EBMT Annual report 2022



ScienceEducationPatient Care & Advocacy

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Partners

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Bénédicte Neven IEWP Chair France

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 <u>IEWP Spring School</u> 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

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

<u>Diseases</u>

Group

Inborn Errors Working Party (IEWP)

Type of treatment

<u>Allogeneic</u>

Principal investigator

Zohreh Nademi

HSCT experience in patients with Interferon gamma receptor deficiencies

Study type

Retrospective Studies

<u>Diseases</u>

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Inborn Errors Working Party (IEWP)

Type of treatment

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Principal investigator

Peter Olbrich

A prospective outcome study on patients with profound combined immune

deficiency (p-CID)

Study type

Retrospective Studies

Diseases

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Group

Inborn Errors Working Party (IEWP)

Type of treatment

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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



Event

Virtual 48th Annual Meeting of the EBMT

Mar 19, 2022 - Mar 23, 2022 / Online <u>Discover more</u>



Event

Inborn Errors Working Party Annual Conference

Sep 23, 2022 - Sep 25, 2022 / Paris, France
Discover more
VISIT THE IEWP WEBPAGE