

Response to immunosuppressive therapy and long-term outcomes for pediatric severe aplastic anemia



Retrospective study (data from 2002-2014)



25 institutions in the North American Pediatric Aplastic Anemia Consortium (NAPAAC)



314 pediatric patients (1-20 years) diagnosed with acquired severe aplastic anemia (SAA) and treated with immunosuppressive therapy (IST) with a minimum of two year-follow up






Treatments



Median follow-up

62 months

Response to IST

-  **264** Horse anti-thymocyte globulin + cyclosporine (hATG+CYA)
-  **15** Horse anti-thymocyte globulin + Tacrolimus (hATG+Tacro)
-  **15** Rabbit anti-thymocyte globulin (rATG) + Tacro or CYA
-  **19** Cyclophosphamide (CTX)
-  **1** Unspecified ATG+CYA

	All treatments	95% CI	hATG+CYA	95% CI
Complete response	60.2 %	54.5, 65.7	59.8 %	53.7, 65.8
Deep response	67.5 %	62.0, 72.7	68.2 %	62.2, 73.8
5-year overall survival	92 %	88, 95	93 %	89, 96
5-year event free survival	62 %	56, 68	64 %	57, 69