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Antonio Risitano

SAAWP Chair (ELECTED APRIL 2023, PREVIOUS CHAIR: Régis Peffault de la Tour)
Italy

Major achievements

Continuation of RACE trial scientific activities and investigation on key questions related to transplant for Severe Aplastic Anaemia

The prospective, randomised 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 2023 the efforts focused on:

- Generation of the final study report (all patients evaluated at the last study visit, at 24 months from treatment starting)
- 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, which aims to investigate the long-term outcome of patients initially enrolled in the RACE trial.

In addition to RACE-related activities, 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 2023 the efforts focused on:

- Outcome of patients transplanted for Fanconi Anaemia
- Outcome of patients transplanted after clonal evolution (ie, transformation to either MDS or AML) of an initial SAA
- Outcome of SAA patients transplanted from haplo-identical donors (vs unrelated and related matched donors)

Principal research studies

[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 Stem Cell Treatment](#)

[Allogeneic](#)

Principal investigator

Juan Montoro

Renal failure in aplastic anemia

Study type

-

Diseases

Bone marrow failure

Group

Severe Aplastic Anaemia Working Party (SAAWP)

Type of Stem Cell Treatment

-

Principal investigator

Beatrice Drexler

Outcomes of treatment with anti thymocyte globulin (ATG) for acquired pure red cell aplasia

Study type

Retrospective Studies

Diseases

Bone marrow failure

Group

Severe Aplastic Anaemia Working Party (SAAWP)

Type of Stem Cell Treatment

-

Principal investigator

Stijn Halkes

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 Stem Cell Treatment

-

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 Stem Cell Treatment](#)

-

[Principal investigator](#)

[Pedro de Lima Prata](#)

[Transplant Outcomes in Children with Fanconi anaemia](#)

[Study type](#)

[Retrospective Studies](#)

[Diseases](#)

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

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

[Type of Stem Cell Treatment](#)

[Allogeneic](#)

[Principal investigator](#)

[Su Han Lum, Sujith Samarasinghe](#)

Key publications

[2023](#)

[The complement alternative pathway in paroxysmal nocturnal hemoglobinuria: From a pathogenic mechanism to a therapeutic target](#)

[Group](#)

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

[1st listed author](#)

[Antonio M Risitano](#)

[Journal](#)

[Immunol Rev.](#)

[2023](#)

[Graft-versus-host disease and relapse/rejection-free survival after allogeneic transplantation for idiopathic severe aplastic anemia: a comprehensive analysis from the SAAWP of the EBMT](#)

[Group](#)

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

[1st listed author](#)

[Raynier Devillier](#)

[Journal](#)

[Haematologica.](#)

[2023](#)

[Current use of androgens in bone marrow failure disorders: a report from the Severe Aplastic Anemia Working Party of the European Society of Blood and Marrow Transplantation](#)

[Group](#)

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

[1st listed author](#)

[Simona Pagliuca](#)

[Journal](#)

[Haematologica.](#)

[2023](#)

[SARS-CoV-2 vaccination in 361 non-transplanted patients with aplastic anemia and/or paroxysmal nocturnal hemoglobinuria](#)

[Group](#)

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

[Infectious Diseases Working Party \(IDWP\)](#)

[1st listed author](#)

[Morag Griffin](#)

[Journal](#)

[Haematologica.](#)

[2023](#)

[Risk factors for thromboembolic events in patients with paroxysmal nocturnal hemoglobinuria \(PNH\): a nested case-control study in the International PNH Registry](#)

[Group](#)

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

[1st listed author](#)

[Britta Höchsmann](#)

[Journal](#)

[Ann Hematol.](#)

2023

Discovering C3 targeting therapies for paroxysmal nocturnal hemoglobinuria:
Achievements and pitfalls

Group

Severe Aplastic Anaemia Working Party (SAAWP)

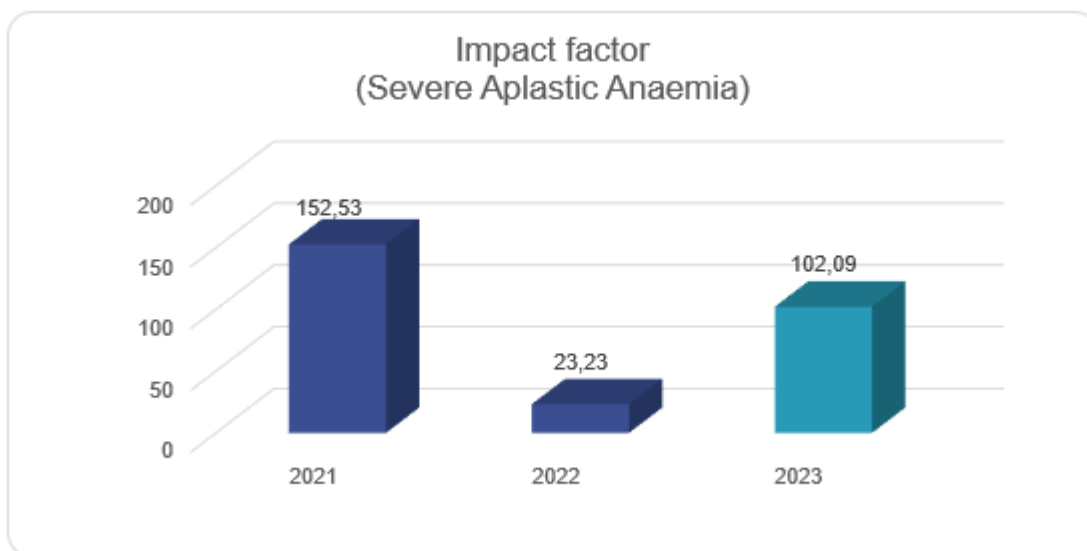
1st listed author

Antonio M Risitano

Journal

Semin Immunol.

See the full list of the SAAWP 2023 publications



2021 2022 2023

Oral presentations 8 22 11

Poster presentations 0 2 0

2021 2022 2023

Educational events 3 4 2



[Event](#)

49th Annual Meeting of the EBMT

[Apr 23, 2023 - Apr 26, 2023 / Paris, France](#)

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- SAAWP Business Meeting (Virtual) - November 14, 2023.

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