This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Endocrine/metabolic disease				
Metabolic disease	ATP-binding cassette sub-family C member 6 (ABCC6; MRP6; PXE)	*In vitro* and mouse studies suggest upregulating plasma levels of pyrophosphate could help treat pseudoxanthoma elasticum (PXE). PXE is an inherited genetic disorder caused by *ABCC6* mutations, which lead to mineralization of the skin, eyes and arteries. In culture medium from a human cell line engineered to express wild-type *ABCC6*, levels of the ATP hydrolysis product pyrophosphate were higher than those in medium from cells expressing mutant *ABCC6*. In *Abcc6* knockout mouse models of PXE, plasma pyrophosphate levels were up to 5.6-fold lower than those in unaltered mice. Ongoing work includes investigating the mechanism by which mutant *ABCC6* leads to reduced plasma levels of pyrophosphate. *SciBX* 7(1); doi:10.1038/scibx.2014.18 Published online Jan. 9, 2014	Unpatented; unlicensed	Jansen, R.S. *et al. Proc. Natl. Acad. Sci. USA*; published online Nov. 25, 2013; doi:10.1073/pnas.1319582110 **Contact:** Koen van de Wetering, The Netherlands Cancer Institute, Amsterdam, the Netherlands e-mail: k.vd.wetering@nki.nl **Contact:** Piet Borst, same affiliation as above e-mail: p.borst@nki.nl