Nanoparticles Deliver Gene Therapy, Killing Tumors

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(PhysOrg.com) -- Given that cancer is ultimately a genetic disease, it has long been the hope of researchers to use gene therapy to attack tumors where they might be most susceptible. Those prospects have taken a significant step forward with the report that a trans-European research team has developed a nanoparticle that transports antitumor genes selectively to cancer cells. The technique, which leaves healthy cells unaffected, could offer hope to people with difficult-to-treat cancers. Although it has so far been tested only in mice, the researchers hope for human trials in 2 years.

Reporting its work in the journal Cancer Research, the research team, headed by Georges Vassaux, Ph.D., Institute National de la Santé et de la Recherche Médicale (INSERM), used poly(propylene imine) dendrimers as carriers for the genes. This particular dendrimer forms stable complexes with DNA that only appear to fall apart when inside tumor cells.

Once taken up by cancer cells, the genes enclosed in the nanoparticles force the cell to produce proteins that can kill the cancer. In this study, the cells were forced to make a protein known as the Na/I symporter, which was then visible in whole-body scans of the mice. These scans revealed that the transfected gene was expressed in cancer cells but not at an observable level in healthy cells.

“Gene therapy has a great potential to create safe and effective cancer treatments, but getting the genes into cancer cells remains one of the big

challenges in this area. This is the first time that nanoparticles have been shown to target tumors in such a selective way, and this is an exciting step forward in the field," said Andreas Schatzlein, Ph.D., University of London, one of the investigators involved in the study. "Once inside the cell, the gene enclosed in the particle recognizes the cancerous environment and switches on. The result is toxic but only to the offending cells, leaving healthy tissue unaffected."

This work is detailed in the paper "Cancer-specific transgene expression mediated by systemic injection of nanoparticles." Investigators from the Queen Mary's School of Medicine and Dentistry in London, The London National Health Service Trust, Cancer Research United Kingdom, Instituto Aragonés de Ciencias de la Salud in Spain, and the University of Bordeaux in France also participated in this study. An abstract of this paper is available at the journal's Web site.

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