

Breadcrumb

Entity Print



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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 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
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

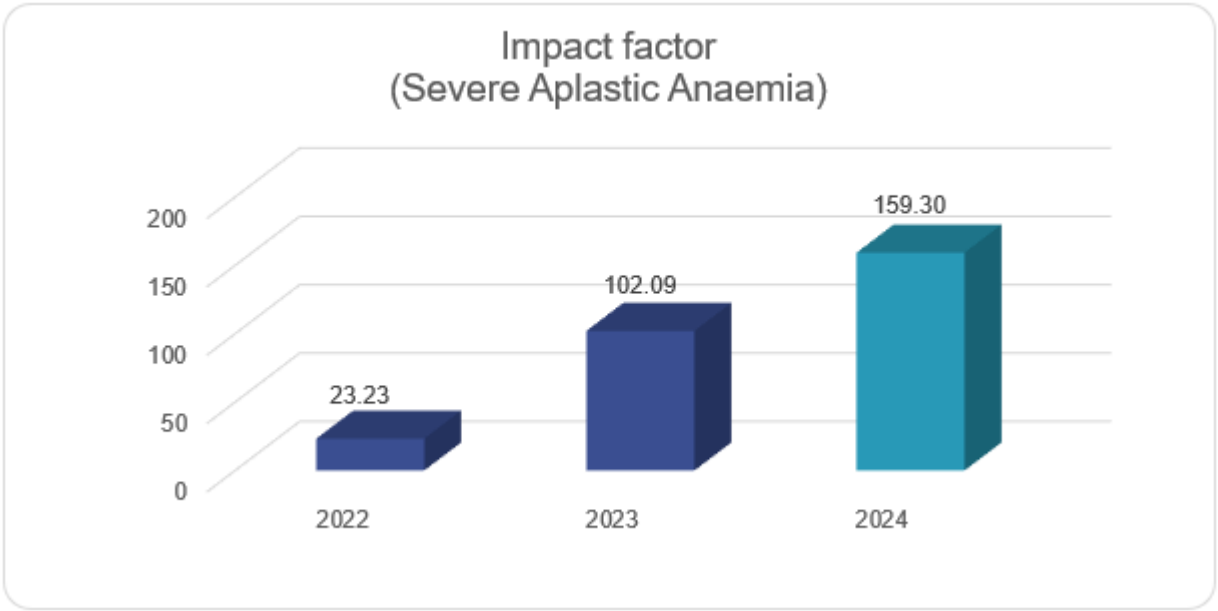
2024

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.

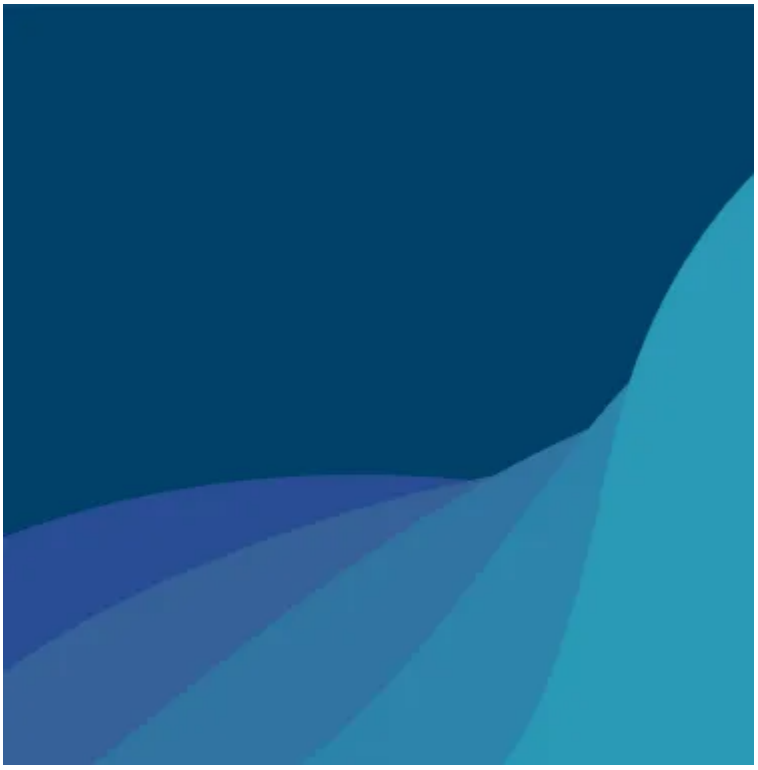
2024
Oral Iptacopan Monotherapy in Paroxysmal Nocturnal Hemoglobinuria
Group
Severe Aplastic Anaemia Working Party (SAAWP)
1st listed author
Régis Peffault de Latour
Journal
N Engl J Med.
See the full list of the SAAWP 2024 publications



Indicators



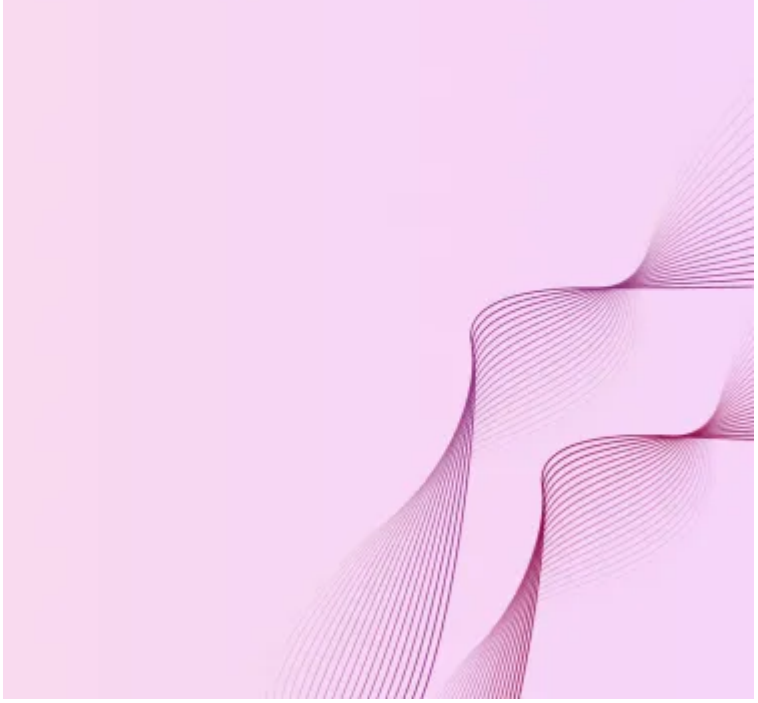
	2022	2023	2024
Oral presentations	22	11	**19**
Poster presentations	2	0	**3**
Educational events	4	2	**2**



Event

50th Annual Meeting of the EBMT

Apr 14, 2024 - Jun 17, 2024 / Glasgow, United Kingdom
Discover more



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

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