

### This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
<b>Renal disease</b>				
Polycystic kidney disease (PKD)	Glucosylceramide synthase (GCS)	<p>Studies in human tissue samples and in mice suggest that inhibiting GCS could help treat PKD. In kidney tissue samples from PKD patients and in three mouse models of PKD, levels of the glycosphingolipid glucosylceramide were higher than those in normal tissues. In the mice, the GCS inhibitor Genz-123346 reduced kidney levels of glucosylceramide and the formation of kidney cysts compared with no treatment. Ongoing work includes investigating the role of other glycosphingolipids in PKD progression.</p> <p>Genzyme Corp.'s eliglustat tartrate (Genz-112638), a ceramide analog that inhibits GCS, is in Phase III testing to treat Gaucher's disease.</p> <p>Otsuka Pharmaceutical Co. Ltd.'s tolvaptan (OPC-41061), a vasopressin 2 (V2) receptor antagonist, is in Phase III testing to treat PKD.</p> <p>PLX5568 (R7376), a Raf kinase inhibitor from Plexxikon Inc. and Roche, is in Phase I testing to treat PKD.</p> <p><b>SciBX 3(25); doi:10.1038/scibx.2010.778</b> Published online June 24, 2010</p>	Patent and licensing status undisclosed	<p>Natoli, T.A. <i>et al. Nat. Med.</i>; published online June 20, 2010; doi:10.1038/nm.2171</p> <p><b>Contact:</b> Oxana Ibraghimov-Beskrovnaya, Genzyme Corp., Framingham, Mass. e-mail: <a href="mailto:oxana.beskrovnaya@genzyme.com">oxana.beskrovnaya@genzyme.com</a></p>