Michigan hospital launches gene therapy study for Parkinson's disease

October 6 2009

A Michigan hospital is embarking on a research study for advanced Parkinson's disease using a state-of-the-art treatment called gene transfer.

The clinical trial will test whether gene transfer therapy is able to restore better mobility in Parkinson's patients who have lost responsiveness to drug therapy.

"The start of this clinical trial provides hope to a Parkinson's disease patient population that has had a long-standing need for better treatment options," says Henry Ford West Bloomfield Hospital neurologist Peter LeWitt, M.D., who is internationally known for his research on Parkinson's disease.

Of the eight sites in the United States involved in the study, Henry Ford West Bloomfield Hospital, part of the Henry Ford Health System, is the only Michigan institution testing this experimental therapy.

Parkinson's disease is a degenerative disorder of the central nervous system that causes tremors and impairs a person's motor skills, speech, balance and posture. Its cause is unknown and it affects 1 percent to 2 percent of people over the age of 60.

A small region deep within the brain is the source for the symptoms of Parkinson's disease. When brain neurons in this part of the brain begin to die, these cells can no longer manufacture the molecule dopamine, a

chemical critical for controlling movement.

Among patients with Parkinson's disease, the pace and extent of progression in neurologic deficits can greatly vary. The burden on quality of life spans a wide spectrum too, Dr. LeWitt says, from minimal discomfort and disability to marked impairment of capabilities such as independence, safety and communication.

Most current therapies and research approaches target dopamine to treat motor symptoms associated with Parkinson's disease. In contrast, the focus of the current gene therapy strategy is on increasing GABA, a brain neurotransmitter that regulates movement. In Parkinson's disease, GABA is reduced in an area of the brain called the subthalamic nucleus, causing it to be overactive. Investigators feel this might be a better way to help advanced Parkinson's disease.

For the clinical trial, the gene therapy product rAAV-GAD will be placed into the subthalamic nucleus by a surgical procedure. The gene transfer is done through a catheter that is removed shortly after its placement.

Participants will be assessed post-treatment at multiple intervals. As an alternative to this experimental treatment, patients with advanced Parkinson's disease have the option of deep brain stimulation. This also involves a surgical procedure in which a pacemaker-like device is placed in the brain to help in control of Parkinson's disease symptoms.

Source: Henry Ford Health System (news : web)

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