

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
14:00-16:00	Session 1 Allogeneic HSCT and HSC-GT for IEM
14:00-14:15	Biomarkers on CSF in ALD
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
16:00-16:30	Coffee Break
16:30-18:30	Session 2 CGD special session from mechanism to novel therapies
16:30-16:55	EROS and new insights into CGD signaling pathways
16:55-17:15	Allo-HSCT
17:15-17:35	Biologics of chronic granulomatous disease
17:35-17:50	Gene editing in HSC for correcting the oxidative burst in CGD
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

09:30-11:30	Session 1 Care Team Challenges in Patient and Family Care
09:30-09:35	Welcome message
09:35-10:05	Challenges we face every day treating private and international patients
10:05-10:35	TBD
10:35-11:05	Improving information resources for informed consent conversations - project with the Gene Therapy families
11:05-11:30	TBD
11:30-12:00	Coffee Break
12:00-13:05	Session 2 Quality of Life and long-term outcomes
12:00-12:05	Welcome message
12:05-12:25	Post-GT experience in WAS patients with a focus on quality of life and long-term follow-up
12:25-12:45	TBD
12:45-13:05	TBD
13:15-14:00	Lunch Break

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PRELIMINARY PROGRAMME – MAIN TRACK

Saturday, 26 September 2026

09:00-10:15 **Session 3 | HSC-Gene therapy for IEIs**

10:15-11:30 **Session 4 | Thymus transplant**

11:30-11:50 **Coffee Break**

11:50-12:30 **Keynote Lecture**

Epstein-Barr Virus, autoimmune disease & big data management

12:30-13:15 **Session 5 | B cells and plasma cell derived treatments**

12:30-12:40 Pathology on early B cells defects

12:40-12:50 HSCT experience in XLA

12:50-13:00 B cell pathology in CVID

13:00-13:15 Anti-CD19 CAR T cells refractory autoimmune disease

13:15-14:00 **Lunch Break**

14:00-15:00 **Session 6 | Advances in GvHD and endothelial complications**

15:00-15:40 **Keynote Lecture**

Immuno-conditioning for SCT and GT

15:40-16:25 **Session 7 | Precision conditioning**

15:40-15:50 TDM Treo Flu

15:50-16:00 Biologicals could include an epitope editing talk

16:00-16:10 Cell (CAR) based conditioning

16:10-16:25 Panel discussion on conditioning approaches (one-size fits all vs MIPD vs TDM) for our current drugs

16:25-16:50 **Coffee Break**

16:50-17:50 **Session 8 | Advanced diagnostics and treatment of infections**

16:50-17:10 Fungal infections in CGD: new antifungal drugs, indications for granulocyte transfusions as bridge to HSCT, when to transplant with an active fungal infection

17:10-17:25 When conventional antimicrobials are not enough: new antimicrobial drugs in HSCT, including antibacterials, antivirals (anti-HSV agents) and phage therapy

17:25-17:40 Atypical viral infections: Aichi virus

17:40-17:50 NGS case report with neurological involvement, case series from Necker's experience with NGS

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PRELIMINARY PROGRAMME – MAIN TRACK

Sunday, 27 September 2026

09:00-10:30 **Session 9 | Juniors session | Management of IELs in cases with unclear/inconclusive Genetic findings**

10:30-10:50 **Coffee Break**

10:50-11:35 **Session 10 | IEWP Studies**

10:50-11:05 DADA2 study

11:05-11:20 P-CID study

11:20-11:35 FHL2/3 study

11:35-13:05 **Session 11 | Advances in immune dysregulation**

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

12:29-12:47 ZNFX1 update on current collaborative study

12:47-13:05 NFKBIA clinical spectrum and HSCT strategies

13:05-13:15 **Final Remarks**