

Breadcrumb

Entity Print



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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
Inherited disorders
Group
Inborn Errors Working Party (IEWP)
Type of treatment
Allogeneic
Principal investigator
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 cartillage hair hypoplasia (CHH); a joint EBMT-CIBMTR study
Study type
Retrospective Studies
Diseases
Inherited disorders
Group
Inborn Errors Working Party (IEWP)
Type of treatment
Allogeneic
Principal investigator
Heljä Lång

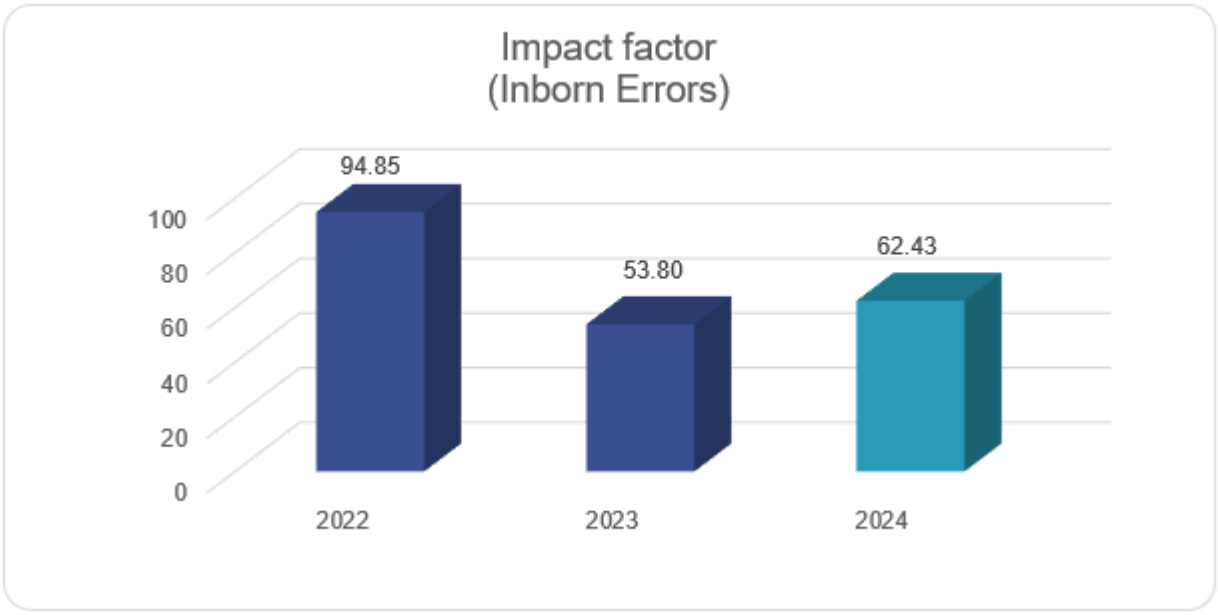
Key publications

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 TCRαβ/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



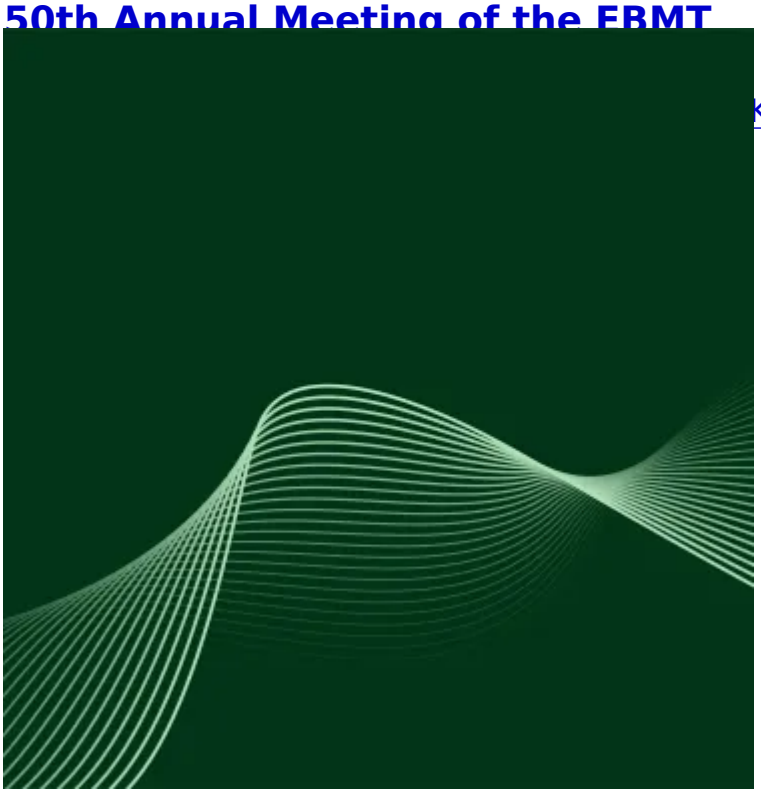
	2022	2023	2024
Oral presentations	7	4	**4**
Poster presentations	0	1	**3**
Educational events	3	4	**2**

Major educational events



Event

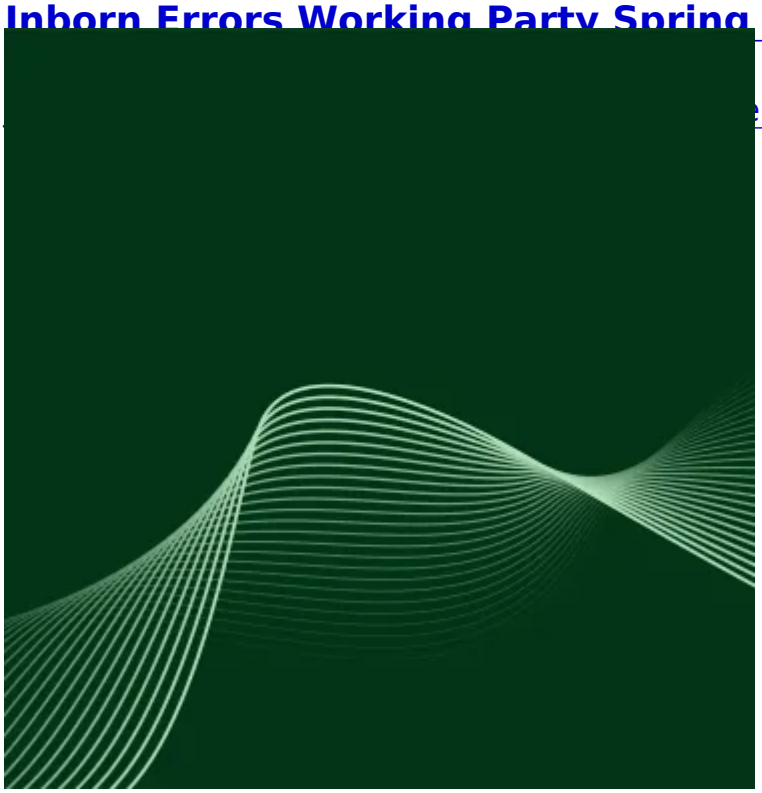
50th Annual Meeting of the EBMT



Kingdom

Event

Inborn Errors Working Party Spring School 2024



elona, 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
VISIT THE IEWP WEBPAGE