This week in techniques

Approach	Summary	Licensing status	Publication and contact information
Drug delivery			
Adeno-associated virus vectors (AAV vectors) for effective delivery of larger genes	A modified AAV vector could potentially incorporate up to twice as much genetic material as can conventional AAV vectors and thus could be useful for treating diseases caused by mutations in large genes. A recombinant AAV vector with an AAV5 capsid was able to package an expression cassette containing up to 8.9 kb of genome. Efficient packaging occurred whether the genome included murine *Abca4*, human *MYO7A* or human *CEP290*—all genes that are implicated in diseases involving blindness. Intraocular administration of a vector encoding *Abca4* into Abca$^{-/-}$ mice significantly improved retinal morphology and function compared with what is seen in wild-type mice. Next steps include clinical testing of AAV vector–based large gene delivery to treat macular degeneration. No fewer than eight companies have AAV vectors for multiple indications in developmental stages ranging from preclinical to Phase II.	U.S. patent applications filed for AAV packaging of large genes to treat Stargardt's disease, Usher syndrome type IB and Leber congenital amaurosis due to *CEP290* mutations; unlicensed	Allocca, M. *et al. J. Clin. Invest.*; published online April 15, 2008; doi:10.1172/JCI34316 **Contact:** Alberto Auricchio, Telethon Institute of Genetics and Medicine, Naples, Italy e-mail: auricchio@tigem.it