

### This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
<b>Cancer</b>				
Acute myeloid leukemia (AML)	miR-155	<p>Studies in mice suggest that reducing levels of miR-155 could potentially help treat AML. Sustained miR-155 expression in bone marrow led to pathological myeloid proliferation and dysplasia in mice reconstituted with miR-155-overexpressing hematopoietic stem cells compared with controls reconstituted with normal hematopoietic stem cells. Transcriptional analysis of myeloid cells overexpressing miR-155 identified several genes implicated in hematopoiesis and disease that are regulated directly by miR-155. Preliminary human gene expression studies further revealed that miR-155 was overexpressed in a subset of AML patients. Next steps include confirming that interference with miR-155 expression <i>in vitro</i> influences AML progression and developing methods to repress miR-155 for therapeutic purposes. At least 15 compounds are in development to treat AML.</p> <p>Leukine sargramostim, a granulocyte/macrophage colony-stimulating factor (GM-CSF), is marketed in the U.S. by Bayer AG to treat AML.</p>	Patent application filed covering the research; available for licensing	<p>O'Connell, R. <i>et al. J. Exp. Med.</i>; published online Feb. 25, 2008; doi:10.1084/jem.20072108</p> <p><b>Contact:</b> David Baltimore, California Institute of Technology, Pasadena, Calif. e-mail: <a href="mailto:baltimo@caltech.edu">baltimo@caltech.edu</a></p>