

References

1. Mellman, I. Endocytosis and molecular sorting. *Annu Rev Cell Dev Biol* **1996**, *12*, 575-625.
2. Conner, S. D.; Schmid, S. L. Regulated portals of entry into the cell. *Nature* **2003**, *422*, (6927), 37-44.
3. Marsh, M.; McMahon, H. T. The structural era of endocytosis. *Science* **1999**, *285*, (5425), 215-20.
4. Parkar, N. S.; Akpa, B. S.; Nitsche, L. C.; Wedgewood, L. E.; Place, A. T.; Sverdllov, M. S.; Chaga, O.; Minshall, R. D. Vesicle formation and endocytosis: function, machinery, mechanisms, and modeling. *Antioxid Redox Signal* **2009**, *11*, (6), 1301-12.
5. McMahon, H. T.; Boucrot, E. Molecular mechanism and physiological functions of clathrin-mediated endocytosis. *Nat Rev Mol Cell Biol* **2011**, *12*, (8), 517-33.
6. Mousavi, S. A.; Malerod, L.; Berg, T.; Kjekken, R. Clathrin-dependent endocytosis. *Biochem J* **2004**, *377*, (Pt 1), 1-16.
7. Muro, S. Challenges in design and characterization of ligand-targeted drug delivery systems. *J Control Release* **2012**, *164*, (2), 125-37.
8. Futerman, A. H.; van Meer, G. The cell biology of lysosomal storage disorders. *Nat Rev Mol Cell Biol* **2004**, *5*, (7), 554-65.
9. Desnick, R. J.; Schuchman, E. H. Enzyme replacement and enhancement therapies: lessons from lysosomal disorders. *Nat Rev Genet* **2002**, *3*, (12), 954-66.
10. Beck, M. New therapeutic options for lysosomal storage disorders: enzyme replacement, small molecules and gene therapy. *Hum Genet* **2007**, *121*, (1), 1-22.
11. Neufeld, E. F. The uptake of enzymes into lysosomes: an overview. *Birth Defects Orig Artic Ser* **1980**, *16*, (1), 77-84.
12. Muro, S. Strategies for Delivery of Therapeutics Into the Central Nervous System for Treatment of Lysosomal Storage Disorders. *Drug Delivery and Translational Research* **2012**, *2*, 169-186.
13. Simons, K.; Gruenberg, J. Jamming the endosomal system: lipid rafts and lysosomal storage diseases. *Trends Cell Biol* **2000**, *10*, (11), 459-62.
14. Fraldi, A.; Annunziata, F.; Lombardi, A.; Kaiser, H. J.; Medina, D. L.; Spampanato, C.; Fedele, A. O.; Polishchuk, R.; Sorrentino, N. C.; Simons, K.; Ballabio, A. Lysosomal fusion and SNARE function are impaired by cholesterol accumulation in lysosomal storage disorders. *Embo j* **2010**, *29*, (21), 3607-20.
15. Ballabio, A.; Gieselmann, V. Lysosomal disorders: from storage to cellular damage. *Biochim Biophys Acta* **2009**, *1793*, (4), 684-96.
16. Fukuda, T.; Ewan, L.; Bauer, M.; Mattaliano, R. J.; Zaal, K.; Ralston, E.; Plotz, P. H.; Raben, N. Dysfunction of endocytic and autophagic pathways in a lysosomal storage disease. *Ann Neurol* **2006**, *59*, (4), 700-8.
17. Marks, D. L.; Pagano, R. E. Endocytosis and sorting of glycosphingolipids in sphingolipid storage disease. *Trends Cell Biol* **2002**, *12*, (12), 605-13.
18. Takahashi, M.; Kobayashi, T. Cholesterol regulation of rab-mediated sphingolipid endocytosis. *Glycoconj J* **2009**, *26*, (6), 705-10.
19. Dhami, R.; Schuchman, E. H. Mannose 6-phosphate receptor-mediated uptake is defective in acid sphingomyelinase-deficient macrophages: implications for Niemann-Pick disease enzyme replacement therapy. *J Biol Chem* **2004**, *279*, (2), 1526-32.

20. Puri, V.; Watanabe, R.; Dominguez, M.; Sun, X.; Wheatley, C. L.; Marks, D. L.; Pagano, R. E. Cholesterol modulates membrane traffic along the endocytic pathway in sphingolipid storage diseases. *Nat Cell Biol* **1999**, *1*, (6), 386-8.
21. Hsu, J.; Northrup, L.; Bhowmick, T.; Muro, S. Enhanced delivery of α -glucosidase for Pompe disease by ICAM-1-targeted nanocarriers: comparative performance of a strategy for three distinct lysosomal storage disorders. *Nanomedicine* **2012**, *8*, (5), 731-9.
22. Muro, S.; Schuchman, E. H.; Muzykantov, V. R. Lysosomal enzyme delivery by ICAM-1-targeted nanocarriers bypassing glycosylation- and clathrin-dependent endocytosis. *Mol Ther* **2006**, *13*, (1), 135-41.
23. Muro, S.; Wiewrodt, R.; Thomas, A.; Koniaris, L.; Albelda, S. M.; Muzykantov, V. R.; Koval, M. A novel endocytic pathway induced by clustering endothelial ICAM-1 or PECAM-1. *J Cell Sci* **2003**, *116*, (Pt 8), 1599-609.
24. Garnacho, C.; Dhami, R.; Simone, E.; Dziubla, T.; Leferovich, J.; Schuchman, E. H.; Muzykantov, V.; Muro, S. Delivery of acid sphingomyelinase in normal and niemann-pick disease mice using intercellular adhesion molecule-1-targeted polymer nanocarriers. *J Pharmacol Exp Ther* **2008**, *325*, (2), 400-8.
25. Hsu, J.; Serrano, D.; Bhowmick, T.; Kumar, K.; Shen, Y.; Kuo, Y. C.; Garnacho, C.; Muro, S. Enhanced Endothelial Delivery and Biochemical Effects of α -Galactosidase by ICAM-1-Targeted Nanocarriers for Fabry Disease. *J Control Release* **2011**, *149*, (3), 323-31.
26. Papademetriou, J.; Garnacho, C.; Serrano, D.; Bhowmick, T.; Schuchman, E. H.; Muro, S. Comparative binding, endocytosis, and biodistribution of antibodies and antibody-coated carriers for targeted delivery of lysosomal enzymes to ICAM-1 versus transferrin receptor. *J Inherit Metab Dis* **2013**, *36*, (3), 467-77.
27. Muro, S., Intercellular Adhesion Molecule-1 and Vascular Cell Adhesion Molecule-1. In *Endothelial Biomedicine*, Aird, W., Ed. Cambridge University Press: New York, 2007; pp 1058-70.
28. Muro, S.; Garnacho, C.; Champion, J. A.; Leferovich, J.; Gajewski, C.; Schuchman, E. H.; Mitragotri, S.; Muzykantov, V. R. Control of Endothelial Targeting and Intracellular Delivery of Therapeutic Enzymes by Modulating the Size and Shape of ICAM-1-targeted Carriers. *Mol Ther* **2008**, *16*, (8), 1450-8.
29. Hsu, J.; Rappaport, J.; Muro, S. Specific Binding, Uptake, and Transport of ICAM-1-Targeted Nanocarriers Across Endothelial and Subendothelial Components of the Blood Brain Barrier. *Pharmaceutical Research* **in press**.
30. Schuchman, E.; Desnick, R., Niemann-Pick disease types A and B: acid sphingomyelinase deficiencies. In *The Metabolic and Molecular Bases of Inherited Disease*, 8 ed.; Scriver, C.; Beaudet, A.; Sly, W.; Valle, D.; Childs, B.; Kinzler, K.; Vogelstein, B., Eds. McGraw-Hill: New York, 2000; pp 3589-3610.
31. Bareford, L. M.; Swaan, P. W. Endocytic mechanisms for targeted drug delivery. *Adv Drug Deliv Rev* **2007**, *59*, (8), 748-58.
32. Doherty, G. J.; McMahon, H. T. Mechanisms of endocytosis. *Annu Rev Biochem* **2009**, *78*, 857-902.
33. Otomo, T.; Higaki, K.; Nanba, E.; Ozono, K.; Sakai, N. Lysosomal storage causes cellular dysfunction in mucopolipidosis II skin fibroblasts. *J Biol Chem* **2011**, *286*, (40), 35283-90.
34. Cardone, M.; Porto, C.; Tarallo, A.; Vicinanza, M.; Rossi, B.; Polishchuk, E.; Donaudy, F.; Andria, G.; De Matteis, M. A.; Parenti, G. Abnormal mannose-6-phosphate receptor

trafficking impairs recombinant alpha-glucosidase uptake in Pompe disease fibroblasts. *Pathogenetics* **2008**, *1*, (1), 6.

35. Qian, Z. M.; Li, H.; Sun, H.; Ho, K. Targeted drug delivery via the transferrin receptor-mediated endocytosis pathway. *Pharmacol Rev* **2002**, *54*, (4), 561-87.

36. Traub, L. M.; Bannykh, S. I.; Rodel, J. E.; Aridor, M.; Balch, W. E.; Kornfeld, S. AP-2-containing clathrin coats assemble on mature lysosomes. *J Cell Biol* **1996**, *135*, (6 Pt 2), 1801-14.

37. Rong, Y.; Liu, M.; Ma, L.; Du, W.; Zhang, H.; Tian, Y.; Cao, Z.; Li, Y.; Ren, H.; Zhang, C.; Li, L.; Chen, S.; Xi, J.; Yu, L. Clathrin and phosphatidylinositol-4,5-bisphosphate regulate autophagic lysosome reformation. *Nat Cell Biol* **2012**, *14*, (9), 924-34.

38. Lieberman, A. P.; Puertollano, R.; Raben, N.; Slaugenhaupt, S.; Walkley, S. U.; Ballabio, A. Autophagy in lysosomal storage disorders. *Autophagy* **2012**, *8*, (5), 719-30.

39. Klein, D.; Yaghoofam, A.; Matzner, U.; Koch, B.; Bräulke, T.; Gieselmann, V. Mannose 6-phosphate receptor-dependent endocytosis of lysosomal enzymes is increased in sulfatide-storing kidney cells. *Biol Chem* **2009**, *390*, (1), 41-8.

40. Muro, S.; Cui, X.; Gajewski, C.; Murciano, J. C.; Muzykantov, V. R.; Koval, M. Slow intracellular trafficking of catalase nanoparticles targeted to ICAM-1 protects endothelial cells from oxidative stress. *Am J Physiol Cell Physiol* **2003**, *285*, (5), C1339-47.

41. Liscum, L.; Faust, J. R. Low density lipoprotein (LDL)-mediated suppression of cholesterol synthesis and LDL uptake is defective in Niemann-Pick type C fibroblasts. *J Biol Chem* **1987**, *262*, (35), 17002-8.

42. Hortsch, R.; Lee, E.; Erathodiyil, N.; Hebbar, S.; Steinert, S.; Lee, J. Y.; Chua, D. S.; Kraut, R. Glycolipid trafficking in *Drosophila* undergoes pathway switching in response to aberrant cholesterol levels. *Mol Biol Cell* **2010**, *21*, (5), 778-90.