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1. Home

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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 (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.

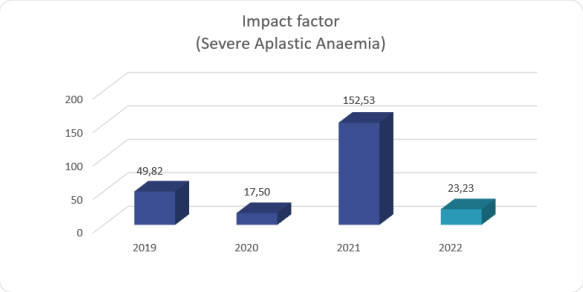
2. The RACE-2 trial has been ongoing since 2021. This retrospective study will assess the long-term follow-up of patients included in RACE which is of outstanding importance to evaluate the long-term safety of the use of Eltrombopag first line in patients with SAA. The trial began early 2022.

Principal research studies

Key publications

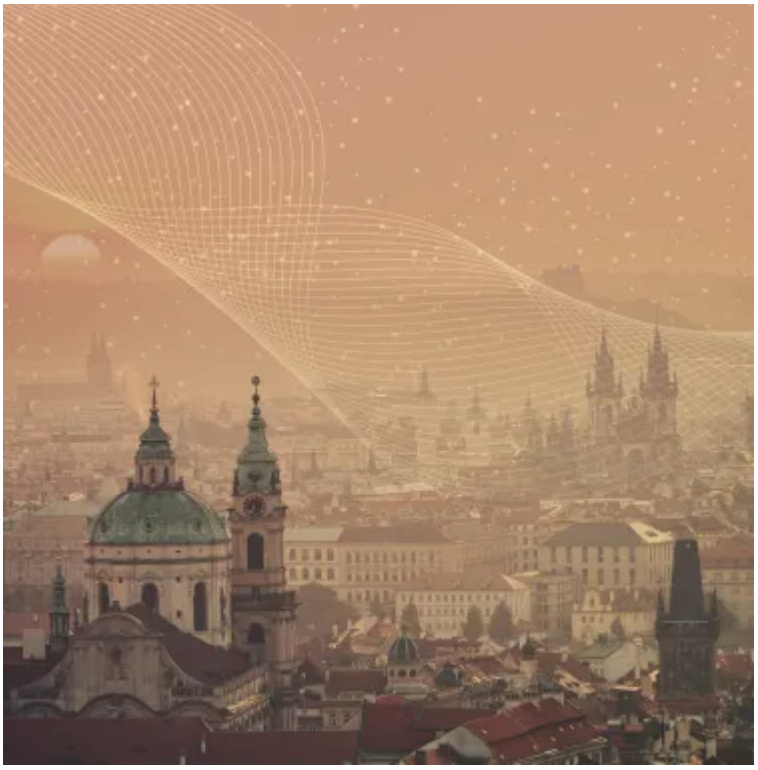
2021
Eltrombopag Added to Immunosuppression in Severe Aplastic Anemia
Group
Severe Aplastic Anaemia Working Party (SAAWP)
1st listed author
Régis Peffault de Latour
Journal
N Engl J Med.
2022
STEM CELL TRANSPLANTATION IN PATIENTS AFFECTED BY SHWACHMAN- DIAMOND SYNDROME: EXPERT CONSENSUS AND RECOMMENDATIONS 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.



	2019	2020	2021	2022
Oral presentations	8	4	8	**22**
Poster presentations	1	6	0	**2**

	2019	2020	2021	2022
Educational events	2	4	3	**4**



Event

Virtual 48th Annual Meeting of the EBMT

Mar 19, 2022 - Mar 23, 2022 / Online
Discover more



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

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