

### This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
<b>Neurology</b>				
Neurology	Cleavage and polyadenylation factor 1 subunit 1 (CLP1)	<p>Studies in patients and zebrafish suggest increasing <i>CLP1</i> expression could help treat neurodegenerative conditions caused by <i>CLP1</i> mutations. Exome sequencing of 2,000 families with children who had neurological disorders identified the loss-of-function R140H mutation in <i>CLP1</i> in 4 independent Turkish families. Zebrafish engineered with the <i>Clp1</i> mutation had neurodegeneration and neuromotor defects. In the engineered zebrafish, injection of wild-type human <i>CLP1</i> mRNA rescued some of the neuromotor defects, whereas injection of mutant human <i>CLP1</i> mRNA did not. Next steps could include developing strategies to increase expression of wild-type <i>CLP1</i> in human cells carrying the R140H mutation.</p> <p><b>SciBX 7(20); doi:10.1038/scibx.2014.592</b>  <b>Published online May 22, 2014</b></p>	Patent and licensing status unavailable	<p>Schaffer, A.E. <i>et al. Cell</i>; published online April 24, 2014;            doi:10.1016/j.cell.2014.03.049  <b>Contact:</b> Joseph G. Gleeson, University of California, San Diego, La Jolla, Calif.            e-mail:  <a href="mailto:jogleeson@ucsd.edu">jogleeson@ucsd.edu</a></p>