



## This week in techniques

| Approach  | Summary   | Licensing status  | Publication and contact information  |
|---|---|---|--|
| Disease models  |   |   |  |
| Intestinal organoid<br>model for cystic<br>fibrosis transmembrane<br>conductance regulator<br>(CFTR) function | An intestinal organoid model for CFTR function could help develop compounds to treat cystic fibrosis. Organoids were cultured from human intestinal stem cells and treated with forskolin, which activates CFTR and induces fluid influx and swelling. In organoids cultured from patients carrying mutant CFTR variants, forskolin-induced swelling was lower than that in patients carrying wild-type CFTR. In ΔF508 mutant CFTR–expressing organoids, compounds that increased CFTR function also increased forskolin-induced swelling compared with vehicle. Next steps include using the organoids to model patient response to CFTR-targeted drugs.  Vertex Pharmaceuticals Inc.'s VX-809, a CFTR corrector, is in Phase III trials to treat ΔF508 mutant cystic fibrosis (CF) in combination with the CFTR potentiator Kalydeco ivacaftor (VX-770). Vertex markets Kalydeco to treat CF. | Patent application<br>filed; available for<br>licensing | Dekkers, J.F. et al. Nat. Med.; published online June 2, 2013; doi:10.1038/nm.3201 Contact: Jeffrey M. Beekman, University Medical Center Utrecht, Utrecht, the Netherlands e-mail: jbeekman@umcutrecht.nl |
|   | SciBX 6(26); doi:10.1038/scibx.2013.669<br>Published online July 11, 2013   |   |  |