New study shows that therapeutic gene expression can be sustainable for 1 year

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Researchers at the Board of Governors Gene Therapeutics Research Institute at Cedars-Sinai Medical Center have shown for the first time that it is possible to sustain therapeutic gene expression in the central nervous system for up to a year, even in the presence of an anti-viral immune response mechanism that is normally present in humans.

The researchers demonstrated in an animal model that the delivery system for the gene, a novel gutted adenoviral vector called HC-Adv, is completely invisible to the immune system. Vectors previously used to deliver genes carried minute amounts of viral proteins that were detected by the immune system, triggering an immune response that rendered the therapeutic gene inactive after a period of weeks.

According to the researchers, this delivery system is safer and more effective than what is currently available, and should therefore advance clinical gene therapy trials for people suffering from central nervous system disorders such as Parkinson's, Alzheimer's, and Multiple Sclerosis. The research was sponsored in part by The National Institutes of Health.

Source: Cedars-Sinai Medical Center