



SEVERE APLASTIC ANAEMIA WORKING PARTY

Antonio M. Risitano (*Chair*)

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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

A total of 22.111 acquired or genetic bone marrow failure diagnoses are registered in the EBMT Registry, corresponding to 21.974 patients (02-2026). The tables below present the numbers per disease.

Acquired BMF	n	Genetic BMF	n
Aplastic anaemia	16.042	Fanconi	2.789
Pure red cell aplasia (non congenital)	183	Diamond-Blackfan (congenital PRCA)	563
Paroxysmal nocturnal haemoglobinuria (PNH)	1000	Shwachman-Diamond	120
Pure white cell aplasia	18	Dyserythropoietic anaemia	64
Amegakaryocytic thrombocytopaenia (non congenital)	74	Dyskeratosis congenita	219
Other acquired cytopenic syndrome	479	Amegakaryocytic thrombocytopaenia (congenital)	180
		Congenital sideroblastic anaemia	41
		Other	339
TOTAL	17.796	TOTAL	4.315

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

RECENT PUBLICATIONS

PNH extravascular hemolysis: exploring clinical significance. Notaro R, Risitano AM. Blood Adv. 2026 Feb 4: bloodadvances.2025019301. doi:10.1182/bloodadvances.2025019301. Online ahead of print.

Allogeneic Hematopoietic Stem Cell Transplantation in ERCC6L2 Disease. Hakkarainen M, et al. Blood Adv. 2026 Feb 2: bloodadvances.2025018349. doi: 10.1182/bloodadvances.2025018349. Online ahead of print.

Complement Biomarkers for Monitoring Last-Generation Complement Inhibitors in Paroxysmal Nocturnal Hemoglobinuria Patients. Risitano AM, et al. Am J Hematol. 2026 Feb 1. doi: 10.1002/ajh.70222. Online ahead of print.

Insights in bone marrow failure syndromes: take home messages from the 3rd ESH-EBMT-EHA-IPIG translational research conference. Cacace F, et al. BMT. 2025 Dec;60(12):1551-1558. doi: 10.1038/s41409-025-02710-2. Epub 2025 Sep 20.

Hematopoietic stem cell transplantation is effective in achieving long-term survival for post-aplastic anemia myeloid neoplasms: the EBMT Severe Aplastic Anemia Report. Prata PH, et al. Haematologica. 2026 Jan 1;111(1):168-176. doi: 10.3324/haematol.2024.287205. Epub 2025 Jul 3.

Donor impact on allogeneic transplant outcomes with PTCy for severe aplastic anemia: a study of the SAAWP EBMT. Montoro J, et al. Bone Marrow Transplant. 2025 Aug;60(8):1152-1159. doi: 10.1038/s41409-025-02633-y. Epub 2025 May 27.

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 EBMT2026

Monday, 23 March 2026

Business Meeting

07:00 - 08:45: On-site (no live stream), N117

Working Party Session

09:00 - 10:15: On-site (live stream), N117

09:00 - 09:10 SAAWP activities, projects and studies
Antonio M. Risitano (Avellino, Italy)

09:10 - 09:25 HCT for Dyskeratosis Congenita: an updated study from the SAAWP | Fabiana Cacace (Naples, Italy)

09:25 - 09:40 Critical decision points in acquired Aplastic Anemia: Diagnosis, therapy selection, and IST management
Philip Scheinberg (Sao Paulo, Brazil)

09:40 - 09:50 Q&A

09:50 - 10:05 Gene therapy for inherited bone marrow failure syndromes
Paula Rio (Madrid, Spain)

10:05 - 10:15 Q&A

RACE STUDIES

RACE trial: final study report Manuscript submitted
(R Peffault de Latour, AM Risitano, A Kulasekararaj, S Iacobelli)

RACE biological study: Clonal Dynamics of Hematopoiesis Manuscript submitted
(R Peffault de Latour, AM Risitano, A Kulasekararaj, S Iacobelli)

RACE biological study: CSMD1 Manuscript preparation
(R Peffault de Latour, AM Risitano, A Kulasekararaj, S Iacobelli)

RACE biological study: Immunology Manuscript preparation
(R Peffault de Latour, AM Risitano, A Kulasekararaj, S Iacobelli)

RACE-2: long-term follow-up of patients participating in RACE Data collection
(R Peffault de Latour, AM Risitano)

CURRENT STUDIES

Comparison of outcomes of IST and HCT using RACE trial data and a registry transplant cohort Data file preparation
(S Iacobelli, AM Risitano)

RACE: Impact of hematological response after IST on Quality of Life in AA Analysis
(IN Lommerse)

Outcomes of Hematopoietic Cell Transplantation in Dyskeratosis Congenita Data collection
(Fabiana Cacace)

Fertility in SAA patients after Allo HCT prepared with TBI 2gy Data file preparation
(A Bagicalupo, N Mordini, F Sicre de Fontbrune, S Giammarco)

Outcome of transplant in PNH patients treated after 2011 Analysis
(C Frieri)

Outcomes of different rabbit ATG brands in patients with AA undergoing Allo HCT Manuscript preparation
(F Beier)

Outcome of transplant in SAA patients older than 40 years treated after 2010 Manuscript preparation
(AM Risitano)

Outcomes of treatment with anti-thymocyte globulin (ATG) for acquired pure red cell aplasia Manuscript preparation
(S Halkes)

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