Major achievements

Top level publication and initiation of the RACE-2 trial:

1. RACE trial comparing standard immunosuppressive therapy (ATG+CsA) plus Eltrombopag vs Standard IST alone in patients with severe aplastic anemia
(SAA) was published in January 2022 (Full text link). This EBMT study, supported financially by Novartis, Pfizer and Alexion, demonstrated that Eltrombopag at the top of standard hATG+CSA was associated with a better and quicker response at 3 and 6 months. At 2 years, event free survival was significantly higher for patients receiving first line the experimental treatment. Horse ATG+CSA+Eltrombopag is now the standard of care for adult patients with SAA. We acknowledge the Clinical Trial Office (CTO) of the EBMT, together with all RACE investigators and nurses for their outstanding work, as well as patients who have accepted to participate in this trial.

2. The RACE-2 trial has been ongoing since 2021. This retrospective study will assess the long-term follow-up of patients included in RACE which is of outstanding importance to evaluate the long-term safety of the use of Eltrombopag first line in patients with SAA. The trial began early 2022.

Principal research studies

Haploidentical stem cell transplantation for congenital bone marrow failure

Study type
Retrospective Studies
Diseases
Bone marrow failure
Group
Severe Aplastic Anaemia Working Party (SAAWP)
Type of Stem Cell Treatment
Allogeneic - Haploidentical
Principal investigator
Dr. Stefano Giardino

Clonal evolution in acquired aplastic anemia

Study type
Retrospective Studies
Diseases
Bone marrow failure
Group
Severe Aplastic Anaemia Working Party (SAAWP)
Type of Stem Cell Treatment

Principal investigator
Pedro de Lima Prata

Key publications

2021
Eltrombopag Added to Immunosuppression in Severe Aplastic Anemia
Group
Severe Aplastic Anaemia Working Party (SAAWP)
1st listed author
Régis Peffault de Latour
Journal

2022
STEM CELL TRANSPLANTATION IN PATIENTS AFFECTED BY SHWACHMAN- DIAMOND SYNDROME: EXPERT CONSENSUS AND RECOMMENDATIONS FROM THE EBMT SEVERE APLASTIC ANAEMIA WORKING PARTY
Group
Severe Aplastic Anaemia Working Party (SAAWP)
1st listed author
Simone Cesaro
Journal
Transplant Cell Ther.

2022
How we('ll) treat paroxysmal nocturnal haemoglobinuria: diving into the future
Group
Severe Aplastic Anaemia Working Party (SAAWP)
1st listed author
Antonio Maria Risitano
Journal
Br J Haematol.

2022
Hemolytic paroxysmal nocturnal hemoglobinuria: 20 years of medical progress
Group
Severe Aplastic Anaemia Working Party (SAAWP)
1st listed author
Regis Peffault de Latour
Journal
Semin Hematol.
2022
Upfront Alternative Donor Transplant versus Immunosuppressive Therapy in Patients with Severe Aplastic Anemia Who Lack a Fully HLA-Matched Related Donor: Systematic Review and Meta-Analysis of Retrospective Studies, on Behalf of the Severe Aplastic Anemia Working Party of the European Group for Blood and Marrow Transplantation

Group
Severe Aplastic Anaemia Working Party (SAAWP)

1st listed author
Hind Alotaibi

Journal
Transplant Cell Ther.

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Major educational events

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2019 2020 2021 2022

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Virtual 48th Annual Meeting of the EBMT
Severe Aplastic Anaemia Working Party Educational Meeting 2022

Jan 28, 2022 - Jan 28, 2022 / Virtual Meeting

Discover more

- SAAWP Business Meeting (Virtual) - May 27, 2022
- SAAWP Business Meeting (Face to face) - Nov. 17, 2022
- 2nd ESH-EBMT Translational Research Conference: Bone Marrow Failure Syndromes: From the cell to the cure of the disease - Nov. 18-20, 2022

VISIT THE SAAWP WEBPAGE