

Breadcrumb

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Major achievements

The SAAWP is extending its international leadership in the field of bone marrow failures continuing top-level investigation in transplant and non-transplant treatment for Aplastic Anaemia and other related conditions.
This was achieved through research through the different main research programs.

1. **RACE trial, with its ancillary studies**

The prospective, randomized phase 3 RACE trial set immunosuppressive therapy (ATG+CsA) plus Eltrombopag (also referred as “triple therapy”, or “ACE therapy”) as the new standard of care for adult SAA patients not eligible for a low-risk allogeneic hematolopoietic stem cell transplantation. This was a seminal study conducted by the EBMT Clinical Trial Office, showing that this experimental treatment resulted in faster and better hematological response as compared with previous standard of care (is, same treatment without eltrombopag). The SAAWP is still generating results and data from this study, which remains a milestone for the SAAWP. In 2025 the efforts focused on:

- Final manuscript reporting the long-term follow up of the study, looking at 2-year follow up data (manuscript under revision)
- Final manuscript reporting biological data on somatic mutations detected by NGS (manuscript accepted for publication in the *New England Journal of Medicine Evidence*)
- Manuscript reporting biological data on immune abnormalities detected by CyTOFF (abstract selected as oral publication at ASH, manuscript in preparation)
- Analysis of Patient Reported Outcomes and comparison with novel PRO instruments (to be presented at EBMT 2026)
- Investigation of infectious risk in the natural history of AA (in collaboration with the IDWP)

<u>The RACE trial also generated some new studies:</u>

- Continuation of the RACE trial within its extension, retrospective “RACE-2” study, which aims to investigate the long-term outcome of patients initially enrolled in the RACE trial
- Retrospective study: comparison of available data from RACE (non-transplant treatment) vs registry data (transplant treatment), in a matched-paired analysis

2. **Retrospective studies from the EBMT Registry**

The SAAWP is conducting a number of studies aiming to address key questions in the field of transplantation for acquired and inherited bone marrow failures. In 2025

the efforts focused on the following key projects:

- Outcome of patients transplanted for AA, looking at the impact of age (by donor type) and other possible factors (manuscript in preparation)
- Outcome of patients transplanted after clonal evolution (ie, transformation to either MDS or AML) of an initial SAA
- Outcome of patients transplanted for PNH, looking at the impact of possible factors
- Outcome of patients transplanted for DKC, looking at the impact of possible factors (selected as poster presentation at ASH)
- Impact of TBI (in the context of conditioning regimen for allo-HCT) on fertility of female patients transplanted for SAA

Click here to see the full list of SAAWP studies.

3. **Future projects.**

The SAAWP is planning to specifically investigate more in detail the differential outcomes of transplant vs non-transplant as (initial) treatment of AA.

- A Prospective Observational Study describing outcomes of Immunosuppressive treatment and Transplantation as first-line treatment for adult severe aplastic anaemia

Principal research studies

Comparison of outcomes of immune-suppression therapy and stem cell transplant using RACE trial data and a registry transplant cohort
Study type
Retrospective Studies
Diseases
Bone marrow failure
Group
Severe Aplastic Anaemia Working Party (SAAWP)
Type of treatment
Allogeneic
Immunosuppressive Treatment (IST)
Principal investigator
Simona Iacobelli, Antonio Risitano

Outcomes of Hematopoietic Cell Transplantation in Dyskeratosis Congenita: A Retrospective Study
Study type
Retrospective Studies
Diseases
Bone marrow failure
Group
Severe Aplastic Anaemia Working Party (SAAWP)
Type of treatment
Allogeneic
Principal investigator
Fabiana Cacace
Fertility in SAA patients after allo-HCT prepared with Total Body Irradiation 2Gy
Study type
Retrospective Studies
Diseases
Bone marrow failure
Group
Severe Aplastic Anaemia Working Party (SAAWP)
Type of treatment
Allogeneic
Principal investigator
Andrea Bagicalupo
Outcome of transplant in PNH patients treated after 2011
Study type
Retrospective Studies
Diseases
Bone marrow failure
Group
Severe Aplastic Anaemia Working Party (SAAWP)
Type of treatment
Allogeneic
Principal investigator
Camilla Frieri
Transplant outcomes in patients over 40 years old with severe aplastic anemia (SAA) treated after 2010
Study type
Retrospective Studies

Diseases
Bone marrow failure
Group
Severe Aplastic Anaemia Working Party (SAAWP)
Type of treatment
Allogeneic
Principal investigator
Antonio Risitano

Key publications

2025
Outpatient subcutaneous alemtuzumab is feasible and safe for aplastic anemia and is associated with high response rates
Group
Severe Aplastic Anaemia Working Party (SAAWP)
1st listed author
Ana Rita da Fonseca
Journal
Blood Adv.
2025
Donor impact on allogeneic transplant outcomes with PTCy for severe aplastic anemia: a study of the SAAWP EBMT
Group
Severe Aplastic Anaemia Working Party (SAAWP)
1st listed author
Juan Montoro
Journal
Bone Marrow Transplant.
2025
Hematopoietic stem cell transplantation is effective in achieving long-term survival for post-aplastic anemia myeloid neoplasms: the EBMT Severe Aplastic Anemia Report
Group
Severe Aplastic Anaemia Working Party (SAAWP)
1st listed author
Pedro Henrique Prata
Journal

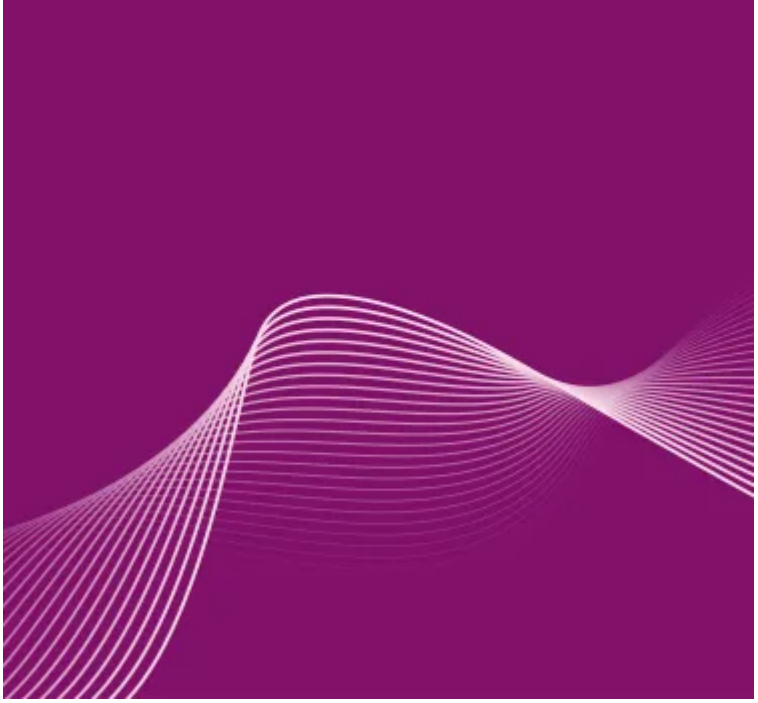
Haematologica.
2025
Advancing the Integration of 'Basic/Fundamental' and Translational Cellular and Gene Therapy Science within the EBMT: Accelerating the Pathway to Progress
Group
Acute Leukemia Working Party (ALWP)
Severe Aplastic Anaemia Working Party (SAAWP)
Autoimmune Diseases Working Party (ADWP)
Chronic Malignancies Working Party (CMWP)
Cellular Therapy & Immunobiology Working Party (CTIWP)
Inborn Errors Working Party (IEWP)
Infectious Diseases Working Party (IDWP)
Complications Working Party (CWP)
Lymphoma Working Party (LWP)
Paediatric Diseases Working Party (PDWP)
Haemoglobinopathies Working Party (HWP)
1st listed author
Donal P McLornan
Journal
Bone Marrow Transplant.
2025
Insights in bone marrow failure syndromes: take home messages from the 3rd ESH-EBMT-EHA-IPIG translational research conference
Group
Severe Aplastic Anaemia Working Party (SAAWP)
Other society
1st listed author
Fabiana Cacace
Journal
Bone Marrow Transplant.
See the full list of the SAAWP 2025 publications



Indicators

	2023	2024	2025
Impact factor	102.09	159.30	25.70
Oral presentations	11	19	14
Poster presentations	0	3	4
Educational events	2	1	1

Major educational events



Event

Severe Aplastic Anaemia Working Party Educational Course

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Event

51st Annual Meeting of the EBMT

Mar 30, 2025 - Apr 02, 2025 / Florence, Italy
Discover more

- SAAWP Business Meeting - 1 April 2025 - Florence, Italy
- SAAWP Business Meeting - 9 September 2025 - Basel, Switzerland

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