



Severe Aplastic Anaemia Working Party

Update and future perspectives

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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 (e.g. SAAWP Recommendations for COVID-19), important clinical information needed to help classify and characterise diseases, data on the natural history of diseases, and late effects that occur after treatment.

CURRENT REGISTRY NUMBERS

In total, 17659 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	Genetic BMF	n
Aplastic anaemia	12980	Fanconi	2259
Pure red cell aplasia (non congenital)	146	Diamond-Blackfan (congenital PRCA)	405
Paroxysmal nocturnal haemoglobinuria (PNH)	705	Shwachman-Diamond	77
Pure white cell aplasia	14	Dyserythropoietic anaemia	52
Amegakaryocytic thrombocytopaenia (non congenital)	62	Dyskeratosis congenita	149
Other	285	Amegakaryocytic thrombocytopaenia (congenital)	136
Unknown	123	Congenital sideroblastic anaemia	28
TOTAL	14315	Other	185
		Unknown	53
		TOTAL	3344

ELTROMBOPAG CLINICAL TRIALS

Current clinical trials with Eltrombopag as part of the initial treatment of AA in Europe (EMAA and RACE). RACE is approaching its final closeout appointment and RACE Follow-Up is scheduled to start this year. EMAA is still recruiting patients.

	moderate AA (EMAA) **RECRUITING**	vSAA / SAA (RACE)
Primary objective	PR + CR at 6 months	CR at 3 months
Inclusion criteria	- age \geq 18 years - Treatment requiring MAA (transfusion dependency or ANC < 1G/l or Thrombo < 30G/l or Hb < 8,5g/dl & Reti < 60G/l)	- age \geq 15 years - SAA/ vSAA - No primary allo-SCT
Treatment	CsA + Eltrombopag versus CsA + Placebo	hATG (ATGAM) + CsA + Eltrombopag versus h ATG + CsA
Eltrombopag dosage	150 mg (225 mg)	150 mg
Design	Placebo controlled	Open label
# Patients	2 x 58	2 x 100
Sponsor	University Hospital Ulm	EBMT

ACTIVE STUDIES – CALL FOR DATA

Impact of SARS-CoV-2 vaccination in non-transplanted AA/PNH patients (in collaboration with IDWP)

PI: Dr. Morag Griffin

There is a reporting bias within the literature favoured towards positive events (e.g. relapse AA). A wider review for patients undergoing vaccination for SARS-CoV-2 with PNH and aplastic anaemia is proposed. Patients are eligible if: 1) they have been diagnosed with aplastic anaemia and/or PNH, irrespective of vaccination status; 2) they have not yet undergone a bone marrow transplant.

Efficacy of ATG treatment in acquired PRCA patients

PI: Dr. Stijn Halkes

Based on old small case series, ATG is proposed to be an effective treatment in acquired PRCA (aPRCA) patients who do not respond to immune suppressive treatment. To verify if ATG indeed provides an enduring response as >second line treatment in aPRCA, we want to retrospectively study the efficacy of ATG treatment in a large cohort of patients with aPRCA. Patients are eligible if: 1) they have been diagnosed with acquired PRCA and 2) received ATG treatment between 2000 and 2018. *Of note:* patients who fulfil these criteria but have not received a transplant are eligible as well!

If you are interested to participate in these studies, we are very grateful if you could indicate your interest to Anne Lippinkhof (saawpebmt@lumc.nl) before April 1st, 2021.

RECENT PUBLICATIONS

COVID-19 Bone Marrow Failure and PNH - Recommendations on behalf of the SAAWP, EBMT: <https://www.ebmt.org/ebmt/documents/covid-19-bone-marrow-failure-and-pnh>

Zubicaray J, Pagliara D, Sevilla J, *et al.* Haplo-identical or mismatched unrelated donor hematopoietic cell transplantation for Fanconi anemia: results from the Severe Aplastic Anemia Working Party of the EBMT. *Am J Hematol.* 2021 Feb 19.

Miano M, Eikema DA, de la Fuente J, *et al.* Stem Cell Transplantation for Diamond-Blackfan Anemia. A Retrospective Study on Behalf of the SAAWP of the EBMT. *Transplantation and Cellular Therapy, Official Publication of the American Society for Transplantation and Cellular Therapy, Volume 0, Issue 0.* 2020 Dec 24.

Giardino, S, de Latour, RP, Aljurf, M, *et al.* Outcome of patients with Fanconi anemia developing myelodysplasia and acute leukemia who received allogeneic hematopoietic stem cell transplantation: A retrospective analysis on behalf of EBMT group. *Am J Hematol.* 2020; 95: 809– 816.

Mohseny AB, Eikema DA, Neven B, *et al.* Hematopoietic Stem Cell Transplantation for Hepatitis-associated Aplastic Anemia Following Liver Transplantation for Nonviral Hepatitis: A Retrospective Analysis and a Review of the Literature by the SAAWP of the EBMT. *J Pediatr Hematol Oncol.* 2020 Dec 2.

ElGohary G, El Fakih R, de Latour R, *et al.* Haploidentical hematopoietic stem cell transplantation in aplastic anemia: a systematic review and meta-analysis of clinical outcome on behalf of the SAAWP of the EBMT. *Bone Marrow Transplant.* 2020 Oct;55(10):1906-1917.

Cesaro S, Pillon M, Sauer M, *et al.* Long-term outcome after allogeneic hematopoietic stem cell transplantation for Shwachman-Diamond syndrome: a retrospective analysis and a review of the literature by the SAAWP of the EBMT. *Bone Marrow Transplant.* 2020 Sep;55(9):1796-1809.

Miller PDE, Snowden JA, De Latour RP, *et al.* Autoimmune cytopenias (AIC) following allogeneic haematopoietic stem cell transplant for acquired aplastic anaemia: a joint study of the Autoimmune Diseases and Severe Aplastic Anaemia Working Parties (ADWP/SAAWP) of the EBMT. *Bone Marrow Transplant.* 2020 Feb;55(2):441-451.

Prata PH, Eikema DJ, Afansyev B, *et al.*; SAA WP of the EBMT. Haploidentical transplantation and posttransplant cyclophosphamide for treating aplastic anemia patients: a report from the EBMT Severe Aplastic Anemia Working Party. *Bone Marrow Transplant.* 2020 Jun;55(6):1050-1058.

EBMT2021 WORKING PARTY SESSION

Available in the on-demand library throughout the entire congress

5 min	Introduction – R. Peffault de Latour, France
5 min	Registry Report – A. Risitano, Italy
25 min	RACE: Clinical update – A. Risitano, Italy
25 min	RACE: What we learn on the biology of SAA? – A. Kulakaseraraj, United Kingdom
15 min	Androgens in AA: Myth or reality in 2021? – S. Pagliuca, United States
15 min	GVHD and relapse free survival (GRFS) after HSCT for AA – R. Devillier, France

*** COMING SOON ***

In 2021 the SAAWP will organise two Business Meetings, one in spring, one in autumn. An Educational Meeting will be organised around the 2nd BM on the topic 'What's new in the field of BMF?'. SAAWP members will be informed of the event dates via email.

Would you want 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