 15:35	<b>Session I: Stem cell therapy in metabolic diseases (I)</b> <b>Chairs:</b> <i>María Ester Bernardo (IT) &amp; Robert Wynn (UK)</i>	
14:00 – 14:20	Update on HSPC-GT for MLD	
14:20 – 14:35	Update on HSPC-GT for MPSIH	Maria Ester Bernardo (IT)
14:35 – 14:50	Update on HSPC-GT for MPSII	Claire Horgan (UK)
14:50 – 15:05	Update on HSPC-GT for MPSIIIA	Robert Wynn (UK)
15:05 – 15:20	Platform approach for LSD with skeletal involvement (MPSIVA, MPSIVB, alphaMAN)	
15:20 – 15:30	<i>Case Presentation</i>	Nathaniel Lucas (NZ)
15:30 – 16:00	<b>Coffee Break</b>	
16:00 – 17:30	<b>Session I: Stem cell therapy in metabolic diseases (II)</b> <b>Chairs:</b> <i>María Ester Bernardo (IT) &amp; Robert Wynn (UK)</i>	
16:00 – 16:15	Retrospective allo-HSCT data on Osteopetrosis	
16:15 – 16:30	Lentiviral-based HSPC-GT for Osteopetrosis	Francesca Tucci (IT)
16:30 – 17:30	<i>To be decided</i>	
17:30 – 18:30	<b>Session II: Pathophysiology of IEL and its role in cellular therapy</b> <b>Chairs:</b> <i>Mikko Seppänen (FI) &amp; Kate Orf (UK)</i>	
17:30 – 17:55	New molecular causes of SCID	Sophie Hambleton (UK)
17:55 – 18:15	STAT1 and STAT3 GOFs- JAKi, HSCT, or both?	Carsten Speckmann (DE)
18:15 – 18:30	In vitro assays for the assessment of thymic stromal cell defects	Alexandra Kreins (UK)

### Saturday, 28 September 2024

09:00 – 10:15	<b>Session III: Infections (focus on viruses)</b> <b>Chairs:</b> <i>Krzysztof Kalwak (PL)</i>	
09:00 – 09:10	Letermovir PK, safety and efficacy data in children	Krzysztof Kalwak (PL)
09:10 – 09:20	Letermovir EBMT study	Katharina Kleinschmidt (DE)
09:20 – 09:35	Metagenomic NGS (London/Paris experience)	
09:35 – 09:50	HPV infection in SCID and beyond	Corentin Le Floch (FR)
09:50 – 10:05	Enteric virus infection in the HSCT setting	Bénédicte Neven (FR)
10:05 – 10:15	<i>To be decided</i>	
10:15 – 10:45	<b>Coffee Break</b>	
10:45 – 11:15	<b>Keynote Lecture</b> Gene therapy for IEL	Donald Kohn (USA)

All participations include time for Q&A.

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<b>11:15 – 12:45</b>	<b>Session IV: Gene therapy</b> <b>Chairs:</b> <i>Sung-Yun Pai (US) &amp; Claire Booth (UK)</i>	
<b>11:15 – 11:35</b>	Lentiviral gene therapy for XLA	David Rawlings (USA)
<b>11:35 – 11:45</b>	Clinical trial of gene edited T cells for CD40L	Daniele Canarutto (IT)
<b>11:45 – 11:55</b>	Gene editing for RAG1-SCID	Anna Villa (IT)
<b>11:55 – 12:05</b>	Lentiviral gene therapy for hypomorphic RAG	Arjan Lankester (NL)
<b>12:05 – 12:15</b>	Clinical trial of T cell gene therapy for XLP	Claire Booth (UK)
<b>12:15 – 12:25</b>	Update on clinical trial for p47-CGD	Claire Booth (UK)
<b>12:25 – 12:35</b>	Artemis SCID ongoing trial update	Marina Cavazzana (FR)
<b>12:35 – 12:45</b>	<i>To be decided</i>	
<b>12:45 – 14:00</b>	<b>Lunch Break</b>	
<b>14:00 – 14:45</b>	<b>Keynote Lecture</b> Genetics of Incomplete Penetrance	Dusan Bogunovic (US)
<b>14:45 – 15:55</b>	<b>Session V: Challenging indications and novel approaches</b> <b>Chairs:</b> <i>Despina Moshous (FR) &amp; Manfred Hönig (DE)</i>	
<b>14:45 – 15:05</b>	The role of somatic variants in IEI	Despina Moshous (FR)
<b>15:05 – 15:25</b>	Immunological reconstitution post-secondary HSCT for SCID: a box of chocolate ( <i>or: you never know what you're gonna get</i> )	Manfred Hönig (DE)
<b>15:25 – 16:00</b>	<i>To be decided</i>	
<b>16:00 – 16:30</b>	<b>Coffee Break</b>	
<b>16:30 – 17:30</b>	<b>Session VI: Characteristic diseases of the Finnish population</b> <b>Chairs:</b> <i>Samppa Ryhänen (FI) &amp; Heljä Lång (FI)</i>	
<b>16:30 – 16:45</b>	Clinical and immunological aspects of CHH	Svetlana Vakkilainen (FI)
<b>16:45 – 17:00</b>	HSCT in CH	Heljä Lång (FI)
<b>17:00 – 17:20</b>	Mechanism-based therapeutic interventions for autoimmunity in APECED patients	Saila Laakso (FI)
<b>17:20 – 17:30</b>	<i>To be decided</i>	
<b>17:30 – 18:35</b>	<b>Session VII: IEWP Studies and proposals</b> <b>Chairs:</b> <i>Bénédicte Neven (FR) &amp; Michael Albert (DE)</i>	
<b>17:30 – 17:45</b>	9/10 permissive HLA mismatch	Reem Elfeky (UK)
<b>17:45 – 18:00</b>	Clinical practice guideline: Long-term follow-up in patients transplanted for IEI	Giulia Prunotto (IT)
<b>18:00 – 18:15</b>	TTC7A	Etai Adam (IL)
<b>18:15 – 18:25</b>	DADA2 proposal	Emmeline Buddingh (NL)
<b>18:25 – 18:35</b>	HSCT in STAT1GOF with/without previous JAKi treatment	

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### Sunday, 29 September 2024

<b>09:00 – 10:15</b>	<b>Session VIII: HSCT for IEI in AYAs (focus on CVID)</b> <b>Chair:</b> <i>Emma Morris (UK)</i>	
<b>09:00 – 09:10</b>	<i>Case presentation</i>	Raffaella Muratori (BR)
<b>09:10 – 09:30</b>	Challenges with diagnosis of CVID and selection of patients for Allo SCT	Neil Halliday (UK)
<b>09:30 – 09:50</b>	<i>GI &amp; Liver disease in CVID and the role of liver transplantation</i>	
<b>09:50 – 10:00</b>	<i>Case presentation</i>	
<b>10:00 – 10:15</b>	Recent CVID Allo SCT outcomes and proposed COCOA clinical study	Emma Morris (UK)
<b>10:15 – 11:15</b>	<b>Session IX – Haplo HSCT for IEI</b> <b>Chair:</b> <i>Andrew Gennery (UK) &amp; Arjan Lankester (NL)</i>	
<b>10:15 – 10:35</b>	PTCY or TCR $\alpha\beta$ : takeaways from the IEWP study - a discussion	Michael Albert (DE) Mary Slatter (UK)
<b>10:35 – 10:50</b>	How to best use T-cell directed serotherapy in haplo HSCT	
<b>10:50 – 11:00</b>	Real-time treosulfan PK	
<b>11:00 – 11:15</b>	Adoptive cellular therapy with CD45RO+ memory T-cell addback?	Su Han Lum (UK)
<b>11:15 – 11:45</b>	<b>Coffee Break</b>	
<b>11:45 – 12:25</b>	<b>Session X: Bridging strategies to HSCT</b> <b>Chairs:</b> <i>Reem Elfeky (UK) &amp; Carsten Speckmann (DE)</i>	
<b>11:45 – 11:55</b>	CTLA4 fusion proteins as bridge to HSCT in LRBA deficiency	Safa Baris (TR)
<b>11:55 – 12:05</b>	CTLA4 fusion proteins as bridge to HSCT in CTLA4 deficiency	Christo Tsilifis (UK)
<b>12:05 – 12:20</b>	<i>To be decided</i>	
<b>12:20 – 12:30</b>	Leniolisib in APDS1/2 patients under named patient program in Europe	Reem Elfeky (UK)
<b>12:30 – 12:40</b>	Emapalumab as a bridge to HSCT	
<b>12:40 – 12:45</b>	<i>To be decided</i>	
<b>12:45 – 13:00</b>	<b>Closing remarks</b> <i>B�nedicte Neven (FR), Michael Albert (DE) &amp; Helj� L�ng (FI)</i>	

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