

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 Maria Ester Bernardo (IT) & Carsten Speckmann (DE)	
14:00–14:45	Keynote lecture Making sense of human immunity by systems immunology	Petter Brodin (SE)
14:45–15:45	Session I: Immune Responses in HSC-GT for IEMs Chair: Maria Ester Bernardo (IT)	
14:45–15:00	Immune Response after HSC-GT for MLD	Valeria Calbi (IT)
15:00–15:15	Immune Response after HSC-GT for X-ALD	Troy Lund (US)
15:15–15:25	Immune Response after HSC-GT for MPS IH, II and IIIA	Maria Ester Bernardo (IT)
15:25–15:45	Mechanisms of Immune response after HSC-GT for metabolic diseases	Silvia Gregori (IT)
15:45–16:15	Coffee break	
16:15–17:30	Clinical Trials of HSC-GT for IEMs Chair: Maria Ester Bernardo (IT)	
16:15–16:30	Phase I/II trial of HSPC-GT for MPSIH: 5-year follow-up analysis	Maria Ester Bernardo (IT)
16:30–16:45	Phase I/II HSPC-GT for MPSIIIA	
16:45–16:55	Phase I/II HSPC-GT for MPSII	Nathan Jeffreys (UK)
16:55–17:10	HSC-GT for Pompe	Pim Pijnappel (NL)
17:10–17:20	Trial design of a Phase I/II clinical of HSC-GT for Osteopetrosis	Francesca Tucci (IT)
17:20–17:30	Proposed EBMT guidelines for infection prophylaxis and vaccination after HSC-GT for IEWM	
17:30–18:22	Session II: IEWP studies and proposals Chair: Maria Ester Bernardo (IT)	
17:30–17:45	Process to initiate IEWP studies and ongoing studies	
17:45–18:00	Hematopoietic Stem Cell Transplant in Osteopetrosis: an IEWP study	Robert Chiesa (UK)
18:00–18:07	HSCT for STATIGOF	
18:07–18:12	IEWP Consensus Guideline on JAK1 treatment for JAK/STAT IEI	Carsten Speckmann (DE)
18:12–18:22	Discussion	

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Saturday, 27 September 2025

09:00–10:15

Session III: CID and related issues

Chairs: Bénédicte Neven (FR) & Michael Albert (DE)

09:00–09:20

PCID Study Update

Carsten Speckmann (DE)

09:20–09:33

Mixed chimerism in Bu exposed young children

Chris Dvorak (US)

09:33–09:42

Flu PK in infants

09:42–09:55

Treo vs Bu conditioning in SCID

Su Han Lum (UK)

09:55–10:15

New insights into IL-7 deficiency and TSLP function

Luigi Notarangelo (US)

10:15–10:45

Coffee break

10:45–11:15

Keynote lecture

Robert Zeiser (DE)

The immunology of GvHD: advances in the understanding of the pathogenesis and novel therapeutic concepts

11:15–12:45

Session IV: Gene therapy

Chair: Toni Cathomen (DE)

11:15–11:35

CGD programme at NIH

Elizabeth Kang (UK)

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

Emma Morris (UK)

12:15–12:25

Munc 13-4 Gene therapy

12:25–12:35

Munc 13-4 gene editing approaches

Toni Cathomen (DE)

12:35–12:40

Wiskott Aldrich Syndrome GT update - progressing to license

Alessandro Aiuti (IT)

12:40–12:45

Update on the multinational gene therapy trial in RAG1 SCID

Arjan Lankester (NL)

12:45–14:00

Lunch break

14:00–14:30

Nurses session

14:30–15:30

Session V: Beyond HLH; HCT for sJIA and Still's Disease

Chair: Rebecca Marsh (US)

14:30–14:40

ZNFX1

Jana Pachlopnik (CH)

14:40–14:50

Allogeneic HCT for sJIA-associated Lung Disease: Collaborative Experience

Rebecca Marsh (US)

14:50–15:00

Allogeneic HCT for Still's Disease-associated Lung Disease: Paris Experience

Benjamin Fournier (FR)

15:00–15:10

Long Term Disease Relapse Risk Following Allogeneic HCT

Juliana Silva (UK)

15:10–15:30

Joint Discussion

15:30–16:00

Coffee break

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Saturday, 27 September 2025 - continued

16:00 – 17:05

Session VI: EBV IEI and EBV driven pathology

Chair: Austen Worth (UK) & Benjamin Fournier (FR)

16:00-16:20

CAEBV – Overview talk

Benjamin Fournier (FR)

16:20-16:35

CAEBV with emphasis on the Cincinnati experience using nivolumab

Ashish Kumar (US)

16:35-16:50

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

Jeremy Rubinstein (US)

16:50-17:05

Adoptive T cell therapy – how to induce a protective antiviral T cell response post transplant

Tobias Feuchtinger (DE)

17:05 – 18:05

Session VII: B-cells

Chair: Marta Rizzi (DE)

17:05-17:25

Regulation of human B lymphopoiesis: lessons from IEI

Marta Rizzi (DE)

17:25-17:40

Effects of rituximab on post HSCT B cell reconstitution

Bénédicte Neven (FR)

17:40-17:55

Repertoire analysis in B cell reconstitution after HSCT

Mirjam van der Burg (NL)

17:55-18:05

B cell reconstitution following HSCT in patients with B-positive SCID

Manfred Hönig (DE)

Sunday, 28 September 2025

09:00 – 10:15

Session VIII: CVID

Chairs: Klaus Warnatz (DE) & Emma Morris (UK)

09:00-09:15

ILD in CVID

09:15-09:30

Enteropathy in CVID

Klaus Warnatz (DE)

09:30-09:45

Novel Biomarkers in CVID

Helen Leavis (NL)

09:45-09:55

NFKB1 mutations (or HPO phenotyping) in CVID

09:55-10:00

HSCT in CVID update

Emma Morris (UK)

10:00-10:15

Q&A

10:15 – 11:25

Session IX: IEI and pancytopenia

Chair: Miriam Erlacher (DE) & Brigitte Strahm (DE)

10:15-10:30

The many connections between immunology and hematology

Miriam Erlacher (DE)

10:30-10:45

What's new in SAMD9/9L syndrome

Marcin Wlodarski (US)

10:45-11:00

GATA2 deficiency: from bone marrow failure and immunodeficiency to MDS and leukemia

Brigitte Strahm (DE)

11:00-11:15

Like a chameleon: ADA2 deficiency

Federica Barzaghi (IT)

11:15-11:25

It's not always as you think

Luca Vinci (DE)

11:25 – 11:55

Coffee break

11:55 – 12:55

Session X

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

Maria Ester Bernardo (IT) & Carsten Speckmann (DE)