Major achievements

The elections for the HWP took place in April 2023 and the election of the Chair was announced during the General Assembly at the 49th Annual Meeting of the EBMT on 25 April.
During the rest of the year to set up the WP and start activities the following actions were carried out:

1. Josu de la Fuente was chosen as the WP Secretary
2. Constitution of the Scientific Board
3. Migration of the six studies already approved by the Paediatric WP. It should be noted that these studies were not discussed by this working group. One of these studies is very difficult to be completed because of the complexity of the questions (see below). Migration issues have been agreed with the PWP chair.
4. On-site meeting with the statistics office in Paris. During this meeting it was noted that the compilation of the questionnaire was very limited due to the complexity and number of questions. A new shortened questionnaire was developed and discussed with the PI. A seventh study has been proposed and will be discussed at the next HWP Meeting.
5. Agreement with EHA for development of EHA/EBMT guidelines for gene therapy in Sickle cell disease
6. The first HWP Meeting took place in Jan 2024.

**Principal research studies**

**Outcomes after graft failure of allogeneic hematopoietic stem cell transplantation in patients with SCD**
- **Study type**: Retrospective Studies
- **Diseases**: Other non-malignant disorders
- **Group**: Paediatric Diseases Working Party (PDWP)
- **Haemoglobinopathies Working Party (HWP)**
- **Type of Stem Cell Treatment**: Allogeneic
- **Principal investigator**: Josu de la Fuente

**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
Diseases
Other non-malignant disorders
Group
Paediatric Diseases Working Party (PDWP)
Haemoglobinopathies Working Party (HWP)
Type of Stem Cell Treatment
Allogeneic
Principal investigator
Katharina Kleinschmidt
Trend and outcome of HSCT in Thalassemia inside the EBMT
Study type
Retrospective Studies
Diseases
Other non-malignant disorders
Group
Paediatric Diseases Working Party (PDWP)
Haemoglobinopathies Working Party (HWP)
Type of Stem Cell Treatment
Allogeneic
Principal investigator
Baronciani Donatella
Allogenic Hematopoietic Stem Cell Transplantation for Adult Sickle Cell Disease: A registry study on behalf of the EBMT Pediatric Disease Working Party
Study type
Retrospective Studies
Diseases
Other non-malignant disorders
Group
Paediatric Diseases Working Party (PDWP)
Haemoglobinopathies Working Party (HWP)
Type of Stem Cell Treatment
Allogeneic
Principal investigator
Can Boga
Treo Flu vs Bu Flu +/-Thio in SCD from 2010-2014 (extended to 2017), part of first Medac Study
Study type
Retrospective Studies
Diseases

- Group
  Paediatric Diseases Working Party (PDWP)
Type of Stem Cell Treatment

- Principal investigator
  Cseh Annamaria
Mixed chimerism after related CB HSCT with hemoglobinopathies
Study type
  Retrospective Studies
Diseases
  Other non-malignant disorders
Group
  Paediatric Diseases Working Party (PDWP)
  Haemoglobinopathies Working Party (HWP)
Type of Stem Cell Treatment
  Allogeneic
Principal investigator
  Vanderson Rocha (Brazil)

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