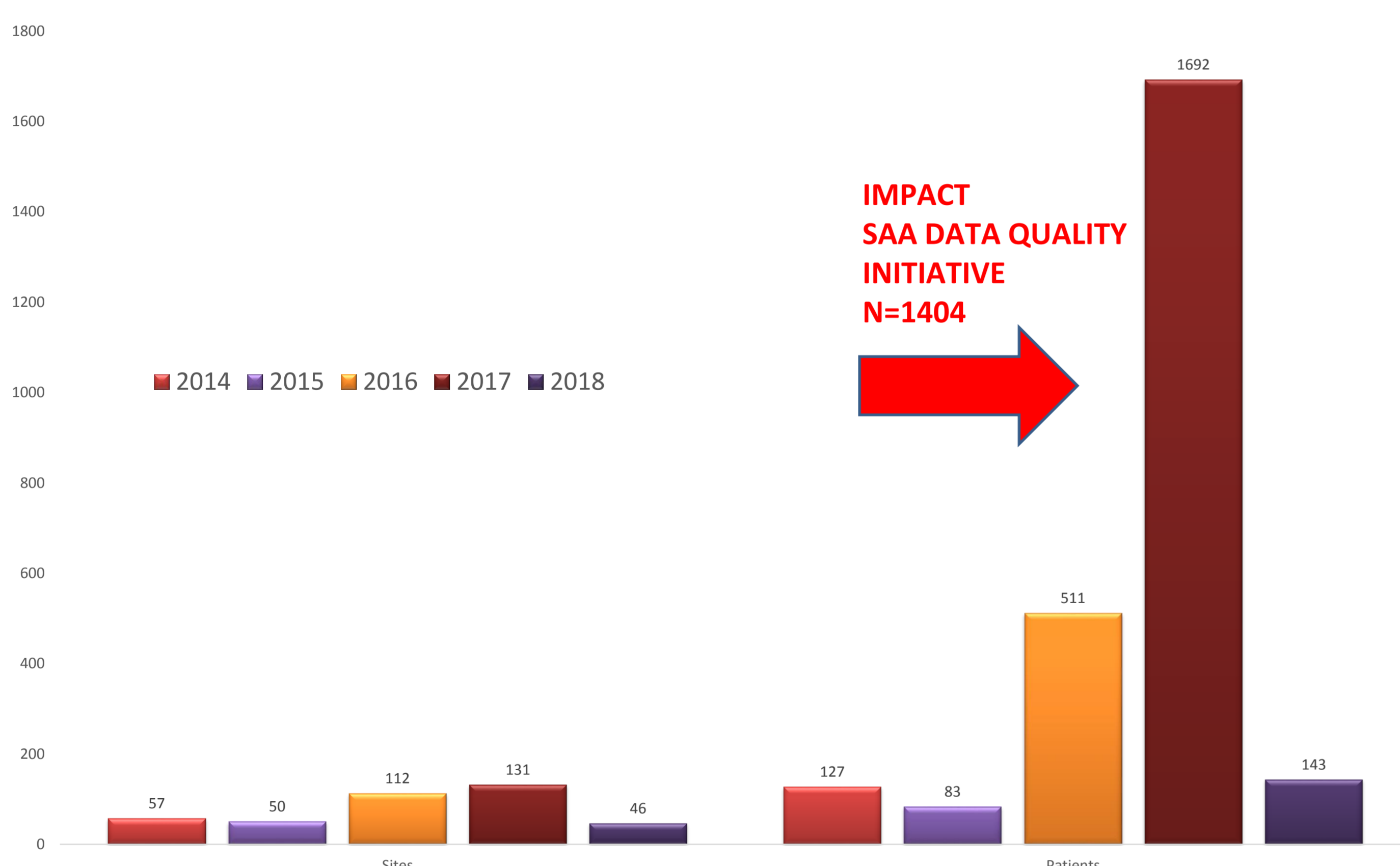


## Clinical trials with Eltrombopag as part of the initial treatment of AA in Europe

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 2014 - 2018



## EMAA trial

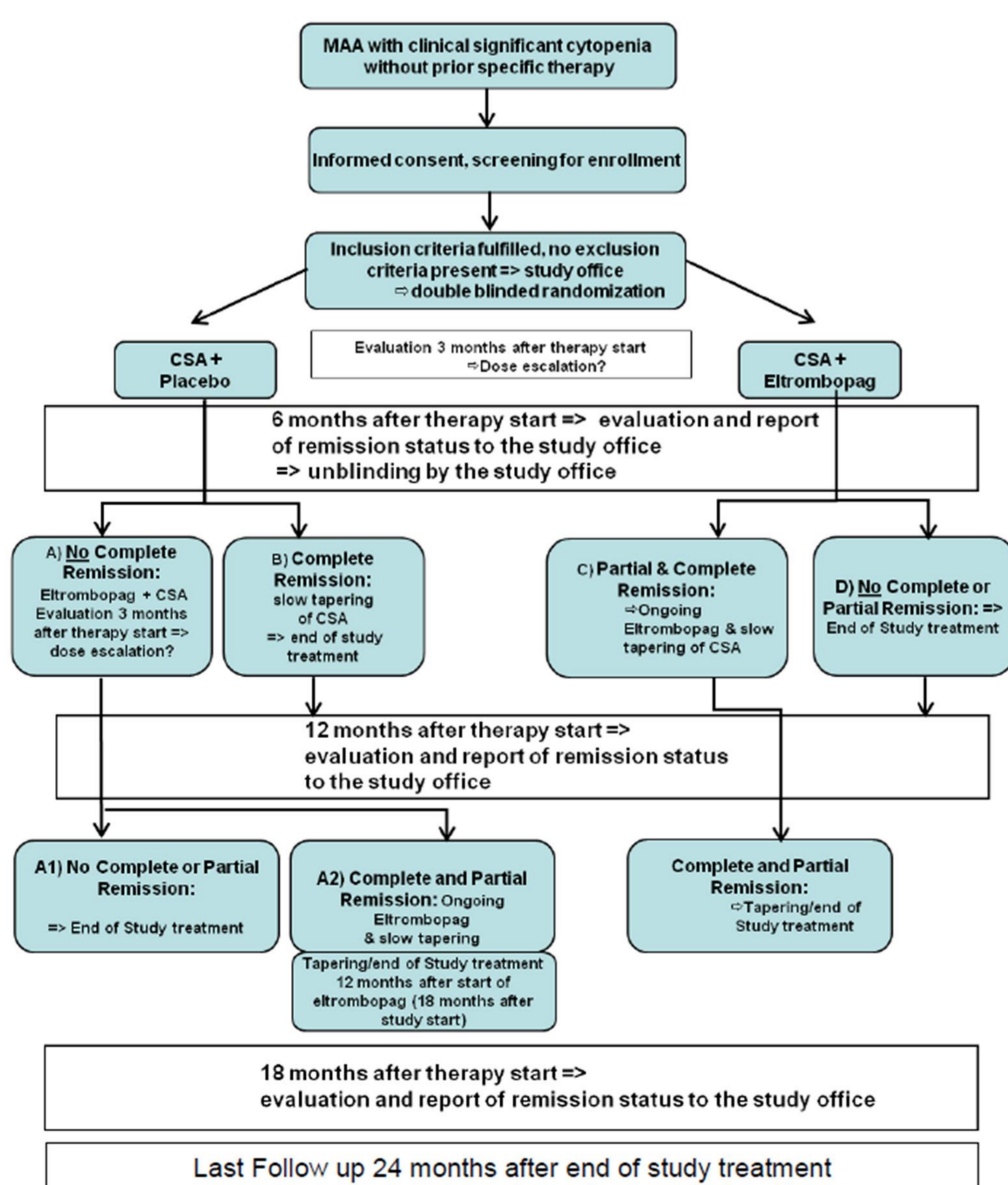


Figure: study schedule EMAA trial. For more information see the EMAA trial poster.

## Numbers in Registry

16216 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	11985	Fanconi	2066
Pure red cell aplasia (non congenital PRCA)	137	Diamond-Blackfan (congenital PRCA)	338
Paroxysmal nocturnal haemoglobinuria (PNH)	663	Shwachman-Diamond	67
Pure white cell aplasia	12	Dyserythropoietic anaemia	42
Ameg. thrombocytopaenia (non congenital)	54	Dyskeratosis congenita	133
Other	258	Ameg. thrombocytopaenia (congenital)	119
Unknown	115	Congenital sideroblastic anaemia	20
<b>TOTAL</b>	<b>13224</b>	<b>TOTAL</b>	<b>2992</b>

## Call for Data !

### ATG vs Campath in Fanconi – S. Samarasinghe (London, UK)

Data manager: Paul Bosman

Accrual until February 2019: N=113

Deadline: April 15<sup>th</sup>, 2019

### Partial Remission after IST – A. Rovo (Bern, Switzerland)

Data manager: Paul Bosman

Accrual until February 2019: n=19

Deadline: April 15<sup>th</sup>, 2019

### HAAA after liver TX – A. Mohseny (Leiden, The Netherlands)

Datamanager: Paul Bosman

Accrual until February 2019: N= 7

### HSCT for Diamond Blackfan Anaemia – M. Miano (Genova, Italy)

Study preparation nearing completion

## Publications 2018/2019

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

Samarasinghe, S., et al (2018). Impact of T-Cell depletion strategies (...) following HSCT. Am J Hematol. 2019 Jan;94(1): 80-86

Rice, C., HSCT in patients aged 50 years or older with severe Aplastic Anemia. BMT 2018 September

Miano, M., et al (2019) HSCT for CDA, Haematologica 2019 jan 24

Fiorredda, F., et al (2018) Outcome HSCT in DKC, British Journal of Haematology, 183(1), 110-118

## Save the date:



Meeting SAAWP  
November, 2019  
Genova, Italy

Time and place are subject to change.

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)