Arsenic trioxide and thalidomide combination produces multi-lineage hematological responses in myelodysplastic syndromes patients, particularly in those with high pretherapy EVI1 expression.

Leuk Res. 2004 Aug; 28(8):791-803.

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Twenty-eight myelodysplastic syndromes (MDS) patients were treated with arsenic trioxide (ATO) and thalidomide. Seven patients responded including one complete hematologic and cytogenetic response and one with regression in spleen size. Two trilineage responses were seen in patients with inv(3)(q21q26.2). Three of five patients who had high pre-therapy EVI1 levels showed unexpectedly good responses while two died early in the first cycle. In vitro studies using 32Dcl3 cells forced to express EVI1 confirmed increased sensitivity of these cells to ATO. Both low/high risk MDS may benefit significantly from therapy with ATO/thalidomide, and those with high pre-therapy EVI1 expression may be uniquely sensitive.

PMID: 15203277