



Severe Aplastic Anemia Working Party

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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 \geq 18 years - Treatment requiring MAA (transfusion dependency or ANC < 1G/l or Thrombo < 30G/l or Hb < 8,5g/dl & Reti < 60G/l)	- age \geq 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

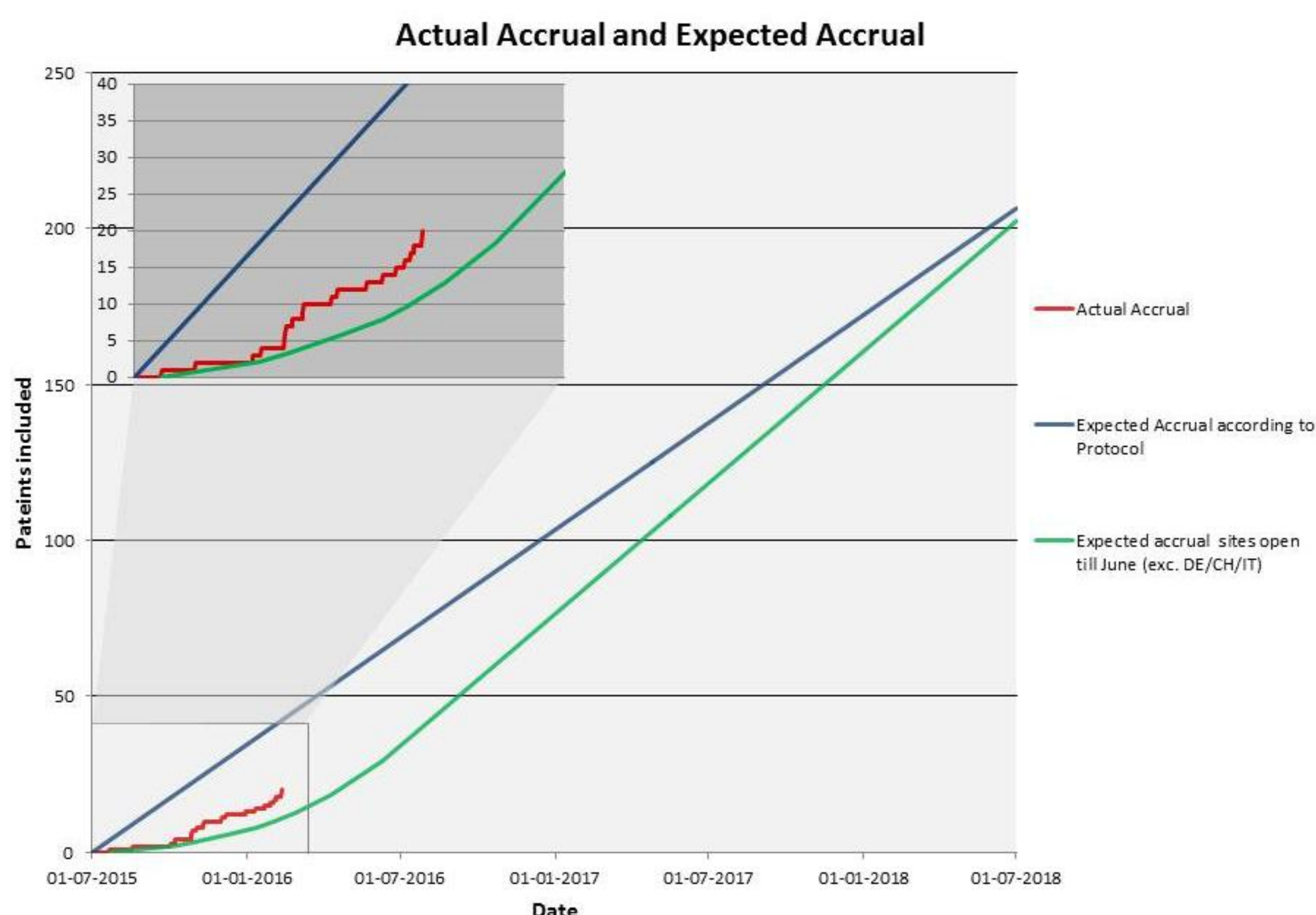


Figure: Actual and expected accrual RACE trial from July 2015 till July 2018 (last update: March 1st 2016). For more information see the RACE trial poster.

EMAA trial

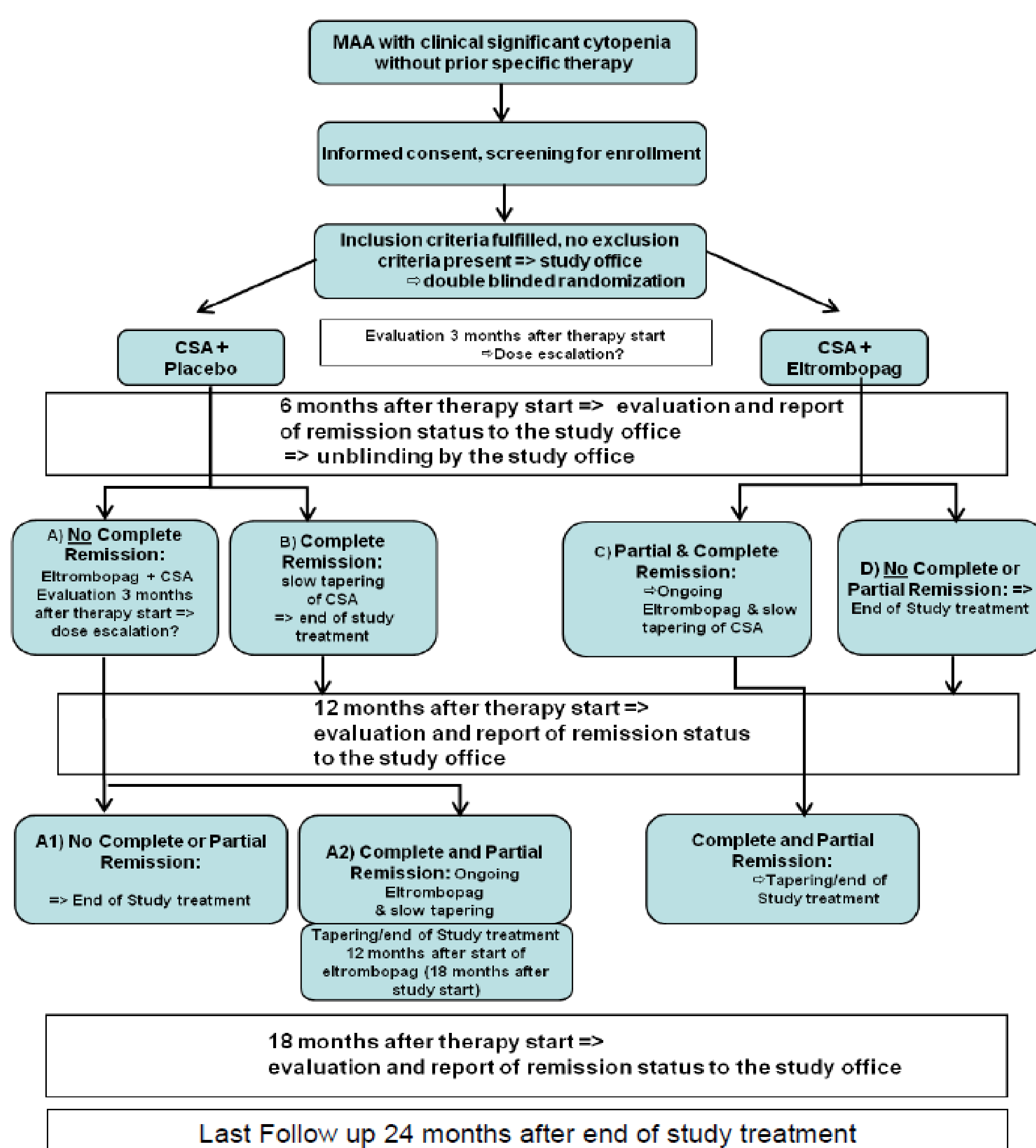


Figure: study schedule EMAA trial.
For more information see the EMAA trial poster

Numbers in registry

13,655 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	Genetic BM failure syndrome	n
Aplastic anaemia	10,136	Fanconi	1,737
Pure red cell aplasia (non congenital PRCA)	133	Diamond-Blackfan (congenital PRCA)	251
Paroxysmal nocturnal haemoglobinuria (PNH)	568	Shwachman-Diamond	53
Pure white cell aplasia	11	Dyserythropoietic anaemia	36
Ameg. thrombocytopaenia (non congenital)	45	Dyskeratosis congenita	94
Other	234	Ameg. thrombocytopaenia (congenital)	84
Unknown	118	Other	130
Unknown		Unknown	20
TOTAL	11,245	TOTAL	2,405

Joint Educational meeting SAAWP and ADWP

What: meet and interact with renowned international experts on auto-immune disorders and severe aplastic anaemia

When and where: two and a half day in the fall of 2016 in Paris

Programme: will be available soon on the EBMT website

More information: please send an e-mail to educationalevents@ebmt.org

Publications 2015/2016

Bacigalupo, A., et al. (2015). "Current outcome of HLA identical sibling versus unrelated donor transplants in severe aplastic anemia: An EBMT analysis." *Haematologica* 100(5): 696-702.

Cesaro, S., et al. (2015). "Second allogeneic stem cell transplant for aplastic anaemia: A retrospective study by the severe aplastic anaemia working party of the European society for Blood and Marrow Transplantation." *British Journal of Haematology* 171(4): 606-614.

Dufour, C., et al. (2015). "Outcome of aplastic anaemia in children. A study by the severe aplastic anaemia and paediatric disease working parties of the European Group Blood and bone Marrow Transplant." *British Journal of Haematology* 169(4): 565-573.

Dufour, C., et al. (2015). "Similar outcome of upfront-unrelated and matched sibling stem cell transplantation in idiopathic paediatric aplastic anaemia. A study on behalf of the UK paediatric BMT working party, paediatric diseases working party and severe aplastic anaemia working party of EBMTt." *British Journal of Haematology* 171(4): 585-594.

Fiorredda, F., et al. (2015). "Stem cell transplantation in severe congenital neutropenia: An analysis from the European society for Blood and Marrow Transplantation." *Blood* 126(16): 1885-1892.

Iori, A. P., et al. (2015). "Stem cell transplant from unrelated donor in patients with severe aplastic anemia: Indications, donor selection, conditioning, GvHD prophylaxis." *Current Drug Targets*: 12 Feb 2015.

Kulasekararaj, A. G. and Marsh, J. C. (2015). "Architectural clones in aplastic anaemia- lessons learned from high throughput sequencing." *Current Drug Targets*: 13 Jan 2015.

Pagliuca, S., et al. (2016). "Characteristics and outcomes of aplastic anemia in HIV patients: A brief report from the severe aplastic anemia working party of the European society of Blood and bone Marrow Transplantation." *Bone Marrow Transplantation* 51(2): 313-315.

Risitano, A. M. (2015). "Aplastic anemia: Alternative immunosuppressive treatments and eltrombopag. A report from the 2014 EBMT educational meeting from the severe aplastic anaemia and infectious diseases working parties." *Current Drug Targets*: 25 Jan 2015.

Schrezenmeier, H., et al. (2015). "Standard first-line immunosuppression for acquired severe aplastic anemia in adults." *Current Drug Targets*: 30 Jun 2015.

SAAWP Data Office

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