

## WELCOME!

This year, the Annual Conference of the Inborn Errors Working Party (IEWP) will be held in Freiburg, Germany, from 26-28 September 2025. It will be a great pleasure to welcome international physicians and experts with a special interest in cellular therapy for inborn errors, on behalf of IEWP EBMT.

The conference will feature comprehensive sessions on stem cell transplantation and gene therapy across inborn errors of immunity, inborn errors of metabolism, haemoglobinopathies, as well as new indications.

There will be special scientific sessions and insights from distinguished keynote speakers.

We are excited that you have joined us for this key event, which offers a unique platform for networking and visibility among leading professionals.

We look forward to welcoming you to Freiburg!

Maria Ester Bernardo, IEWP Chair

Claire Booth, IEWP Secretary

Bénédicte Neven, former IEWP Chair

Michael Albert, former IEWP Secretary

Carsten Speckmann, Local organiser

Stephan Ehl, Local organiser

## Friday, 26 September 2025

13:00 – 13:45

**Registration & Welcome Coffee**

13:45 – 14:00

**Welcome from the organisers**

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

14:00 – 14:45

**Keynote lecture**

**Chair: Carsten Speckmann (DE)**

*Making sense of human immunity by systems immunology*

**Petter Brodin (UK/SE)**

14:45 – 15:45

**Session I: Immune Responses in HSC-GT for IEMs & Clinical Trials of 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 – 15:25

Case Presentation: Hematopoietic Stem Cell Transplantation in a Patient with Mucopolipidosis Type II

Gizem Zengin Ersoy (TR)

15:25 – 15:45

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

Giulia Consiglieri (IT)

15:45 – 16:00

Phase I/II HSPC-GT for MPSIIIA

Ramya Nataraj (UK)

16:00 – 16:15

Phase I/II HSPC-GT for MPSII

Nathan Jeffreys (UK)

16:15 – 16:30

HSC-GT for Pompe

Pim Pijnappel (NL)

16:30 – 16:45

Trial design of a Phase I/II clinical of HSC-GT for Osteopetrosis

Francesca Tucci (IT)

16:45 – 17:00

Proposed EBMT guidelines for infection prophylaxis and vaccination after HSC-GT for IEWM

Jenna Nunn (UK)

17:00 – 17:30

**Coffee Break**

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)

## 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 – 09:35

Mixed chimerism in Bu exposed young children

Chris Dvorak (US)

09:35 – 09:48

Treo vs Bu conditioning in SCID

Su Han Lum (UK)

09:48 – 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

**Chair: Carsten Speckmann (DE)**

*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

**Chairs:** Toni Cathomen (DE) & Claire Booth (UK)

11:15 – 11:35

CGD programme at NIH

Elizabeth Kang (UK)

11:35 – 11:45

X-CGD in vivo GT

Claire Booth (UK)

11:45 – 11:55

p47 CGD Gene therapy consortium

Claire Booth (UK)

11:55 – 12:05

LV X-SCID trial update and planned new trial

Sung-Yun Pai (US)

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 (I): Beyond Transplantation: Nursing in Immunology & Gene Therapy

**Chairs:** Maria Ester Bernardo (IT) & Marina Ben Izhak (IL)

14:00 – 14:15

Transition to Immunology post BMT - Nursing Perspective

Susan Farish (UK)

14:15 – 14:30

Supporting Patients and Families through the Gene Therapy Process: A Clinical Research Nurse Perspective

Federico Frascchetta (IT)

14:30 – 15:30

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

**Chairs:** Rebecca Marsh (US) & Carsten Speckmann (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	<b>Nurses Session (II): Advanced Treatment Options in Complex Pediatric Cases</b>	
08:30 – 08:40	<b>Chairs:</b> Evey Howley (UK) & Federico Fraschetta (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	<b>Session VIII: CVID</b>	
09:00 – 09:07	<b>Chairs:</b> 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)
09:22 – 09:37	Enteropathy in CVID	Francesco Cinetto (IT)
09:37 – 09:52	Novel Biomarkers in CVID	Klaus Warnatz (DE)
09:52 – 10:02	NFKB1 mutations (or HPO phenotyping) in CVID	Helen Leavis (NL)
10:02 – 10:07	HSCT in CVID update	Luiza Campos (UK)
10:07 – 10:15	Q&A	Emma Morris (UK)
10:15 – 11:25	<b>Session IX: IEI and pancytopenia</b>	
10:15 – 10:30	<b>Chairs:</b> Miriam Erlacher (DE) & Brigitte Strahm (DE) 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	
11:00 – 11:15	Like a chameleon: ADA2 deficiency	Brigitte Strahm (DE)
11:15 – 11:25	It's not always as you think	Federica Barzaghi (IT)
11:25 – 11:55	<b>Coffee Break</b>	Luca Vinci (DE)
11:55 – 12:35	<b>Session X: Emerging Indications/New Developments</b>	
11:55 – 12:05	<b>Chairs:</b> Maria Ester Bernardo (IT) & Carsten Speckmann (DE) PLAID Syndrom	Paul Szabolcs (US)
12:05 – 12:15	HSCT in NfKB2	Catharina Schütz (DE)
12:15 – 12:25	The spectrum from chronic-active EBV infection to EBV+ T-cell lymphoma presenting with HLH – HSCT or no HSCT	
12:25 – 12:35	Flu dosing in infants	Helena Lichtenfeld (DE)
12:35 – 12:45	<b>Final Remarks</b>	Sujal Ghosh (DE)
	Tayfun Güngör (CH), Maria Ester Bernardo (IT) & Carsten Speckmann (DE)	

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