First-line azacitidine in advanced pediatric myelodysplastic syndrome leads to reduction or suppression of bone marrow blasts in seven of eight patients, has a favorable toxicity profile, and could improve event-free survival

n=22 pediatric patients with advanced myelodysplastic syndrome

diagnosed between January 2000 and December 2015 Azacitidine before Untreated before hematopoietic stem cell transplantation hematopoietic stem cell transplantation 21.4% achieved marrow remission had progressive disease 0% >50% increase in blast counts progressed • progression to leukemia Bone marrow blast decrease (p=0.02) from 15% (9-31%) to 5.5% (0-12%) Azacitidine-treated patients had significantly increased 4-year event-free survival (p=0.04) Azacitidine treatment Event-free survival No Azacitidine treatment Log-rank: 0.04

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Months from presentation