SEVERE APLASTIC ANAEMIA WORKING PARTY

Antonio M. Risitano *(Chair)*
Brian Piepenbroek *(Study coordinator)*
Dirk-Jan Eikema *(Statistician)*
Kelly Hennessy *(Nurse)*
Austin Kulasekararaj *(Secretary)*
Joe Tuffnell *(Data manager)*
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, 19797 patients are registered in the Registry with an acquired or genetic bone marrow failure (03-2024). The tables below present the numbers per disease.

Acquired BMF	*n*
Aplastic anaemia	14414
Pure red cell aplasia (non congenital)	165
Paroxysmal nocturnal haemoglobinuria (PNH)	887
Pure white cell aplasia	15
Amegakaryocytic thrombocytopaenia (non congenital)	64
Other acquired cytopenic syndrome	305
Unknown	139
TOTAL	**15989**

Genetic BMF	*n*
Fanconi	2468
Diamond-Blackfan (congenital PRCA)	487
Shwachman-Diamond	107
Dyserythropoietic anaemia	58
Dyskeratosis congenita	197
Amegakaryocytic thrombocytopaenia (congenital)	160
Congenital sideroblastic anaemia	32
Other	242
Unknown	57
TOTAL	**3808**

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

RECENT PUBLICATIONS

Umbilical Cord Blood Transplantation for Fanconi Anemia with a special focus on late complications: a Study on Behalf of Eurocord and SAAWP-EBMT. Rafii H, *et al*. Transplant Cell Ther. 2024 Mar 5:S2666-6367(24)00248-3. doi: 10.1016/j.jtct.2024.02.024.

Plain language summary of RACE study results: addition of eltrombopag to standard treatment of severe aplastic anemia. Peffault de Latour R, *et al*. Immunotherapy. 2024 Feb;16(3):135-142. doi: 10.2217/imt-2023-0200. Epub 2023 Dec 13.

The complement alternative pathway in paroxysmal nocturnal hemoglobinuria: From a pathogenic mechanism to a therapeutic target. Risitano AM, *et al*. Immunol Rev. 2023 Jan;313(1):262-278. doi: 10.1111/imr.13137. Epub 2022 Sep 15.

Graft- versus-host disease and relapse/rejection-free survival after allogeneic transplantation for idiopathic severe aplastic anemia: a comprehensive analysis from the SAAWP of the EBMT. Devillier R, *et al*. Haematologica. 2023 Sep 1;108(9):2305-2315. doi: 10.3324/haematol.2022.281876.

Current use of androgens in bone marrow failure disorders: a report from the Severe Aplastic Anemia Working Party of the European Society of Blood and Marrow Transplantation. Pagliuca S, *et al*. Haematologica. 2023 May 18. doi: 10.3324/haematol.2023.282935.

SARS-CoV-2 vaccination in 361 non-transplanted patients with aplastic anemia and/or paroxysmal nocturnal hemoglobinuria. Griffin M, *et al*. Haematologica. 2024 Jan 1;109(1):283-286. doi: 10.3324/haematol.2023.283863.

Risk factors for thromboembolic events in patients with paroxysmal nocturnal hemoglobinuria (PNH): a nested case-control study in the International PNH Registry. Höchsmann B, *et al*. Ann Hematol. 2023 Nov;102(11):2979-2988. doi: 10.1007/s00277-023-05402-3. Epub 2023 Sep 5.

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

SAAWP SESSIONS at EBMT2024

Monday, 15 April 2024

Business Meeting

07:00 - 08:45 (BST): On-site (no live stream), ALSH

Working Party Session

11:00 - 12:15 (BST): On-site (live stream), FORTH

11:00 - 11:10	Current and future SAAWP studies *Antonio Risitano (Italy)*
11:10 - 11:30	Case-based discussion: haplo-HSCT upfront? EPAG dependency? Somatic mutations? *Austin Kulasekararaj (United Kingdom)*
11:30 - 11:45	Outcome of HSCT from different types of alterative donors *Juan Montoro Gómez (Spain)*
11:45 - 12:00	RACE biological study: somatic mutations in SAA patients *Deniz Ece Kaya (United Kingdom)*
12:00 - 12:15	RACE biological study: immune abnormalities in SAA patients *Giorgio Napolitani (United Kingdom)*

RACE STUDIES

RACE trial: final study report *(R Peffault de Latour, AM Risitano, A Kulasekararaj, S Iacobelli)* Oral presentation by Antonio Risitano at the Presidential Symposium on 15 April at 17:24 (BST)	Manuscript preparation (Letter to Blood)
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

Outcome of transplant in PNH patients treated after 2011 *(C Frieri)*	Data file preparation
Outcome of transplant in SAA patients older than 40 years treated after 2010 *(AM Risitano)*	Analysis
Allogeneic Hematopoietic Stem Cell Transplantation for ERCC6L2 disease *(U Wartiovaara-Kautto, O Kilpivaara, FS de Fontbrune)*	Data collection
Allogeneic HSCT in ICU setting *(T Güngör)*	Data collection
Post-transplant cyclophosphamide as GVHD prophylaxis in patients with aplastic anemia with different donors *(J Montoro)*	Analysis
Outcomes of treatment with anti-thymocyte globulin (ATG) for acquired pure red cell aplasia *(S Halkes)*	Analysis
Clonal evolution in acquired aplastic anemia *(P de Lima Prata)*	Writing
Renal failure in aplastic anemia *(B Drexler)*	Writing
Comparison of outcomes after mismatched unrelated donor and haploidentical donor stem cell transplantation in aplastic anemia *(J Montoro)*	Manuscript revision
Haploidentical stem cell transplantation for congenital bone marrow failure *(S Giardino)*	Manuscript accepted
Transplant Outcomes in Children with Fanconi anaemia *(SH Lum)*	Submitted