



Parkinson Association of Orange County

Stem Cell Research and Treatment

The news has recently provided information on some amazing breakthroughs in stem cell research. On September 24th, 2013, Dr. Peter Saunders writing on LifeNews.com had the following to say: “Researchers have for the first time converted cultured skin cells into stem cells with near-perfect efficiency. The discovery could clear the way for scientists to produce large volumes of stem cells (induced pluripotent stem cells (iPS)) on demand, hastening the development of new treatments for conditions like Parkinson’s Disease, spinal injury and diabetes.

Since 2006, the production of stem cells remained mysteriously inefficient with many failing to grow as desired. Now, as reported in Nature (Nature 502, 65-70 (03 October 2013)) a team at the Weizmann Institute in Israel has been able to increase the conversion rate to almost 100% (ten times normal) by removing a single protein, called Mbd3, from the cells. Moreover, the researchers show that the cells all transition to pluripotency on a synchronized schedule in about seven days. The work has been proven using both mouse and human cells.”

Ethical concerns from the past about using embryonic stem cells are no longer an issue since induced pluripotent stem cells were discovered in 2006, a feat which was honored by a Nobel Prize given to John B. Gurdon and Shinya Yamanaka last year. Another advantage of IPS cells is that they should be less prone to rejection by the patients’ immune systems, because the transplanted cells come from the individuals themselves.

Using Induced Pluripotency Cells is a key to stem cell technology making a difference in the lives of people with Parkinson’s Disease as well as many other disorders. In a June 12th, 2013 interview with KPBS, a San Diego radio station, Melissa Houser, MD, a neurologist and medical director of Scripps Clinic’s Parkinson’s Disease and Movement Disorders Center, commented on research done in the Scripps Clinic.

Under her leadership, skin cells taken from Parkinson’s Disease patients who meet select criteria are being cultivated in vitro and turned into pluripotent stem cells. “By using your own skin cells, we devolve them into what’s called a pluripotent stem cell that is able to convert into any cell we want it to be, a bone cell, brain cell, a heart cell. And once we get the cell primed to become a brain cell, we turn it into a dopamine-producing cell. And dopamine is what’s missing in Parkinson’s,” says Dr. Houser.

When asked by KPBS how often this particular procedure has been used in any kind of medical research, Dr. Houser replied: “Well the use of iPS is quite common. There are lots of institutes doing this sort of cell work. But most of them are doing it to check how drugs can work on that cell line. And some of them are doing animal trials. Our trial is trying to take it into the rodent phase with a view toward using it as actual therapy for Parkinson’s Disease, instead of keeping it in the lab.”

The plans, which will require FDA approval, are to implant these cells back into the donor patients’ brains. Preliminary research will likely be completed in 2013. The clinical trial is slated to launch two or three years later, depending upon the timing of FDA approval.

While these breakthroughs are wonderful and give hope to the many people with Parkinson’s and other neurological disorders, take notice of the timetable above for clinical trials. There are currently no treatments based on these breakthroughs being offered which have been through the clinical trials needed to ensure their safety for humans and there has been no FDA approval yet for this type of stem cell treatment.

According to the International Society for Stem Cell Research (ISSCR), in a statement made on September 12, 2013, “the current uses of stem cells for treatment of blood or skin-related problems have proven successful. However for virtually all other diseases or conditions, researchers have not yet proven the clinical safety and effectiveness of the collection and administration of a patient’s own cells.” To assist patient decision-making the ISSCR published the *Top Ten Things to Know About Stem Cell Treatments*. (see <http://www.isscr.org>) A few notable items from the list include the following:

#6. To be used in treatments, stem cells will have to be instructed to behave in specific ways. (Be wary of claims that stem cells will somehow just know where to go and what to do to treat a specific condition.)



Parkinson Association of Orange County

#7. Just because stem cells came from your body doesn't mean they are safe.

#9. An experimental treatment offered for sale is not the same as a clinical trial.

The fact that a procedure is experimental does not automatically mean that it is part of a research study or clinical trial. Beware of expensive treatments that have not passed successfully through clinical trials.

Clearly, patients interested in becoming involved with new Parkinson's research programs should realize these are experimental in nature. Great care must be taken to thoroughly investigate what is offered and read the "fine print" about the treatment. Common disclaimers posted by these programs in their literature and on their websites include the following:

- "(Our) treatment centers are not offering stem cell therapy as a cure for any condition, disease, or injury."
- "No statements or implied treatments ... have been evaluated or approved by the FDA."
- "We do not claim that these treatments work for any listed nor unlisted condition, intended or implied."

Conclusion:

There have been spectacular discoveries in just the last few years which have moved research past some difficult hurdles, but FDA approval of clinical trials for human treatment with stem cells for Parkinson's Disease is still a few years away. In the meantime, patients eager for help must be very cautious about the many claims made by clinics in the marketplace. There is no magic solution yet but there is good reason to have hope for the future.

Ted Napolitano



Parkinson Association of Orange County