

THE DISTILLERY

This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Cancer				
Neurofibromatosis	p21 protein (Cdc42 Rac)-activated kinase 1 (PAK1)	<i>In vitro</i> and mouse studies identified PAK1 inhibitors that could help treat neurofibromatosis type 2 (NF2). PAK1 is highly expressed in Schwann cells in primary NF2 tumors. High throughput screening, chemistry and SAR studies identified a pyridopyrimidinone as a nanomolar-potent inhibitor of PAK1. In a Schwann cell–based model of NF2, the compound decreased proliferation compared with vehicle. In mice bearing orthotopic NF2 tumors, the compound decreased tumor growth. Ongoing work at the Genentech Inc. unit of Roche includes further optimizing the compound.	Patented by the Massachusetts Institute of Technology; licensed to Genentech	Licciulli, S. <i>et al. J. Biol. Chem.</i> ; published online Aug. 19, 2013; doi:10.1074/jbc.M113.510933 Contact: Joseph L. Kissil, Scripps Florida Jupiter, Fla. e-mail: jkissil@scripps.edu

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