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Huntington's disease breakthrough equals hope for patients

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UCF Professor Ella Bossy-Wetzel has made a huge contribution to understanding Huntington's disease with her research published in *Nature Medicine* this week. Credit: UCF/ Jacque Brund

A huge leap forward in understanding Huntington's disease may give patients hope for a cure.

Laboratory tests on skin cells and post-mortem brain tissue of Huntington's disease patients determined that an overactive protein triggers a chain reaction that causes brain nerve cells to die. Toning down the activity of that protein, known as DRP1, prevented the chain reaction and kept those cells alive, according to the research team led by University of Central Florida Professor Ella Bossy-Wetzel.

Huntington's is an inherited, incurable neurodegenerative disease

affecting 35,000 people annually. The disease gradually kills nerve cells in the brain, stripping away a person's physical abilities and causing hallucinations, antisocial behavior and paranoia.

People diagnosed with the disease usually die 15 to 20 years from the onset of symptoms, and there is an increased rate of suicide among those struggling with the disease.

"The next step will be to test the DRP1 function in animals and patients to see whether the protein also protects the brain," Bossy-Wetzel said. "This could be done before the onset of disease in patients who have the mutant Huntington gene, but have no neurological symptoms. The hope is that we might be able to delay the onset of disease by improving the energy metabolism of the brain."

The research findings were published online this week in the journal *Nature Medicine*, and they will be featured in the cover story of the March edition.

Until now, little has been known about how Huntington's works. Scientists knew that people with the mutant Huntington gene develop the disease. They also knew that a cell's powerhouse– mitochondria, which turn food into energy – was somehow involved. But until Bossy-Wetzel's team completed its work, little else was known.

"Mitochondria require balanced cycles of division and fusion to maintain their ability to produce energy," Bossy-Wetzel said. "The protein DRP1 is needed for mitochondrial division. We found that in Huntington's disease, DRP1 becomes overactive and causes too much mitochondrial division without balancing fusion."

That production error causes the brain's nerve cells to die. The UCF team toned down the activity of DRP1, which restored a normal balance

of mitochondrial division and fusion and improved the energy metabolism and survival of neurons.

Other scientists in the field say the discovery is an important step toward eventually finding a cure.

"It is an outstanding piece of work, which further implicates mitochondrial dysfunction in the pathogenesis of Huntington's disease," said Flint Beal, a professor of neurology and neuroscience at the Weill Medical College of Cornell University who specializes in the disease and is a practicing physician. "It opens new therapeutic targets for therapies aimed at disease modification."

Provided by University of Central Florida

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