Severe Aplastic Anaemia Working Party

Chair: Carlo Dufour (Genova)
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

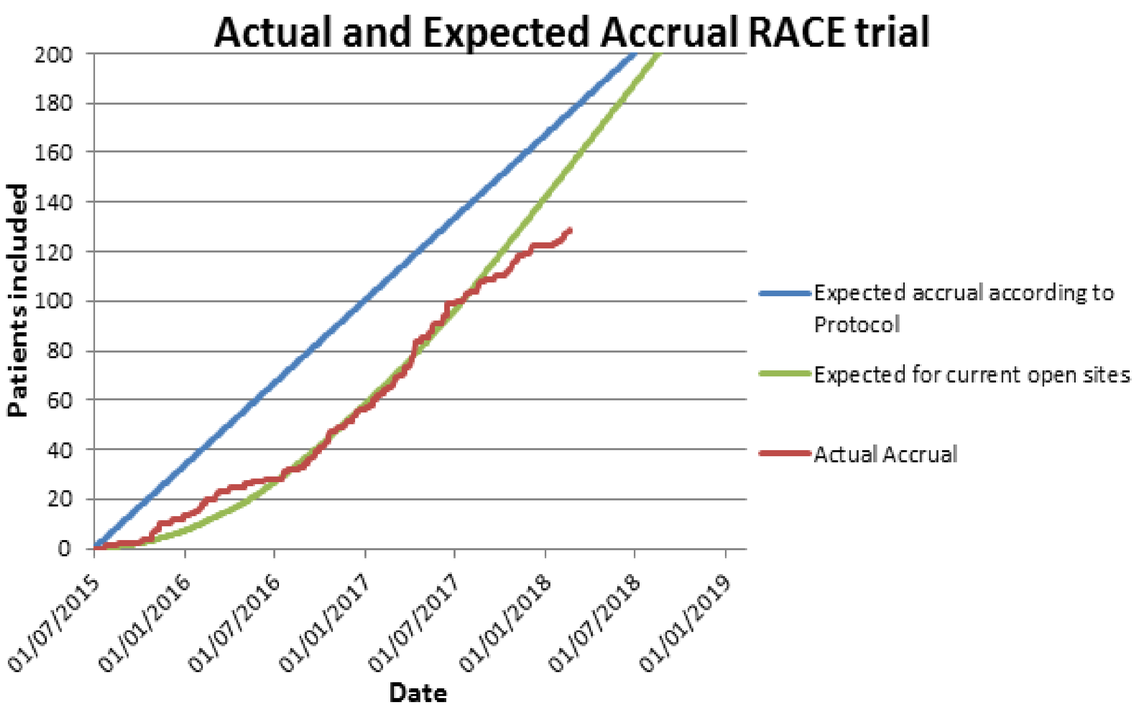

Figure: Actual and expected accrual RACE trial from July 2015 till February 2018 (last update: February 21st 2018). For more information see the RACE trial poster.

EMAA trial

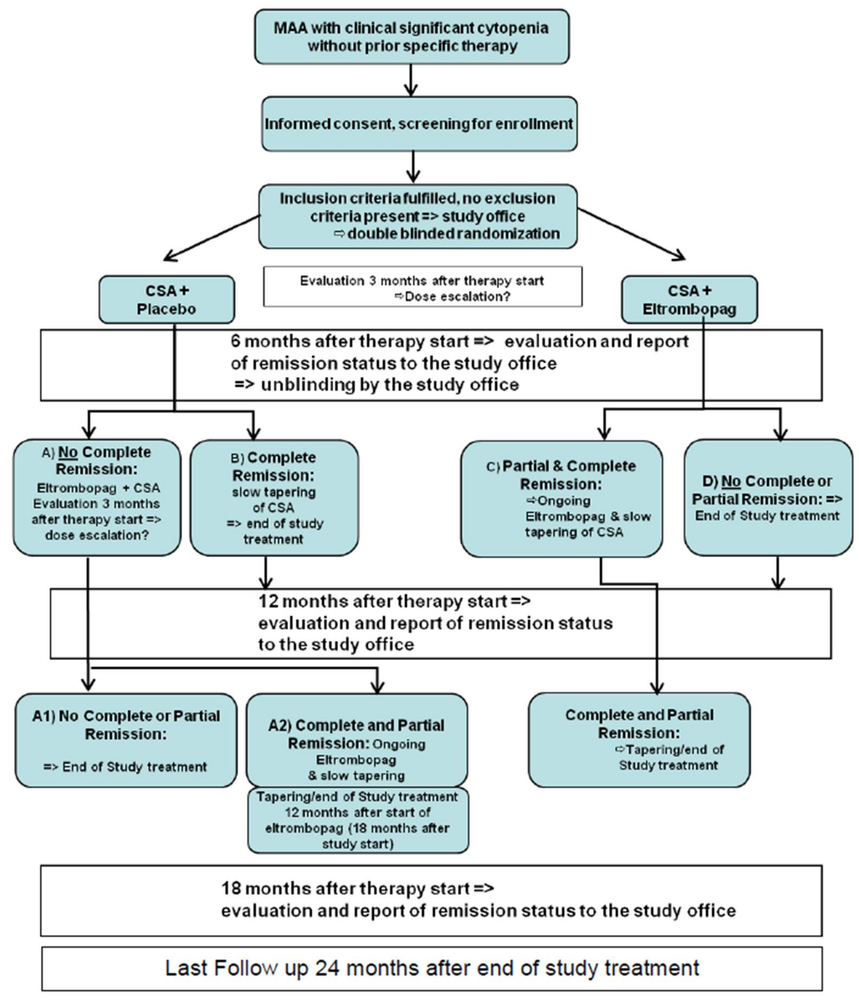

Figure: study schedule EMAA trial. For more information see the EMAA trial poster.

Numbers in registry

15450 patients are registered in the EBMT registry database with some type of Bone Marrow Failure. The tables below present the numbers per type of disease.

Acquired BMF	n
Aplastic anaemia	11249
Pure red cell aplasia (non congenital PRCA)	133
Paroxysmal nocturnal haemoglobinuria (PNH)	626
Pure white cell aplasia	11
Ameg. thrombocytopaenia (non congenital)	50
Other	249
Unknown	116
TOTAL	**12434**

Genetic BMF	n
Fanconi	1948
Diamond-Blackfan (congenital PRCA)	308
Shwachman-Diamond	62
Dyserythropoietic anaemia	40
Dyskeratosis congenita	118
Ameg. thrombocytopaenia (congenital)	108
Congenital sideroblastic anaemia	20
Other	150
Unknown	63
TOTAL	**2817**

Call for data!

Follow up of G-CSF trial – A. Tichelli (Basel, Switzerland)
Data manager: Paul Bosman and Nelleke van 't Veer
Accrual until February 2018: n=85
Deadline: June 15th, 2018

Transformed Fanconi Anaemia – S. Giardino (Genova, Italy)
Data manager: Paul Bosman
Accrual until February 2018: n=19

NEW: Haplo transplants in SAA – P. De Lima Prata (Paris, France)
Deadline: April 30th, 2018

Publications 2017/2018

Bacigalupo, A. (2017). Antithymocyte globulin and transplants for aplastic anaemia. Haematologica, 102(7), 1137-1138.

Bacigalupo, A. (2017). How I treat acquired aplastic anaemia. Blood, 129(11), 1428-1436.

Bierings, M., et al (2018). Transplant results in adults with Fanconi anaemia. British Journal of Haematology, 180(1), 100-109.

Dufour, C. (2017, April). Classical inherited bone marrow failure syndromes with high risk for myelodysplastic syndrome and acute myelogenous leukemia. Seminars in Hematology, 54(2), 105-114.

Dufour, C. (2017). How I manage patients with Fanconi anaemia. British Journal of Haematology, 178, 32-47.

Pagliuca, S., et al (2017). Long-Term Outcomes of Cord Blood Transplantation from an HLA-Identical Sibling for Patients with Bone Marrow Failure Syndromes: A Report From Eurocord, Cord Blood Committee and Severe Aplastic Anemia Working Party of the European Society for Blood and Mar. Biology of Blood and Marrow Transplant, 23(11), 1939-1948.

Risitano, A. (2017). Immune insights into AA. Blood, 129, 2824-2826.

Rovó, A., et al (2017). Association of aplastic anaemia and lymphoma: a report from the severe aplastic anaemia working party of the European Society of Blood and Bone Marrow Transplantation. British Journal of Haematology, Epub ahead of print.

SAAWP Data Office

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