

# 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 and Régis Peffault de Latour.

Grant providers: Novartis and Pfizer



#### **Trial Objective**

The primary objective of this trial is to investigate whether Eltrombopag added to standard immunosuppressive treatment increases the rate of early (at three months) complete response in untreated aplastic anaemia patients.

The Race trial is a prospective open label randomised phase III trial aiming to change standard practice in SAA.

	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)

Primary endpoint: Rate of Complete Response at 3 months since start of treatment in naive severe AA patients.

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

Country Status (data per 21 February 2018)				
Country	N° of open sites	N° of enrolling sites	N° of randomised subjects	
France	7	7	58	
Italy	5	3	14	
Netherlands	4	4	19	
Spain	3	2	4	
Switzerland	1	1	6	
United Kingdom	4	3	23	
Total	24	20	124	

### **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<sup>9</sup>/L
    - ✓ Reticulocyte counts <60 x 10<sup>9</sup>/L
  - Hypocellular bone marrow (<30% cellularity without evidences of fibrosis or malignant cells)
- 2. Male or female age ≥ 15 years

#### **Treatment schema** Consent and routine tests to assess pt eligibility must be done Registration within 30 days before registration Randomisation max 7 days Randomisation after registration ARM B: (hATG+CsA) (hATG+CsA+EPAG) Start hATG on same day or day 4 days hATG 4 days hATG after randomisation Start eltrombopag at day 14 from start hATG 2,5 months EPAG Dose: 150 mg/day (Asian pts ½ dose) PR/NR 3 month evaluation Continue with Stop EPAG 3 months EPAG CR/PR NR Relapse Stop EPAG 6 month evaluation within 6m ifter stop EPAG option restart EPAG for 6m Relapse within 6m Option: open label EPAG for after stop EPAG option restart up to EPAG for 6m 6 months 12 month: taper CsA Last FU 24 month evaluation

Legenda: EPAG = Eltombopag hATG = ATGAM CsA = Cyclosporin

Note 1: time is not depicted linearly. With we indicate a variable time span between 2 weeks and 6 months after stop EPAG treatment.

Note 2: patients with NR at 6 months FU are considered treatment failures and should receive rescue treatment chosen by PI. Only patients in arm A are eligible for 3 up to 6 month EPAG as salvage treatment within the RACE trial.

#### Accrual **Actual and Expected Accrual RACE trial** 200 180 160 140 **Subjects included** Expected accrual 120 according to Protocol Expected for current open sites 60 —Actual Accrual 40 20 7/1/2017 1/1/2018 1/1/2019 1/1/2016 7/1/2016 1/1/2017 7/1/2018 **Date**

## The CTO team

**Clinical Trials Operating Manager** 

Marianne Mol ebmtcto@lumc.nl

**Clinical Trials Coordinators** 

Alain Barrois, Dilyana Georgieva, Annelies Kleijne, Marleen van Os, Janette Symons and

Sofie Terwel (RACE Trial Project Coordinator) racetrial@lumc.nl



EBMT Clinical Trials
Office Leiden

Rijnsburgerweg 10 2333 AA Leiden The Netherlands

Tel: +31 (0)71 526 5005 Fax: +31 (0)71 526 6185

