

Breadcrumb

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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

Renal failure in aplastic anemia
Study type

Diseases
Bone marrow failure
Group
Severe Aplastic Anaemia Working Party (SAAWP)
Type of 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 treatment
Allogeneic
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 treatment
Immunosuppressive Treatment (IST)
Principal investigator
Régis Peffault de Latour, Antonio M. Risitano

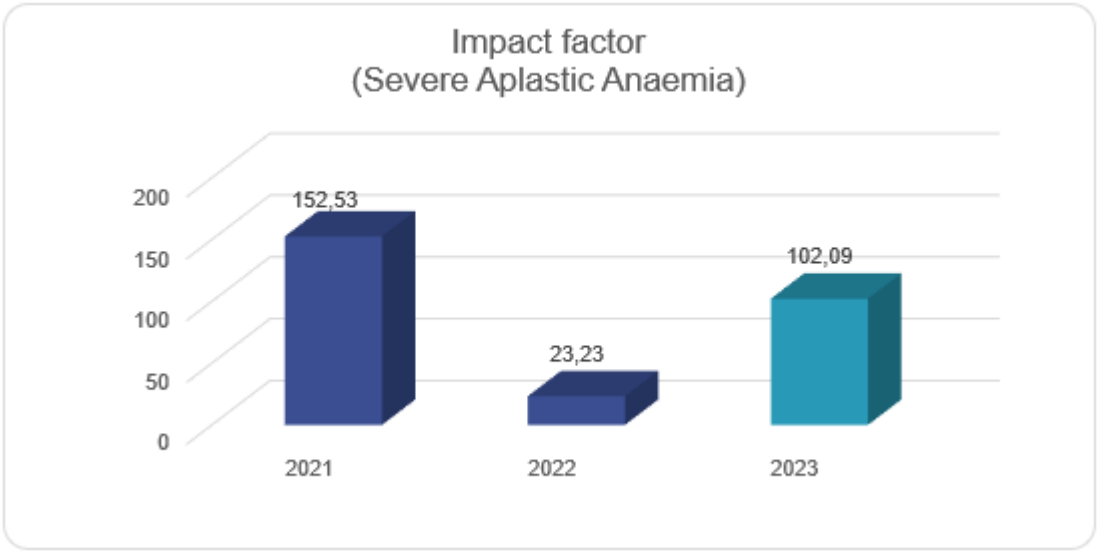
Key publications

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

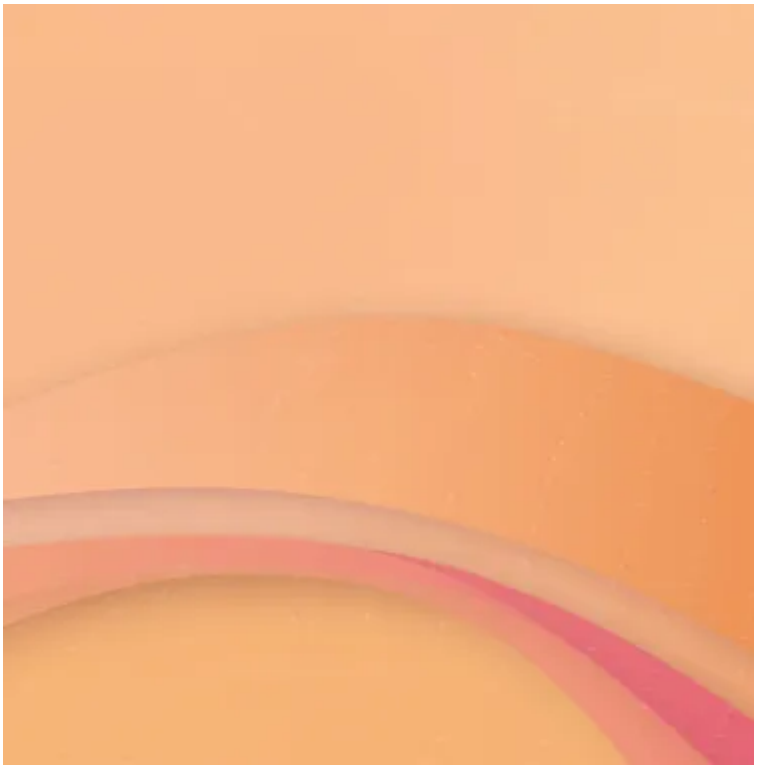
Journal
Ann Hematol.
2023
Discovering C3 targeting therapies for paroxysmal nocturnal hemoglobinuria: Achievements and pitfalls
Group
Severe Aplastic Anaemia Working Party (SAAWP)
1st listed author
Antonio M Risitano
Journal
Semin Immunol.
See the full list of the SAAWP 2023 publications





2021 2022 2023

Oral presentations	8	22	**11**
Poster presentations	0	2	**0**
Educational events	3	4	**2**



Event

49th Annual Meeting of the EBMT

Apr 23, 2023 - Apr 26, 2023 / Paris, France
Discover more

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

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