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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 IEIs 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 IEIs will finally take place in April 2023 with 22 attendees and 7 faculty members at Frauenchiemsee in Germany.

Principal research studies

[HSCT experience in patients with Interferon gamma receptor deficiencies](#)

[Study type](#)

[Retrospective Studies](#)

[Diseases](#)

[Inherited disorders](#)

[Group](#)

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

[Type of treatment](#)

[Allogeneic](#)

[Principal investigator](#)

[Peter Olbrich](#)

[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 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

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



2019 2020 2021 2022

Oral presentations	9	3	3	7
Poster presentations	0	1	1	0
Educational events	3	3	3	3

Major educational events



[Event](#)

[Virtual 48th Annual Meeting of the EBMT](#)



[Event](#)

Inborn Errors Working Party Annual Conference

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

Discover more

VISIT THE IEWP WEBPAGE