National Stem Cell Foundation and Michael J. Fox Foundation Partner to Support Parkinson's Cell Replacement Therapy

The National Stem Cell Foundation (NSCF) and The Michael J. Fox Foundation for Parkinson's Research (MJFF) have announced a partnership to fund development of a cutting-edge therapy to replace the dopamine neurons that degenerate in Parkinson's disease, the second most common brain disease after Alzheimer's.

The Foundations will collectively grant $625,000 to Ole Isacson, MD, director of the Neuroregeneration Research Institute at McLean Hospital and principal faculty at Harvard Stem Cell Institute, to support pre-clinical work exploring implantation of dopamine neurons created from engineered stem cells. The treatment aims to alleviate or prevent Parkinson's motor symptoms of tremor, rigidity and slowness.

"Advances in stem cell engineering offer a new route to replace what is lost in Parkinson's disease," says Paula Grisanti, DMD, National Stem Cell Foundation Chairman. "As a leader in the field, Dr. Isacson is poised to refine this treatment toward widespread application, and we are glad to partner with The Michael J. Fox Foundation to fund this important work."

Therapy Replacing Machinery, Not Only Product

Dopamine neuron loss causes Parkinson's motor symptoms and may play a role in some non-motor symptoms. Currently available treatments introduce replacement dopamine, but these medications lose efficacy over time and can cause debilitating side effects such as involuntary movements called dyskinesia. Replacing the dopamine neurons may provide a regulated stream of dopamine that would alleviate motor symptoms.

"The limitations of current therapies call for the evolution of treatment options for the millions of people living with Parkinson's disease," said Todd Sherer, PhD, CEO of The Michael J. Fox Foundation. "This collaboration with the National Stem Cell Foundation and the innovation of Dr. Isacson's team moves us closer to that next-generation offering."

The NSCF/MJFF-funded study will further explore implantation of dopamine neurons made from induced pluripotent stem cells (iPSCs) in the brains of non-human primates. In 2015, Dr. Isacson published that implanted iPSC-derived dopamine neurons survived and that the therapy was associated with motor improvement.

"The restoration of the dopamine supply system would be a significant step forward in our treatment of Parkinson's disease," said Dr. Isacson. "This project brings us closer to realizing such a therapy, though there is still much work to be done."
Cells Reborn with New Direction

Stem cells form a more specialized type of cell (e.g., muscle or red blood cell). There are two natural categories of these cells: Embryonic stem cells (also called pluripotent) are found in fertilized eggs and can become any type of cell. Adult stem cells are found among specialized cells in a tissue or organ and can differentiate only into the cell types found in that tissue or organ. In the brain, regions involved in memory formation and smell function contain pockets of adult stem cells that help those systems replace neurons. Research to this point has shown other regions — including the substantia nigra (site of Parkinson’s degenerating dopamine neurons — do not contain stem cells and therefore do not renew.

In 2007, researchers realized another category. With genetic manipulation, scientists engineered embryonic stem cells from an adult fibroblast (cell found in connective tissue). The creation of these induced pluripotent stem cells has transformed medical research. iPSCs are easier to access and may be the source of a personalized therapy that the body is less likely to reject (i.e., dopamine neurons made from one's own blood or skin cell).

These cells also have utility for disease modeling for biological investigation and early-stage drug development. The MJFF-led Parkinson’s Progression Markers Initiative, a 33-site observational study in pursuit of biological markers of Parkinson’s, is making available to qualified researchers iPSC from its varied cohorts (de novo Parkinson’s, genetic mutation carriers, those with Parkinson’s clinical risk factors, and control volunteers). Learn more at www.ppmi-info.org.