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THE DISTILLERY

This week in therapeutics

Indication	Target/marker/ pathway	Summary	Licensing status	Publication and contact information
Cardiovascular disease				
Cerebral cavernous malformation (CCM); vascular dysplasia	Cerebral cavernous malformation 2 (CCM2); ras homolog gene family, member A (RHOA); HMG-CoA reductase (HMGCR)	A study in mice suggests that statins may be useful for treating CCM-associated vascular dysplasias. Humans with heterozygous loss-of-function mutations in *CCM2* have CCM and are prone to vascular hemorrhage and leakage. In *CCM2* heterozygous knockout mice, simvastatin—an HMGCR inhibitor that is also known to inhibit Rho GTPases—significantly lowered VEGF-associated vascular permeability compared with that seen using vehicle control ($p<0.01$). However, simvastatin did not affect VEGF-associated vascular permeability in wild-type mice. In two human endothelial cell lines, small interfering RNA–mediated depletion of CCM2 resulted in CCM-like cellular pathologies and increased activation of the RHOA GTPase compared with what was seen in nondepleted controls. Next steps include a clinical trial to evaluate the effect of marketed statins in patients with CCM. Zocor simvastatin, a HMGCR inhibitor from Merck & Co. Inc., is marketed to treat acute coronary syndrome (ACS), dyslipidemia, hypercholesterolemia and hyperlipidemia. At least eight other companies market HMGCR reductase inhibitors to treat metabolic and cardiovascular diseases. ***SciBX*** **2(4); doi:10.1038/scibx.2009.140** **Published online Jan. 29, 2009**	Patent pending covering findings; available for licensing from The University of Utah Technology Commercialization Office **Contact:** The University of Utah Technology Commercialization Office, Salt Lake City, Utah phone: 801-581-7792 e-mail: info@tco.utah.edu	Whitehead, K.J. *et al. Nat. Med.*; published online Jan. 18, 2009; doi:10.1038/nm.1911 **Contact:** Dean Y. Li, University of Utah, Salt Lake City, Utah e-mail: dean.li@hmbg.utah.edu **Contact:** Kevin J. Whitehead, same affiliation as above e-mail: kevin.whitehead@hsc.utah.edu