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THE DISTILLERY

This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Cardiovascular disease				
Cardiovascular disease	Apolipoprotein C-III (APOCIII; APOC3)	Human sample studies suggest *APOC3* mutations could help predict the risk of developing cardiovascular disease and that the gene may be a therapeutic target. In a cohort of 3,734 individuals, loss-of-function mutations in *APOC3* correlated with ~40% lower plasma triglyceride levels and 40% reduced risk of coronary heart disease compared with wild-type *APOC3*. In a cohort of 75,725 individuals, nonfasting triglyceride levels <1.0 mM correlated with a decreased incidence of cardiovascular disease compared with levels >4.0 mM. In the same cohort, a heterozygous, loss-of-function mutation in *APOC3* was associated with reduced triglyceride levels. Next steps could include testing whether lowering APOC3 or triglyceride levels can lower risk for cardiovascular disease. Isis Pharmaceuticals Inc. has ISIS-APOCIIIRx, an antisense inhibitor of *APOC3*, in Phase II testing to treat hypertriglyceridemia.	Patent and licensing status unknown for findings from both studies	TG and HDL Working Group of the Exome Sequencing Project, National Heart, Lung, and Blood Institute. *N. Engl. J. Med.*; published online June 18, 2014; doi:10.1056/NEJMoa1307095 **Contact:** Sekar Kathiresan, Massachusetts General Hospital, Boston, Mass. e-mail: skathiresan@partners.org Jørgensen, A.B. *et al. N. Engl. J. Med.*; published online June 18, 2014; doi:10.1056/NEJMoa1308027 **Contact:** Anne Tybjærg-Hansen, Copenhagen University Hospital, Copenhagen, Denmark e-mail: anne.tybjaerg.hansen@regionh.dk

SciBX 7(30); doi:10.1038/scibx.2014.892
Published online Aug. 7, 2014