

This week in therapeutics

| Indication | Target/marker/pathway | Summary | Licensing status | Publication and contact information |
|------------------|--|---|---|--|
| Neurology | | | | |
| Ataxia | Ribosomal protein S6 kinase 90 kDa polypeptide 5 (RPS6KA5; MSK1); ataxin 1 (ATXN1) | <p>Fly and mouse studies suggest inhibiting MSK1 could help treat spinocerebellar ataxia type 1 (SCA1), which is caused by a polyglutamine expansion in <i>ATXN1</i>. Fly- and human cell-based screens identified <i>MSK1</i> as a gene in which knockdown leads to decreased levels of mutant ATXN1 and mutant ATXN1-associated toxicity. In mice, small molecule inhibitors of the Mapk pathway or MSK1 decreased Atxn1 levels compared with vehicle. In a mouse model for SCA1, a deficiency in <i>Rps6ka5</i> resulted in less neurodegeneration than no deficiency. Next steps include developing brain-permeable MSK1 inhibitors and conducting additional screens for modifiers of other proteins associated with neurodegenerative disease.</p> <p>SciBX 6(25); doi:10.1038/scibx.2013.632 Published online June 27, 2013</p> | Patent application filed; available for licensing | <p>Park, J. <i>et al. Nature</i>; published online May 29, 2013; doi:10.1038/nature12204 Contact: Huda Y. Zoghbi, Baylor College of Medicine, Houston, Texas e-mail: hzoghbi@bcm.edu</p> |