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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) has been accepted for publication in the NEJM on October 14<sup>th</sup> 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

## 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 \(SAAWP\)](#)

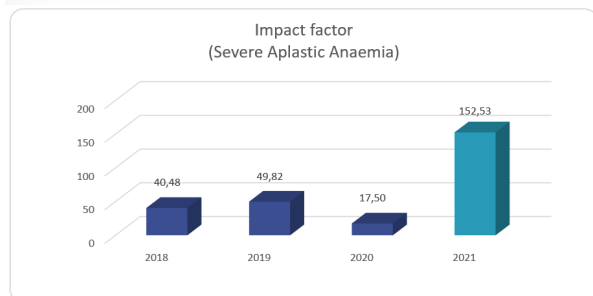
[1st listed author](#)

[Pierre-Edouard Debureaux](#)

[Journal](#)

[Bone Marrow Transplant.](#)

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



**2019 2020 2021**

<b>Oral presentations</b>	<b>8</b>	<b>4</b>	<b>8</b>
<b>Poster presentations</b>	<b>1</b>	<b>6</b>	<b>0</b>
<b>Educational events</b>	<b>2</b>	<b>4</b>	<b>3</b>