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Major achievements

In this unusual year, and despite the COVID-19 related limitations to meet each other during regular meetings, the IEWP has been able to organize several educational and scientific virtual events that have proven to be very successful and well attended.

In July, the first educational Midterm Focus Meeting (MFM) on stem cell transplantation in non-malignant diseases was organized in collaboration with the SAAWP, PDWP and the Nurses Group. The three IEWP sessions focused on Immune Dysregulation, non-SCID Inborn Errors of Immunity and Inherited Metabolic Disorders. The thematic and interactive Q&A sessions were each attended by the speakers panel and over hundred colleagues. Based on the very positive evaluation a second version of this MFM will be scheduled for the summer of 2021.

The annual Inborn Errors Working Party scientific and business meeting was this year meant to take place in Milano (host: Alessandro Aiuti). Because of the COVID-19 restrictions, the regular meeting was postponed until next year, and replaced by a virtual extended business meeting. More than one hundred international colleagues participated in this event in which current activities and reports on ongoing studies were discussed with the IEWP members. In addition, two excellent seminars were given by Raffaella Di Micco and Bernhard Gentner on behalf of the Milano team. In 2021, we hope that the circumstances will allow us to organize a regular IEWP autumn meeting in Milano.

In 2020, a series of excellent studies were published on behalf of IEWP, plus a number of reviews as part of a Golden Research Topic in Frontiers in Pediatrics on HSCT for Primary Immunodeficiencies and Rare Metabolic Diseases. For 2021 IEWP has the aim to further expand its active study program, which includes both retrospective cohort studies as well as biological studies, mostly performed in collaboration with ESID, SCETIDE, PIDTC and other international partners. In addition, a revised, updated version of the IEWP guidelines for HSCT in Inborn Errors of Immunity will be made available early 2021.

Principal research studies

1. Clinical and immunological outcome after HSCT in SCID: IEWP-SCETIDE study
2. HSCT in Wiskott-Aldrich syndrome comparing Bu-Flu and Treo-Flu conditioning: IEWP-SCETIDE study
3. Long term outcome of HSCT for SCID: SCETIDE-IEWP-PIDTC study
4. HSCT for Erythropoietic Porphyria: EBMT-CIBMTR study
5. HSCT in Inherited Metabolic Diseases with focus on immune cytopenia: IEWP-Eurocord study
6. HSCT in LAL-Wolman’s disease

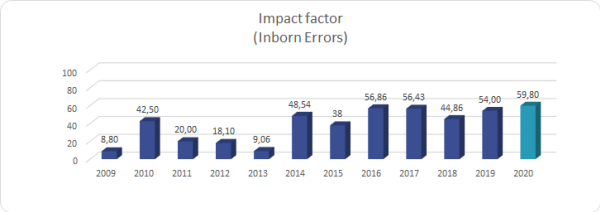
7. HSCT in patients with IFNg receptor deficiencies
8. HSCT in adolescent and adult patients with primary immunodeficiencies
9. Haploidentical SCT in PID comparing PT-Cy and TCRαβ depletion approaches
10. HSCT outcomes in hypomorphic RAG deficiency
11. Characteristics and HSCT outcome in Immunodeficiency, Centromer instability and Facial anomalies (ICF) syndrome
12. HSCT outcome in PNP SCID
13. Prediction outcome based on patient and donor factors (PREPAD)
14. HSCT outcome in mevalonate kinase deficiency
15. Second HSCT in Inborn Errors of Immunity

Key publications

2020
Hematopoietic cell transplantation in chronic granulomatous disease: a study of 712 children and adults
Group
Inborn Errors Working Party (IEWP)
1st listed author
Robert Chiesa
Journal
Blood.
2020
Long-term outcome of LRBA deficiency in 76 patients after various treatment modalities as evaluated by the immune deficiency and dysregulation activity (IDDA) score.
Group
Inborn Errors Working Party (IEWP)
Other society
1st listed author
Tesch VK
Journal
J Allergy Clin Immunol.
2020
Extended clinical and immunological phenotype and transplant outcome in CD27 and CD70 deficiency
Group

Inborn Errors Working Party (IEWP)
1st listed author
Sujal Ghosh
Journal
Blood.
2020
Targeted busulfan-based reduced-intensity conditioning and HLA-matched HSCT cure hemophagocytic lymphohistiocytosis
Group
Inborn Errors Working Party (IEWP)
1st listed author
Matthias Felber
Journal
Blood Adv.
2020
Hematopoietic Stem Cell Transplantation for DNA Double Strand Breakage Repair Disorders
Group
Inborn Errors Working Party (IEWP)
1st listed author
Beata Wolska-Kuśnierz
Journal
Front Pediatr.
See the full list of the IEWP 2020 publications





	2015	2016	2017	2018	2019	2020
Oral presentations	5	2	8	8	9	**3**
Poster presentations	2	3	2	1	0	**1**
International educational events	3	4	4	4	3	**3**

Major educational courses

1. IEWP session at ESID Biannual meeting - 14-17 Oct, 2020.
2. Midterm Focus Meeting on Non-Malignant Diseases - 8-10 July, 2020 / [Virtual].
3. IEWP meeting – 25 Sep, 2020 / [Virtual].