Autologous Brain Cell Transplant for Parkinson Disease

A patient with idiopathic Parkinson disease safely received replacement dopamine neurons in 2 brain cell transplants. The transplanted cells were derived from his own skin cells, eliminating the need for immunosuppressing drugs.

Researchers reprogrammed skin fibroblasts from the 69-year-old man into induced pluripotent stem cells, which they then differentiated into the dopamine-producing cells that are lost in Parkinson disease. They implanted millions of the cells into the patient’s left and right brain hemispheres in minimally invasive procedures in 2017 and 2018.

Brain imaging suggests the cells have survived. The patient, who funded early research leading to the treatment, has reported no adverse events or decline in function. His daily “off” time, when symptoms return between medication doses, decreased from 3 hours to less than 1 hour.

Two years after the first procedure, his reduction in medication was equal to a 6% decrease in levodopa. His parkinsonian motor signs and quality-of-life scores also improved over the follow-up period. However, the researchers said these findings should be interpreted cautiously because the study was not blinded and did not include a control comparison.

They also emphasized in a statement that the therapy is not currently available. Although the procedure was a technical success, further studies are needed “to address how this approach will perform in a variety of patients with diverse genetic backgrounds and disease phenotypes over a period longer than 24 months,” they wrote in the New England Journal of Medicine.

Researchers Home in on COVID-19 Severity Biomarkers

About one-fifth of patients with coronavirus disease 2019 (COVID-19) have clinically severe or life-threatening infections requiring interventions such as immediate oxygen therapy or mechanical ventilation. Knowing early on which patients are likely to develop severe disease could help save lives.

To that end, several research teams are working to discover telltale blood biomarkers. In a study recently published in Cell, researchers in China found 93 proteins and 204 metabolites whose levels correlated with severe COVID-19. The scientists analyzed sera from 53 healthy people and 46 patients with severe and nonsevere COVID-19 to find the molecular markers.

They then trained a machine learning model to stratify disease severity using 29 of these serum factors. The model correctly classified 29 of 31 patients in the training cohort, for an overall accuracy of 93.5%, and 23 of 29 patients in 2 independent test cohorts.

Larger studies of patient samples collected at more time points will be needed to develop a clinical test that predicts severe cases before they develop. Still, the study provides “some of the first evidence that such a test might be possible,” National Institutes of Health Director Francis Collins, MD, PhD, wrote in a recent blog post.

Meanwhile, European researchers have designed a high-throughput platform to analyze serum and plasma proteins from COVID-19 samples. The system identified 27 potential protein biomarkers that are expressed differently in hospitalized patients depending on their case severity. Another team, led by New York University researchers, has developed a point-of-care mobile app that provides a COVID-19 severity score based on patients’ biomarker measurements and clinical risk factors.

COVID-19 Antibody Trials Have Begun

Potential antibody treatments for coronavirus disease 2019 (COVID-19) are being tested in early trials, companies recently announced.

Pharmaceutical giant Lilly, in collaboration with Vancouver-based AbCellera Biologics, disclosed on June 1 that the first patients had been dosed in a phase 1 trial. A week later, Lilly and Chinese partner Junshi Biosciences announced a phase 1 study of another investigational antibody treatment, JS016. Regeneron’s dual-antibody cocktail, REGN-COV2, entered trials during the second week of June.

Lilly’s first investigational drug, LY-CoV555, is an intravenously administered neutralizing IgG monoclonal antibody directed against the severe acute respiratory syndrome coronavirus 2 (SARS-CoV-2) spike protein. Researchers at AbCellera and the Vaccine Research Center at the National Institute of Allergy and Infectious Diseases identified the antibody produced by immune cells in a blood sample taken from an early patient who recovered from COVID-19.

The double-blind trial will test safety and tolerability over 2 months. An estimated 40 adult patients diagnosed with COVID-19 within the previous 3 days who do not require mechanical ventilation will be randomized to receive LY-CoV555 or a placebo. The first patients were dosed at the New York University Grossman School of Medicine and Cedars-Sinai in Los Angeles.

If the experimental treatment is found to be safe, a phase 2 trial will test it among nonhospitalized patients. Lilly also expects to study the antibody as a preventive approach for people who cannot receive vaccines. It’s already being manufactured at large scale, “with the goal of having several hundred thousand doses available by the end of the year,” Daniel Skovronsky, MD, PhD, Lilly’s chief scientific officer, said in a statement. – Jennifer Abbasi

Note: Source references are available through embedded hyperlinks in the article text online.