SEVERE APLASTIC ANAEMIA WORKING PARTY

Antonio M. Risitano *(Chair)*
Brian Piepenbroek *(Study Coordinator Lead)*
Dirk-Jan Eikema *(Statistician)*
Kelly Hennessy *(Nurse)*
Austin Kulasekararaj *(Secretary)*
Joe Tuffnell *(Study Coordinator)*
Simona Iacobelli *(Statistician)*

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.

EBMT REGISTRY

In total, 21.357 patients are registered in the Registry with an acquired or genetic bone marrow failure (01-2025). The tables below present the numbers per disease.

Acquired BMF	*n*
Aplastic anaemia	15.726
Pure red cell aplasia (non congenital)	186
Paroxysmal nocturnal haemoglobinuria (PNH)	975
Pure white cell aplasia	18
Amegakaryocytic thrombocytopaenia (non congenital)	72
Other acquired cytopenic syndrome	321
TOTAL	**17.298**

Genetic BMF	*n*
Fanconi	2.671
Diamond-Blackfan (congenital PRCA)	532
Shwachman-Diamond	114
Dyserythropoietic anaemia	62
Dyskeratosis congenita	209
Amegakaryocytic thrombocytopaenia (congenital)	171
Congenital sideroblastic anaemia	34
Other	266
TOTAL	**4.059**

Please register all patients with an SAA diagnosis, including those who have <u>only</u> received IST.

RECENT PUBLICATIONS

Outpatient subcutaneous alemtuzumab is feasible and safe for aplastic anemia and associated with high response rates. da Fonseca ARB, *et al*. Blood Adv. 2024 Dec 18:bloodadvances.2024014159. doi: 10.1182/bloodadvances.2024014159. Online ahead of print.

Outcomes of hematopoietic stem cell transplantation in 813 pediatric patients with Fanconi anemia. Lum SH, *et al*. Blood. 2024 Sep 19;144(12):1329-1342. doi: 10.1182/blood.2023022751.

Alternative donor transplantation for severe aplastic anemia: a comparative study of the SAAWP EBMT. Montoro J, *et al*. Blood. 2024 Jul 18;144(3):323-333. doi: 10.1182/blood.2024024173.

HLA-haploidentical stem cell transplantation in children with inherited bone marrow failure syndromes: A retrospective analysis on behalf of EBMT severe aplastic Anemia and pediatric diseases working parties. Giardino S, *et al*. Am J Hematol. 2024 Jun;99(6):1066-1076. doi: 10.1002/ajh.27293. Epub 2024 Mar 18.

Oral Iptacopan Monotherapy in Paroxysmal Nocturnal Hemoglobinuria. Peffault de Latour R, *et al*. N Engl J Med. 2024 Mar 14;390(11):994-1008. doi: 10.1056/NEJMoa2308695.

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: saawp@ebmt.org

SAAWP SESSIONS at EBMT2025

Monday, 31 March 2025

Business Meeting

07:00 - 08:45: On-site (no live stream), Michelangelo (S-1)

Working Party Session

09:00 - 10:15: On-site (live stream), Michelangelo (S-1)

09:00 - 09:10	SAAWP Activities *Austin Kulasekararaj (London, United Kingdom)*
09:10 - 09:20	Impact of age and donor type in transplant for SAA in the decade 2011-2020: a retrospective SAAWP study *Speaker to be confirmed*
09:20 - 09:30	HSCT vs IST as front-line treatment for SAA *Speaker to be confirmed*
09:30 - 09:45	Round Table: How I treat SAA in real life: burning question 1- How do I role out inherited BMF? *Flore Sicre de Fontbrune (Paris, France)*
09:45 - 10:00	Round Table: How I treat SAA in real life: burning question 2- Do I offer haplo HSCT upfront? To which patients? *Amy De Zern (Baltimore, United States of America)*
10:00 - 10:15	Round Table: How I treat SAA in real life: burning question 3- How do I manage Tpo-mimetics long-term in patients lacking/losing complete response *Beatrice Drexler (Basel, Switzerland)*

RACE STUDIES

RACE trial: final study report *(R Peffault de Latour, AM Risitano, A Kulasekararaj, S Iacobelli)*	Manuscript preparation
RACE biological study *(R Peffault de Latour, AM Risitano, A Kulasekararaj, S Iacobelli)*	Manuscript preparation
RACE-2: long-term follow-up of patients participating in RACE *(R Peffault de Latour, AM Risitano)*	Ongoing

CURRENT STUDIES

Fertility in SAA patients after Allo HCT prepared with TBI 2gy *(A Bagicalupo, N Mordini, F Sicre de Fontbrune, S Giammarco)*	Feasibility
Outcome of transplant in PNH patients treated after 2011 *(C Frieri)*	Data collection
Outcomes of different rabbit ATG brands in patients with AA undergoing Allo HCT *(F Beier)*	Analysis
Outcome of transplant in SAA patients older than 40 years treated after 2010 *(AM Risitano)*	Analysis
Outcomes of treatment with anti-thymocyte globulin (ATG) for acquired pure red cell aplasia *(S Halkes)*	Analysis
Post-transplant cyclophosphamide as GVHD prophylaxis in patients with aplastic anemia with different donors *(J Montoro)*	Manuscript submitted
Clonal evolution in acquired aplastic anemia *(P de Lima Prata)*	Manuscript revision

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