

This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Neurology				
Ataxia	MicroRNA-19 (miRNA-19); miRNA-101; miRNA-130; ataxin 1 (ATXN1)	Cell-culture studies suggest that enhancing the activity of miRNA-19, miRNA-101 and miRNA-130 could help treat ataxia and other polyglutamine (polyQ) diseases. Spinocerebellar ataxia type 1 is caused by high levels of polyQ-modified ATXN1. In three different human cell lines, the three miRNAs separately lowered ATXN1 levels compared with those seen using control miRNA. Next steps could include genomic analysis of diseased cells to determine whether mutations in the miRNA binding sites or the miRNA genes are associated with ataxia and other polyQ diseases. Idebenone (SNT-MC17) from Santhera Pharmaceuticals Holding AG and Takeda Pharmaceutical Co. Ltd. is marketed in the EU to treat Friedrich's ataxia.	Patent and licensing status unavailable	Lee, Y. <i>et al. Nat. Neurosci.</i> ; published online Aug. 31, 2008; doi:10.1038/nn.2183 Contact: Huda Y. Zoghbi, Department of Molecular and Human Genetics, Baylor College of Medicine, Houston, Texas e-mail: hzoghbi@bcm.tmc.edu