

This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Renal disease				
Polycystic kidney disease (PKD)	Cystic fibrosis transmembrane conductance regulator (CFTR)	<p>A screen study identified a pyrimido-pyrrolo-quinoxalinedione CFTR inhibitor that could help treat PKD. In a mouse embryonic kidney culture model of PKD, the inhibitor, dubbed PPQ-102, produced dose-dependent reductions in the number and size of renal cysts compared with no inhibitor ($p < 0.001$). PPQ-102 inhibited the CFTR chloride current with an IC_{50} value of about 90 nM. Next steps include evaluating the CFTR inhibitors in animal models of PKD.</p> <p>Samsca tolvaptan, a vasopressin 2 (V2) receptor antagonist from Otsuka Pharmaceutical Co. Ltd., is in Phase II testing for PKD. The compound is approved to treat hyponatremia.</p> <p>PLX5568, a Raf kinase inhibitor from Plexxikon Inc. and Roche, is in Phase I testing for PKD.</p> <p>SciBX 2(39); doi:10.1038/scibx.2009.1484 Published online Oct. 8, 2009</p>	Patent application filed; licensing status undisclosed	<p>Tradtrantip, L. <i>et al. J. Med. Chem.</i>; published online Sept. 28, 2009; doi:10.1021/jm9009873</p> <p>Contact: A.S. Verkman, University of California, San Francisco, Calif. e-mail: alan.verkman@ucsf.edu</p>