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# Entity Print



Bénédicte Neven  
IEWP Chair  
France

## Major achievements

The year 2024 has been another pivotal year for the Inborn Errors Working Party (IEWP) of the EBMT, marked by groundbreaking studies and collaborative achievements in the field of hematopoietic stem cell transplantation (HSCT) for Inborn Error of Immunity (IEI) and non-malignant genetic disorders.

Several key studies have been published. The retrospective analysis of **HLA-mismatched HSCT with TCRαβ/CD19 depletion or post-transplant cyclophosphamide** (*Blood*) provides valuable insights into these approaches applied to IEI. Other studies investigated HSCT outcomes for **CTLA-4 insufficiency** (*J Allergy Clin Immunol*), **ICF syndrome** (*J Clin Immunol*), **JAGN1 severe congenital neutropenia** (*Blood Advances*) and **C1q deficiency** (*J Clin Immunol*) and have significantly expanded the understanding of transplant strategies in these rare conditions. Another notable publication, in *Blood Advances*, focused on **permissible HLA mismatches in 9/10 HLA-matched pediatric HSCT** using the HLA-EMMA approach, paving the way for improved donor selection practices in pediatric patients.

Moreover, important ongoing studies continue to shape the landscape of HSCT for inborn errors of immunity, including research on **FHL2 and FHL3**, and **long-term outcomes in Chediak-Higashi syndrome, and osteopetrosis**.

In terms of educational initiatives, the **second IEWP Spring School** on HSCT in Inborn Errors of Immunity, held in Montserrat, Spain in June 2024, brought together over 30 international experts and attendees to deepen knowledge and share expertise on cellular therapies in these disorders. This event follows the success of the inaugural edition in 2023 at Frauenchiemsee, Germany.



Inborn Errors Working Party Spring School 2024 - Montserrat Barcelona, Spain - 23-26 June 2024

The IEWP continues to push the boundaries of scientific knowledge and clinical practice, fostering collaboration across Europe and intercontinental to improve patient outcomes and refine transplant protocols.

## Principal research studies

[Outcome of haematopoietic stem cell transplant \(HSCT\) in patients with Chediak Higashi syndrome](#)

[Study type](#)

[Retrospective Studies](#)

[Diseases](#)

[Inherited disorders](#)

[Group](#)

[Inborn Errors Working Party \(IEWP\)](#)

[Type of treatment](#)

[Allogeneic](#)

[Principal investigator](#)

[Reem Elfeky](#)

[Setting the stage for newborn screening and gene therapy: a disease-specific retrospective survey of FHL2 and 3](#)

[Study type](#)

[Retrospective Studies](#)

[Diseases](#)

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[Stephan Ehl, Despina Moshous, Claire Booth, Sujal Ghosh](#)

[Allogeneic haematopoietic stem cell transplantation in infantile osteopetrosis](#)

[Study type](#)

[Retrospective Studies](#)

[Diseases](#)

[Inherited disorders](#)

[Group](#)

-  
[Type of treatment](#)

[Allogeneic](#)

[Principal investigator](#)

[Robert Chiesa](#)

[Allogeneic hematopoietic stem cell transplantation in cartilage hair hypoplasia \(CHH\); a joint EBMT-CIBMTR study](#)

[Study type](#)

[Retrospective Studies](#)

[Diseases](#)

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[Inborn Errors Working Party \(IEWP\)](#)

[Type of treatment](#)

[Allogeneic](#)

[Principal investigator](#)

[Heljä Lång](#)

## **Key publications**

2024

Hematopoietic Stem Cell Transplantation for C1q Deficiency: A Study on Behalf of the EBMT Inborn Errors Working Party

Group

Inborn Errors Working Party (IEWP)

1st listed author

Helena Buso

Journal

J Clin Immunol.

2024

Haematopoietic stem cell transplantation for CTLA-4 insufficiency across Europe: an EBMT Inborn Errors Working Party study

Group

Inborn Errors Working Party (IEWP)

1st listed author

Christo Tsilifis

Journal

J Allergy Clin Immunol.

2024

Allogeneic Hematopoietic Stem Cell Transplantation in Immunodeficiency-Centromeric Instability-Facial Dysmorphism (ICF) Syndrome: an EBMT/ESID Inborn Errors Working Party Study

Group

Inborn Errors Working Party (IEWP)

Other society

1st listed author

Dagmar Berghuis

Journal

J Clin Immunol.

2024

Outcomes of HLA-mismatched HSCT with TCR $\alpha\beta$ /CD19 depletion or post-HSCT cyclophosphamide for inborn errors of immunity

Group

Inborn Errors Working Party (IEWP)

1st listed author

Su Han Lum

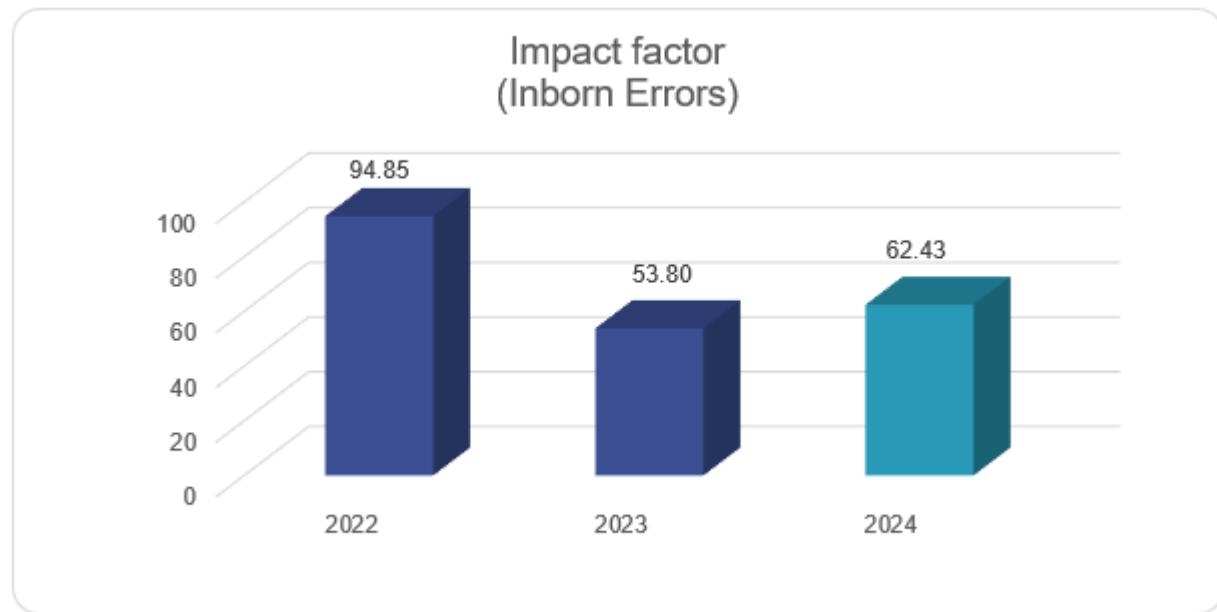
Journal

Blood.

[See the full list of the IEWP 2024 publications](#)



## Indicators



2022 2023 2024

<b>Oral presentations</b>	7	4	4
<b>Poster presentations</b>	0	1	3
<b>Educational events</b>	3	4	2

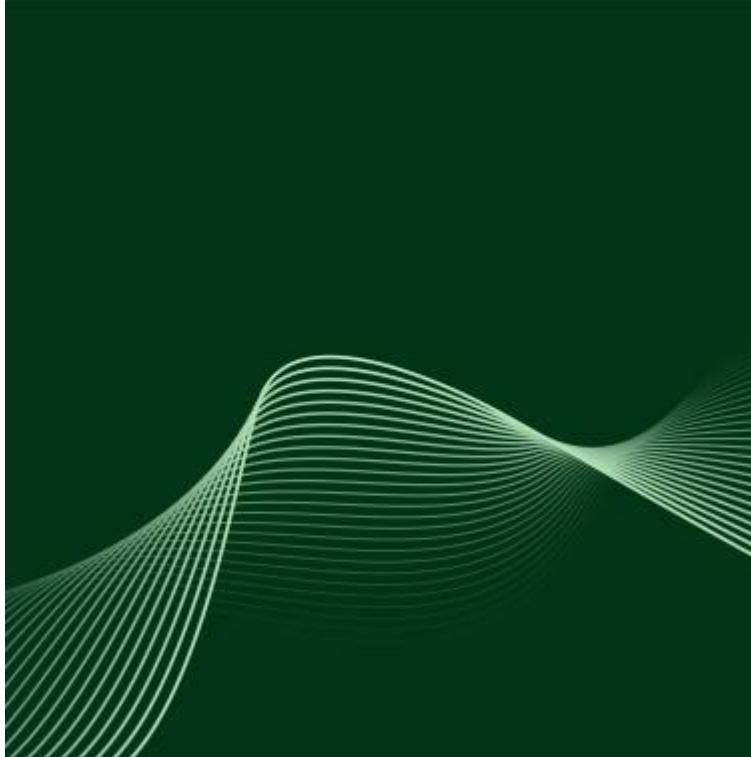
## Major educational events



## Event

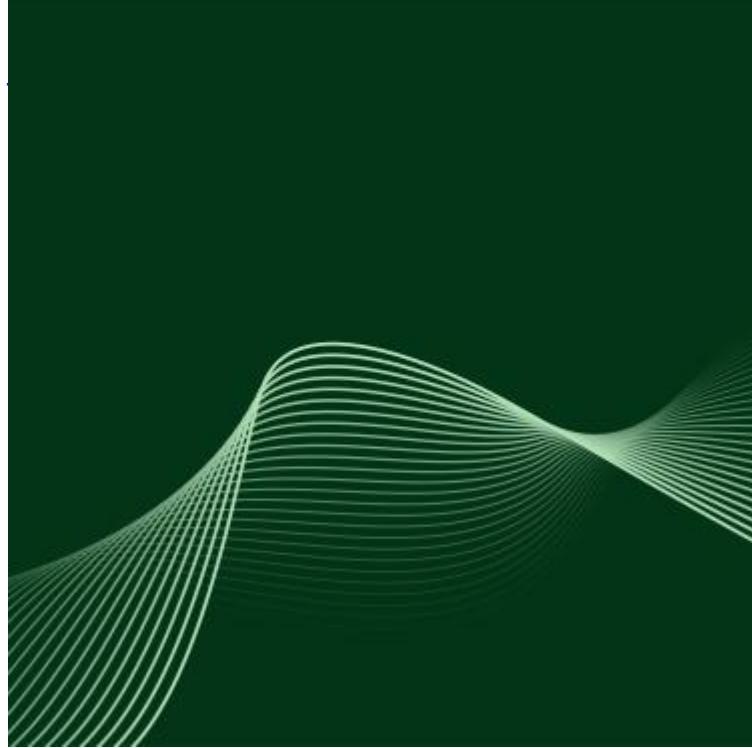
### **50th Annual Meeting of the EBMT**

[Kingdom](#)



## Event

## **Inborn Errors Working Party Spring School 2024**



[Barcelona, Spain](#)

[Event](#)

## **Inborn Errors Working Party Annual Conference**

[Sep 27, 2024 - Sep 29, 2024 / Helsinki, Finland](#)

[Discover more](#)



[Event](#)

## **9th International Transplant and Cellular Therapy Course**

[Sep 06, 2024 - Sep 08, 2024 / Barcelona, Spain](#)

[Discover more](#)

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