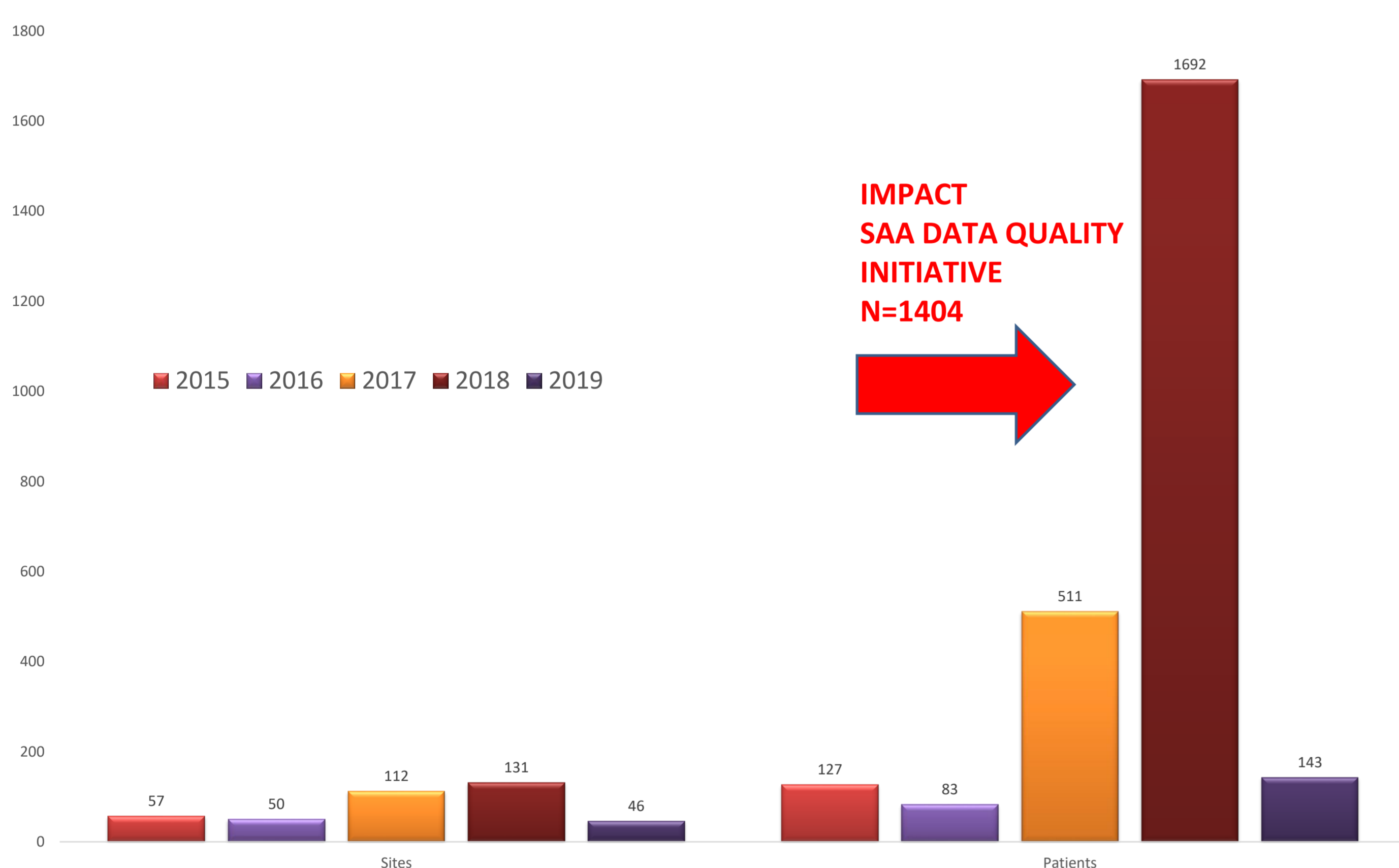


## Clinical trials with Eltrombopag as part of the initial treatment of AA in Europe

Table: Overview of the EMAA trial and RACE trial : study objective, inclusion criteria, treatment, eltrombopag dosage, design, number of patients and sponsor. For SAA Trial see separate poster

	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

## SAA Participating Overview 2015 - 2019



## EMAA trial

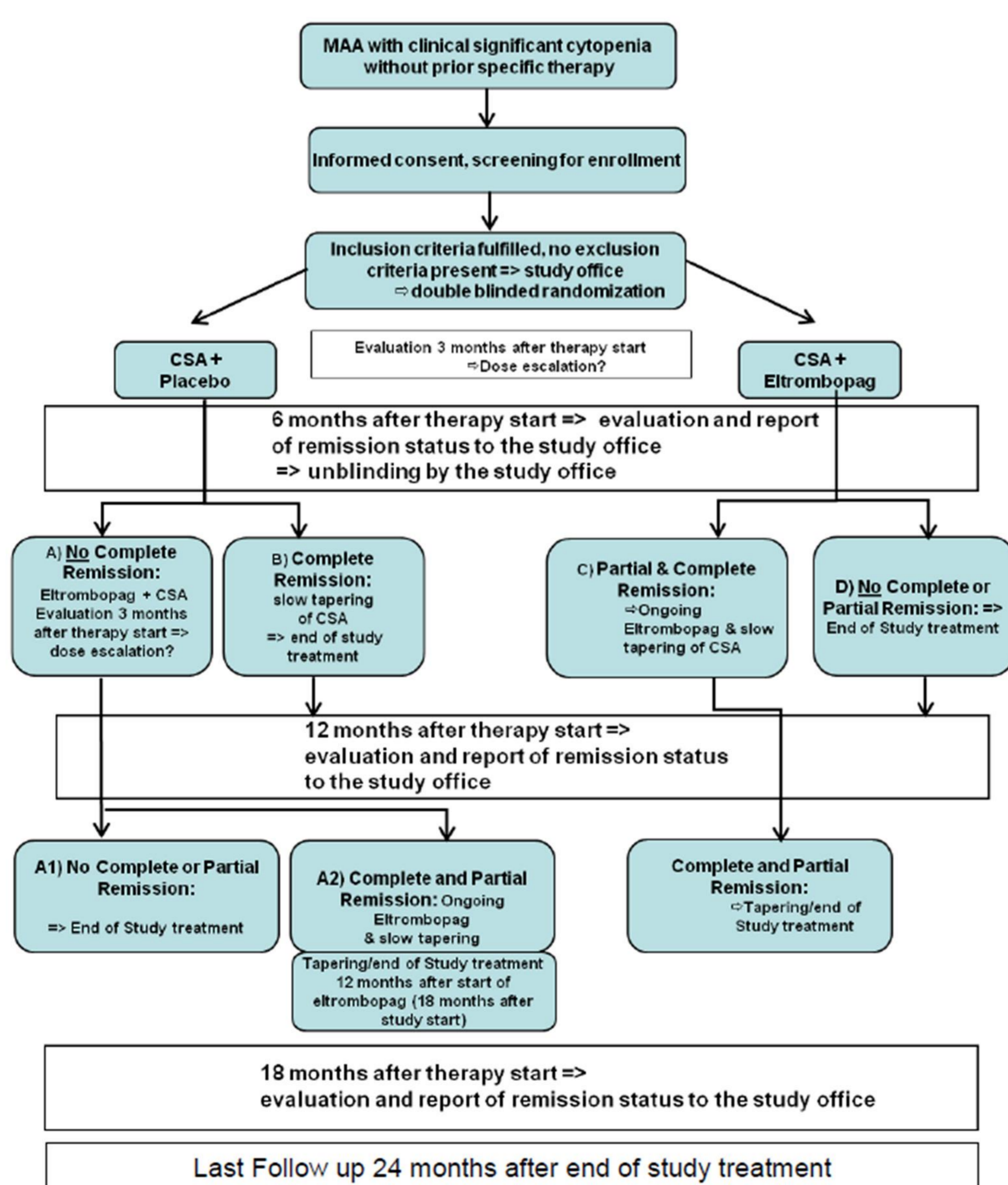


Figure: study schedule EMAA trial. For more information see the EMAA trial poster.

## Numbers in Registry

17213 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	Genetic BMF	n
Aplastic anaemia	12534	Fanconi	2164
Pure red cell aplasia (non congenital PRCA)	142	Diamond-Blackfan (congenital PRCA)	377
Paroxysmal nocturnal haemoglobinuria (PNH)	685	Shwachman-Diamond	75
Pure white cell aplasia	14	Dyserythropoietic anaemia	48
Ameg. thrombocytopaenia (non congenital)	55	Dyskeratosis congenita	141
Other	263	Ameg. thrombocytopaenia (congenital)	126
Unknown	120	Congenital sideroblastic anaemia	25
<b>TOTAL</b>	<b>13813</b>	<b>TOTAL</b>	<b>3182</b>

## Change of Staff !

As of this summer the SAA WP will have a new studycoordinator. Her name is Anne Lippinkhof-Kozijn. And also welcome Brian Piepenbroek, the SAA datamanager.



## Publications 2019/2020

[Bone Marrow Transplant](#). 2019 Sep 25. doi: 10.1038/s41409-019-0680-4. [Epub ahead of print] Autoimmune cytopenias (AIC) following allogeneic haematopoietic stem cell transplant for acquired aplastic anaemia, [Miller PDE](#)

[Biol Blood Marrow Transplant](#). 2019 Mar;25(3):488-495. doi: 10.1016/j.bbmt.2018.08.029. Epub 2018 Sep 5.

Allogeneic Hematopoietic Cell Transplantation in Patients Aged 50 Years or Older with Severe Aplastic Anemia. [Rice C](#)

[Bone Marrow Transplant](#). 2019 Dec 16. doi: 10.1038/s41409-019-0773-0. [Epub ahead of print] Haploidentical transplantation and posttransplant cyclophosphamide for treating aplastic anemia patients: a report from the EBMT Severe Aplastic Anemia Working Party. [Prata PH](#)

[Am J Hematol](#). 2019 Jan;94(1):80-86. doi: 10.1002/ajh.25314. Epub 2018 Nov 25. Impact of T-cell depletion strategies on outcomes following hematopoietic stem cell transplantation for idiopathic aplastic anemia: A study on behalf of the European blood and marrow transplant severe aplastic anemia working party. [Samarasinghe S](#)

[Haematologica](#). 2019 Oct 3. pii: haematol.2019.222562. doi: 10.3324/haematol.2019.222562. [Epub ahead of print]

Long-term outcome of a randomized controlled study in patients with newly diagnosed severe aplastic anemia treated with antithymocyte globuline, cyclosporine, with or without G-CSF: a Severe Aplastic Anemia Working Party Trial from the European Group of Blood and Marrow Transplantation. [Tichelli A](#)



SAAWP Business Autumn Meeting will take place in Paris, France on

Thursday 12<sup>th</sup> November 2020

Exact details will follow...



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