

# Using bone marrow to protect the brain

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The ability to produce neuroprotectors, proteins that protect the human brain against neurodegenerative disorders such as Parkinson's and ALS, is the holy grail of brain research. A technology developed at Tel Aviv University does just that, and it's now out of the lab and in hospitals to begin clinical trials with patients suffering from amyotrophic lateral sclerosis (ALS), also known as Lou Gehrig's disease.

Developed by Prof. Daniel Offen and Prof. Eldad Melamed of TAU's Sackler Faculty of Medicine and Felsenstein Medical Research Center, the technology is now a patent-pending process that takes <u>stem cells</u> from a patient's own bone marrow and causes them to differentiate into astrocyte-like cells, which are responsible for the well-being of the brain's neurons. The cells release neurotrophic factors, or neuroprotectants, which have been shown to play a key role in reducing the progress of ALS, a debilitating disease characterized by the progressive degeneration of motor neurons, resulting in paralysis of a patient's limbs and <u>organ function</u>.

The research has appeared in the *Journal of Stem Cells Reviews and Reports* and a number of other publications.

#### **Trials in Jerusalem and Boston**

This stem cell technology, says Prof. Offen, represents 10 years of development. Inspired by advances in <u>embryonic stem cell research</u> and its huge potential – but trying to bypass the ethical and safety issues – Prof. Offen and his fellow researchers turned to stem cells derived from



a patient's own bone marrow.

After coaxing the cells to differentiate into astrocyte-like cells, whose natural function is to guard the brain's neurons and prevent deterioration, the researchers began testing the concept in animal models. "In the mouse model," Prof. Offen explains, "we were able to show that the bone marrow derived stem cells prevent degeneration in the brain following injection of selective neurotoxins." Researchers also demonstrated that transplantation of these cells increased the survival rate in the mouse model of ALS and significantly delayed the progress of motor dysfunction.

According to Prof. Offen, this is a uniquely successful method for differentiating bone marrow stem cells into astrocyte-like cells without manipulating the genetic material of the cell itself. They are the first team of researchers to demonstrate the efficacy of this technology in vivo in various models of neurodegenerative diseases.

The technology was licensed to BrainStorm Cell Therapeutics that has developed it into a clinical grade product called NurOwn<sup>TM</sup>, which is now being used in a clinical trial at Jerusalem's Hadassah Medical Center. BrainStorm Cell Therapeutics has recently struck an agreement to expand clinical trials to Massachusetts General Hospital in collaboration with the University of Massachusetts Medical School.

## Home-grown therapy — and talent

The ongoing clinical studies are aimed at evaluating the safety and the efficacy of this treatment, says Prof. Offen. Because the original cells are drawn from the patients themselves, he adds, the body should have no adverse reactions.

Although the current study targets ALS, these cells have the potential to



treat a broad range of neurodegenerative conditions, including Parkinson's and Huntington's diseases. For many conditions, explains Prof. Offen, the current available treatments only attempt to alleviate the symptoms of these diseases rather than repair existing damage.

### Provided by Tel Aviv University

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