First treatment for Huntington's disease shows promise in rats, Van Andel Institute scientist says

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A stem cell treatment investigated for Huntington's disease holds out hope that scientists will someday be able to reverse damage caused by the degenerative brain disorder.

The technique, which uses reprogrammed skin cells from a Huntington's patient, successfully restored motor functions in rats, said Dr. Patrik Brundin, a Van Andel Institute researcher who was involved in the study.

"It's an interesting step, one we've been hoping for," he said. "It's exciting."

The technique also will be tested in treatments for Parkinson's disease, said Brundin, who came to VAI from Sweden in October to lead the institute's Parkinson's research.

Scientists from Sweden, South Korea and the U.S. collaborated on the study, which was published online Monday in the journal Stem Cells.

Brundin said researchers took stem cells derived from the skin of a patient with Huntington's disease and converted them to brain cells – or nerve cells – in culture dishes in the lab. The cells were transplanted into the brains of rats that had an experimental form of Huntington's, and the rats' motor functions improved.

"The unique features of the (stem cell approach) means that the transplanted cells will be genetically identical to the patient," Jihwan Song, an associate professor at CHA University in Seoul and co-author of the study, said in a statement released by VAI. "Therefore, no medications that dampen the immune system to prevent graft rejection will be needed."

Brundin estimated the research might lead to treatments for humans in five to 10 years, although he acknowledged a timeframe is difficult to predict. Researchers are eager to find a new treatment for Huntington's because "there is nothing really powerful to offer currently," he said.

Huntington's is a genetic disorder affecting one in every 10,000 Americans that slowly diminishes a person's ability to walk, talk and reason. A child of a parent who has Huntington's has a 50 percent chance of inheriting the gene that causes it.

Medications can relieve some symptoms in some cases, but there are no treatments available that can slow the disease, according to the Huntington's Disease Society of America.

The study also showed that patient's stem cells can be used to study the disease process – and potential treatments — in a culture dish, Brundin said. The cells formed protein clumps similar to what is seen in the brains of Huntington's disease patients.

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