



Novel Technique to Enhance Ribozyme Specificity and Delivery

The University of Florida is seeking companies interested in commercializing a novel technique for discovery of gene function using AAV-delivered ribozymes. Ribozymes are RNA enzymes that bind and cleave other RNA or DNA molecules. Target recognition domains of ribozymes can be altered to bind and cleave almost any messenger RNA. Thus, ribozymes have the potential to cleave most messenger RNA molecules and prevent them from functioning in the cell. By capitalizing on these properties of ribozymes, this invention provides methods to inactivate genes of unknown function in cells or tissue and obtain evidence of function based on the resulting phenotype. This novel technique enables identification of novel genes that are involved, either directly or indirectly, in a wide range of cellular, biological and physiological processes.

Applications

Useful for the elucidation of gene function, which is important for:

- ◆ Identifying new tumor suppressor genes that function to prevent cancer
- ◆ Determining genes involved in pathogenesis
- ◆ Identifying genes required for specific regulatory functions
- ◆ Development of new therapeutics or treatments for disease

Advantages

- ◆ Enables screening of unknown gene function without any prior assumptions of gene sequence or function, allowing the discovery of unique genes
- ◆ *In vivo* screening provides a more representative look at gene function manifested in normal animal cells, affording valuable insight into gene function in health and disease
- ◆ Provides a method to “knock-out” genes of unknown function to determine their specific disease-causing potential, thereby identifying new disease-causing genes
- ◆ Enables “knock-out” animals to be created in adults, allowing genes essential to embryonic development (which cause lethality in embryonic “knock-outs”) to be studied in adult animals

The Technology

In this invention, DNA copies of ribozymes are delivered using recombinant adeno-associated virus (rAAV). Target binding sites of the ribozyme are designed with conserved nucleotides, and RNA copies are generated using either strong general promoters or cell-type specific promoters. The library of ribozyme encoding genes is delivered as high-titer preparations of rAAV by inoculation of specific organs. After a period of time, tissue is collected, ribozyme-containing sequences are purified, and the genes identified. As a result of the combination of ribozyme specificity and their delivery in a tissue and cell-type specific manner *in vivo*, this invention enables the assignment of function to a large number of newly discovered genes.

contact

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