Severe Aplastic Anaemia Working Party

SAAWP Chair: Régis Peffault de Latour

SAAWP Secretary: Antonio M. Risitano

SAAWP Team: Brian Piepenbroek, Dirk-Jan Eikema, Joe Tuffnell

SAAWP MISSION

The SAAWP strives to share experiences and develop collaborative studies to increase the knowledge in the field of aplastic anaemia and other rare acquired/inherited bone marrow failure disorders. We provide essential data on outcomes after treatment from large numbers of patients that can only be obtained from large registries like the EBMT registry. We also provide guidelines, important clinical information needed to help classify and characterize diseases, data on the natural history of diseases, and late effects that occur after treatment.

CURRENT REGISTRY NUMBERS

In total, 19848 patients are registered in the EBMT registry database with some type of bone marrow failure. The tables below present the numbers per type of disease.

Acquired BMF	n
Aplastic anaemia	14553
Pure red cell aplasia (non congenital)	163
Paroxysmal nocturnal haemoglobinuria (PNH)	797
Pure white cell aplasia	14
Amegakaryocytic thrombocytopaenia (non congenital)	61
Other acquired cytopenic syndrome	303
Unknown	132
TOTAL	**16023**

Genetic BMF	n
Fanconi	2530
Diamond-Blackfan (congenital PRCA)	482
Shwachman-Diamond	101
Dyserythropoietic anaemia	56
Dyskeratosis congenita	189
Amegakaryocytic thrombocytopaenia (congenital)	154
Congenital sideroblastic anaemia	32
Other	227
Unknown	54
TOTAL	**3825**

CURRENT STUDIES

RACE-2: long-term follow-up of patients participating in RACE *(R Peffault de Latour, AM Risitano)*	Ongoing
Umbilical cord blood transplantation in patients with Fanconi anemia *(H Raffi)*	Ongoing
Impact of SARS-CoV-2 vaccination in non-transplanted AA/PNH patients *(M Griffin)*	Analysis
Comparison of outcomes after mismatched unrelated donor and haploidentical donor stem cell transplantation in aplastic anemia *(J Montoro)*	Analysis
Clonal evolution in acquired aplastic anemia *(P de Lima Prata)*	Analysis
Haploidentical stem cell transplantation for congenital bone marrow failure *(S Giardino)*	Manuscript preparation
Transplant Outcomes in Children with Fanconi anaemia *(SH Lum)*	Manuscript preparation
Outcomes of treatment with anti thymocyte globulin (ATG) for acquired pure red cell aplasia *(S Halkes)*	Manuscript preparation
Renal failure in aplastic anemia *(B Drexler)*	Manuscript preparation
Androgens in bone marrow failure disorders *(S Pagliuca, A Kulasekararaj)*	Submitted
GvHD and relapse free survival after allogeneic transplantation for idiopathic severe aplastic anemia *(R Devillier)*	Accepted

SAAWP SESSIONS at EBMT2023

Tuesday, April 25th

Business Meeting
07:00 - 08:45: On-site (no live stream), Room 251

Working Party Session
14:30 - 15:45: On-site (live stream), Room 143

14:30 - 14:50	AA PNH and COVID: all what you want to know in 2023 *Morag Griffin (United Kingdom)*
14:50 - 15:00	HLA-haploidentical stem cell transplantation in children with inherited bone marrow failure syndromes *Stefano Giardino (Italy)*
15:00 - 15:10	BMT in paediatric FA: an update *Su Han Lum (United Kingdom)*
15:10 - 15:30	RACE bio: state of the art *Austin Kulasekararaj (United Kingdom)*
15:30 - 15:45	Questions and Conclusion

RECENT PUBLICATIONS

How we('ll) treat paroxysmal nocturnal haemoglobinuria: diving into the future. Risitano AM, Peffault de Latour R. Br J Haematol. 2022 Jan;196(2):288-303. doi: 10.1111/bjh.17753. Epub 2021 Aug 5.

Stem Cell Transplantation in Patients Affected by Shwachman-Diamond Syndrome: Expert Consensus and Recommendations From the EBMT Severe Aplastic Anaemia Working Party. Cesaro S, *et al*. Transplant Cell Ther. 2022 Oct;28(10):637-649. doi: 10.1016/j.jtct.2022.07.010. Epub 2022 Jul 20.

Hemolytic paroxysmal nocturnal hemoglobinuria: 20 years of medical progress. Peffault de Latour R, *et al*. Semin Hematol. 2022 Jan;59(1):38-46. doi: 10.1053/j.seminhematol.2022.01.001. Epub 2022 Jan 11.

Correction to: Special issues related to the diagnosis and management of acquired aplastic anemia in countries with restricted resources, a report on behalf of the Eastern Mediterranean Blood and Marrow Transplantation (EMBMT) Group and Severe Aplastic Anemia Working Party of the European Society for Blood and Marrow Transplantation (SAAWP of EBMT). Iftikhar R, Ahmed P, *et al*. Bone Marrow Transplant. 2022 Feb;57(2):331. doi: 10.1038/s41409-021-01534-0.

Upfront Alternative Donor Transplant versus Immunosuppressive Therapy in Patients with Severe Aplastic Anemia Who Lack a Fully HLA-Matched Related Donor: Systematic Review and Meta-Analysis of Retrospective Studies, on Behalf of the Severe Aplastic Anemia Working Party of the European Group for Blood and Marrow Transplantation. Alotaibi H, *et al*. Transplant Cell Ther. 2022 Feb;28(2):105.e1-105.e7. doi: 10.1016/j.jtct.2021.10.006. Epub 2021 Oct 11.

Eltrombopag Added to Immunosuppression in Severe Aplastic Anemia. Peffault de Latour R, *et al*. N Engl J Med. 2022 Jan 6;386(1):11-23. doi: 10.1056/NEJMoa2109965.

CONTACT SAAWP

Would you like to receive information on our studies, submit a research proposal, or become a SAAWP member and help advance our research?

Feel free to contact us at: saawpebmt@lumc.nl

Or scan the QR Code to visit the SAAWP webpage:

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