

A new minimally invasive device to deliver stem cells to the spinal cord of patients with ALS.

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Introduction

Amyotrophic lateral sclerosis (ALS) results in loss of motor neurons in the spinal cord and there is no known etiology, treatment, or cure. Preclinical studies have shown that glial cell line-derived neurotrophic growth factor (or GDNF) can protect motor neurons from damage in animal models that may include ALS. There is an ongoing Phase 1 clinical trial

<https://clinicaltrials.gov/show/NCT02943850> designed to deliver GDNF producing stem cells into the spinal cord of patients with ALS that could potentially slow the death of motor neurons causing paralysis.

Methods

Patients with ALS are treated with an investigational new device that utilizes a minimally invasive surgical retractor that is anchored to the operating room table. The device has precision stereotactic adjustment capabilities designed for delivery of stem cells into the lumbar region of the spinal cord in these FDA approved experimental procedures. Radiologic localization of the 4th and 5th lumbar segments of the spinal cord are targeted from MRI measurements in the ventral horn region of the spinal cord.

Results

Six patients with ALS have been treated so far in this Phase 1 non-randomized study, and no significant adverse events related to the device have been seen. There has been short-term transient pain in the lower extremity. Post-operative imaging has not demonstrated any changes in the spinal cord parenchyma

Conclusions

Precision delivery of these stem cells into the spinal cord with a new minimally invasive device appears to be feasible based upon early results of this study. Completion of this Phase1 study is ongoing and will be completed in the next calendar year and then final results will be reported. At this early stage, technical and safety goals associated with this FDA approved minimally invasive investigational device appear to be attainable.

Learning Objectives

1. New minimally invasive device is being used for stem cell delivery
2. GDNF producing stem cells are a first in man for patients with ALS

References