

U-M researchers successfully use DNA as a drug

U-M Medical Center researchers in June 1992 made headlines when they became the first to attempt direct gene transfer-taking DNA off the shelf and introducing it directly into the body-by injecting a cancer-fighting gene into the tumor of a woman with advanced melanoma. Their goal: to prod the woman's immune system to attack the malignancy.

The researchers successfully demonstrated gene expression, lack of toxicity and the therapeutic potential of direct gene transfer in the above-mentioned woman and in all others who participated in the trial. The results of the study were published in the Dec. 1, 1993, issue of *Proceedings of the National Academy of Science*.

"We have successfully given DNA directly to patients, using it as a drug to express a gene. This approach will make it simpler and safer to treat cancer and other disease with gene therapy", says principal investigator Gary J. Nabel, M.D., Ph.D., professor of internal medicine and of biological

chemistry and a Howard Hughes Medical Institute investigator.

Nabel and his colleagues injected modified genetic material into the malignant skin lesions of five patients with metastatic melanoma.

No complications were noted and in each patient an immune response was triggered. Additionally, actual tumor regression was observed on two occasions in one of the study subjects.

The genetic material that triggered such a response is a gene for a transplantation antigen called HLAB7. The antigen, found on the surface of cells in transplanted organs, triggers the immune system to attack foreign cells. This rejection like response is what's responsible for the tumor shrinkage.

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