#### <u>EBMT</u> <u>Annual report</u> <u>2021</u>



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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**

Clinical and immunological outcome of CID patients with hypomorphic RAG mutations following HSCT Study type **Retrospective Studies** Diseases Other non-malignant disorders Group Inborn Errors Working Party (IEWP) Type of 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

<u>Group</u>

Inborn Errors Working Party (IEWP) Type of 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 Journal | 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 International retrospective study of allogeneic hematopoietic cell transplantation for activated PI3K-delta syndrome 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

<b>Oral presentations</b>	9	3	3
Poster presentations	0	1	1
<b>Educational events</b>	3	3	3

#### **Major educational events**



#### <u>Event</u>

#### **Inborn Errors Working Party Annual Conference**

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

#### Webinars and e-courses

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