New Study Shows Stem Cells’ Promise as Future ALS Treatment

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Durham, NC (PRWEB) February 14, 2013 -- A new study in the current issue of STEM CELLS Translational Medicine demonstrates how human stem cells can successfully engrat, survive and differentiate into mature neurons in the spinal cord of a rat with amyotrophic lateral sclerosis (ALS). The results offer new hope for those suffering from this disease, which generally ends in death within three to five years after diagnoses.

ALS (commonly known as Lou Gehrig’s disease) is characterized by the degeneration and death of the body’s motor neurons, leading to muscle atrophy, paralysis and death due to failure of the respiratory muscles. Despite studies that have improved our understanding of how ALS develops, there are no effective treatments. However, stem cell based-therapies have emerged as a potential solution.

“The transplantation of stem-cell derived neural progenitors may have beneficial effect not only for the replacement of motor neurons already lost, but also in counteracting degeneration and death of motor neurons,” said Roland Pochet, Ph.D., of the Université libre de Bruxelles, Belgium. He headed up the research team that included scientists from INSERM et Université Paris-Sud, and the Pasteur Institute, also in Paris, and Hannover Medical School in Germany.

Spinal motor neurons have been successfully generated from various sources such as embryonic stem cells (ESCs) and neural stem cells (NSCs). Studies also have evaluated the therapeutic potential of bone marrow-derived human mesenchymal stem cells (MSCs) and human umbilical cord blood cells (UCBCs), but modest or no therapeutic benefit was obtained when transplanted in ALS patients.

In theory, induced pluripotent stem cells (iPSCs) derived from patients with neurodegenerative diseases, such as ALS, could be used to reverse the diseases. However, no report had yet described the fate of transplanted iPSCs into an ALS environment.

In the current study, the team wanted to learn how human-induced pluripotent stem cell- (iPSc) derived neural progenitors might affect ALS. The idea was inspired by a previous study in which they injected ALS rats with NSCs derived from other rats. “Although these cells undergo a massive apoptosis, after a few days of injection several survived, crossed the blood-brain barrier, differentiated and engrafted into the animals’ spinal cords,” Dr. Pochet explained.

Sixty days after transplantation, the iPSc-derived cells had efficiently engrat in the rat’s spinal cord and were surviving, the team reported. Different neural progenitor, tissue and neuronal markers indicated that, over time, the transplanted cells differentiated into cells displaying a neuronal phenotype, the team learned.

“Our results,” Dr. Pochet said, “demonstrate proof-of-principle of survival and differentiation of human iPSc-derived neural progenitors in in vivo ALS environment, offering perspectives for the use of iPSc-based therapy in ALS.”
“This report of the ability of iPSCs to survive and differentiate in an ALS environment is certainly encouraging,” said Anthony Atala, MD, Editor of STEM CELLS Translational Medicine and director of the Wake Forest Institute for Regenerative Medicine. “The results suggest the potential of cell therapy for the field of neurobiology and disease treatment.”

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The full article, “Neural progenitors derived from human induced pluripotent stem cells survive and differentiate upon transplantation into a rat model of Amyotrophic Lateral Sclerosis,” can be accessed at [http://www.stemcellstm.com](http://www.stemcellstm.com).

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