EBMT Annual report 2021



ScienceEducationPatient Care & Advocacy

Our SocietyOur Mission, Vision & ValuesEquality, Diversity & InclusionEBMT

Membership 2021Organisational Structure 2021Financial Highlights 2021EBMT

Partners

Breadcrumb

1. Home

Entity Print



Bénédicte Neven IEWP Chair France

Major achievements

It is difficult to talk about 2021 without mentioning the difficulties related to the COVID19 pandemic, which severely restricted travel and interactions. In the first part of the year, the organization of two webinars dedicated to the management of severe combined immunodeficiency and cell therapy in metabolic diseases were

nevertheless very successful and allowed, despite the virtual format, very fruitful exchanges between attendees and experts.

In September, we took up the challenge of holding our annual WP in face to face and we met in Milan. The 125 participants coming from 15 countries, mainly European, enjoyed an outstanding scientific program and the return of social interaction!

The year 2021 also saw the finalization of several important publications performed on behalf of the IEWP. in particular, the update recommendations for conditioning regimen in Inborn Errors of Immunity and the latest results of HSCT in severe combined immunodeficiency were finalized and published. A large retrospective study of HSCT in IEIs in more than 300 teenagers and young adults as well as an important study of HSCT in Wiskott-Aldrich syndrome are in their final steps.

In 2022, the IEWP continues its studies activities on important topics in close collaborations with partners such as ESID, RITA, SCETIDE and PIDTC.

Principal research studies

Outcomes of HLA-mismatched HSCT in patients with PID following in vitro T-cell depletion with CD3+TCR $\alpha\beta$ /CD19 depleted PBSC or in vivo T-cell depleted HSCT with post-transplant cyclophosphamide

Study type

Retrospective Studies

Diseases

Other non-malignant disorders

Group

Inborn Errors Working Party (IEWP)

Type of Stem Cell Treatment

Allogeneic - Haploidentical

Principal investigator

Mary Slatter & Michael Albert

Clinical and immunological outcome of CID patients with hypomorphic RAG mutations following HSCT

Study type

Retrospective Studies

Diseases

Other non-malignant disorders

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Inborn Errors Working Party (IEWP)

Type of Stem Cell Treatment

Allogeneic

Principal investigator

Catharina Schuetz & Benedicte Neven

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

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Group

Inborn Errors Working Party (IEWP)

Type of Stem Cell Treatment

Allogeneic

Principal investigator

Zohreh Nademi

Key publications

2021

Hematopoietic cell transplantation in severe combined immunodeficiency: the SCETIDE 2006-2014 European cohort

Group

Inborn Errors Working Party (IEWP)

1st listed author

Arjan C Lankester

<u>Journal</u>

J Allergy Clin Immunol.

2021

EBMT/ESID inborn errors working party guidelines for hematopoietic stem cell transplantation for inborn errors of immunity

Group

Inborn Errors Working Party (IEWP)

Other society

1st listed author

A C Lankester

Journal

Bone Marrow Transplant.

2021

<u>International retrospective study of allogeneic hematopoietic cell transplantation for activated PI3K-delta syndrome</u>

Group

Inborn Errors Working Party (IEWP)

1st listed author

Dimana Dimitrova

Journal

J Allergy Clin Immunol.

2021

Allogeneic hematopoietic stem cell transplantation in leukocyte adhesion deficiency type I and III

Group

Inborn Errors Working Party (IEWP)

1st listed author

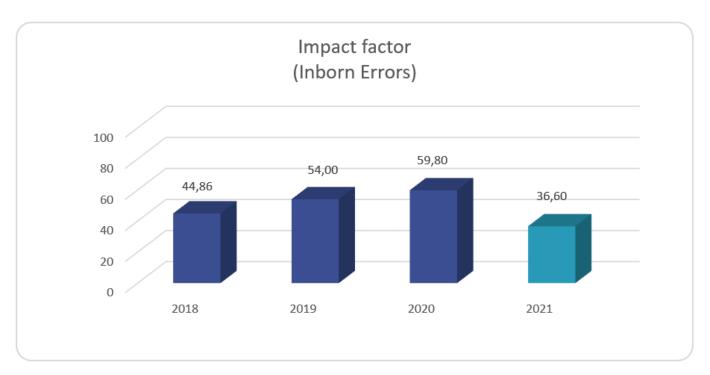
Shahrzad Bakhtiar

Journal

Blood Adv.

See the full list of the IEWP 2021 publications





2019 2020 2021

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



Event

Inborn Errors Working Party Annual Conference

Sep 24, 2021 - Sep 26, 2021 / Milan, Italy Discover more

Webinars and e-courses

<u>Update on diagnostic and treatment of severe combined immunodeficiencies</u> Cellular therapy in inborn errors of metabolism: indications and perspectives