European Society for Blood and Marrow Transplantation

EBMT Clinical Trials Office

RACE Trial

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 anemia patients

Working party	Principal investigators	Trial Coordinator
SAA-WP	Antonio M Risitano Regis Peffault De Latour	Marleen van Os
	To investigate whether **Eltrombopag** (Revolade, Novartis) added to standard immunosuppressive treatment, CsA + hATG (ATGAM, Pfizer), increases the rate of early complete response in untreated AA patients	
Participating countries		

NIH Phase II Study on Eltrombopag

At the ASH congress 2015 Neal Young presented promising results from the NIH **phase II** study on Eltrombopag. Addition of Eltrombopag to immuno-suppressive therapy markedly increases overall and complete hematologic response rates in treatment-naive SAA.
See: bloodjournal.org/content/126/23/LBA-2.

The Race trial investigates Eltrombopag in a **phase III** randomised setting, aiming to change standard practice in SAA. Purpose of the trial is to improve the efficacy of the current treatment by combining standard immunosuppression with Eltrombopag in order to rescue or improve the function of residual hematopoiesis.

Country Status

Country	Approval	Nr of sites	Sites open
France	NCA & EC	6	4
United Kingdom	NCA & EC	5	2
Spain	NCA & EC	5	0
Italy	NCA & local EC	6	2
Netherlands	NCA & EC	4	1
Germany	submitting	5	0
Switzerland	submitting	1	0
Total		32	9

In Italy we obtained ethics approval in 3 sites; pending approval in 3 sites.
For sites with ethics approval we are negotiating site agreements. As soon as approvals and contracts are finalised, we can formally open the site, ideally within 2 weeks.

Main Inclusion Criteria

1. **Diagnosis of severe or very severe aplastic anemia, defined by:**
   - At least two of the following:
     - ✓ Absolute neutrophil counts <0,5 x 10^9/L (severe) or <0,2 x 10^9/L (very severe)
     - ✓ Platelets counts <20 x 10^9/L
     - ✓ Reticulocyte counts <60 x 10^9/L
   - Hypocellular bone marrow (<30% cellularity without evidences of fibrosis or malignant cells)
2. **Male or female age ≥ 15 years**

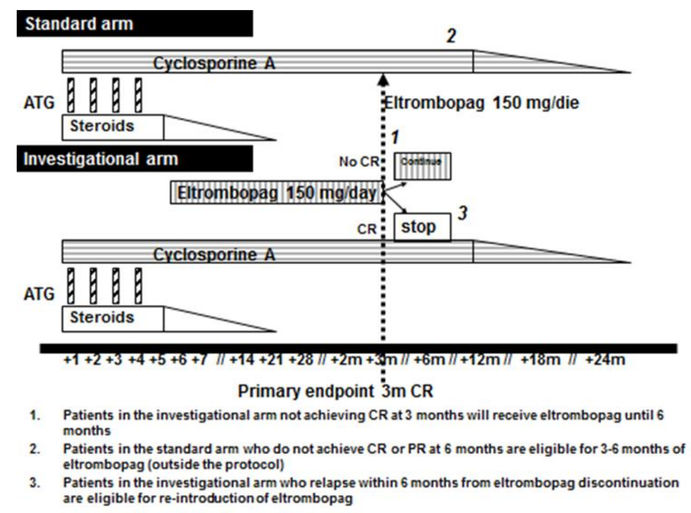
Primary Endpoint

Rate of Complete Response at 3 months

since start of treatment in untreated severe AA patients.

CR is defined as: Hb>10 g/dL, ANC>1,000/µL and Platelets>100,000 µL

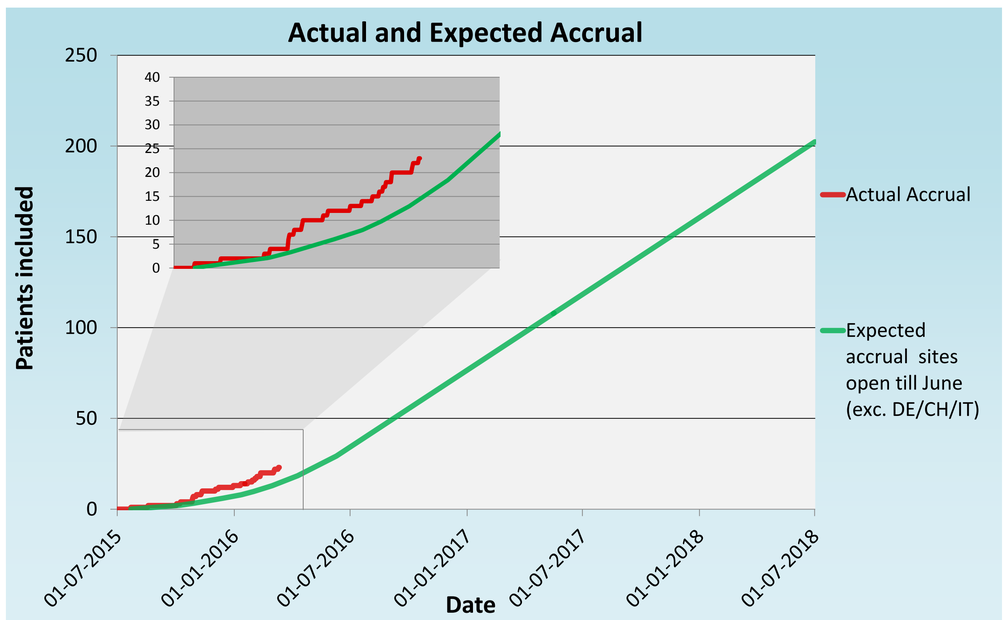
Treatment schedule



1. Patients in the investigational arm not achieving CR at 3 months will receive eltrombopag until 6 months
2. Patients in the standard arm who do not achieve CR or PR at 6 months are eligible for 3-6 months of eltrombopag (outside the protocol)
3. Patients in the investigational arm who relapse within 6 months from eltrombopag discontinuation are eligible for re-introduction of eltrombopag

	Treatment	Dose (units)	Treatment Period
Arm A (standard):	ATGAM	40 mg/kg/day, iv	Day 1, 2, 3 and 4
	Cyclosporine A	5 mg/kg/day, po	Day 1-365
Arm B: arm A + Investigational drug	Eltrombopag	150 mg every 24 h, po	Day 14-90 (or 14-180)

Accrual



RACE Team