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'Very promising' treatment for Huntington disease discovered

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Medical researchers at the University of Alberta have discovered a promising new therapy for Huntington disease that restores lost motor skills and may delay or stop the progression of the disease based on lab model tests, says the lead researcher. Because the new therapy uses a molecule already being used in clinical trials for other diseases, it could be used in a clinical trial for Huntington disease within the next one to two years.

"We didn't expect to see such dramatic changes after administering this therapy," said Simonetta Sipione, the Principal Investigator "We expected to see improvement, but not complete restoration of motor skills. When we saw this, we were jumping with excitement in the lab. This is very promising and should give hope to those with Huntington disease. I think it's a treatment that deserves to go to clinical trials because it could have huge potential."

Those with this inherited brain disorder, where a mutant protein triggers brain cell death causing loss of motor and cognitive skills and eventually death, have slightly lower levels of a brain molecule known as GM1. When U of A medical researchers restored GM1 to normal levels in lab models with the disease, motor skills in the lab models returned to normal within days, said Sipione, a researcher in the Department of Pharmacology and the Centre for Neuroscience, both within the Faculty of Medicine & Dentistry.

Her team's research was published in the peer-reviewed journal

Proceedings of the National Academy of Sciences today.

The molecule used in the lab tests at the U of A was produced both naturally and synthetically through chemical production. This same molecule has been used in clinical trials for the treatment of Parkinson's and other neurodegenerative diseases, so using this molecule to treat patients with Huntington disease in a small first stage clinical trial could happen relatively quickly. Details are still being worked out about where the trial would take place, but researchers are hoping it will be at the U of A and are in discussions with a University of Alberta Hospital neurologist.

During the research stage, lab models at the U of A were given the GM1 molecule therapy for four weeks. During the first two weeks after the treatment finished, the lab models still had normal motor function. But after that, motor function started to decline and return to pre-treatment levels by the end of the fourth week. So a potential treatment with this molecule would involve repeated treatments over the long-term, says Sipione.

Sipione and her team are continuing their research to see if restored levels of the GM1 molecule can also reverse cognitive damage in lab models with Huntington disease. They hope to publish the results from these tests within one year. It seems the GM1 therapy improves the way neurons work and makes the mutant huntingtin protein less toxic.

"Because of the way it works, we think it will work on cognitive symptoms of the disease too," says Sipione, a Canada Research Chair Tier 2 in Neurobiology of Huntington disease and an Alberta Innovates-Health Solutions Scholar.

The Huntington Society of Canada funded the research and the CEO said she is excited about the promising results.

"The Huntington Society of Canada is proud to support the excellent research of Dr. Sipione," said Bev Heim-Myers, CEO, Huntington Society of Canada. "Dr. Sipione, for the first time, has demonstrated that in a Huntington disease laboratory model, the treatment reverts the lab model back to normal, not just slightly better.

"It is important to understand that some treatments may work in laboratory models, but not in people. The applicability of the treatment discovered by Dr. Sipione to Huntington disease patients will be determined in clinical trials. We are optimistic that this research demonstrates real potential for a Huntington disease therapy."

Provided by University of Alberta