SciBX
Science-Business eXchange

THE DISTILLERY

This week in therapeutics

Indication	Target/marker/pathway	Summary	Licensing status	Publication and contact information
Hematology				
Myeloproliferative disorder	*Calreticulin* (*CALR*)	Genetic studies suggest neutralizing *CALR* mutations associated with myeloproliferative neoplasms could help treat the disease. Mutations in *Janus kinase-2* (*JAK-2*) and other genes cause the majority of myeloproliferative neoplasms, but genetic causes for about 30%–45% of cases are unknown. In the first study, exome sequencing identified frameshift mutations in *CALR* that altered the C-terminal peptide in all six patients lacking known mutations. The *CALR* mutations were confirmed in 67% of patients with thrombocythemia and 88% of patients with myelofibrosis in a validation cohort. In the second study, exome sequencing identified *CALR* mutations in 70%–84% of samples from 151 patients with myeloproliferative neoplasms that lacked *JAK-2* mutations but not in patients with other cancers. In mouse B cells, expression of the most common Calr mutant increased cell proliferation compared with wild-type Calr expression. Next steps include designing mAbs targeting the new C-terminal peptide sequence of mutant CALR. Authors from the first study plan to start a company to develop anti-CALR antibodies. *SciBX* 7(3); doi:10.1038/scibx.2014.89 Published online Jan. 23, 2014	For findings in first study, patent application filed for diagnostic applications and for mutant CALR as a therapeutic target; diagnostic applications available for licensing Patent and licensing status unavailable for findings in second study	Klampfl, T. *et al. N. Eng. J. Med.*; published online Dec. 10, 2013; doi:10.1056/NEJMoa1311347 **Contact:** Robert Kralovics, Research Center for Molecular Medicine of the Austrian Academy of Sciences, Vienna, Austria e-mail: robert.kralovics@cemm.oeaw.ac.at Nangalia, J. *et al. N. Eng. J. Med.*; published online Dec. 10, 2013; doi:10.1056/NEJMoa1312542 **Contact:** Anthony R. Green, Cambridge Institute for Medical Research, Cambridge, U.K. e-mail: arg1000@cam.ac.uk