

This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Hematology				
Paroxysmal nocturnal hemoglobinuria (PNH)	Complement 5 (C5)	<p>Genetic sequencing studies identified a C5 mutation that could help predict response to Soliris eculizumab. In 345 Japanese patients with PNH, a substitution of C5 Arg885 for glycine was identified in all 11 patients who had a poor response to Soliris. In C5-depleted serum reconstituted with recombinant C5 proteins, Soliris blocked the activity of wild-type C5 but not the mutant, whereas an alternative C5 antibody that binds at a distinct site blocked activity of both wild-type and mutant C5. Next steps could include validating the finding in additional patients.</p> <p>Alexion Pharmaceuticals Inc. markets the C5 mAb Soliris to treat PNH and hemolytic uremic syndrome. At least four other companies have therapeutics targeting C5 in Phase II or earlier testing.</p> <p>SciBX 7(12); doi:10.1038/scibx.2014.347 Published online March 27, 2014</p>	Unpatented; unavailable for licensing	<p>Nishimura, J. <i>et al. N. Eng. J. Med.</i>; published online Feb. 13, 2014; doi:10.1056/NEJMoa1311084</p> <p>Contact: Jun-ichi Nishimura, Osaka University Graduate School of Medicine, Osaka, Japan e-mail: junnishi@bldon.med.osaka-u.ac.jp</p>