

THE DISTILLERY

This week in techniques

| Approach | Summary | Licensing status | Publication and contact information |
|-------------------------------------|--|---|---|
| Disease models | | | |
| Cellular model for Down syndrome | A cellular model for trisomy 21 Down syndrome could be useful for studying disease pathophysiology and could help guide the development of new therapeutics. In induced pluripotent stem (iPS) cells derived from a patient with Down syndrome, designed zinc finger nucleases were used to insert inducible <i>X inactive specific transcript (XIST)</i> into chromosome 21. In six clones, induced transgene expression resulted in <i>XIST</i> coating the extra chromosome 21 and triggering chromosome inactivation. In the patient-derived iPS cells, neural differentiation of those with induced <i>XIST</i> expression resulted in normal neural rosette formation, whereas differentiation of cells without induced <i>XIST</i> expression resulted in delayed neural rosette formation. Next steps could include characterizing gene expression and signaling pathway differences between neural progenitor cells from the two different neural rosette populations. | Patent and licensing status unavailable | Jiang, J. <i>et al. Nature</i> ; published online July 17, 2013; doi:10.1038/nature12394 Contact: Jeanne B. Lawrence, University of Massachusetts Medical School, Worcester, Mass. e-mail: jeanne.lawrence@umassmed.edu |

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