



The ALS Association
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Media Release

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ALS Association Funded Lead Investigators Demonstrate That Modified Adult Stem Cells Show Promise in a Rat Model of ALS

CALABASAS HILLS, Calif. (September 22) – Lead investigators at the University of Wisconsin, Madison demonstrate improved survival by 28 days in a rat model of ALS after direct muscle administration of modified adult stem cells carrying a growth factor, glial cell-line derived neurotrophic factor (GDNF). This work builds on previous studies in which the team delivered modified stem cells expressing this factor to the spinal cord. Although direct injection into the spinal cord successfully spared the motor neurons, there was no change in survival of the animals.

The investigators suggested that the lack of increased survival could be explained by the inability of motor neurons to reconnect at the muscle. Indeed growing evidence suggests that loss of connections at the neuromuscular junction occurs early on in the disease process and attempts to restore this process could have therapeutic benefit. See <http://www.ncbi.nlm.nih.gov/pubmed/18797452>.

ALS Association-funded lead investigator Masatoshi Suzuki, Ph.D., and Clive Svendsen, Ph.D., attempted to address the inability of surviving motor neurons to reconnect at the neuromuscular junction by isolating human mesenchymal stem cells found in bone marrow and engineered them to express a marker, green fluorescent protein (GFP) and GDNF. They first determined whether

such transplanted cells could survive after injection into skeletal muscle and whether GDNF production could be measured at the injection site.

Not only did this approach increase survival, with increased neuromuscular connections and increased numbers of motor neurons, there was also functional improvement of the rats. These preliminary findings provide important data for consideration when applying stem cell therapeutic approaches in the clinic. Investigators are working on methods to enhance the effect in the model system and cautioned that there is still a great deal of work to be done to bring this approach to the clinic.

“We are very pleased to be providing the necessary support for these and other stem cell projects at the University of Wisconsin. The investigators together with their international collaborators including Patrick Aebischer, Ph.D., have embarked on key studies and their rigor and attention to detail ensures reliable data for further development of these therapeutic approaches,” said Lucie Bruijn, Ph.D., senior vice president, research and development.

There has been much excitement about the potential of stem cell therapy for ALS and other neurological disorders. Although there are still many hurdles to overcome in these complex disorders, findings such as these are critical to move the field forward. Investigators in the stem cell field will be coming together today and tomorrow in Madison, Wis., to discuss opportunities in stem cell therapy at the 2008 World Stem Cell Summit. For more information <http://www.worldstemcellsummit.com/>

For a list of recently funded projects at the University of Wisconsin:

- ▶ [Modeling Human Motor Neuron Degeneration Using Stem Cells](#)
- ▶ [Using iPS cells to generate ALS patient specific stem cell lines](#)