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Major achievements

In 2022, a major focus of the WP activities was both the increase of the educational as well as of the research activity of the PDWP subcommittees. In that view, a dedicated Pediatric Infectious Diseases subcommittee has been established, with Elif Ince and Krzysztof Kalwak as co-chairs, in order to cover pediatric aspects of post-

transplant infectious complications.

On the educational side, “CART in first line ALL therapy, pros and cons”, “Primary treatment of acute GvHD – time for a risk adapted approach?” and “PK and antigen target differences of variant ATGs” are only some exemplary topics that were offered via a pre-recorded webinar and consecutively discussed in a moderated 45 min live session. Internationally recognized speakers with outstanding expertise in their respective field participated, e.g. Tobias Feuchtinger, Francis Ayuk or Jaap Jan Boelens.

From November 10th to 12th the ninth international Haemoglobinopathies Meeting was hosted for the second time by the PDWP, as an extension of the formerly well-established Regensburg’s Sickle Cell Meeting. During the three-days meeting, all aspects of sickle cell disease and thalassemia syndromes were presented by more than 30 international experts worldwide. Each session offered the opportunity to interact directly with the speakers in order to discuss and ask questions. The meeting was attended by more than 130 participants from 24 countries.

As a further focus on haemoglobinopathies, and in order to fill the gap of the EBMT registry, a very extensive questionnaire for haemoglobinopathies was designed and distributed to the centers. With the collection of these very detailed data, five distinguished scientific contributions, which are already evaluated and accepted by the scientific committee of the PDWP, can be elaborated and prepared for publication.

The scientific output in 2022 reached an IF of 61, that was only topped once during the term of the current WP chair with an IF of 86 in 2020. One of the most important publications of this year is the review of Haematopoietic Stem Cell Transplantation (HSCT) for severe autoimmune diseases in children on behalf of the ADWP and the PDWP published by Raffaella Greco et al. on the British Journal of Hematology. The oral presentations are also well above the average of the preceding decade.

Moreover, a Midterm Meeting on New indications in Immune Dysregulatory, Autoinflammatory and Autoimmune Diseases, in collaboration with both the ADWP and IEWP is organised. The meeting will take place in Brescia, Italy from 22 – 24 June 2023.

Principal research studies

Haploidentical stem cell transplantation using PTCY for children with acute leukemia
Study type
Retrospective Studies
Diseases
Acute Lymphatic Leukaemia (ALL)
Acute Myeloid Leukaemia (AML)
Group
Paediatric Diseases Working Party (PDWP)
Type of treatment
Allogeneic - Haploidentical
Principal investigator
Annalisa Ruggeri
Evaluation of Outcomes following Allogeneic Hematopoietic Cell Transplantation in Pediatric Patients with High-Risk Acute Myeloid Leukemia:
Study type
Retrospective Studies
Diseases
Acute Myeloid Leukaemia (AML)
Group
Paediatric Diseases Working Party (PDWP)
Type of treatment
Allogeneic
Allogeneic - Related
Allogeneic - Unrelated
Allogeneic - Haploidentical
Principal investigator
Akshay Sharma (Memphis, US)
HSCT for Congenital Amegakaryocitic thrombopecytopenia (CAMT)
Study type
Closed Survey/Study
Diseases
Bone marrow failure
Group
Paediatric Diseases Working Party (PDWP)
Type of treatment
Allogeneic
Principal investigator
Clémence Aldebert

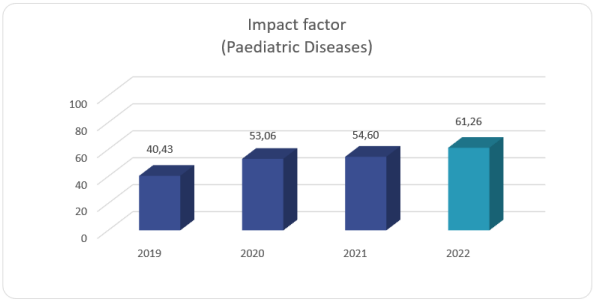
Outcome of children developing grade III-IV aGVHD after allogeneic HSCT
Study type
Retrospective Studies
Diseases
Acute Myeloid Leukaemia (AML)
Group
Paediatric Diseases Working Party (PDWP)
Type of treatment
Allogeneic
Allogeneic - Related
Allogeneic - Unrelated
Allogeneic - Haploidentical
Principal investigator
Giovanna Lucchini

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
Busulfan-fludarabine- or treosulfan-fludarabine-based myeloablative conditioning for children with thalassemia major
Group
Inborn Errors Working Party (IEWP)
Paediatric Diseases Working Party (PDWP)
1st listed author
Roswitha Lüftinger
Journal
Ann Hematol.

2022
Extracorporeal membrane oxygenation in children receiving haematopoietic cell transplantation and immune effector cell therapy: an international and multidisciplinary consensus statement
Group
Paediatric Diseases Working Party (PDWP)
Other society
1st listed author
Matteo Di Nardo
Journal
Lancet Child Adolesc Health.
2022
Safe transfer of pediatric patients from hematopoietic stem cell transplant unit into the pediatric intensive care unit: views of nurses and physicians
Group
Paediatric Diseases Working Party (PDWP)
Nursing Scientific Committee
1st listed author
Daphna Hutt
Journal
Bone Marrow Transplant.
2022
Haematopoietic stem cell transplantation for severe autoimmune diseases in children: A review of current literature, registry activity and future directions on behalf of the autoimmune diseases and paediatric diseases working parties of the European Society for Blood and Marrow Transplantation
Group
Autoimmune Diseases Working Party (ADWP)
Paediatric Diseases Working Party (PDWP)
1st listed author
Federica R. Achini-Gutzwiller
Journal
BJHaem
See the full list of the PDWP 2022 publications





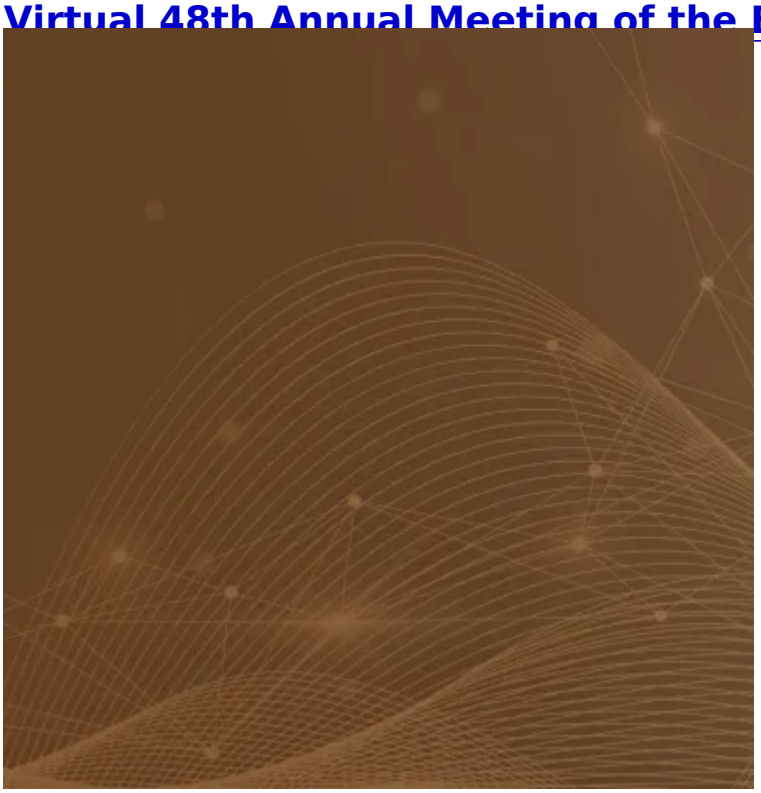
	2019	2020	2021	2022
Oral presentations	8	8	1	**10**
Poster presentations	1	3	1	**3**
Educational events	1	1	1	**1**

Major educational events



Event

Virtual 48th Annual Meeting of the EBMT



Event

Paediatric Diseases Working Party Educational Meeting on Haemoglobinopathies 2022

Nov 10, 2022 - Nov 12, 2022 / Regensburg, Germany
Discover more

PDWP Webinars

1. **How to transplant children and AYAs with ALL**: Christina Peters (speaker) and Katharina Kleinschmidt (moderator).
2. **The injured lung in HSCT – the nowadays diagnostic and therapeutic challenges**: Tayfun Güngör (speaker) and Marianne Ifversen (moderator).
3. **PK and AntigenTarget differences of variant ATGs**: Jaap Jan Boelens (speaker) and Selim Corbacioglu (moderator).
4. **First line treatment of acute GVHD - time for a risk adapted approach?**: Francis Ayuk (speaker) and Daniel Wolff (moderator).
5. **Diagnosis, Grading and Management of Toxicities from Immunotherapies in Children and AYA Patients with Cance**r: Kris M. Mahadeo (speaker) and Hisham Abdel-Azim (moderator).
6. **Vaccination post cellular therapy in children**: Roland Meisel (speaker) and Elif Ince (moderator).
7. **Role and status quo of CART in non-ALL**: Patrick Schlegel (speaker) and Antonio Pérez-Martínez (moderator).
8. **CART in first line ALL Therapy, pros and cons**: Tobias Feuchtinger, Gunnar Cario (speakers) and Adriana Balduzzi (moderator).

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