

THE DISTILLERY

This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Cancer				
Myelodysplastic syndrome (MDS)	p53	Studies in cell culture and patients suggest antagonizing p53 could help treat the chromosome 5q deletion (del(5q)) subtype of MDS. Patients with MDS can develop anemia and require blood transfusions. In erythroid precursors from patients with del(5q) MDS, an antisense oligonucleotide that decreases p53 levels increased erythroid cell proliferation compared with a control oligonucleotide. In patients with Revlimid lenalidomide–resistant del(5q) MDS who are dependent on blood transfusions, Revlimid plus the p53 antagonist dexamethasone increased erythropoiesis compared with baseline and restored transfusion independence in five of eight patients. Next steps include starting a clinical trial this month in patients with lower-risk MDS. Celgene Corp. markets Revlimid to treat MDS, multiple myeloma (MM) and anemia.	Provisional patent application filed; available for licensing	Caceres, G. <i>et al. Proc. Natl. Acad. Sci.</i> <i>USA</i> ; published online Sept. 16, 2013; doi:10.1073/pnas.1311055110 Contact: Alan F. List, H. Lee Moffitt Cancer Center & Research Institute, Tampa, Fla. e-mail: alan.list@moffitt.org

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