EBMT
European Society
for Blood and Marrow Transplantation

RACE trial

EBMT Clinical Trials Office

RACE trial

A prospective Randomized multicenter study comparing horse Antithymocyte globuline (hATG) + Cyclosporine A (CsA) with or without Eltrombopag as front-line therapy for severe aplastic anaemia patients

A study by the Severe Aplastic Anaemia Working Party (SAAWP)
Principal Investigators:
Antonio Risitano, Carlo Dufour and Régis Peffault de Latour
Grant providers: Novartis and Pfizer

Trial set-up

Currently, the standard immunosuppressive therapy (IST) for patients with (very) severe aplastic anemia ((v)SAA) who are not eligible for allogeneic stem cell transplantation is horse antithymocyte globulin (hATG) plus ciclosporin (CsA).

The RACE trial is an investigator initiated, open-label, randomized phase III trial comparing hATG and CsA with or without the thrombopoietin-mimetic agent Eltrombopag (EPAG) as frontline treatment of patients with (v)SAA.

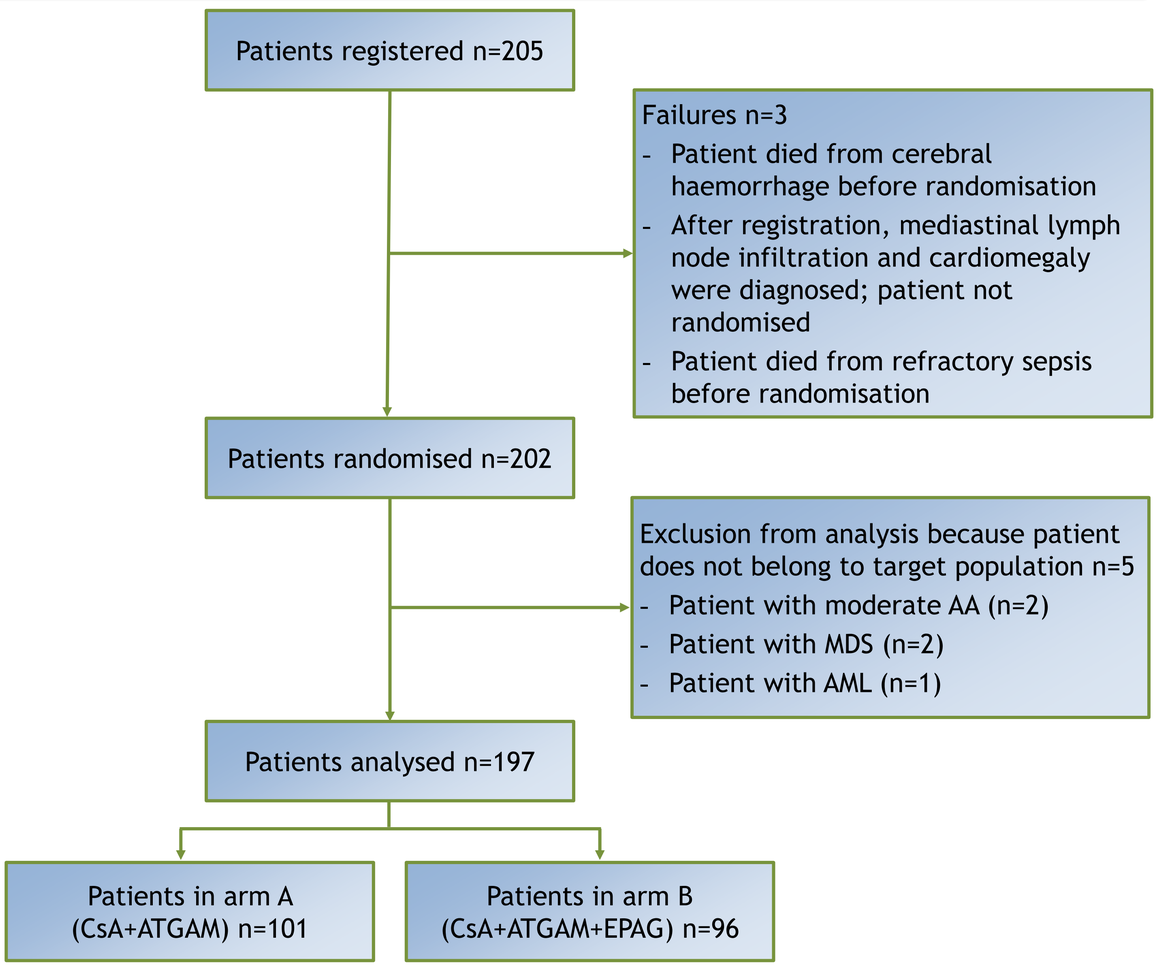
From July 2015 to April 2019, **197 treatment-naive patients** were enrolled in **6 countries and 24 sites**. Stratification for treatment was based on disease severity, age and center. Patients were randomized to either **standard IST** (**hATG** 40 mg/kg x4d and **CsA** 5 mg/kg/d; arm A) or **standard IST + EPAG** (experimental arm B) at the dose of 150 mg/d from day +14 until 6 months (or 3m, in case of early complete response (CR)).

Primary endpoint is the **rate of Complete Response (CR) at 3 months** since start of treatment in naive SAA patients. CR is defined as: haemoglobin >10 g/dL, absolute neutrophil count >1,000/µL and platelets >100,000 µL.

RACE goes Virtual

- **EBMT presidential symposium**
  Monday 31 August
  General, Auditorium 1, 14:30-16:00
  First presentation of RACE trial primary endpoint data
- **Bone marrow failure: From diagnosis to treatment in 2020**
  Tuesday 1 September
  Educational, Auditorium 1, 09:30-10:30

Study Population



Van Bekkum Award!

We are proud to share with you the excellent news that the RACE trial has been awarded the **Van Bekkum Award** for the best abstract submitted to the physician's programme of the EBMT annual congress. The **RACE trial primary endpoint data will be presented** for the first time as part of the **Presidential Symposium, Monday 31 August 14:30-16:00**.

This major achievement is the result of the hard work done by our many collaborators, first and foremost the research teams of the participating sites, but also the grant givers Novartis and Pfizer, and our external partners. We are grateful for the good collaboration and support: the short communication lines, the willingness to share expertise and the unabating enthusiasm for the RACE project. We look forward to finalizing the RACE trial and hope that we can count on continued support in this great endeavor of improving SAA patients' lives.

"Seven years after the first discussions, we feel proud to have achieved the primary endpoint of the RACE study improving the response rate of treatment-naïve patients with Severe Aplastic Anemia. We made it here thanks to the outstanding support, but the story does not end: RACE2 (long-term follow-up study) is on its way."

Baseline characteristics RACE participants

	Arm A	Arm B	Total
No. of patients	101 (51.3%)	96 (48.7%)	197 (100%)
Age (median, min-max)	52 (15-81)	55 (16-77)	53 (15-81)
Age categories (n, %)			
<18 y	7 (6.9%)	2 (2.1%)	9 (4.6%)
18-<40	29 (28.7%)	27 (28.1%)	56 (28.4%)
40-<65	43 (42.6%)	43 (44.8%)	86 (43.7%)
>65	22 (21.8)	24 (25.0%)	46 (23.4%)
Sex (n, %)			
Male	52 (51.5%)	56 (58.3%)	108 (54.8%)
Female	49 (48.5%)	40 (41.7%)	89 (45.2%)
Severity of AA (n, %)			
SAA	67 (66.3%)	62 (64.6%)	129 (65.5%)
vSAA	34 (33.7%)	34 (35.4%)	68 (34.5%)
Platelets $x10^9$/L (median, min-max)	18 (1-76)	15 (1-109)	17 (1-109)
Neutrophils $x10^9$/L (median, min-max)	0.30 (0.00-1.54)	0.46 (0.00-4.3)	0.39 (0.00-4.3)
Lymphocytes $x10^9$/L (median, min-max)	1.43 (0.64-3.45)	1.36 (0.14-2.73)	1.40 (0.14-3.45)
Reticulocytes $x10^9$/L (median, min-max)	20.0 (0.0-85.0)	25.5 (0.0-121.0)	21.1 (0.0-121.0)
Haemoglobin g/L (median, min-max)	8.5 (2.3-10.8)	8.3 (3.3-12.2)	8.4 (2.3-12.2)
PNH granulocytes >0.1% (n, %)	58 (59.2%)	42 (45.2%)	100 (52.4%)
PNH granulocytes >1.0% (n, %)	44 (44.9%)	33 (35.5%)	77 (40.3%)

Contact

Questions about RACE?
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New Clinical Trial or Non-Interventional Study Proposal?
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