

This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Neurology				
Alzheimer's disease (AD)	MicroRNA-29a; miRNA-29b-1; β -secretase (BACE1); amyloid precursor protein (APP)	<p>An analysis of cortical tissue from AD patients identified two miRNAs that might be useful as diagnostics or therapeutic targets for sporadic AD. In a subgroup of AD patients with high BACE1 levels, miRNA-29a and miRNA-29b-1 levels were lower than those in healthy controls or AD patients with normal BACE1 levels. In human embryonic kidney cells, overexpression of either miRNA downregulated BACE1 with a consequent decrease in APP terminal fragment and β-amyloid ($A\beta$), which can contribute to AD. These suppressor effects were reversed when cells were treated with anti-miRNA oligonucleotides. Next steps include developing an miRNA-29a and miRNA-29b-1 knockout mouse and vectors to target or to overexpress miRNA-29a and miRNA-29b-1 in the brains of mice.</p> <p>CTS-2116, a small molecule BACE1 inhibitor from CoMentis Inc., is in Phase I trials.</p> <p>ATG-Z1, an orally active BACE1 inhibitor also from CoMentis, is in preclinical development to treat AD.</p>	Patent application filed; available for licensing through the Vlaams Institute for Biotechnology	<p>Hébert, S. <i>et al. Proc. Natl. Acad. Sci. USA</i>; published April 21, 2008; doi:10.1073/pnas.0710263105</p> <p>Contact: Bart De Strooper, Center for Human Genetics and Department of Molecular and Developmental Genetics, Katholieke Universiteit Leuven, Leuven, Belgium</p> <p>e-mail: bart.destrooper@med.kuleuven.ac.be</p>