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, Régis Peffault de Latour and Austin Kulasekararaj

Grant providers: Novartis and Pfizer

Trial set-up

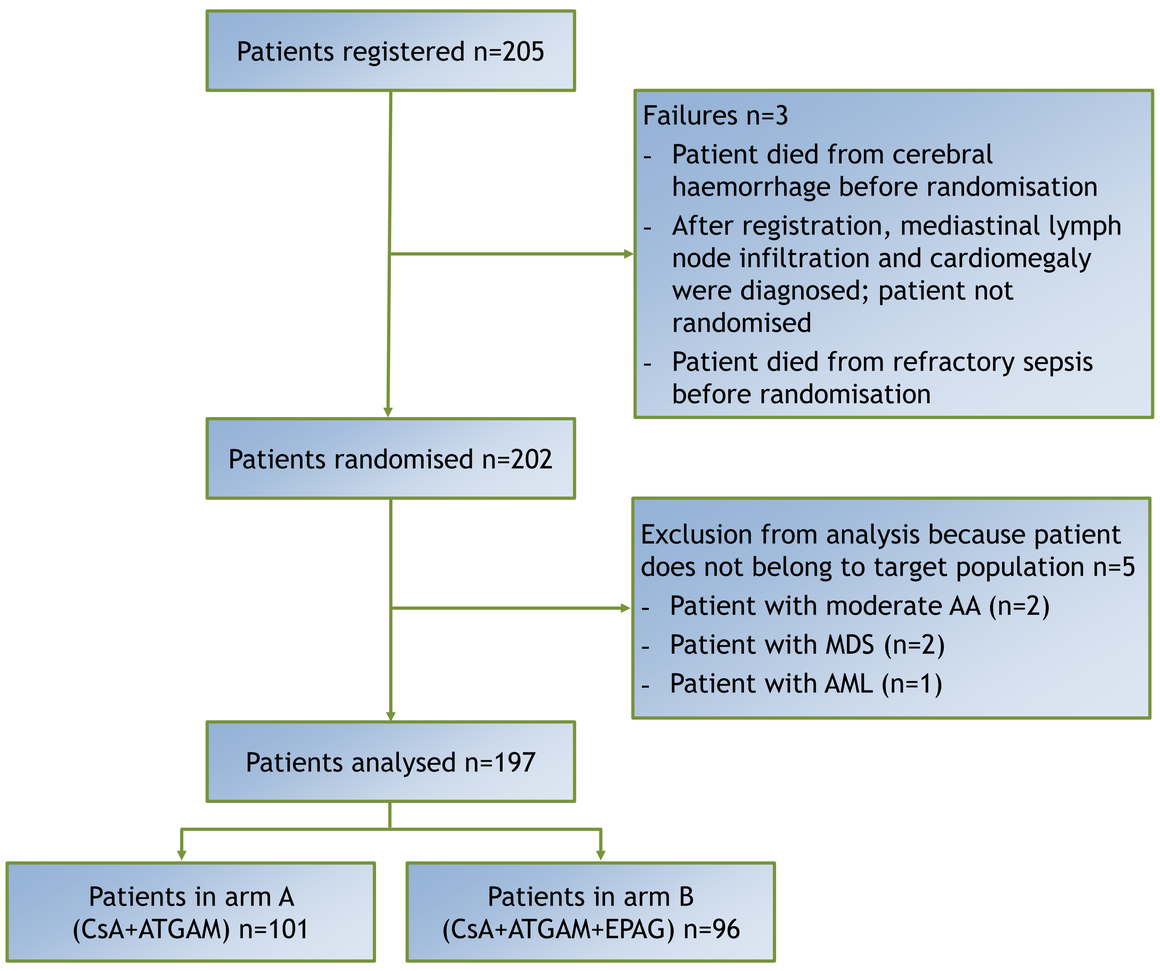
Currently, the standard immunosuppressive therapy (IST) for patients with (very) severe aplastic anemia ((v)SAA) who are not eligible for allogeneic stem cell transplantation is horse antithymocyte globulin (hATG) plus ciclosporin (CsA).

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.

Study Population



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%)

RACE Finish inside!

RACE is in the final phase. The Last Patient Last Visit is expected in April 2021. To avoid a huge workload at the end it is of great importance to complete and send all outstanding CRFs to the RACE trial team. Be informed that the RACE trial team will send out Data Queries more frequently.

COVID has caused an enormous workload on your sites in the last year and we can not take it easy yet. Due to COVID Monitoring Visits were cancelled and Close Out Visits were postponed. The RACE team is hoping to be able to schedule the on-site visits again.

Special thanks to all the sites participating in the RACE trial for their support and commitment !

RACE2 is coming!

RACE patients' outcomes will be followed up long term via the EBMT Registry

Contact

Questions about RACE?
Contact Johanneke Heeres (RACE trial coordinator): +31 71 526 1183, RACEtrial@lumc.nl

New Clinical Trial or Non-Interventional Study Proposal?
Contact Marianne Mol (Clinical Research Director): +31 71 526 3953, marianne.mol@ebmt.org

EBMT Clinical Trials Office Leiden

Rijnsburgerweg 10
2333 AA Leiden
The Netherlands