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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) has been accepted for publication in the NEJM on October 14th 2021. 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 to this trial.

2. The RACE-2 trial is ready in 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 will begin early 2022.

Principal research studies

[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

N Engl J Med.

2021

Upfront unrelated donor Hematopoietic stem cell transplantation in patients with idiopathic aplastic anemia: a retrospective study of the Severe Aplastic Anemia Working Party of European Bone Marrow Transplantation

Group

Severe Aplastic Anaemia Working Party (SAAWP)

1st listed author

Audrey Françoise Petit

Journal

Am J Hematol.

2021

Haplo-identical or mismatched unrelated donor hematopoietic cell transplantation for Fanconi anemia: results from the Severe Aplastic Anemia Working Party of the EBMT

Group

Severe Aplastic Anaemia Working Party (SAAWP)

1st listed author

Josune Zubicaray

Journal

Am J Hematol.

2021

Stem Cell Transplantation for Diamond-Blackfan Anemia. A Retrospective Study on Behalf of the Severe Aplastic Anemia Working Party of the European Blood and Marrow Transplantation Group (EBMT)

Group

Severe Aplastic Anaemia Working Party (SAAWP)

1st listed author

Maurizio Miano

Journal

Transplant Cell Ther.

2021

Categorizing hematological response to eculizumab in paroxysmal nocturnal hemoglobinuria: a multicenter real-life study

Group

Severe Aplastic Anaemia Working Party (SAAWP)

1st listed author

[Pierre-Edouard Debureaux](#)

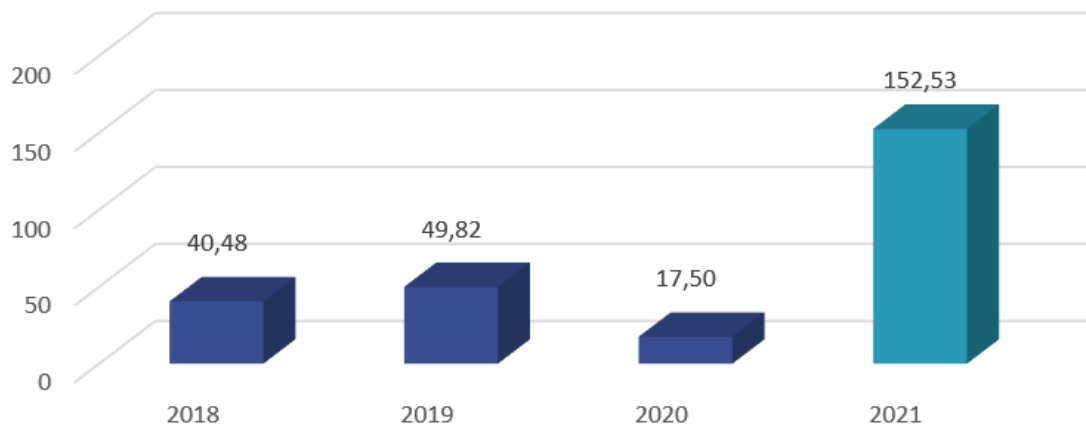
[Journal](#)

[Bone Marrow Transplant.](#)

[See the full list of the SAAWP 2021 publications](#)



Impact factor
(Severe Aplastic Anaemia)



2019 2020 2021

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