This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Cancer				
Brain cancer	Activin receptor-like kinase 2 (ALK2; ACVR1); SMAD family member 1 (MADH1; SMAD1); inhibitor of DNA binding 1 (ID1)	Three studies in primary tumors and cell culture suggest inhibiting ALK2 could help treat a subset of pediatric brain tumors. Whole-genome sequencing of diffuse intrinsic pontine gliomas and midline high-grade astrocytomas identified <i>ALK2</i> mutations in 19 of 101 tumors. In human astrocyte cell lines, the mutant <i>ALK2</i> increased cell growth, levels of activated SMAD1 and expression of <i>ID1</i> compared with wild-type <i>ALK2</i> . In cultures of primary diffuse intrinsic pontine glioma cells, a research compound that inhibited ALK2 blocked proliferation at low micromolar to high nanomolar GI_{50} values. Ongoing work includes additional <i>in vitro</i> and <i>in vivo</i> studies of mutant ALK2 function and optimization of the ALK2 inhibitor to treat diffuse intrinsic pontine glioma. SciBX 7(19); doi:10.1038/scibx.2014.551 Published online May 15, 2014	For findings from first study, ALK2 inhibitor patented by Harvard University; unlicensed Patent and licensing status unavailable for findings from second study Findings from third study unpatented; unlicensed	Taylor, K.R. <i>et al. Nat. Genet.</i> ; published online April 6, 2014; doi:10.1038/ng.2925 Contact: Chris Jones, The Institute of Cancer Research, Sutton, U.K. e-mail: chris.jones@icr.ac.uk Buczakowicz, P. <i>et al. Nat. Genet.</i> ; published online April 6, 2014; doi:10.1038/ng.2936 Contact: Cynthia Hawkins, The Hospital for Sick Children, Toronto, Ontario, Canada e-mail: cynthia.hawkins@sickkids.ca Fontebasso, A.M. <i>et al. Nat. Genet.</i> ; published online April 6, 2014; doi:10.1038/ng.2950 Contact: Nada Jabado, McGill University, Montreal, Quebec, Canada e-mail: nada.jabado@mcgill.ca