

## Breadcrumb

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## Entity Print



Antonio Risitano  
SAAWP Chair  
Italy

## 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 confirmed by four oral presentations at ASH 2024 (all the 4 studies submitted were selected for oral sessions. See [HERE](#)).

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 hematopoietic 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 haematological 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 2024 the efforts focused on:

- Final manuscript reporting the long-term follow up of the study, looking at 2-year follow up data
- Analysis of biological data from patients samples collected during the study (at baseline, 6 and 24 months from treatment)
- Continuation of the RACE trial within its extension, retrospective “RACE-2” study (see below)
- Investigation of infectious risk in the natural history of AA (in collaboration with the IDWP)

### **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 2024 the efforts focused on the following key projects:

- 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
- Outcome of patients transplanted for AA, looking at the impact of age (by donor type) and other possible factors
- 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 SAA patients transplanted with a PTCY GVHD prophylaxis

For the full list of studies see the section below.

### 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.

- Retrospective study: comparison of available data from RACE (non-transplant treatment) vs registry data (transplant treatment), in a matched-paired analysis
- Prospective study: A Prospective Observational Study describing outcomes of Immunosuppressive treatment and Transplantation as first-line treatment for adult severe aplastic anaemia
- 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.

## **Principal research studies**

[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

Retrospective analysis of different rabbit ATG brands on outcome and occurrence of acute and chronic GVHD in patients with aplastische anemia undergoing allogeneic transplantation

Study type

Retrospective Studies

Diseases

Bone marrow failure

Group

Severe Aplastic Anaemia Working Party (SAAWP)

Type of treatment

Allogeneic

Allogeneic - Related

Allogeneic - Unrelated

Principal investigator

Fabian Beier

Post-transplant cyclophosphamide as GvHD prophylaxis in patients with aplastic anemia with different donors: a comparative study of the SAAWP EBMT

Study type

Retrospective Studies

Diseases

Bone marrow failure

Group

Severe Aplastic Anaemia Working Party (SAAWP)

Type of treatment

Allogeneic

Principal investigator

Juan Montoro

RACE-2: long-term follow-up of patients participating in RACE: the prospective Randomized multicenter study comparing horse Antithymocyte globuline (hATG) + Cyclosporine A (CsA) with or without Eltrombopag as front-line therapy for Severe Aplastic Anemia patients

Study type

Non-interventional Prospective Studies

Diseases

[Bone marrow failure](#)

[Group](#)

[Severe Aplastic Anaemia Working Party \(SAAWP\)](#)

[Type of treatment](#)

[Immunosuppressive Treatment \(IST\)](#)

[Principal investigator](#)

[Régis Peffault de Latour, Antonio M. Risitano](#)

[Clonal evolution in acquired aplastic anemia](#)

[Study type](#)

[Retrospective Studies](#)

[Diseases](#)

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[Severe Aplastic Anaemia Working Party \(SAAWP\)](#)

[Type of treatment](#)

[Allogeneic](#)

[Principal investigator](#)

[Pedro de Lima Prata](#)

## **Key publications**

[2024](#)

[Outcomes of hematopoietic stem cell transplantation in 813 pediatric patients with Fanconi anemia](#)

[Group](#)

[Severe Aplastic Anaemia Working Party \(SAAWP\)](#)

[Paediatric Diseases Working Party \(PDWP\)](#)

[1st listed author](#)

[Su Han Lum](#)

[Journal](#)

[Blood.](#)

[2024](#)

[Alternative donor transplantation for severe aplastic anemia: a comparative study of the SAAWP EBMT](#)

[Group](#)

[Severe Aplastic Anaemia Working Party \(SAAWP\)](#)

[1st listed author](#)

[Juan Montoro](#)

[Journal](#)

[Blood.](#)

[2024](#)

[HLA-haploidentical stem cell transplantation in children with inherited bone marrow failure syndromes: A retrospective analysis on behalf of EBMT severe aplastic Anemia and pediatric diseases working parties](#)

[Group](#)

[Severe Aplastic Anaemia Working Party \(SAAWP\)](#)

[Paediatric Diseases Working Party \(PDWP\)](#)

[1st listed author](#)

[Stefano Giardino](#)

[Journal](#)

[Am J Hematol.](#)

[2024](#)

[Umbilical Cord Blood Transplantation for Fanconi Anemia with a special focus on late complications: a Study on Behalf of Eurocord and SAAWP-EBMT](#)

[Group](#)

[Severe Aplastic Anaemia Working Party \(SAAWP\)](#)

[1st listed author](#)

[Hanadi Rafii](#)

[Journal](#)

[Transplant Cell Ther.](#)

[2024](#)

[Oral Iptacopan Monotherapy in Paroxysmal Nocturnal Hemoglobinuria](#)

[Group](#)

[Severe Aplastic Anaemia Working Party \(SAAWP\)](#)

[1st listed author](#)

[Régis Peffault de Latour](#)

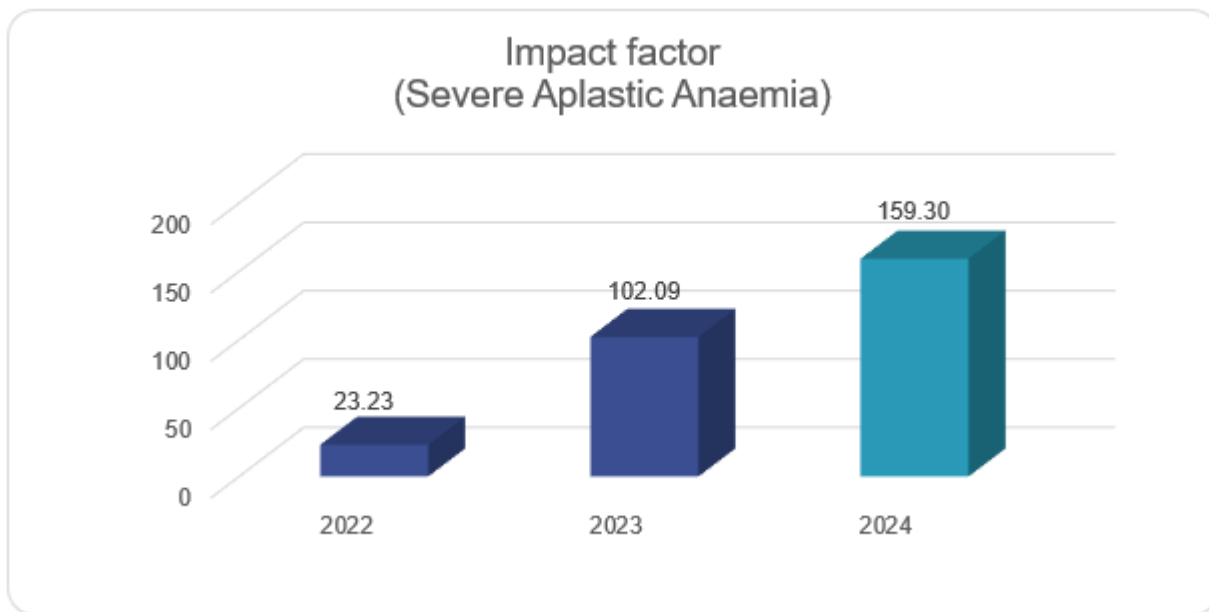
[Journal](#)

[N Engl J Med.](#)

[See the full list of the SAAWP 2024 publications](#)



## Indicators



### 2022 2023 2024

<b>Oral presentations</b>	22	11	<b>19</b>
<b>Poster presentations</b>	2	0	<b>3</b>
<b>Educational events</b>	4	2	<b>2</b>

## Major educational events



[Event](#)

**50th Annual Meeting of the EBMT**



[Kingdom](#)

[Event](#)



## **Cellular Therapies In Non-Malignant Haematology - Haemoglobinopathies and Severe Aplastic Anaemia Working Parties Joint Educational Meeting**

Sep 11, 2024 - Sep 13, 2024 / Genova, Italy

Discover more

- ESH-EBMT-EHA-IPIG 3rd Translational Research Conference: Bone Marrow Failure and Leukaemia Predisposition Syndromes - 15-17 November 2024 - Paris, France
- SAAWP Business Meeting - 16 April 2024 - Glasgow, UK
- SAAWP Business Meeting - 11 September 2024 - Genova, Italy

VISIT THE SAAWP WEBPAGE