

# Inborn Errors Working Party Annual Conference

Zurich, Switzerland | 25-27 September 2026



## PRELIMINARY PROGRAMME – MAIN TRACK

**Friday, 25 September 2026**

<b>13:00-13:45</b>	Registration & Welcome Coffee	
<b>13:45-14:00</b>	<b>Welcome from the organisers</b> Maria Ester Bernardo (IT), Claire Booth (UK) and Tayfun Güngör (CH)	
<b>14:00-16:00</b>	<b>Session 1   Allogeneic HSCT and HSC-GT for IEM</b> Chairs: Giulia Consiglieri (IT)	
14:00-14:15	Biomarkers in ALD, Gene Therapy and Allo-HCT	Troy Lund (US)
14:15-14:30	Biomarkers on CSF in MLD	
14:30-14:45	Update on the MPS II clinical trial/immune response to transgene and assays	
14:45-15:00	Update on the MPS III clinical trial	
15:00-15:15	Real-world data for MLD	
15:15-15:30	Umbilical cord blood transplant outcomes in NMD in the modern era	
15:30-15:45	Platform approach for LSDs with skeletal and neurological involvement: clinical trial design	
15:45-16:00	Mechanisms of immune response after HSC-GT for metabolic diseases	
<b>16:00-16:30</b>	<b>Coffee Break</b>	
<b>16:30-18:30</b>	<b>Session 2   CGD special session from mechanism to novel therapies</b> Chairs: Tayfun Güngör (CH)	
16:30-16:55	EROS and new insights into CGD signaling pathways	David Thomas (UK)
16:55-17:15	Allo-HSCT	Tayfun Güngör (CH)
17:15-17:35	Biologics of chronic granulomatous disease	
17:35-17:50	Gene editing in HSC for correcting the oxidative burst in CGD	Giorgia Santilli (UK)
17:50-18:00	TBD	
18:00-18:10	TBD	
18:10-18:30	Panel Discussion	

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## PRELIMINARY PROGRAMME – NURSES TRACK

**Saturday, 26 September 2026**

### **Session 1 | Care Team Challenges in Patient and Family Care**

Welcome message

Challenges we face every day treating private and international patients

TBD

Improving information resources for informed consent conversations - project with the Gene Therapy families

TBD

### **Session 2 | Quality of Life and long-term outcomes**

Welcome message

Post-GT experience in WAS patients with a focus on quality of life and long-term follow-up

TBD

TBD

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## PRELIMINARY PROGRAMME – MAIN TRACK

**Saturday, 26 September 2026**

<b>09:00-10:30</b>	<b>Session 3   HSC-Gene therapy for IEIs</b> Chairs: Claire Booth (UK) and Francesca Ferrua (IT)	
09:00-09:15	Overview of current open trials	Claire Booth (UK)
09:15-09:25	Preclinical development of gene therapy for Major Histocompatibility Complex Class II (MHC II) Deficiency	
09:25-09:35	Gene editing for STAS disorders	
09:35-09:40	International XIAP GT consortium	Claire Booth (UK)
09:40-09:50	CD40L moving to the clinic	Daniele Canarutto (IT)
09:50-10:00	P47 CGD LV clinical trial update	Jasmeen Dara (UK)
10:00-10:10	Artemis -SCID	Marina Cavazzana (FR)
10:10-10:20	WASKYRA approval - current update about rollout/availability and results of EAS	Francesca Ferrua (IT)
10:20-10:30	Initiatives and regulatory innovation to improve access to GT in Europe	
<b>10:30-10:45</b>	<b>Coffee Break</b>	
<b>10:45-11:25</b>	<b>Keynote Lecture</b> Epstein-Barr Virus, autoimmune disease & big data management Prof. Christian Münz (CH)	
<b>11:25-12:10</b>	<b>Session 4   B cells and plasma cell derived treatments</b> 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)
<b>12:10-13:00</b>	<b>Lunch Break</b>	
<b>13:00-14:15</b>	<b>Session 5   Thymus transplant</b> Chairs:	
13:00-13:20	Rebuilding Immunity after Pediatric HCT: The Role of Thymic Hormones	Coco de Koning (NL)
13:20-13:40	Sex differences in the human thymus	Viktoria Hennings (SE)
13:40-14:00	Two cases on Challenging referrals for thymic transplants	Coco de Koning (NL) and Viktoria Hennings (SE)
14:00-14:15	Discussions on the current state algorithm for decision-making	

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**14:15-15:15**     **Session 6 | Advances in GvHD and endothelial complications**

Chairs: Andrew Gennery (UK)

TBD

TBD

TBD

TBD

**15:15-15:35**     **Coffee Break**

**15:35-16:15**     **Keynote Lecture**

Immuno-conditioning for SCT and GT

Prof. Markus Manz (CH)

**16:15-17:00**     **Session 7 | Precision conditioning**

Chairs: Thomas Fox (UK)

16:15-16:30     Advances in antibody-based conditioning

16:30-16:45     PK and model-informed precision dosing of chemotherapy  
to optimize conditioning

16:45-17:00     TBD

**17:00-18:00**     **Session 8 | Advanced diagnostics and treatment of infections**

Chairs:

17:00-17:20     Fungal infections in CGD: new antifungal drugs, indications for granulocyte  
transfusions as bridge to HSCT, when to transplant with an active fungal infection

17:20-17:35     When conventional antimicrobials are not enough: new antimicrobial drugs  
in HSCT, including antibacterials, antivirals (anti-HSV agents)  
and phage therapy

17:35-17:50     Atypical viral infections: Aichi virus

17:50-18:00     NGS case report with neurological involvement, case series from Necker's experience with NGS

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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: Sujal Ghosh (DE)

Summary of the webinars held so far

Newborn Screening and SCT indication

Case Presentation 1 by IEWP junior committee member

Case Presentation 2 by IEWP junior committee member

Case Presentation 3 by IEWP junior committee member

Interactive discussion (voting on management decisions among the audience) and Panel Discussion

**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

Juliana Silva (UK)

11:00-11:15

P-CID study

Stephan Ehl (DE)

11:15-11:25

FHL2/3 study

Stephan Ehl (DE)

11:25-11:35

CHH study

Helja Lång (FI)

**11:35-13:05**

### **Session 11 | Advances in immune dysregulation**

Chairs:

11:35-11:53

ADA2 updates, including management of patients with dominant negative disease

11:53-12:11

Autophagy/inflammation in WAS

12:11-12:29

STAT6 GOF updates on the current ESID registry study

Julia Körholz (DE)

12:29-12:47

ZNFX1 update on current collaborative study

Julius Köppen (CH)

12:47-13:05

NFKBIA clinical spectrum and HSCT strategies

Winnie Ip (UK)

**13:05-13:15**

### **Final Remarks**

Maria Ester Bernardo (IT), Claire Booth (UK), Tayfun Güngör (CH)