PublisherInfo		
PublisherName	:	BioMed Central
PublisherLocation	:	London
PublisherImprintName	:	BioMed Central

Targeting human transgenes

ArticleInfo		
ArticleID	:	4519
ArticleDOI	:	10.1186/gb-spotlight-20020702-01
ArticleCitationID	:	spotlight-20020702-01
ArticleSequenceNumber	:	185
ArticleCategory	:	Research news
ArticleFirstPage	:	1
ArticleLastPage	:	2
ArticleHistory	:	RegistrationDate : 2002–7–2 OnlineDate : 2002–7–2
ArticleCopyright	:	BioMed Central Ltd2002
ArticleGrants	:	
ArticleContext	:	130593311

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Adeno-associated viruses (AAVs) are single-stranded DNA vectors that have shown promise as gene-targeting vectors for experimental and therapeutic applications. In the July issue of Nature Biotechnology, Roli Hirata and colleagues at the University of Washington, Seattle, describe a way of using AAV to introduce a functional transgene cassette into defined genomic loci in human cells in culture (*Nature Biotechnology* 2002, **20:**735-738). The AAV vectors can deliver gene cassettes of up to 1.5 kb. Hirata *et al.* designed an AAV vector containing a selectable neomycin cassette within the hypoxanthine phosphoribosyl transferase (*HPRT*) gene. They then infected diploid male human fibroblasts, or HT1080 fibrosarcoma cells, and selected for neomycin resistance and functional HPRT expression. The targeting efficiency was as high as 1% of the total cell population. Hirata *et al.* also used a similar strategy to disrupt the autosomal type I collagen (*COL1A1*) gene in human fibroblasts. The high efficiency and accuracy of this procedure provides an effective tool for experimental and therapeutic gene targeting of specific human loci.

References

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