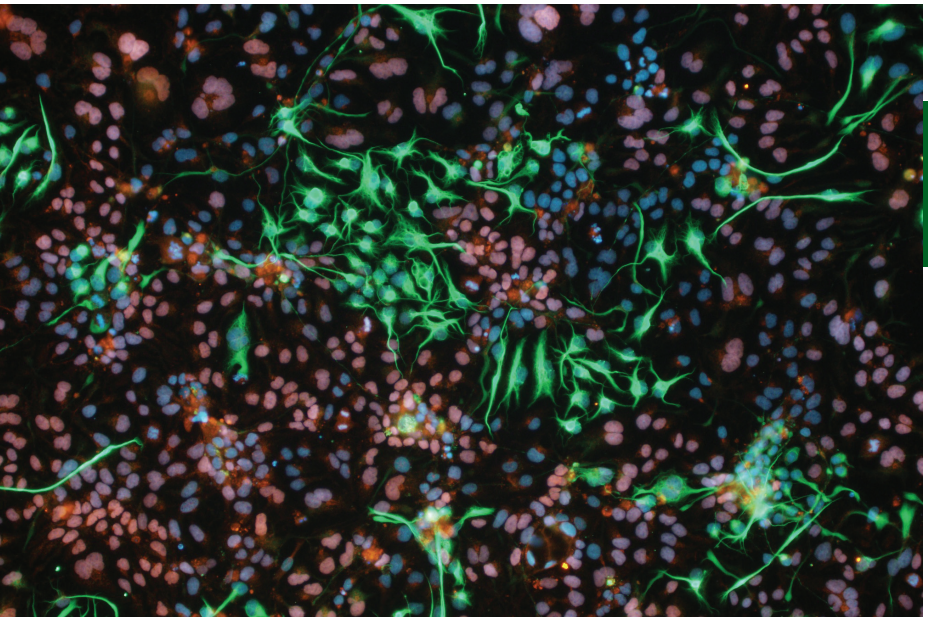

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Parkinson's Disease

Overview

Parkinson's Disease (PD) is a neurodegenerative disease that afflicts more than one million people in the United States, with over 60,000 new cases being diagnosed each year. PD causes the nerve cells that generate dopamine to degenerate and die, leaving patients with limited muscle control. PD generally affects the elderly with the average onset occurring around age 60, although early onset cases do occur. Patients suffer from numerous side effects that have a strong and continued impact on their quality of life. These include uncontrollable muscle tremors and twitching, loss of facial expression, poor balance, trouble swallowing, pain and loss of movement control.

While PD is not lethal and patients can live with the disease for over 20 years, the burden on patients and their families is significant. There is currently no cure for Parkinson's and treatments are limited to managing symptoms rather than addressing the underlying cause of the disease. Patients have to cope with frequent medication changes and side effects to manage (and not eliminate) the primary symptoms of this disease, resulting in a condition that is extraordinarily difficult to control on a chronic basis.

Parkinson's Disease and Regenerative Medicine

Regenerative medicine technologies have the potential to shift the Parkinson's treatment and discovery paradigm.

Currently there are two major cell-based approaches for treating PD. One approach hopes to replace the damaged or lost tissues via cell injections and several studies have shown that mesenchymal stem cells and neural stem cells can be directed into dopaminergic neurons. The second approach utilizes the trophic effect of cells to aid in the endogenous repair and rescue of the PD affected tissue.

Disease modeling is proving to be another useful tool in the research and development of PD therapies. Disease modeling allows scientists to better understand the root cause of the disease as it permits the study of the manifestation and underlying mechanisms of PD. The models are created by reprogramming adult cells from PD patients into induced pluripotent stem cells. Once the cells are reprogrammed, scientists can differentiate them into dopaminergic nerve cells that are affected by the disease. These modeled cells show distinct differences when compared to dopaminergic cells from healthy people.

While Parkinson's Disease is not lethal and patients can live with the disease for more than 20 years, the burden on patients and their families is significant.

The California Institute for Regenerative Medicine (CIRM) grantees at Stanford University and The Parkinson's Institute became the first research team to model Parkinson's with reprogrammed iPSCs from a woman with a genetic form of the disease. The cells initially behaved normally, but 30–60 days later started to exhibit diseased cell traits found in PD patients. Such disease models will be useful for PD patients with a variety of genetic mutations; comparing normal and diseased models may determine commonalities and differences that advance treatments for each patient individually. CIRM is also supporting Dr. Fred H. Gage at the Salk Institute for Biological Studies. Gage has grants from CIRM to mature embryonic stem cells into neurons in order to develop new cell lines from people with the disease and more closely study the mechanisms behind Parkinson's.

Parkinson's Disease: Economic Impact

$23 Billion Annually

Estimated combined direct and indirect costs for Parkinson's Disease patients in the U.S.[1]

Beyond disease modeling, many international groups of scientists are creating dopaminergic neurons from stem cells to replace damaged cells in people afflicted with PD. U.S.-based researcher Lorenz Studer and his colleagues at the Sloan-Kettering Cancer Center have recently succeeded in making highly efficient dopamine-producing neurons from human embryonic stem cells and have transplanted them into the brains of rats and mice with PD. The cells did not multiply abnormally and improved some symptoms. However, work is still needed before human studies begin. The scientists hope to initiate early clinical trials in 2014 or 2015. Additionally, Anne Rosser and colleagues at Cardiff University, UK are exploring ways to help transplanted nerve cells survive inside the brains of Parkinson's patients.

Regenerative medicine is proving to play an important role in our understanding of the disease and the early development of next generation PD technologies and therapies.

[1] Deborah Boland and Mark Stacy, "The Economic and Quality of Life Burden Associated with Parkinson's Disease: A Focus on Symptoms," *The American Journal of Managed Care* Vol. 18, September 2012, pp. 168-175 available at www.ajmc.com/publications/supplement/2012/408_12sep_Parkinsons/A408_12sep_Boland_S168to75