



*EBMT Pediatric Diseases Working Party*  
**BMF and MAS Subcommittee**  
Goals and action plan 2018 - 2022

Aligned with the mission of the Pediatric Diseases Working Party (PDWP) of the European Group for Blood and Marrow Transplantation (EBMT) the activities of the subcommittee of BMF and MAS of the PDWP will focus on:

- **Bone marrow failure syndromes (BMF):** Improving and extending the transplant procedure for patients affected by congenital BMF, including those of ethnical minorities and those who do not have HLA identical donors.
- **Macrophage activation syndrome (MAS):** MAS is increasingly recognized as a post-transplant complication, in particular with the dramatic raise of haploidentical HSCT. Identifying the patients at risk, understanding the pathophysiology and consequently improving diagnosis and treatment is pivotal.
- The safety and quality of the transplant programs.

**Main objectives:**

- Interact with the Aplastic Anemia Working Party (AAWP) and Inborn Errors Working Party (IEWP), that share common objectives to establish collaborations and education programs.
- Establish recommendations for congenital bone marrow failure syndrome patients, including alternative donor transplants in collaboration with the AAWP.
- Collaborate with the outreach subcommittee of the PDWP in education programs on BMF syndromes.
- Increase the knowledge about MAS post transplant.

**Action plan:**

Investigate in the EBMT registry about haploidentical donor transplants in patients with BMF.

Make a survey for EBMT members to learn about MAS post transplant. In particular, we want to know in which patients is it presented, how is it diagnosed and how is it managed.