

Drug shows promise for Huntington's disease

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An early stage clinical trial of the experimental drug dimebon (latrepirdine) in people with Huntington's disease appears to be safe and may improve cognition. That is the conclusion of a study published today in the *Archives of Neurology*.

"This is the first clinical trial that has focused on what is perhaps the most disabling aspect of the disease," said University of Rochester Medical Center neurologist Karl Kieburtz, M.D., the lead author of the study. "While more investigation needs to be done, these results are encouraging and show, for the first time, a statistically significant benefit in terms of improved cognitive function in patients with [Huntington's disease](#)."

Huntington's disease is a progressive [neurodegenerative disorder](#) that impacts movement, behavior, cognition, and generally results in death within 20 years of the disease's onset. The disease steadily erodes a person's memory and their ability to think and learn. Over time, this cognitive impairment contributes to the loss of the ability to work and perform the activities of daily life. There are no treatments current available that effectively alter the course of the disease or improve cognition.

It is believed that mitochondria - the part of the cell that helps convert food to energy - plays a role in the development of Huntington's disease. Lampridine stabilizes and enhances mitochondrial function, a result that has been shown to improve behavioral, cognitive, and functional outcomes in Alzheimer's disease. Scientists speculate that this may have the same effect in patients with Huntington's disease.

The investigators studied the impact of the drug on 91 patients over a 90 day period. Half were given the drug and the other half a placebo. The patients were then evaluated using a cognitive tool called the Mini-Mental State Examination. This exam - which is used by clinicians to evaluate the stage

and severity of [dementia](#) and Alzheimer's disease - consists of series of questions used to test an individual's orientation, memory, and ability to follow commands. The researchers found that the drug on average improved the scores of people taking the drug vs. those who received the placebo.

Dimebon is being developed by Medivation, Inc., a San Francisco-based pharmaceutical company which funded the study. The drug is also being testing in patients with Alzheimer's disease. In July of last year, Medivation and Pfizer, Inc. launched a Phase 3 clinical trial of the drug for Huntington's disease. The study, called the Horizon Trial, is being conducted in sites throughout North America, Australia, and Europe. Kieburtz is the principal investigator of the Horizon Trial.

Provided by University of Rochester

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