

RNAi shows promise in gene therapy, researcher says

Three years ago Mark Kay, MD, PhD, published the first results showing that a biological phenomenon called RNA interference could be an effective gene therapy technique. Since then he has used RNAi gene therapy to effectively shut down the viruses that cause hepatitis and HIV in mice.

With three human RNAi gene therapy trials now under way - two in macular degeneration and one in RSV pneumonia - the technique Kay pioneered may be among the first to find widespread use for treating human diseases. "We've worked on a gene therapeutic approach against viral hepatitis for about 10 years and this is the first thing we've done that really looks promising," said Kay, professor of genetics and of pediatrics at the Stanford University School of Medicine.

Kay will talk about future uses for RNAi gene therapy at the American Association for the Advancement of Sciences annual meeting in San Francisco during a session titled "RNAi for emerging pandemics and biosecurity."

RNAi is a biological phenomenon in which a strand of RNA in the cell can cause the destruction of another strand of RNA that is relaying a protein-coding message from a gene. With that protein-coding message removed, the gene's message is effectively destroyed. When used as gene therapy, RNAi turns off genes that are overactive in such diseases as cancer or macular degeneration, or disables genes needed by an invading virus. With key genes shut off, viruses such as hepatitis or HIV are unable to multiply and cause disease.

In early RNAi experiments, researchers saw some hints that the technique could induce an immune reaction or switch off the wrong gene or genes. In work last year, Kay confirmed those findings but also showed a possible way around those toxic effects by selecting particular RNA sequences.

"One benefit of RNAi gene therapy is that it uses the body's own machinery, making it an effective approach," Kay said at the time. "However, the detriment of RNAi gene therapy turns out to be that it uses the body's own machinery." He said he expects the current trials will help him and others figure out the best way to bring RNAi gene therapy safely to humans.

Source: Stanford University

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