



Method of Targeting Dendritic Cells

Dendritic cells are responsible for mounting immune responses against invading foreign pathogens as well as against harmful responses of the body. Gene transfer in dendritic cells can be especially useful for a variety of therapeutic purposes such as cancer therapy, as a cure for a variety of autoimmune diseases, and to improve transplantation success. University of Florida researchers have successfully demonstrated the ex vivo transduction of dendritic cells by a viral vector containing a gene of interest. In addition, these researchers have discovered that transduction of dendritic cells is most successful using adeno-associated virus serotype 1 (AAV1). This technology has the potential to cure or ameliorate a wide variety of diseases, and at the very least to reduce the side effect profile associated with current therapies.

Applications

This technology can be used to alter dendritic cells for a variety of therapeutic applications, such as:

- ◆ Producing a protein as a treatment for a disease involving a deficiency of that protein
- ◆ Inducing an immune response against various cancers
- ◆ Preventing transplant rejection or autoimmune conditions

Advantages

- ◆ Useful for a wide variety of clinical uses, ensuring broad applicability
- ◆ Can be used to improve therapy for cancer, autoimmune disease, and transplant rejection, reducing morbidity associated with standard treatments for these conditions
- ◆ Successful treatment of these costly diseases through transduction of dendritic cells translates into lower health care costs to patients and insurance companies

The Technology

In *ex vivo* experiments, University of Florida researchers have successfully transduced murine bone marrow-derived dendritic cells with recombinant adeno-associated virus (rAAV) containing the gene for human alpha-1-antitrypsin. Contrary to previously published work, they also demonstrated that AAV serotype 1 is clearly superior to the other rAAV serotypes in transducing dendritic cells. This technology has a wide range of clinical uses, and may be pivotal in developing a cure or an ameliorative therapy for a number of devastating human diseases.

contact

Elizabeth Garami
University of Florida
Office of Technology Licensing
352/392-8929 • email: egarami@ufl.edu
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