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THE DISTILLERY

This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Cancer				
Pancreatic cancer; neuroendocrine tumors	Multiple endocrine neoplasia I (MEN1; menin); death-domain associated protein (DAXX); α-thalassemia/mental retardation syndrome X-linked (ATRX); mammalian target of rapamycin (mTOR; FRAP; RAFT1)	Genomic studies identified mutations that could help guide treatment of pancreatic neuroendocrine tumors. In pancreatic neuroendocrine tumors isolated from patients, mutation rates for the *MEN1*, *DAXX* and *ATRX* genes were 44%, 25% and 18%, respectively. Also, components of the mTOR pathway had a 14% mutation rate. In patients with pancreatic neuroendocrine tumors, *MEN1* mutations together with *ATRX* or *DAXX* mutations correlated with 100% survival for at least 10 years compared with 60% in patients lacking the mutations. Next steps include validating the usefulness of the genetic alterations as prognostic markers in a larger set of patients and a possible clinical trial to correlate response to mTOR inhibitors with gene mutation status. Afinitor everolimus, an oral mTOR inhibitor from Novartis AG, is marketed for a number of cancer and transplant indications and is in registration to treat pancreatic neuroendocrine tumors. *SciBX* 4(8); doi:10.1038/scibx.2011.224 Published online Feb. 24, 2011	Patent and licensing status undisclosed	Jiao, Y. *et al. Science*; published online Jan. 20, 2011; doi:10.1126/science.1200609 **Contact:** Nickolas Papadopoulos, The Sydney Kimmel Comprehensive Cancer Center at Johns Hopkins, Baltimore, Md. e-mail: npapado1@jhmi.edu **Contact:** Ralph H. Hruban, same affiliation as above e-mail: rhruban@jhmi.edu **Contact:** Kenneth W. Kinzler, same affiliation as above e-mail: kinzlke@jhmi.edu