

Finding the ZIP-code for gene therapy: Scientists imitate viruses to deliver therapeutic genes

August 31 2009

A research report featured on the cover of the September 2009 print issue of *The FASEB Journal* describes how Australian scientists developed a new gene therapy vector that uses the same machinery that viruses use to transport their cargo into our cells. As a result of this achievement, therapeutic DNA can be transferred to a cell's nucleus far more efficiently than in the past, raising hopes for more effective treatment of genetic disorders and some types of cancers.

"Through the use of proteins that mimic key functions of viruses for the packaging and transport of therapeutic DNA, we hope to improve the efficiency, and above all, the specificity of human gene therapy," said David Jans, from the Nuclear Signaling Laboratory at Monash University in Victoria, Australia and one of the researchers involved in the work. "Following the creation of efficient, specific and safe DNA delivery vectors, the challenges in human gene therapy will be able to move on from questions of delivery to actual clinical application."

In short, a gene therapy vector is used to deliver a therapeutic gene or a portion of DNA into a [cell nucleus](#) similar to how a syringe is used to inject medicines. To create the new gene therapy vector, Jans and colleagues used pieces of different genes to create a protein called a "modular DNA carrier," which can be produced by bacteria. This protein carries therapeutic DNA and delivers it to a cell's nucleus, where it reprograms a cell to function properly. In the laboratory, these carrier

proteins were combined with therapeutic DNA and attached to cell membrane receptors and the nuclear import machinery of target cells. In turn, the packaged DNA moved into the cell through the cytoplasm and into the nucleus.

"Effective [gene therapy](#) is clearly the best way to treat heritable diseases. It's also an approach to other diseases where the environment or infection messes up our genes." said Gerald Weissmann, M.D., Editor-in-Chief of The [FASEB Journal](#). "The Australians have worked out how viruses identify our nuclear ZIP-code, and have delivered therapeutic genes to the same address. This work opens up a new era of pharmaceutical development."

More information: Dominic J. Glover, Su May Ng, Adam Mechler, Lisandra L. Martin, and David A. Jans. Multifunctional protein nanocarriers for targeted nuclear gene delivery in nondividing [cells](#). *FASEB J.* 2009 23: 2996-3006.
www.fasebj.org/cgi/content/abstract/23/9/2996

Source: Federation of American Societies for Experimental Biology
([news](#) : [web](#))

Citation: Finding the ZIP-code for gene therapy: Scientists imitate viruses to deliver therapeutic genes (2009, August 31) retrieved 20 March 2024 from <https://phys.org/news/2009-08-zip-code-gene-therapy-scientists-imitate.html>

This document is subject to copyright. Apart from any fair dealing for the purpose of private study or research, no part may be reproduced without the written permission. The content is provided for information purposes only.
