

Inborn Errors Working Party Annual Conference

Zurich, Switzerland | 25-27 September 2026



PRELIMINARY PROGRAMME – MAIN TRACK

Friday, 25 September 2026

13:00-13:45	Registration & Welcome Coffee	
13:45-14:00	Welcome from the organisers Maria Ester Bernardo (IT), Claire Booth (UK) and Tayfun Güngör (CH)	
14:00-16:07	Session 1 Allogeneic HSCT and HSC-GT for IEM Chairs: Giulia Consiglieri (IT) and Robert Wynn (UK)	
14:00-14:07	Case presentation: Disseminated Mycobacterium avium complex infection and Immune Reconstitution Inflammatory Syndrome following Haematopoietic Stem Cell Transplantation for Mucopolysaccharidosis Type II: a complex post-transplant course	Aisling McCann (IE)
14:07-14:27	Biomarkers in ALD, Gene Therapy and Allo-HCT	Troy Lund (US)
14:27-14:47	Biomarkers on CSF in MLD	Valeria Calbi (IT)
14:47-15:02	Update on the MPS II clinical trial/immune response to transgene and assays	Robert Wynn (UK)
15:02-15:17	Update on the MPS III clinical trial	Robert Wynn (UK)
15:17-15:32	Real-world data for MLD	Valeria Calbi (IT)
15:32-15:47	Platform approach for LSDs with skeletal and neurological involvement: clinical trial design	Maria Ester Bernardo (IT)
15:47-16:07	Mechanisms of immune response after HSC-GT for metabolic diseases	Silvia Gregori (IT)
16:10-16:40	Coffee Break	
16:40-18:40	Session 2 CGD special session from mechanism to novel therapies Chairs: Tayfun Güngör (CH) and Emma Morris (UK)	
16:40-16:47	Case presentation: Chronic Granulomatous Disease: a Cohort of 173 Patients—10-Years Single Center Experience from Egypt	Sohilla Lotfy (EG)
16:47-17:12	EROS and new insights into CGD signaling pathways	David Thomas (UK)
17:12-17:32	CGD - a tough candidate for allo HSCT	Tayfun Güngör (CH)
17:32-17:52	Biologics of chronic granulomatous disease	Jennifer Leiding (US)
17:52-18:07	Gene editing in HSC for correcting the oxidative burst in CGD	Giorgia Santilli (UK)
18:07-18:17	The complexities of surviving CGD	
18:17-18:27	First inhuman in vivo gene therapy	Emma Morris (UK)
18:27-18:40	Panel Discussion	

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PRELIMINARY PROGRAMME – NURSES TRACK

Saturday, 26 September 2026

10:30-12:30	Session 1 Care Team Challenges in Patient and Family Care Chairs: Marina Ben Izhak (IL) and Karen Nicholson (UK) Welcome message	
10:30-11:00	Challenges we face every day treating private and international patients (25+5)	Tanja Stankova (UK)
11:00-11:30	Stakeholder perspectives on Stem Cell Therapy for Patients with Inborn Errors of Immunity (25+5)	Hilda Mekelenkamp (NL)
11:30-12:00	Partnering with Families: Co-Designing information resources for paediatric Gene Therapy (25+5)	Evey Howley (UK)
12:00-12:30	Psycho-educational intervention for parents after their child was diagnosed with an IEI following a positive NBS test (25+5)	Patricia Luck (CH)
12:30-13:00	Lunch Break	
13:00-14:15	Session 5 Thymus transplant (Main Track)	
14:15-15:15	Session 6 Advances in GvHD and endothelial complications (Main Track)	
15:15-15:45	Coffee Break	
15:45-16:45	Session 2 Quality of Life and long-term outcomes Chair: Evey Howley (UK) Welcome message	
15:45-16:05	Post-GT experience in WAS patients with a focus on quality of life and long-term follow-up	Federico Fraschetta (IT)
16:05-16:25	Developing a pathway for transition from the paediatric to adult long-term follow-up clinic	Karen Nicholson (UK)
16:25-16:45	TBD	TBD

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Saturday, 26 September 2026

09:00-10:30

Session 3 | HSC-Gene therapy for IEIs

Chairs: Sung-Yun Pai (US) and Francesca Ferrua (IT)

09:00-09:10

Overview of current open trials

Claire Booth (UK)

09:10-09:20

Preclinical development of gene therapy for Major Histocompatibility

Complex Class II (MHC II) Deficiency

Alessandra Magnani (ES)

09:20-09:30

Personalized Gene Correction for Inborn Errors of Immunity

Emma Haapaniemi (FI)

09:30-09:35

International XIAP GT consortium

Claire Booth (UK)

09:35-09:45

CD40L moving to the clinic

Daniele Canarutto (IT)

09:45-09:55

P47 CGD LV clinical trial update

Jasmeen Dara (UK)

09:55-10:05

Artemis -SCID

Marina Cavazzana (FR)

10:05-10:15

WASKYRA approval - current update about rollout/availability and results of EAS

Francesca Ferrua (IT)

10:15-10:25

Initiatives and regulatory innovation to improve access to GT in Europe

Arjan Lankester (NL)

10:25-10:45

Coffee Break

10:45-11:25

Keynote Lecture

Epstein-Barr Virus, autoimmune disease & big data management

Prof. Christian Münz (CH)

11:25-12:10

Session 4 | B cells and plasma cell derived treatments

Chairs: Carsten Speckmann (DE) and Julian Thalhammer (DE)

11:25-11:35

Pathology on early B cells defects

Mirjam van der Burg (NL)

11:35-11:45

HSCT experience in XLA

Andrew Gennery (UK)

11:45-11:55

B cell pathology in CVID

11:55-12:10

Anti-CD19 CAR T cells refractory autoimmune disease

Franco Locatelli (IT)

12:10-13:00

Lunch Break

13:00-14:22

Session 5 | Thymus transplant

Chairs: Alexandra Kreins (UK) and Mathias Hauri-Hohl (CH)

13:00-13:07

Case presentation: Delayed thymic immune reconstitution in paediatric patients with Chronic Granulomatous Disease that underwent allogeneic hematopoietic stem cell transplantation

Laura Marsiglio (CH)

13:07-13:27

Rebuilding Immunity after Paediatric HCT: The Role of Thymic Hormones

Coco de Koning (NL)

13:27-13:47

Sex differences in the human thymus

Viktoria Hennings (SE)

13:47-14:07

Two cases on Challenging referrals for thymic transplants

Coco de Koning (NL) and Viktoria Hennings (SE)

14:07-14:22

Discussions on the current state algorithm for decision-making

Alexandra Kreins (UK)

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14:22-15:24	Session 6 Advances in endothelial complications	
	Chairs: Andrew Gennery (UK) and Virginia Meda Spaccamela (CH)	
14:22-14:29	Case presentation: Impact of Pulmonary Disease on Survival and Long-Term Respiratory Outcomes in Children undergoing Haematopoietic Cell Transplantation for Inborn Errors of Immunity	Ho Wai Koo (MY)
14:29-14:39	Light Therapy for oral mucositis: the nurse experience	Karin Schoch (CH) & Svenja Schweizer (CH)
14:39-14:54	Endothelial biomarker dynamics in Sinusoidal Obstruction Syndrome following paediatric allogeneic HSCT	
14:54-15:09	Paediatric VOD: Still a concern?	
15:09-15:24	Pulmonary Vasculopathy after paediatric HSCT - a review of the disease and proposal for a monitoring strategy and early treatment	Antonio Grasso (UK)
15:25-15:45	Coffee Break	
15:35-16:15	Keynote Lecture	
	Immuno-conditioning for SCT and GT	Prof. Markus Manz (CH)
16:25-17:17	Session 7 Precision conditioning	
	Chairs: Thomas Fox (UK) and Arjan Lankester (NL)	
16:25-16:32	Case presentation: Comparison of Cyclophosphamide and Fludarabine in alloreactive settings in vitro	Antonia Reers (DE) Persis Amrolia (UK)
16:32-16:47	Advances in targeted conditioning	
16:47-17:02	PK and model-informed precision dosing of chemotherapy to optimize conditioning	Janel Long-Boyle (US)
17:02-17:17	Alemtuzumab PK/PD to optimize conditioning	Thibault César (FR)
17:17-18:24	Session 8 Advanced diagnostics and treatment of infections	
	Chairs: Bénédicte Neven (FR) and Jacques Rivière (ES)	
17:17-17:24	Case presentation: Successful Allogeneic HSCT Following Peri-Transplant IFN-γ Blockade With Emapalumab in Complete IFNGR2 Deficiency	Linda Rossini (IT)
17:24-17:44	Fungal infections in CGD: new antifungal drugs, indications for granulocyte transfusions as bridge to HSCT, when to transplant with an active fungal infection	Christa Zerbe (US)
17:44-17:59	When conventional antimicrobials are not enough: new antimicrobial drugs in HSCT, including antibacterials, antivirals (anti-HSV agents) and phage therapy	Natalia Mendoza (ES) Muriel Schmutz (FR)
17:59-18:14	Atypical viral infections: Aichi virus	
18:14-18:24	NGS case report with neurological involvement, case series from Necker's experience with NGS	Cassandre Cremades (FR)

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Sunday, 27 September 2026

09:00-10:30	Session 9 Juniors session Management of IEIs in cases with unclear/inconclusive genetic findings Chairs: Lisa Ott de Bruin (NL) and Sujal Ghosh (DE) 09:00-09:20 Transplant decision-making in severe T cell immunodeficiency 09:20-09:27 Case Presentation 1 by IEWP junior committee member 09:27-09:34 Case Presentation 2 by IEWP junior committee member 09:34-09:41 Case Presentation 3 by IEWP junior committee member 09:41-10:30 Panel Discussion	Sophie Hambleton (UK)
10:30-10:50	Coffee Break	
10:50-11:35	Session 10 IEWP Studies Chairs: Maria Ester Bernardo (IT) and Claire Booth (UK) 10:50-11:00 DADA2 study 11:00-11:15 P-CID study 11:15-11:25 FHL2/3 study 11:25-11:35 CHH study	Juliana Silva (UK) Stephan Ehl (DE) Stephan Ehl (DE) Helja Lång (FI)
11:35-13:11	Session 11 Advances in immune dysregulation Chairs: Tiphaine Arlabosse (CH) 11:35-11:42 Case presentation: First haematopoietic cell transplantation as a curative treatment for monogenic SFK-related multisystem vasculitis 11:42-12:00 ADA2 updates, including management of patients with dominant negative disease 12:00-12:17 Autophagy/inflammation in WAS 12:17-12:35 STAT6 GOF updates on the current ESID registry study 12:35-12:53 ZNFX1 update on current collaborative study 12:53-13:11 NFKBIA clinical spectrum and HSCT strategies	Isabelle Meyts (BE) Elizabeth Rivers (UK) Julia Körholz (DE) Julius Köppen (CH) Winnie Ip (UK)
13:11-13:20	Final Remarks Maria Ester Bernardo (IT), Claire Booth (UK) and Tayfun Güngör (CH)	