DNA nanoparticles hold promise in gene therapy for Parkinson's disease

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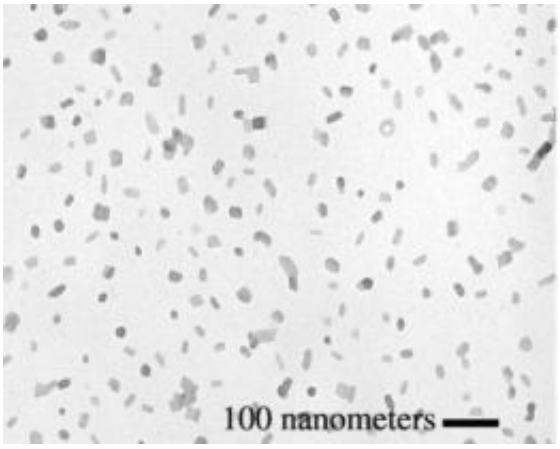

Nanoparticle Gene Therapy for Parkinson's Disease -- This electron micrograph shows the minute scale of DNA compacted into nanoparticles. After it is condensed, the DNA is delivered to the brain in an attempt to repair faulty genes in hopes of halting or preventing the neurodegenerative process. Credit: David Yurek, University of Kentucky

University of Kentucky researcher David Yurek was recently awarded $66,000 by The Michael J. Fox Foundation for Parkinson's Research under the foundation's Rapid Response Innovation Awards program. The goal of this newly launched initiative is to move quickly to support innovative research focused on the cause of and cure for Parkinson's disease (PD). In particular, MJFF seeks to fund high-risk, high-reward projects tackling critical scientific roadblocks that if successful, can open new avenues for PD therapy development.

Yurek's project, titled "Nanoparticle Gene Therapy for Parkinson's Disease," examines a relatively new gene therapy approach for treating neurodegenerative disorders. He is testing the feasibility of using a novel technology to condense DNA plasmids into nanoparticles and deliver them to the brain as a means to halt or prevent the neurodegenerative process.

The technology comes from Copernicus Therapeutics, Inc., a biotechnology company in Cleveland, Ohio. Yurek, whose laboratory is one of the first to apply this technology to central nervous system disorders, said this relatively new gene therapy strategy holds potential to help repair faulty genes. It entails transduction, a technique for expressing a particular gene in a cell by delivering DNA into the cell and making the cell synthesize the protein that corresponds to that DNA.

"We plan to use this technology to transduce brain cells so that they express proteins beneficial to the cell's survival," Yurek said.

The MJFF award will allow Yurek to test the feasibility of delivering condensed DNA nanoparticles that encode for a neurotrophic factor to the brain as a means to halt or prevent the neurodegenerative process in an animal model of PD. Neurotrophic factors are capable of protecting neurons from dying, thereby rescuing essential neurons in the brain. In animal studies, neurotrophic factors have revived dormant brain cells, caused them to produce dopamine, and prompted dramatic improvement of symptoms.

PD is a chronic, progressive disorder of the central nervous system, and is the direct result of the loss of cells in a section of the brain called the substantia nigra. Those cells produce dopamine, a chemical messenger responsible for transmitting signals within the brain. Loss of dopamine causes critical nerve cells in the brain, or neurons, to fire out of control, leaving patients unable to direct or control their movement in a normal

manner.

The Michael J. Fox Foundation for Parkinson's Research is dedicated to ensuring the development of a cure for PD within this decade through an aggressively funded research agenda. Enormous progress toward finding a cure has been made on many neurological fronts, and scientists' understanding of the brain and how disease affects it has increased dramatically. The foundation seeks to hasten progress further by awarding grants that help guarantee that new and innovative research avenues are thoroughly funded and explored.

The MJFF Rapid Response Innovation Awards support projects that may have little to no existing preliminary data, but that hold potential to significantly impact understanding or treatment of PD.

"Given the extremely tight budget of federal government research funding, MJFF's work in prioritizing and funding new and innovative projects is extremely valuable," Yurek said.

Source: University of Kentucky