Severe Aplastic Anemia Working Party

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Secretary: Régis Peffault de Latour (Paris)
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Clinical trials with Eltrombopag as part of the initial treatment of AA in Europe

Table: *Overview of the EMAA trial and RACE trial : study objective, inclusion criteria, treatment, eltrombopag dosage, design, number of patients and sponsor.*

	moderate AA (EMAA)	vSAA / SAA (RACE)
Primary objective	PR + CR at 6 months	CR at 3 months
Inclusion criteria	- age ≥18 years - Treatment requiring MAA (transfusion dependency or ANC < 1G/l or Thrombo < 30G/l or Hb < 8,5g/dl & Reti < 60G/l)	- age ≥ 15 years - SAA/ vSAA - No primary allo-SCT
Treatment	CsA + Eltrombopag versus CsA + Placebo	hATG (ATGAM) + CsA + Eltrombopag versus h ATG + CsA
Eltrombopag dosage	150 mg (225 mg)	150 mg
Design	Placebo controlled	Open label
# Patients	2 x 58	2 x 100
Sponsor	University Hospital Ulm	EBMT

RACE trial

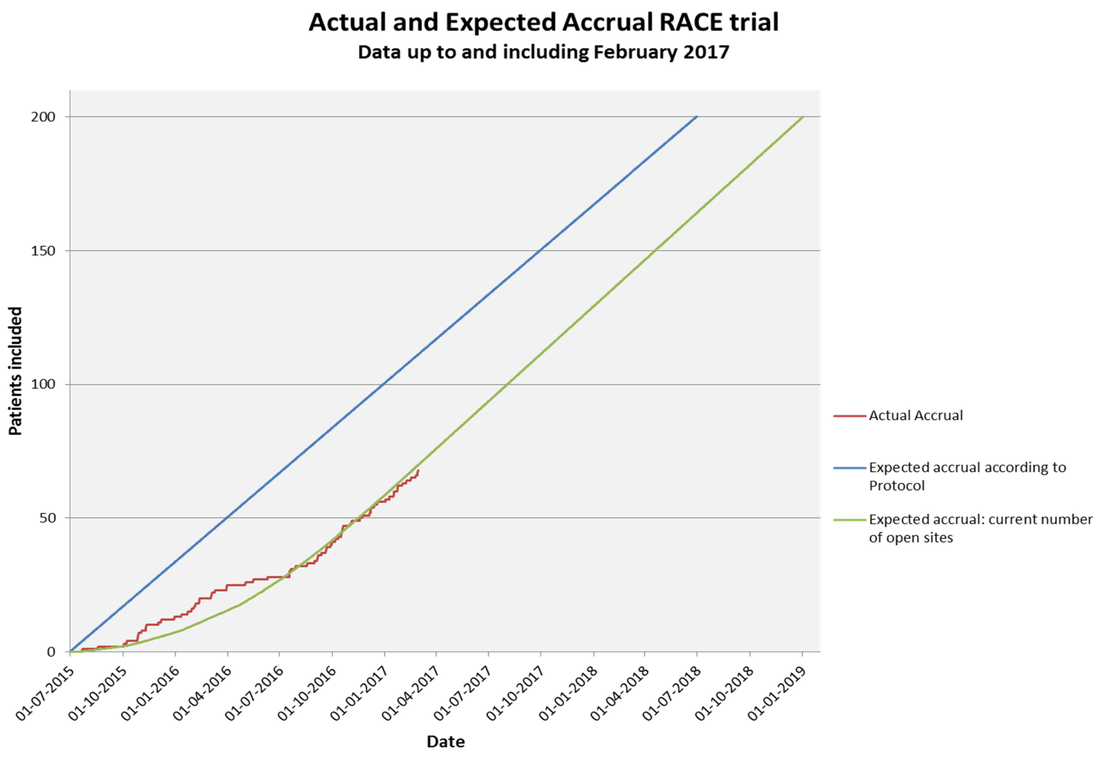

Figure: *Actual and expected accrual RACE trial from July 2015 till July 2018 (last update: March 1st 2017). For more information see the RACE trial poster.*

EMAA trial

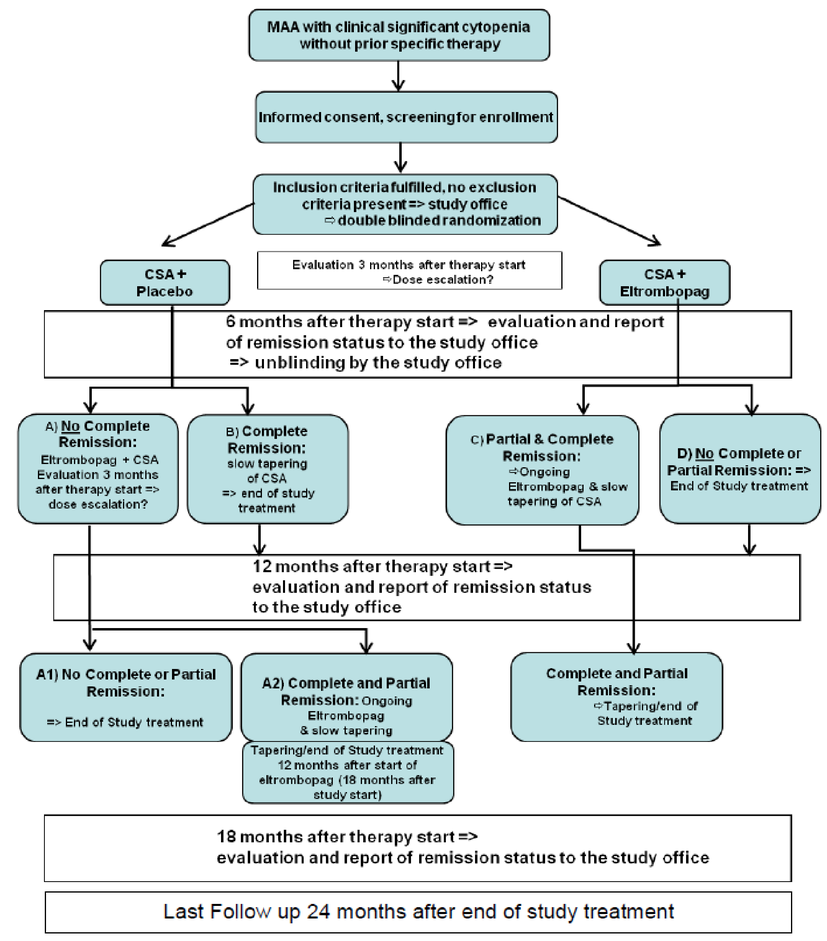

Figure*: study schedule EMAA trial.*

Numbers in registry

14.518 patients are registered with some type of Bone Marrow Failure in the EBMT registry . The tables below present the numbers per type of disease.

Acquired BM failure syndrome	n
Aplastic anaemia	10.749
Pure red cell aplasia (non congenital PRCA)	129
Paroxysmal nocturnal haemoglobinuria (PNH)	600
Pure white cell aplasia	11
Ameg. thrombocytopaenia (non congenital)	48
Other	238
Unknown	122
TOTAL	**11.897**

Genetic BM failure syndrome	n
Fanconi	1.860
Diamond-Blackfan (congenital PRCA)	284
Shwachman-Diamond	60
Dyserythropoietic anaemia	38
Dyskeratosis congenita	106
Ameg. thrombocytopaenia (congenital)	95
Congenital sideroblastic anaemia	19
Other	132
Unknown	27
TOTAL	**2.621**

Shwachman-Diamond and Eltrombopag

SDS : SAAWP-EBMT retrospective analysis of characteristics and outcome of patients affected by Shwachman-Diamond disease (SDS) who underwent HSCT

PI: Simone Cesaro, Verona, Italy

DM: Paul Bosman, EBMT Data Office, Leiden

Actual accrual till March 2017: N= 28

Eltrombopag: to investigate the safety and efficacy of Eltrombopag in patients with SAA receiving the drug for initial immunosuppressive treatment or for incomplete response or to treat relapse.

PI: Jakob Passweg, Basel, Switzerland

DM: Paul Bosman, EBMT Data Office, Leiden

Actual accrual till March 2017: N= 34

Publications 2016

Kelly RJ, Kulasekararaj A, Risitano AM, Peffault de Latour R. Eculizumab in Pregnant Patients with Paroxysmal Nocturnal Hemoglobinuria. N Engl J Med. 2015 Sep 10;373(11):1032-9. 2016.

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Risitano AM, Marotta S. Therapeutic complement inhibition in complement-mediated hemolytic anemias: Past, present and future. Semin Immunol.;28(3):223-40. 2016

Devillier R, Dalle JH, Kulasekararaj A, Dufour C, Peffault de Latour R. Unrelated alternative donor transplantation for severe acquired aplastic anemia: a study from the French Society of Bone Marrow Transplantation and Cell Therapies and the EBMT Severe Aplastic Anemia Working Party. Haematologica.;101(7):884-90.2016

Alternative donor transplants for severe aplastic anemia: current experience. Bacigalupo A, Sica S. Semin Hematol. 2016

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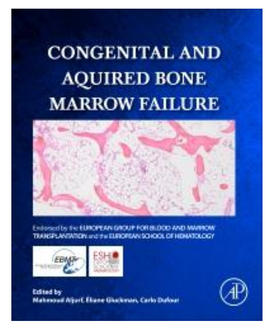

SAAWP Data Office

For participation in, or information on SAA studies, please contact the the EBMT Data Office in Leiden, The Netherlands: SAAwpEBMT@lumc.nl.