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 characterize diseases, data on the natural history of diseases, and late effects that occur after treatment.

CURRENT REGISTRY NUMBERS

In total, 18892 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	13851
Pure red cell aplasia (non congenital)	153
Paroxysmal nocturnal haemoglobinuria (PNH)	759
Pure white cell aplasia	14
Amegakaryocytic thrombocytopaenia (non congenital)	60
Other acquired cytopenic syndrome	296
Unknown	128
TOTAL	**15261**

Genetic BMF	n
Fanconi	2428
Diamond-Blackfan (congenital PRCA)	444
Shwachman-Diamond	93
Dyserythropoietic anaemia	55
Dyskeratosis congenita	170
Amegakaryocytic thrombocytopaenia (congenital)	146
Congenital sideroblastic anaemia	31
Other	212
Unknown	52
TOTAL	**3631**

ELTROMBOPAG CLINICAL TRIALS

An update about the clinical trials with Eltrombopag as part of the initial treatment of AA in Europe (RACE and EMAA).

The RACE trial has been concluded and published in the New England Journal of Medicine. CRF collection is still ongoing for the latest follow up.

RACE-2: the long-term follow-up of RACE is on its way. RACE-2 is not a clinical trial, but a non-interventional observational cohort study performed by the Working Party using the EBMT Registry database. More information about RACE-2 will follow soon.

The EMAA trial (performed outside EBMT) is still ongoing.

ACTIVE STUDIES

RACE-2: long-term follow-up of patients participating in RACE

PI: Dr. Régis Peffault de Latour, Dr. Antonio M. Risitano

To evaluate the long-term outcome and survival of patients who have received standard IST with or without Eltrombopag within the RACE trial. Patients are eligible if they participated in the RACE trial during which patient received ATGAM, Cyclosporine A with or without Eltrombopag.

Clonal evolution in acquired aplastic anemia

PI: Dr. Pedro de Lima Prata

Describe the natural history of patients with SAA and/or PNH that evolve into a myeloid disease. Patients are eligible if they have been diagnosed with AML/MDS or any karyotypic abnormality secondary to a SAA or PNH diagnosis from 2000 onwards.

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.

RECENT PUBLICATIONS

Iftikhar R, Ahmed P, *et al*. 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). Bone Marrow Transplant. 2022 Feb;57(2):331.

Alotaibi H, *et al*. 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. Transplant Cell Ther. 2022 Feb;28(2):105.e1-105.e7.

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

Petit A, *et al*. Upfront unrelated donor Hematopoietic stem cell transplantation in patients with idiopathic aplastic anemia: a retrospective study of the Severe Aplastic Anemia Working Party of European Bone Marrow Transplantation. Am J Hematol. 2022 Jan 1;97(1):E1-E3.

Tichelli A, Peffault de Latour R, Dufour C, Rovó A, *et al*. Adding Eltrombopag to immunosuppression: the importance of predicting outcome. Haematologica. 2022 Jan 1;107(1):46-48.

Risitano AM, Peffault de Latour R. How we('ll) treat paroxysmal nocturnal haemoglobinuria: diving into the future. Br J Haematol. 2022 Jan;196(2):288-303.

Debureaux PE, Kulasekararaj AG, Cacace F, *et al*. Categorizing hematological response to eculizumab in paroxysmal nocturnal hemoglobinuria: a multicenter real-life study. Bone Marrow Transplant. 2021 Oct;56(10):2600-2602.

Iftikhar R, Ahmed P, *et al*. Special issues related to diagnosis and management of acquired Aplastic anemia in countries with restricted resources 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) . Bone Marrow Transplant. 2021 Oct;56(10):2518-2532.

EBMT2022 WORKING PARTY SESSION

Wednesday, March 23: In-person hybrid session (in Prague and live-streamed, with live discussion)

09:00–09:20 Somatic mutations in acquired aplastic anemia: what do we learn from the RACE trial? – *A. Kulakaseraraj, United Kingdom*

09:20–09:40 Current standard of IST in SAA in Europe in the EPAG era? – *A. Risitano, Italy*

09:40–10:00 Transplantation modalities in acquired aplastic anemia: any change? – *R. Peffault de Latour, France*

10:00–10:15 Q&A

*** COMING SOON ***

In 2022, the SAAWP will organize a Scientific Meeting on Thursday April 14th. SAAWP members will be informed of the event 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