OSU trying out cure for Huntington's Disease

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COLUMBUS - In a worldwide first, surgeons at The Ohio State University Wexner Medical Center have treated two patients with Huntington's disease using a novel gene therapy treatment as part of a multi-center, double-blind randomized clinical trial.

Huntington's disease is an inherited genetic disorder that results in progressive physical and cognitive deterioration, ultimately leading to death. The cause is a mutant protein which damages brain cells, said Ohio State Wexner Medical Center neurosurgeon Dr. James "Brad" Elder who performed the surgeries.

"The overall goal of this gene therapy treatment strategy is to stop the neurologic deterioration associated with Huntington's disease by blocking production of the mutant protein. Targeting specific areas of the brain with gene therapy will hopefully help patients maintain their existing level of function, and be reflected in a halting of deterioration of the brain structures that are most affected by this disease," Elder said.

People are born with the defective gene, but symptoms usually don't appear until middle age. Early symptoms of Huntington's disease may include uncontrolled movements, clumsiness and balance problems. Later, Huntington's disease can take away the ability to walk, talk and swallow. It affects movement, cognitive function and behavior.

The five-year Phase I/II clinical trial will explore the safety, tolerability and effectiveness of an investigational gene therapy by uniQure called AMT-130. Researchers will enroll 26 patients with early stage Huntington's disease. Patients will be randomized to either receive the treatment or an imitation surgery without treatment.

The clinical trial consists of a blinded 18-month core study period followed by un-blinded long-term follow-up. Patients receive a single administration of gene therapy through MRI-guided neurosurgical delivery directly into the striatum. This is an area of the brain that regulates movement.

The first two patients treated at Ohio State will be observed for an initial period of 90 days, and their data will be reviewed to determine continued dosing of the next patients.

"Gene therapy represents a viable solution for both rare and common forms of complex disease," said Peter Mohler, chief scientific officer for Ohio State Wexner Medical Center and Ohio State's Health Sciences Colleges, and vice dean of Research at The Ohio State University College of Medicine. "Innovative and collaborative clinical trials like this one offer us the ability to tackle diseases like Huntington's that were previously viewed to be incurable." If someone's parent has Huntington's disease, then they have a 50% chance of developing it. A blood test can detect the gene that causes Huntington's disease to develop.

"The direct delivery of gene therapy that is being done in this trial provides the opportunity to now treat diseased brain circuits in a targeted permanent manner via a minimally-invasive approach. Using this approach, Ohio State is also performing first-in-patient gene therapy trials for other incurable neurologic diseases, as well as Huntington's Disease," said Dr. Russell Lonser, co-director of The Ohio State University Wexner Medical Center's Neurological Institute and chair of the department of neurological surgery.