

Breadcrumb

Entity Print



Bénédicte Neven
IEWP Chair
France

Major achievements

2023 has once again given us great opportunities to push back the frontiers of our knowledge in the fields of transplantation and cell therapy for immune deficiencies and non-malignant genetic diseases.

Several important studies conducted within the IEWP have been published, such as the results of allograft transplantation in patients with hypomorphic RAG deficiency (in collaboration with the PIDTC), the PREPAD study (Predicting Patient Death after Allogeneic Stem Cell Transplantation for Inborn Errors Using Machine learning), the multicentric retrospective experience of JAK inhibitor treatment for inborn errors of JAK/STAT signalling (in collaboration with ESID). The retrospective study on outcome of HLA-mismatched HSCT in patients with PID following in vitro T-cell depletion with CD3+TCRαβ/CD19 depleted PBSC or in vivo T-cell depleted HSCT with post-transplant cyclophosphamide is under revision.

Important studies are on-going (on FHL2 and 3, haplo-transplant in CGD) or about to start (osteopetrosis, long term outcome in Chediak-Higashi syndrome…).

In June 2023, we met in Brescia for the midterm meeting co-organised with the PDWP and the AIWP. We all enjoyed our annual working party that took place in the nice city of Graz. The 140 participants enjoyed an outstanding scientific program.

Education remained also our top priority. The very first international IEWP spring school on HSCT in IEIs took place in April at Frauenchiemsee in Germany. 22 attendees and 7 faculty members met for 4 days to cover the main specificities of cellular therapies in this field. This event was very much appreciated by attendees. The second edition will take place in Montserrat, close to Barcelona in June 2024.

Principal research studies

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

Key publications

2023
Predicting Patient Death after Allogeneic Stem Cell Transplantation for Inborn Errors Using Machine Learning (PREPAD): A European Society for Blood and Marrow Transplantation Inborn Errors Working Party Study
Group
Inborn Errors Working Party (IEWP)
1st listed author
Erik G J von Asmuth
Journal
Transplant Cell Ther.

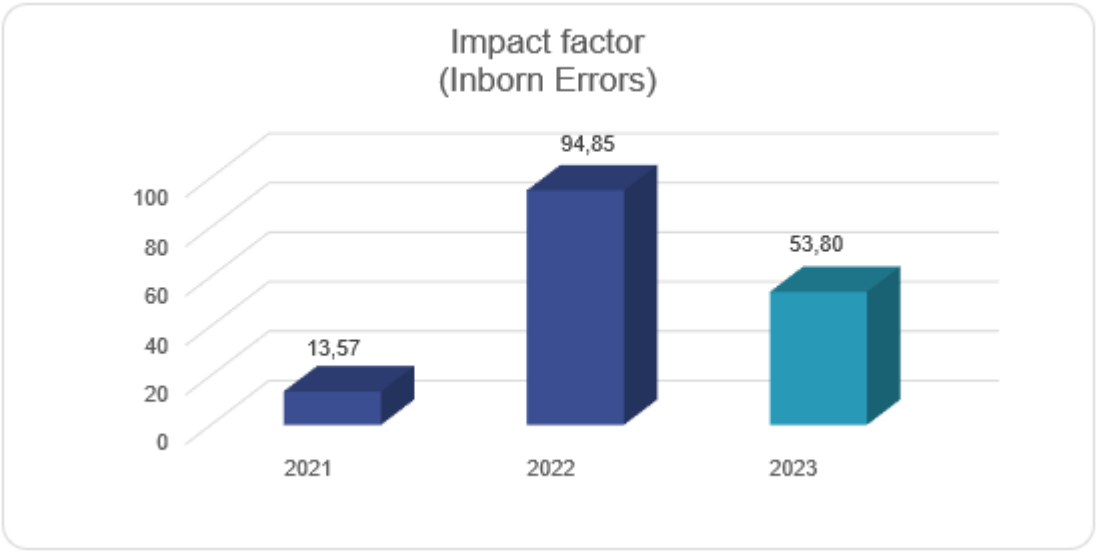
2023
Hypomorphic RAG deficiency: impact of disease burden on survival and thymic recovery argues for early diagnosis and HSCT
Group
Inborn Errors Working Party (IEWP)
1st listed author
C Schuetz
Journal
Blood.

2023
JAK inhibitor treatment for inborn errors of JAK/STAT signaling: An ESID/EBMT-IEWP retrospective study
Group
Inborn Errors Working Party (IEWP)
Other society
1st listed author
Marco Fischer
Journal
J Allergy Clin Immunol.

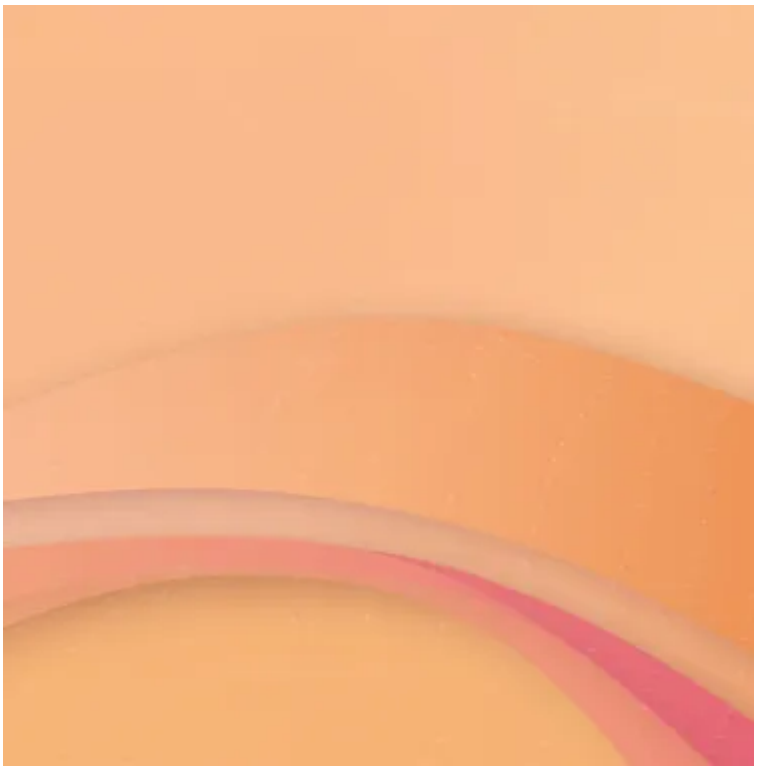
2023
Busulfan-fludarabine- or treosulfan-fludarabine-based conditioning before allogeneic HSCT from matched sibling donors in paediatric patients with sickle cell disease: A study on behalf of the EBMT Paediatric Diseases and Inborn Errors Working Parties
Group
Inborn Errors Working Party (IEWP)

Paediatric Diseases Working Party (PDWP)
1st listed author
Annamária Cseh
Journal
Br J Haematol.
2023
Outcome of haematopoietic cell transplantation in children with lysosomal acid lipase deficiency: a study on behalf of the EBMT Inborn Errors Working Party
Group
Inborn Errors Working Party (IEWP)
1st listed author
Su Han Lum
Journal
Bone Marrow Transplant.
See the full list of the IEWP 2023 publications



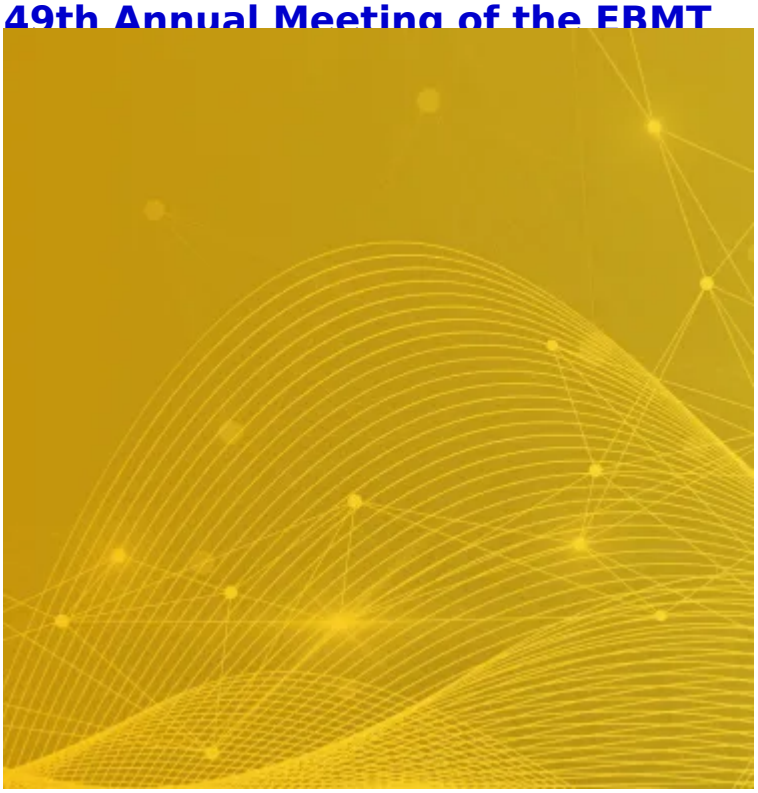


	2021	2022	2023
Oral presentations	3	7	**4**
Poster presentations	1	0	**1**
Educational events	3	3	**4**



[Event]

49th Annual Meeting of the EBMT



Event

Inborn Errors Working Party Spring School on Cellular Therapy for Inborn Errors of Immunity and Hematopoiesis 2023

Apr 02, 2023 - Apr 05, 2023 / Frauenchiemsee, Germany
Discover more
VISIT THE IEWP WEBPAGE