

Preliminary Programme

Freiburg, Germany
26-28 September 2025

Friday, 26 September 2025

13:00–13:45 Registration and Welcome Coffee

13:45–14:00 Welcome from the organizers

14:00–14:45 Keynote lecture

14:45–15:45 Session I: Immune Responses in HSC-GT for IEMs

14:45–15:00 Immune Response after HSC-GT for MLD

15:00–15:15 Immune Response after HSC-GT for X-ALD

15:15–15:25 Immune Response after HSC-GT for MPS IH, II and IIIA

15:25–15:45 Mechanisms of Immune response after HSC-GT for metabolic diseases

15:45–16:15 Coffee break

16:15–17:30 Clinical Trials of HSC-GT for IEMs

16:15–16:30 Phase I/II trial of HSPC-GT for MPSIH: 5-year follow-up analysis

16:30–16:45 Phase I/II HSPC-GT for MPSIIIA

16:45–16:55 Phase I/II HSPC-GT for MPSII

16:55–17:10 HSC-GT for Pompe

17:10–17:20 Trial design of a Phase I/II clinical of HSC-GT for Osteopetrosis

17:20–17:30 Proposed EBMT guidelines for infection prophylaxis and vaccination after HSC-GT for IEWM

17:30–18:47 Session II: IEWP studies and proposals

17:45–18:00 Process to initiate IEWP studies and ongoing studies

18:00–18:15 Osteopetrosis study

18:15–18:22 To be informed

18:22–18:27 To be informed

18:27–18:37 To be informed

18:37–18:47 Chediak-Higashi study

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Saturday, 27 September 2025

09:00–10:15 **Session III: CID and related issues**

09:00–09:20	PCID
09:20–09:35	Mixed chimerism in Bu exposed young children
09:35–09:50	Treo vs Bu conditioning in SCID
09:50–10:15	To be informed
10:15–10:45	Coffee break

10:45–11:15 **Keynote lecture**

11:15–12:45 **Session IV: Gene therapy**

11:15–11:35	CGD programme at NIH
11:35–11:45	X-CGD in vivo GT
11:45–11:55	p47 CGD Gene therapy consortium
11:55–12:05	LV X-SCID trial update and planned new trial
12:05–12:15	Gene editing strategies for APDS1 and STAT1
12:15–12:25	Munc 13-4 Gene therapy
12:25–12:35	Munc 13-4 gene editing approaches
12:35–12:40	Wiskott Aldrich Syndrome GT update - progressing to license
12:40–12:45	RAG1 trial update/new trial plans

12:45–14:00 Lunch break

14:00–14:30 **Nurses session**

14:30–15:15 **Session V: HLH related diseases**

14:30–14:50	To be informed
14:50–15:15	To be informed

15:15–15:45 Coffee break

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15:45 – 16:50

Session VI: EBV IEI and EBV driven pathology

- 15:45-16:05 CAEBV – Overview talk
- 16:05-16:20 CAEBV with emphasis on the Cincinnati experience using nivolumab
- 16:20-16:35 VSTs for EBV associated issues namely viremia and PTLD in patients following HSCT and SOT along with pre-transplant use in patients with inborn errors
- 16:35-16:50 Adoptive T cell therapy – how to induce a protective antiviral T cell response post transplant

16:50 – 17:50

Session VII: B-cells

- 16:50-17:10 Regulation of human B lymphopoiesis: lessons from IEI
- 17:10-17:25 Effects of rituximab on post HSCT B cell reconstitution
- 17:25-17:40 Repertoire analysis in B cell reconstitution after HSCT
- 17:40-17:50 B cell chimerism in SCID

Sunday, 28 September 2025

09:00 – 10:15

Session VIII: CVID

- 09:00-09:15 ILD in CVID
- 09:15-09:30 Enteropathy in CVID
- 09:30-09:45 Novel Biomarkers in CVID
- 09:45-09:55 NFKB1 mutations (or HPO phenotyping) in CVID
- 09:55-10:00 HSCT in CVID update
- 10:00-10:15 Q&A

10:15 – 11:15

Session IX: IEI and pancytopenia

- 10:15-10:30 The many connections between immunology and hematology
- 10:30-10:45 What's new in SAMD9/9L syndrome
- 10:45-11:00 GATA2 deficiency: from bone marrow failure and immunodeficiency to MDS and leukemia
- 11:00-11:15 Like a chameleon: ADA2 deficiency
- 11:15-11:25 It's not always as you think

11:25 – 11:55

Coffee break

11:55 – 12:55

Session X: Autoinflammatory conditions

- 11:55-12:10 To be informed
- 12:10-12:25 To be informed
- 12:25-12:40 To be informed
- 12:40-12:55 To be informed

12:55 – 13:05

Final Remarks