

### This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
<b>Ophthalmic disease</b>				
Age-related macular degeneration (AMD)	<i>Complement factor H (CFH)</i>	<p>A genotyping study identified a loss-of-function mutation in <i>CFH</i> that could help predict AMD risk. In a cohort of 2,423 patients and 1,121 controls, an arginine-to-cysteine loss-of-function mutation in <i>CFH</i> was significantly associated with the development of AMD and early disease onset (<math>p=7\times 10^{-6}</math> and <math>p=2.3\times 10^{-6}</math>, respectively). Next steps include conducting studies to determine how the loss-of-function mutation leads to the development of AMD.</p> <p>Eculizumab, an intravitreal formulation of a humanized mAb targeting complement 5 (C5) from Alexion Pharmaceuticals Inc., is in Phase II testing to treat AMD.</p> <p>At least four other companies have compounds targeting the complement system in Phase I or earlier to treat AMD.</p> <p><b>SciBX 4(43); doi:10.1038/scibx.2011.1217</b>  <b>Published online Nov. 3, 2011</b></p>	Work unpatented; unavailable for licensing	<p>Raychaudhuri, S. <i>et al. Nat. Genet.</i>; published online Oct. 23, 2011; doi:10.1038/ng.976</p> <p><b>Contact:</b> Johanna M. Seddon, Tufts Medical Center, Boston, Mass.  e-mail: <a href="mailto:jseddon@tuftsmedicalcenter.org">jseddon@tuftsmedicalcenter.org</a></p> <p><b>Contact:</b> Soumya Raychaudhuri, Brigham and Women's Hospital, Boston, Mass.  e-mail: <a href="mailto:soumya@broadinstitute.org">soumya@broadinstitute.org</a></p>