





Evaluation of the long-term effect of G-CSF added to ATG and CSA in patients with newly diagnosed severe aplastic anemia

 Open-label multicenter randomized study (NCT01163942)

 192 patients with newly diagnosed severe aplastic anemia (SAA), median age: 46 years (2-81)



 Median follow-up 11.7 (10.9-12.5) years

	 ALL	 G-CSF	 non-G-CSF	
Overall survival (OS) at 15 years	60±9%	57±12%	63±12%	P=0.92
Event free survival (EFS) at 15 years	24±7%	24±10%	23±10%	P=0.36
<i>Development of:</i>				
• Myelodysplastic syndrome or acute myeloid leukemia(*)	n=9	• Solid tumor	n=10	
• Clonal cytogenetic abnormality(*)	n=10	• Osteonecrosis	n=8	
• Clinical paroxysmal nocturnal hemoglobinuria	n=18	• Chronic kidney disease	n=12	
Cumulative incidence at 15 years(*)		8.5±3%	8.2±3%	P=0.90
Cumulative incidence at any late event		50±12%	49±12%	P=0.65

G-CSF unlikely impacts on the outcome of severe aplastic anemia