INTRASPINAL STEM CELL TRANSPLANTATION IN ALS: RESULTS OF A PHASE 1/2 CLINICAL TRIAL

E.L. Feldman[1], N.M. Boulis[2], S. Goutman[1], K. Johe[3], S.B. Rutkove[4], P. Patil[5], J.D. Glass[6]
[1] *Department of Neurology, University of Michigan, Ann Arbor, MI*
[2] *Department of Neurosurgery, Emory University, Atlanta, GA*
[3] *Neuralstem, Inc., Rockville, MD*
[4] *Department of Neurology, Beth Israel Deaconess Medical Center, Boston, MA*
[5] *Department of Neurosurgery, University of Michigan, Ann Arbor, MI*
[6] *Department of Neurology, Emory University School of Medicine, Atlanta, GA*

The FDA-approved trial, "A Phase 1, Open-label, First-in-human, Feasibility and Safety Study of Human Spinal Cord-derived Neural Stem Cell Transplantation for the Treatment of Amyotrophic Lateral Sclerosis, Protocol Number: NS2008-1," has been completed in 15 patients with amyotrophic lateral sclerosis (ALS). Our overall objective was to assess the safety and feasibility of stem cell transplantation into lumbar and/or cervical spinal cord regions in ALS. Patient cohorts consisting of 3 ALS patients each followed a "risk escalation" paradigm progressing from non-ambulatory to ambulatory patients receiving unilateral (n=5) or bilateral (n=10 total) lumbar or cervical injections. The final cohort of 3 patients, Group E, received cervical injections and had previously received bilateral lumbar injections. All injections delivered 100,000 cells in a 10 µl volume, for a dosing range between 500,000 to 1.5 million cells over the 18 surgeries. The procedure was well-tolerated by all patients with minimal perioperative or postoperative complications. Although this was a safety trial, clinical progression was monitored and will be reported. Advanced analyses on Group E outcome data revealed preliminary insight into potential windows of stem cell biological activity and identified assessment measures that closely correlate with disease progression. Overall, results demonstrate that lumbar, cervical and dual-targeted intraspinal transplantation of stem cells in ALS patients is feasible and well-tolerated, supporting future trial phases examining therapeutic dosing and efficacy. Phase 2 of the trial commenced September 2013 and was completed on July 30 2014; initial results from this trial will also be reported.

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