Major achievements

In 2022, we managed to finish and publish three major studies for our community in *Blood*: outcome of HSCT in adult patients with IEIs, outcome of HSCT in Wiskott-Aldrich, and HSCT in hypomorphic RAG deficiency. The latter was a collaborative study with PIDTC.
The large retrospective study on haplo-transplant with TCRab/CD19 depletion or PTCY is in its final steps of manuscript preparation. The study on outcome of patients with IEI after second transplant is progressing very well.

In 2023, the IEWP will continue its scientific activities on important topics in close collaborations with partners such as ESID, ERN RITA, SCETIDE and PIDTC. New studies on HLH2 and HLH3 in collaboration with the HLH-registry have been initiated and data requests to centres should be disseminated soon. A retrospective study on haplo-transplant in CGD is also about to start. A steering committee for writing guidelines regarding late outcome monitoring of transplanted patients with IEs is starting in collaboration with the clinical working party of ESID.

In September 2022, we held our Annual Conference in person in Paris. The 150 participants coming from 15 countries, mainly European, enjoyed an outstanding scientific program. Education remains also a top priority. Postponed by COVID, the very first international IEWP Spring School on HSCT in IEs will finally take place in April 2023 with 22 attendees and 7 faculty members at Frauenchiemsee in Germany.

**Principal research studies**

The outcome of second and third allogeneic conditioned HSCT in patients with non-SCID primary immune deficiency (PID) following primary or secondary graft failure

**Study type**
Retrospective Studies

**Diseases**

- Group
  Inborn Errors Working Party (IEWP)

**Type of Stem Cell Treatment**
Allogeneic

**Principal investigator**
Zohreh Nademi

Outcomes 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

**Study type**
Retrospective Studies
Other non-malignant disorders
Group
Inborn Errors Working Party (IEWP)
Type of Stem Cell Treatment
Allogeneic - Haploidentical
Principal investigator
Mary Slatter & Michael Albert
HSCT experience in patients with Interferon gamma receptor deficiencies
Study type
Retrospective Studies
Diseases

- Group
Inborn Errors Working Party (IEWP)
Type of Stem Cell Treatment

- Principal investigator
Peter Olbrich
Immunodeficiency – centromeric instability – facial dysmorphism (ICF) syndrome: evaluation of characteristics and outcome after allogeneic haematopoietic stem cell transplantation
Study type
Retrospective Studies
Diseases

- Group
Inborn Errors Working Party (IEWP)
Type of Stem Cell Treatment
Allogeneic
Principal investigator
Dagmar Berghuis
A prospective outcome study on patients with profound combined immune deficiency (p-CID)
Study type
Retrospective Studies
Diseases
Group
Inborn Errors Working Party (IEWP)
Type of Stem Cell Treatment
-
Principal investigator
Stephan Ehl

Key publications

2022
Use of eculizumab in children with allogeneic haematopoietic stem cell transplantation associated thrombotic microangiopathy - a multicentre retrospective PDWP and IEWP EBMT study
Group
Inborn Errors Working Party (IEWP)
Paediatric Diseases Working Party (PDWP)
1st listed author
Peter Svec
Journal
Bone Marrow Transplant.

2022
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
Catharina Schuetz
Journal
Blood.

2022
The efficacy and safety of allogeneic stem cell transplantation in Mevalonate Kinase Deficiency
Group
Inborn Errors Working Party (IEWP)
1st listed author
Jerold Jeyaratnam
Journal
Pediatr Rheumatol Online J.
2022
Hematopoietic stem cell transplantation for adolescents and adults with inborn errors of immunity: an EBMT IEWP study

Group
Inborn Errors Working Party (IEWP)

1st listed author
Michael H Albert

Journal
Blood.

2022
Hematopoietic stem cell transplantation for Wiskott-Aldrich syndrome: an EBMT inborn errors working party analysis

Group
Inborn Errors Working Party (IEWP)

1st listed author
Michael H Albert

Journal
Blood.

See the full list of the IEWP 2022 publications
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**Impact factor (Inborn Errors)**

- 2019: 54.00
- 2020: 59.80
- 2021: 13.57
- 2022: 94.85

*Image of Prag cityscape with a sunset*
Event

**Virtual 48th Annual Meeting of the EBMT**

Mar 19, 2022 - Mar 23, 2022 / Online

Discover more

Event

**Inborn Errors Working Party Annual Conference**

Sep 23, 2022 - Sep 25, 2022 / Paris, France

Discover more

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