

THE DISTILLERY

This week in techniques

Approach	Summary	Licensing status	Publication and contact information
Disease models			
Macrophage model of Gaucher's disease	A macrophage model of Gaucher's disease could help identify new therapies to treat the condition, which is caused by a genetic deficiency in glucocerebrosidase (GBA; GCase). Induced pluripotent stem (iPS) cells and monocytes derived from patients with Gaucher's disease or healthy subjects were used to generate macrophages. Patient-derived macrophages showed impaired bacteria-induced reactive oxygen species production, decreased GBA activity and increased lysosomal storage of GBA substrates compared with macrophages generated from healthy subjects. In the patient-derived macrophages, a chaperone protein that restores mutant GBA folding increased GBA activity with potency comparable to that of recombinant GBA. Next steps could include developing screening assays that use the macrophages to identify new therapeutic candidates.	Patent and licensing status unavailable	Aflaki, E. <i>et al. Sci. Transl. Med.</i> ; published online June 11, 2014; doi:10.1126/scitranslmed.3008659 Contact: Ellen Sidransky, National Institutes of Health, Bethesda, Md. e-mail: sidranse@mail.nih.gov

SciBX 7(27); doi:10.1038/scibx.2014.808 Published online July 17, 2014