Stem cell treatment for Lou Gehrig's disease may be safe

June 29 2016

A phase II clinical trial in people with amyotrophic lateral sclerosis (ALS), or Lou Gehrig's disease, suggests that transplanting human stem cells into the spinal cord may be done safely. The research is published in the June 29, 2016, online issue of *Neurology*, the medical journal of the American Academy of Neurology. While the study was not designed to determine whether the treatment was effective, researchers noted that it did not slow down the progression of the disease.

ALS is a disorder in which motor neurons, found in the brain and spinal cord, degenerate. The disease results in progressive loss of muscle control, including breathing and swallowing, leading to death. There are currently no treatments that can stop the disease.

"Though there were two serious complications related to the treatment, the level of acceptable risk for treating patients with ALS, where the prognosis is poor and treatments are limited, is arguably higher than that for more benign disorders," said Jonathan D. Glass, MD, a professor of neurology at Emory University School of Medicine in Atlanta and a member of the American Academy of Neurology.

The study involved 15 people with ALS at three university hospitals. All had their first ALS symptoms within two years of the start of the study. The participants were divided into five treatment groups that received increasing doses of stem cells by increasing numbers of injections. The trial was open-label, meaning the participants knew they were getting the active stem cell treatment.

All participants received bilateral injections into the cervical spinal cord between the C3 and C5 regions. The final group received injections into both the lumbar (L2-L4) and cervical cord through two separate surgical procedures. The numbers of injections ranged from 10 to 40, and the number of cells injected ranged from two million to 16 million. During the nine months of follow-up, researchers collected information about side effects and measured progression of the disease using the ALS functional rating scale.

Most of the side effects were related to temporary pain associated with surgery and to medications that suppress the immune system. Two people developed serious complications related to the treatment. One person developed spinal cord swelling that caused pain, sensory loss and partial paralysis and one person developed central pain syndrome.

The participants' functioning was compared to three historical control groups, and there was no difference in how fast the disease progressed between those who received stem cells and those who did not. However, Glass cautioned that a conclusion should not be made about effectiveness of the treatment from the small study.

"This study was not designed, nor was it large enough, to determine the effectiveness of slowing or stopping the progression of ALS. The importance of this study is that it will allow us to move forward to a larger trial specifically designed to test whether transplantation of human stem cells into the spinal cord will be a positive treatment for patients with ALS," Glass said.

Provided by American Academy of Neurology