EBMT Annual report 2023



ScienceEducationPatient Care & Advocacy

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# **Entity Print**



Krzysztof Kalwak PDWP Chair (ELECTED APRIL 2023, PREVIOUS CHAIR: Selim Corbacioglu) Germany

# **Major achievements**

### **Leadership Change**

In April 2023, Krzysztof Kalwak assumed the role of PDWP Chair, succeeding Selim Corbacioglu. Katharina Kleinschmidt was reconfirmed as Secretary.

#### **Organisational Restructuring**

The PDWP underwent a slight reorganisation, including updates to specific subcommittees and the establishment of new subcommittees such as Young Investigators and PICU (see organigram).

#### **Educational Initiatives**

The PDWP prioritised educational efforts this year, participating at two significant events:

- **Midterm Meeting on Immune Diseases:** Held from June 22 24 in Brescia, Italy, this event brought together three EBMT working parties to discuss therapeutic approaches to immune dysregulatory, autoinflammatory, and autoimmune diseases. The meeting aimed to facilitate interdisciplinary discussions among transplant physicians and disease specialists to evaluate transplant and cellular therapy options.
- International Transplant Course: The eighth edition of this course took
  place in September, 2023 in Barcelona, Spain. Notably, a dedicated paediatric
  track was included in this edition, occupying nearly one day. Future editions
  aim to further expand the paediatric program to establish a one and a half day
  event.

#### **Registry Data Quality Improvement**

A major focus of the PDWP is to enhance the quality of registry-based data. Currently, over 20 PDWP studies are either under evaluation or already ongoing, emphasizing the commitment to advancing research in the field.

#### **Prospective Trials**

The PDWP plans to intensify collaborative international prospective trials in the coming years, underscoring the commitment to driving forward research and clinical advancements in paediatric transplant and cellular therapy.

Overall, the PDWP's activities and initiatives demonstrate a multifaceted approach to advancing knowledge, enhancing education, improving data quality, and fostering collaboration in the field of paediatric transplantation and cellular therapy.

# **Principal research studies**

Phase 2 trial to assess haploidentical a/ß T-depleted stem cell transplantation in patients with sickle cell disease with no available sibling donor (T-Haplo-HSCT for SCD)

Study type

-

**Diseases** 

Other non-malignant disorders

Group

Paediatric Diseases Working Party (PDWP)

Type of treatment

Allogeneic - Haploidentical

Principal investigator

Selim Corbacioglu

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

Impact of AB0 Incompatibility on HSCT outcome in hemoglobinopathies: A

Retrospective Registry Study from the Paediatric Diseases Working Party (PDWP) of

the European Society for Blood and Marrow Transplantation (EBMT)

Study type

**Retrospective Studies** 

<u>Diseases</u>

Other non-malignant disorders

<u>Group</u>

Paediatric Diseases Working Party (PDWP)

Haemoglobinopathies Working Party (HWP)

Type of treatment

<u>Allogeneic</u>

Principal investigator

Katharina Kleinschmidt

Myeloablative conditioning for allo-HSCT in pediatric ALL and AML below 2 years

Study type

**Retrospective Studies** 

Diseases

\_

Group

Paediatric Diseases Working Party (PDWP)

Type of treatment

\_

Principal investigator

André Willasch

Prevention and management of conditioning related skin toxicity in paediatric HSCT

patients

Study type

Closed Survey/Study

<u>Diseases</u>

All diseases

Group

Nursing Research Committee

**Nursing Paediatric Committee** 

Paediatric Diseases Working Party (PDWP)

Type of treatment

Allogeneic

<u>Autologous</u>

Principal investigator

Mihaela Hartmann, Hilda Mekelenkamp

# **Key publications**

2023

Specific Cytogenetic Abnormalities at Diagnosis Predict Survival after Hematopoietic Cell Transplant in Poor-Risk Pediatric Acute Myeloid Leukemia: A PDWP/EBMT Study

Group

Paediatric Diseases Working Party (PDWP)

1st listed author

**Akshay Sharma** 

Journal

Bone Marrow Transplant.

2023

Matched unrelated donor transplantation versus haploidentical transplantation with post-transplant cyclophosphamide in children with acute myeloid leukemia: a PDWP-

EBMT study

Group

Cellular Therapy & Immunobiology Working Party (CTIWP)

Paediatric Diseases Working Party (PDWP)

1st listed author

Annalisa Ruggeri

**Journal** 

Haematologica.

2023

Busulfan-fludarabine- or treosulfan-fludarabine-based conditioning before allogeneic HSCT from matched sibling donors in paediatric patients with sickle cell disease: A study on behalf of the EBMT Paediatric Diseases and Inborn Errors Working Parties Group

Inborn Errors Working Party (IEWP)

Paediatric Diseases Working Party (PDWP)

1st listed author

Annamária Cseh

Journal

Br J Haematol.

2023

Decision making on HSCT in patients with hemoglobinopathies; an EBMT Pediatric Diseases Working Party and Inborn Errors Working Party scenario-based survey on physicians' perspectives

Group

**Inborn Errors Working Party (IEWP)** 

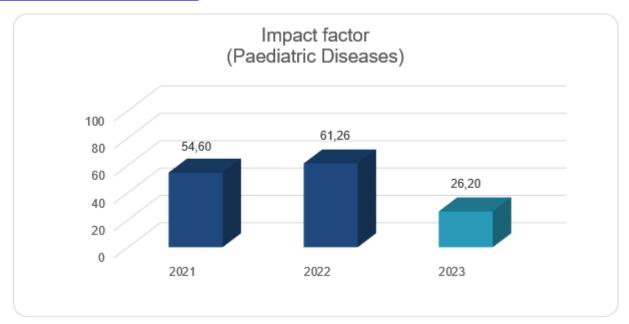
Paediatric Diseases Working Party (PDWP)

1st listed author

Hilda Mekelenkamp

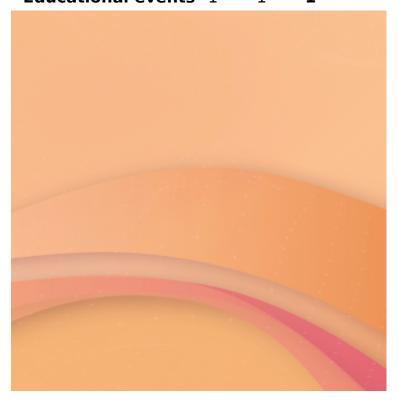
<u>Journal</u>

### Bone Marrow Transplant.



### 2021 2022 2023

Oral presentations 1 10 2
Poster presentations 1 3 2
Educational events 1 1 1



### **Event**



### **Event**

Midterm Meeting on New indications in Immune Dysregulatory,
Autoinflammatory and Autoimmune Diseases

Jun 22, 2023 - Jun 24, 2023 / Brescia, Italy Discover more



### **Event**

# 8th International Transplant and Cellular Therapy Course

Sep 08, 2023 - Sep 10, 2023 / Barcelona, Spain <u>Discover more</u> VISIT THE PDWP WEBPAGE