

Preliminary Programme

Freiburg, Germany
26-28 September 2025

Friday, 26 September 2025

13:00 – 13:45	Registration & Welcome Coffee	
13:45 – 14:00	Welcome from the organizers Maria Ester Bernardo (IT) & Carsten Speckmann (DE)	
14:00 – 14:45	Keynote lecture <i>Making sense of human immunity by systems immunology</i>	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 – 16:22	Case Presentation: Hematopoietic Stem Cell Transplantation in a Patient with Mucopolysaccharidosis Type II	Gizem Zengin Ersoy (TR) Maria Ester Bernardo (IT)
16:22 – 16:35	Phase I/II trial of HSPC-GT for MPSIH: 5-year follow-up analysis	
16:35 – 16:49	Phase I/II HSPC-GT for MPSIIIA	
16:49 – 16:58	Phase I/II HSPC-GT for MPSII	Nathan Jeffreys (UK)
16:58 – 17:12	HSC-GT for Pompe	Pim Pijnappel (NL)
17:12 – 17:21	Trial design of a Phase I/II clinical of HSC-GT for Osteopetrosis	Francesca Tucci (IT)
17:21 – 17:30	Proposed EBMT guidelines for infection prophylaxis and vaccination after HSC-GT for IEWM	
17:30 – 19:00	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	Osteopetrosis study	Rob Chiesa (UK)
18:00 – 18:07	HSCT for STAT1GOF	Emmeline Buddingh (NL)
18:07 – 18:12	IEWP Consensus Guideline on JAK1 treatment for JAK/STAT IEI	Carsten Speckmann (DE)
18:12 – 18:22	To be informed	
18:22 – 18:32	To be informed	
18:32 – 18:42	RALD study	
18:42 – 18:52	Chediak-Higashi study	
18:52 – 19:00	Discussion	Bénédicte Neven (FR)

Preliminary Programme

Freiburg, Germany
26-28 September 2025

Saturday, 27 September 2025

09:00 – 10:15

Session III: CID and related issues

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

09:00 – 09:07

Case Presentation: *Allogenic Hematopoietic Stem Cell Transplantation for ARPC1B Deficiency: A Single Center Experience from Resource-Constrained Settings*

Dharmagat Bhattarai (NP)

09:07 – 09:22

PCID Study Update

Carsten Speckmann (DE)

09:22 – 09:32

Mixed chimerism in Bu exposed young children

Chris Dvorak (US)

09:32 – 09:42

Flu PK in infants

Sujal Ghosh (DE)

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

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

Robert Zeiser (DE)

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

Jean Sebastien Diana (FR)

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

To be announced

Preliminary Programme

Freiburg, Germany
26-28 September 2025

14:30 – 15:30	Session V: Beyond HLH; HCT for sJIA and Still's Disease Chair: <i>Rebecca Marsh (US)</i>	
14:30 – 14:40	ZNFXI	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	
16:00 – 17:05	Session VI: EBV IEI and EBV driven pathology Chairs: <i>Austen Worth (UK) & Benjamin Fournier (FR)</i>	
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: <i>Marta Rizzi (DE)</i>	
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)

Preliminary Programme

Freiburg, Germany
26-28 September 2025

Sunday, 28 September 2025

09:00 – 10:15

Session VIII: CVID

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

09:00 – 09:07

Case Presentation: *Unmasking Familial Hemophagocytic Lymphohistiocytosis type 3: Chronic Disseminated Rubella Infection Following MMR Vaccination*

Ruben Van Paemel (BE)

09:07 – 09:22

ILD in CVID

Francesco Cinetto (IT)

09:22 – 09:37

Enteropathy in CVID

Klaus Warnatz (DE)

09:37 – 09:52

Novel Biomarkers in CVID

Helen Leavis (NL)

09:52 – 10:02

NFKB1 mutations (or HPO phenotyping) in CVID

Luiza Campos (UK)

10:02 – 10:07

HSCT in CVID update

Emma Morris (UK)

10:07 – 10:15

Q&A

10:15 – 11:25

Session IX: IEI and pancytopenia

Chairs: 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

Chairs: To be announced

11:55 – 12:10

To be announced

12:10 – 12:25

To be announced

12:25 – 12:40

To be announced

12:40 – 12:55

To be announced

12:55 – 13:05

Final Remarks

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