This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Autoimmune disease				
Autoimmune disease	Phosphoinositide kinase FYVE finger containing (PIKFYVE)	<p>Cell culture studies identified the molecular target of apilimod as PIKFYVE, suggesting the target could be inhibited to treat autoimmune diseases. Apilimod was previously shown to inhibit the production of IL-12 and IL-23, but its molecular target was unknown. <i>In vitro</i>, apilimod inhibited PIKFYVE kinase activity with an IC₅₀ value of 14 nM. In cell culture, small hairpin RNA-mediated knockdown of PIKFYVE decreased expression of the p40 subunit of IL-12 compared with no knockdown. Next steps could include designing and optimizing PIKFYVE inhibitors.</p> <p>Synta Pharmaceuticals Corp. discontinued development of apilimod in 2009 after the compound missed the primary endpoints in multiple Phase II trials. The company has an IL-12 and IL-23 inhibitor program in preclinical development for autoimmune and inflammatory disease.</p> <p>Novartis AG, which ran the current cell culture studies, did not disclose if it is developing inhibitors of PIKFYVE.</p> <p>SciBX 6(33); doi:10.1038/scibx.2013.884 Published online Aug. 29, 2013</p>	Unpatented; licensing status not applicable	<p>Cai, X. <i>et al. Chem. Biol.</i>; published online July 25, 2013; doi:10.1016/j.chembiol.2013.05.010 Contact: Qian Huang, Novartis Institutes for BioMedical Research, Cambridge, Mass. e-mail: qian.huang@novartis.com</p>