This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Cardiovascular disease				
Cerebral cavernous malformation (CCM)	KRIT1 ankyrin repeat containing (KRIT1; CCM1); cerebral cavernous malformation 2 (CCM2); programmed cell death 10 (PDCD10; CCM3)	*In vitro* and mouse studies suggest that antiangiogenic agents could help treat CCM, which is caused by mutations in the *CCM1*, *CCM2* or *CCM3* genes that lead to the formation of highly dilated blood vessels and symptoms including headaches, seizures and hemorrhage. Mice receiving *CCM1*-silenced human endothelial cell transplants had vascular abnormalities similar to those seen in patients with CCM. In those mice, Nexavar sorafenib reduced the formation of abnormal blood vessels compared with vehicle control. Planned work includes testing other antiangiogenic compounds in mice receiving *CCM1*-silenced cell transplants. Onyx Pharmaceuticals Inc. and Bayer AG market Nexavar sorafenib (BAY 43-9006), an inhibitor of VEGF, CRAF (RAF1), and multiple receptor tyrosine kinases, to treat liver and renal cancer.	Unpatented; unlicensed	Wüstehube, J. *et al. Proc. Natl. Acad. Sci. USA*; published online June 21, 2010; doi:10.1073/pnas.1000132107 **Contact:** Andreas Fischer, Heidelberg University, Heidelberg, Germany e-mail: andreas.fischer@medma.uniheidelberg.de

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