EBMT Annual report 2022



ScienceEducationPatient Care & Advocacy

Our SocietyOur Mission, Vision & ValuesEquality, Diversity & InclusionEBMT

Membership 2022Organisational Structure 2022Financial Highlights 2022EBMT

Partners

Breadcrumb

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Entity Print



Régis Peffaut de Latour SAAWP Chair France

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 (<u>Full text link</u>). 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 treatment

Allogeneic - Haploidentical

Principal investigator

Dr. Stefano Giardino

Clonal evolution in acquired aplastic anemia

Study type

Retrospective Studies

<u>Diseases</u>

Bone marrow failure

Group

Severe Aplastic Anaemia Working Party (SAAWP)

Type of treatment

<u>Allogeneic</u>

Principal investigator

Pedro de Lima Prata

Androgens in bone marrow failure disorders

Study type

Retrospective Studies

Diseases

Bone marrow failure

Group

Severe Aplastic Anaemia Working Party (SAAWP)

Type of treatment

- None -

Principal investigator

Simona Pagliuca, Austin Kulasekararaj

GvHD and relapse free survival after allogeneic transplantation for idiopathic severe

aplastic anemia

Study type

Retrospective Studies

Diseases

Bone marrow failure

Group

Severe Aplastic Anaemia Working Party (SAAWP)

Type of treatment

<u>Allogeneic</u>

Principal investigator

Raynier Devillier

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

<u>Journal</u>

N Engl J Med.

2022

STEM CELL TRANSPLANTATION IN PATIENTS AFFECTED BY SHWACHMAN- DIAMOND

SYNDROME: EXPERT CONSENSUS AND RECOMMENDATIONS FROM THE EBMT

SEVERE APLASTIC ANAEMIA WORKING PART	SEVERE	APLASTIC	ANAEMIA	WORKING	PART'
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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

<u>Upfront Alternative Donor Transplant versus Immunosuppressive Therapy in Patients</u> <u>with Severe Aplastic Anemia Who Lack a Fully HLA-Matched Related Donor:</u>

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

<u>Transplantation</u>

<u>Group</u>

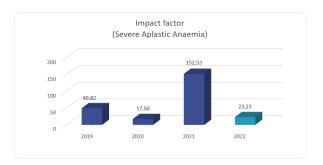
Severe Aplastic Anaemia Working Party (SAAWP)

1st listed author

Hind Alotaibi

<u>Journal</u>

Transplant Cell Ther.



2019 2020 2021 2022

Oral presentations 8 4 8 22
Poster presentations 1 6 0 2
Educational events 2 4 3 4



Event

Virtual 48th Annual Meeting of the EBMT

<u>Mar 19, 2022 - Mar 23, 2022 / Online</u> <u>Discover more</u>



Event

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