

A Phase I, Open-label, Single-site, Safety Study of Human Spinal Cord-derived Neural Stem Cell Transplantation for the Treatment of Chronic Spinal Cord Injury

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Introduction

Spinal cord injury (SCI) resulting in paraplegia or quadriplegia is a significant burden in the world. It is estimated that there are approximately 250,000 people living with SCI in the United States alone. Our study offered the direct implantation of human-derived stem cells into the spinal cord of subjects who suffer from chronic SCI. The primary objective of the study is to determine the safety and toxicity of human spinal stem cell transplantation for the treatment of paralysis. The secondary objectives of the study are to evaluate (1) graft survival in the transplant site (2) effectiveness of transient immunosuppression as determined by absence of donor-specific HLA antibodies (3) potential therapeutic role of implantation on motor and sensory function in SCI.

Methods

This is a Phase I, open-label, single-site, study of human spinal cord-derived neural stem cell (HSSC) transplantation for the treatment of chronic SCI. 4 subjects with chronic SCI with ASIA A SCI who met eligibility criteria were enrolled. All subjects received spinal cord injections of HSSC. The treatment consisted of removal of spinal instrumentation followed by direct injections into spinal parenchyma. 6 HSSC injections were administered in each subject. Each injection consisted of 2×10^5 cells in $10 \mu\text{L}$.

Learning Objectives

By the conclusion of this session, participants should be able to: 1) Describe the background, hypothesis, goal and results of our trial. 2) Understand and advance their own knowledge of current potential therapeutic options for patients with SCI. 3) Continue to understand how stem cell therapies and research may advance the field of neurological surgery and neural regeneration.

Results

4 subjects have been implanted to date. All subjects tolerated the procedure well and there have been no major adverse events to date. Prospective data has been collected including ISNCSCI scores, ASIA level, functional and pain surveys, SCIM scores, Sensory and Motor Evoked potentials, EMG, and MRI including a novel sequence of MR spinal diffusion tensor imaging (DTI). These metrics are continuing to be collected and evaluated.

Conclusions

We can conclude that (1) HSSC transplanted into the injury site of a spinal cord in chronic spinal cord injury patients can be done safely. (2) The HSSC graft has been shown to have no major adverse event to date.

References

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