

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 Chairs: Maria Ester Bernardo (IT) & Caroline Lindemans (NL)	
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 – 15:45	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 Chairs: Maria Ester Bernardo (IT) & Caroline Lindemans (NL)	
16:15 – 16:22	Case Presentation: Hematopoietic Stem Cell Transplantation in a Patient with Mucopolysaccharidosis Type II	Gizem Zengin Ersoy (TR)
16:22 – 16:35	Phase I/II trial of HSPC-GT for MPSIIH: 5-year follow-up analysis	Maria Ester Bernardo (IT)
16:35 – 16:49	Phase I/II HSPC-GT for MPSIIIA	Ramya Nataraj (UK)
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	Jenna Nunn (UK)
17:30 – 19:00	Session II: IEWP studies and proposals Chairs: Maria Ester Bernardo (IT) & Claire Booth (UK)	
17:30 – 17:45	Process to initiate IEWP studies and ongoing studies	Claire Booth (UK)
17:45 – 18:00	Osteopetrosis study	Rob Chiesa (UK)
18:00 – 18:07	HSCT for STAT1GOF	Emmeline Buddingh (NL)
18:07 – 18:14	IEWP Consensus Guideline on JAK1 treatment for JAK/STAT IEI	Carsten Speckmann (DE)
18:14 – 18:17	STAT Joint Discussion	
18:17 – 18:27	MHC Class II deficiency study update	Kanchan Rao (UK)
18:27 – 18:37	HLH in sibling pairs: how did the 2018 data evolve?	Violante Gustuti (IT)
18:37 – 18:47	RALD study	Srividya Senthil (UK)
18:47 – 19:00	Joint Discussion	Alexandra Laberko (RU)

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Saturday, 27 September 2025

09:00 – 10:15	Session III: CID and related issues	
09:00 – 09:07	Chairs: Bénédicte Neven (FR) & Michael Albert (DE)	
09:07 – 09:22	Case Presentation: Allogenic Hematopoietic Stem Cell Transplantation for ARPC1B Deficiency: A Single Center Experience from Resource-Constrained Settings	Dharmagat Bhattarai (NP)
09:22 – 09:35	PCID Study Update	Carsten Speckmann (DE)
09:35 – 09:48	Mixed chimerism in Bu exposed young children	Stephan Ehl (DE)
09:48 – 10:15	Treo vs Bu conditioning in SCID	Chris Dvorak (US)
	New insights into IL-7 deficiency and TSLP function	Su Han Lum (UK)
10:15 – 10:45	Coffee Break	Luigi Notarangelo (US)
10:45 – 11:15	Keynote lecture	Robert Zeiser (DE)
	<i>The immunology of GvHD: advances in the understanding of the pathogenesis and novel therapeutic concepts</i>	
11:15 – 12:45	Session IV: Gene Therapy	
11:15 – 11:35	Chairs: Toni Cathomen (DE) & Claire Booth (UK)	
11:35 – 11:45	CGD programme at NIH	Elizabeth Kang (UK)
11:45 – 11:55	X-CGD in vivo GT	Claire Booth (UK)
11:55 – 12:05	p47 CGD Gene therapy consortium	Claire Booth (UK)
12:05 – 12:15	LV X-SCID trial update and planned new trial	Sung-Yun Pai (US)
12:15 – 12:25	Gene editing strategies for APDS1 and STAT1	Emma Morris (UK)
12:25 – 12:35	Munc 13-4 Gene therapy	Jean Sebastien Diana (FR)
12:35 – 12:40	Munc 13-4 gene editing approaches	Toni Cathomen (DE)
12:40 – 12:45	Wiskott Aldrich Syndrome GT update - progressing to license	Alessandro Aiuti (IT)
	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 (I)	
14:00 – 14:10	Chairs: Hilda Mekelenkamp (NL) & Marina Ben Izhak (IL)	
14:10 – 14:20	Introduction of the nursing group, its role, and future plans	Hilda Mekelenkamp (NL)
14:20 – 14:30	Transition to Immunology post BMT - Nursing Perspective	Susan Farish (UK)
	Supporting Patients and Families through the Gene Therapy Process: A Clinical Research Nurse Perspective	Federico Frascchetta (IT)

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14:30 – 15:30

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

Chairs: Rebecca Marsh (US) & Stephan Ehl (DE)

14:30 – 14:40

ZNFX1

14:40 – 14:50

Allogeneic HCT for sJIA-associated Lung Disease: Collaborative Experience

14:50 – 15:00

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

15:00 – 15:10

Long Term Disease Relapse Risk Following Allogeneic HCT

15:10 – 15:30

Joint Discussion

Jana Pachlopnik (CH)

Rebecca Marsh (US)

Benjamin Fournier (FR)

Juliana Silva (UK)

15:30 – 16:00

Coffee Break

16:00 – 17:05

Session VI: EBV IEI and EBV driven pathology

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

16:00 – 16:20

CAEBV – Overview talk

16:20 – 16:35

CAEBV with emphasis on the Cincinnati experience using nivolumab

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

16:50 – 17:05

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

Benjamin Fournier (FR)

Ashish Kumar (US)

Jeremy Rubinstein (US)

Tobias Feuchtinger (DE)

17:05 – 18:05

Session VII: B-cells

Chairs: Marta Rizzi (DE) & Arjan Lankester (NL)

17:05 – 17:25

Regulation of human B lymphopoiesis: lessons from IEI

17:25 – 17:40

Effects of rituximab on post HSCT B cell reconstitution

17:40 – 17:55

Repertoire analysis in B cell reconstitution after HSCT

17:55 – 18:05

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

Marta Rizzi (DE)

Bénédicte Neven (FR)

Mirjam van der Burg (NL)

Manfred Hönig (DE)

Sunday, 28 September 2025

08:30 – 09:00	Nurses Session (II)	
08:30 – 08:40	Chairs: Evey Howley (UK) & Federico Frascchetta (IT) Decision-Making in Treatment Options: Gene Therapy versus BMT - A Patients Experience	Jinhua Xu Bayford (UK) Helen Braggins (UK) Evey Howley (UK) Marina Ben Izhak (IL)
08:40 – 08:50	Thymus Transplantation: Challenges to Admission	
08:50 – 09:00	Hematopoietic Stem Cell Transplantation for Osteopetrosis: Clinical Experience	
09:00 – 10:15	Session VIII: CVID	
09:00 – 09:07	Chairs: Klaus Warnatz (DE) & Emma Morris (UK) Case Presentation: <i>Unmasking Familial Hemophagocytic Lymphohistiocytosis type 3: Chronic Disseminated Rubella Infection Following MMR Vaccination</i>	
09:07 – 09:22	ILD in CVID	Ruben Van Paemel (BE) 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	
10:15 – 10:30	Chairs: Miriam Erlacher (DE) & Brigitte Strahm (DE) The many connections between immunology and hematology	Miriam Erlacher (DE) Marcin Wlodarski (US)
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	Brigitte Strahm (DE) Federica Barzaghi (IT) Luca Vinci (DE)
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: Emerging Indications/New Developments	
11:55 – 12:05	Chairs: Maria Ester Bernardo (IT) & Carsten Speckmann (DE) PLAID Syndrom	Paul Szabolcs (US) Catharina Schütz (DE)
12:05 – 12:15	HSCT in NfKB2	
12:15 – 12:25	The spectrum from chronic-active EBV infection to EBV+ T-cell lymphoma presenting with HLH – HSCT or no HSCT	Helena Lichtenfeld (DE) Sujal Ghosh (DE)
12:25 – 12:35	Flu dosing in infants	
12:35 – 12:45	Final Remarks Tayfun Güngör (CH), Maria Ester Bernardo (IT) & Carsten Speckmann (DE)	