

This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Cancer				
Acute megakaryoblastic leukemia (AMKL)	One twenty-two protein (RBM15; OTT); megakaryocytic acute leukemia (MKL1; MAL); myeloproliferative leukemia virus oncogene (MPL); recombination signal binding protein for immunoglobulin κ J region (RBPJ)	<p>Studies in mice suggest that targeting both RBPJ and MPL could treat infants with AMKL. In infants, AMKL is characterized by the expression of an OTT-MAL fusion protein. In OTT-MAL knock-in mice, all mice had abnormal hematopoiesis and abnormal activation of the cancer-associated transcription factor <i>rbpj</i>, but only those with a concomitant mutation in the <i>mpl</i> gene developed symptoms similar to human AMKL. The results indicate that both OTT-MAL activation of RBPJ and the presence of a cooperating MPL mutation are required to induce AMKL. Future studies could include testing the combined inhibition of RBPJ and MPL to treat AMKL.</p> <p>At least six companies have products marketed or approved to treat AML.</p> <p>SciBX 2(13); doi:10.1038/scibx.2009.522 Published online April 2, 2009</p>	Patent and licensing status undisclosed	<p>Mercher, T. <i>et al. J. Clin. Invest.</i>; published online March 16, 2009; doi:10.1172/JCI35901</p> <p>Contact: D. Gary Gilliland, Harvard Medical School, Boston, Mass. e-mail: ggilliland@rics.bwh.harvard.edu</p> <p>Contact: Olivier A. Bernard, Hospital Necker, Paris, France e-mail: olivier.bernard@inserm.fr</p>