UNIVERSITY OF FLORIDA *seeks partner to license*

Insulation Cassette that Enables Controlled, Sustained Gene Therapy Delivery

The University of Florida is seeking a company interested in commercializing a novel gene delivery cassette allowing for the controlled and sustained expression of a gene. The field of gene therapy offers a promising solution for the treatment of a wide spectrum of human diseases. Research and Markets estimates that "collectively, the value of markets for gene therapy technologies in 2002 was approximately $20.2 billion and is estimated to increase to $35.7 billion by the year 2007 and $81.3 billion by the year 2012. The largest expansion will be in diseases of the central nervous system," including among others Alzheimer's, Parkinson's, Huntington's Disease, and Fragile-X Mental Retardation Syndrome. Thus far, developing effective gene therapies for these diseases has proven difficult, because the body's native genetic material tends to repress the introduced, therapeutic genes. Our researchers have engineered a gene expression cassette that can insulate the therapeutic genetic material inside from the influence of surrounding genetic material. The cassette is also highly controllable and can be targeted to specific cell types, representing a major technological step and a promising business opportunity in the field of gene therapy.

Applications

Insulation cassette for sustained and regulatable gene delivery

Advantages

- Permits maintenance of persistent, highly controllable, long-term gene expression, significantly improving quality and effectiveness of gene therapy products
- Enables cell-type specificity, assuring therapeutic resources are used directly on the cells needing them, while leaving non-target cells unaffected
- Vector system packages large DNA insertions, efficiently providing the needed space for complex genetic information
- Can be used for the production of large amounts of a needed gene for pharmacological or agricultural purposes in animals or plants
- Can be engineered to combat non-central nervous system disorders, offering opportunities in a broad range of other markets

The Technology

The technology at hand employs a defective form of the herpes virus as the vector to carry the gene expression cassette for gene transfer to the central and peripheral nervous systems. Specifically, the technology allows a transgene to maintain persistent, long-term and highly regulatable gene expression, unhindered by host cell silencing mechanisms such as histone methylation/deacetylation, DNA methylation, position effects, or transgene copy number. The delivery system is thus composed of (1) the insulated gene expression cassette and (2) a defective herpesvirus-I-based virus vector for delivery of the transgene to the central nervous system. The insulation cassette has the potential to become a powerful tool in the field of gene therapy, basic gene expression assays, and development of animal disease models. Note that the insulated gene cassette can be used alone, such as in transgenic applications, or be used with other DNA-based or viral delivery systems.

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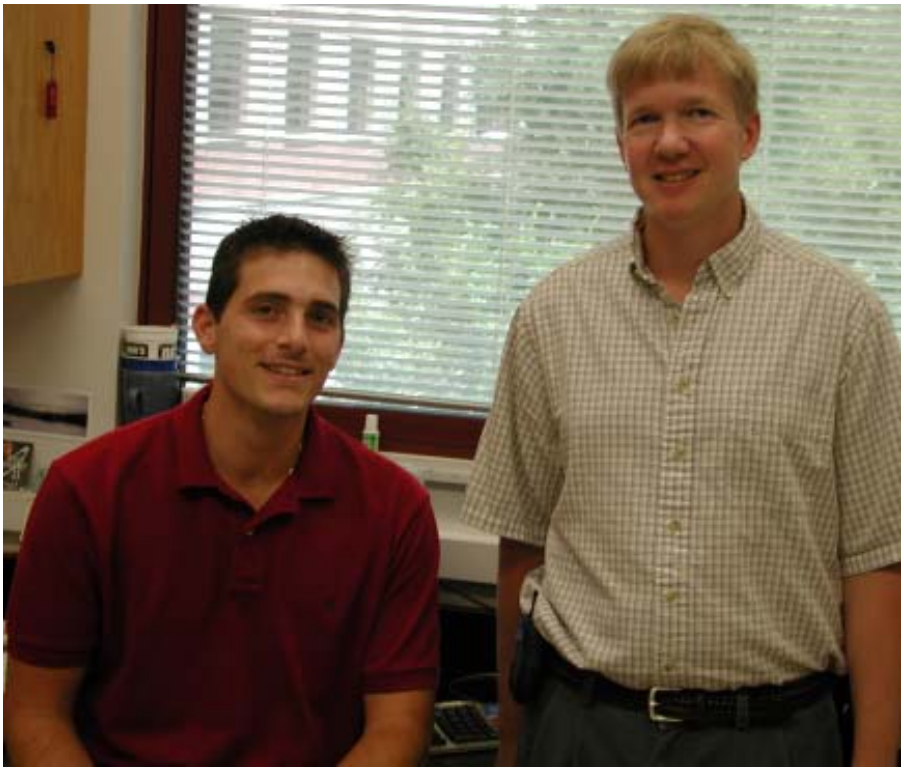

Dr. David Bloom (standing, at right) is an Assistant Professor in Molecular Genetics and Microbiology at the University's College of Medicine. He obtained his Ph.D from Vanderbilt University and completed a postdoctoral fellowship at UCLA. He was nominated for the prestigious Howard Hughes Medical Institute Investigator and received the Burroughs Wellcome Fund Investigator in Pathogenesis Award.

Antonio L. Amelio is a Ph.D candidate in the University of Florida Interdisciplinary Program in Biomedical Sciences, with an advanced concentration in Genetics. He is a concurrent degree student completing a Master of Science in Business Administration, with a concentration in Management. Mr. Amelio has served as President of the College of Medicine Graduate Student Organization and is a member of the Phi Theta Kappa and Golden Key National Honor Societies. He was Student of the Year in 1997-98.

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