This week in techniques

Approach	Summary	Licensing status	Publication and contact information
Assays & screens			
Diagnosing and monitoring treatment responses in patients with Niemann-Pick disease type C1 (NPC1) using fluorescence-based volumetric measurement of lysosomes	A fluorescence-based assay in B cells could help monitor treatment responses and diagnose patients with NPC1. The assay measured uptake of a fluorescent probe by the acidic compartment of lysosomes to determine the volume of that compartment relative to total cell volume. In B cells from pediatric patients with NPC1, the method identified positive correlations between the relative compartment volume and NPC1 disease severity. In B cells from patients with NPC1 receiving Zavesca miglustat or bone marrow transplantation, the method identified a correlation between decreased relative compartment volume and treatment response. Future studies could include testing the assay in patients with other lysosomal storage disorders. Actelion Ltd.'s and UCB Group's Zavesca, a glucosylceramide synthase (GCS) inhibitor, is marketed to treat Gaucher's disease and Niemann-Pick disease.	Patent and licensing status unavailable	te Vruchte, D. *et al. J. Clin. Invest.*; published online Feb. 3, 2014; doi:10.1172/JCI72835 **Contact:** Frances M. Platt, University of Oxford, Oxford, U.K. e-mail: frances.platt@pharm.ox.ac.uk **Contact:** Mario Cortina-Borja, University College London, London, U.K. e-mail: m.cortina@ucl.ac.uk

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