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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.

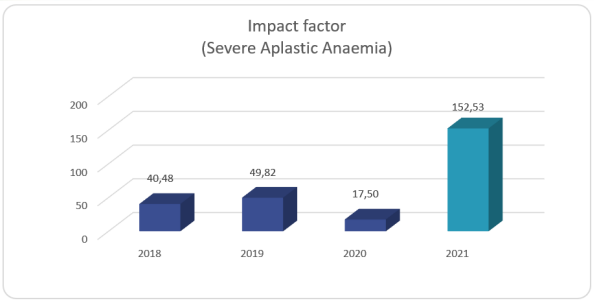
Principal research studies

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