
Stem Cell Therapy For Cerebral Palsy

In his brilliant and award-winning, yet slightly disturbing and controversial novel “Stuck in Neutral”, Terry Trueman narrates the fictional story of Shawn McDaniel, a fourteen-year-old boy with Cerebral Palsy. Shawn, self-portrayed as happy and more intelligent than most, is robbed of his ability to control his muscles, suffers from frequent seizures, and is viewed by others as retarded. This leaves him feeling trapped in his own body, and completely dependent on caregivers, mostly his mother, for his activities of daily living. What’s more? Shawn believes his father, out of love, intends to murder him to end his supposed suffering.

Trueman’s depiction of cerebral palsy, though fictional and perhaps slightly distorted, paints a very vivid picture of the devastating nature of this disorder both for patients and relatives/caregivers of children with cerebral palsy. Sadly, despite the neuroscience community’s giant strides in recent years, currently available treatments for cerebral palsy remain largely supportive and directed at rehabilitation with very limited efficacy. Recent attempts at developing more novel and effective therapies targeting cerebral injury have focused on the use of stem cells, special human cells that can develop into many different cell types. These stem cells, present in different locations in the body including the bone marrow, umbilical cord etc., also have regenerative and anti-inflammatory properties making them very attractive strategies for developing a possible cure for cerebral palsy.

Studies using this stem cell approach have reported improvements in gross motor function and alleviation of brain lesions in animal models. However, there have been relatively fewer clinical research involving humans, leaving a bench-to-bedside gap that needs bridging.

In a recent research, Jiaowei Gu and her colleagues in China conducted a randomized trial to determine the safety and efficacy of stem cells derived from the human umbilical cord with concomitant rehabilitation in the treatment of cerebral palsy. The team recruited 40 children with cerebral palsy aged 2-12 years and randomly assigned them, in a double blinded manner, to one of two groups: a test (umbilical cord-derived stem cell transplant) group and a control (placebo) group.

Participants in the test group received a total of 4 doses of stem cell transplantation via intravenous administration, each dose 7 days apart, while the control group received an equal number of doses of Albumin in Normal Saline infusion as placebo. Both groups of patients were placed on a form of physical rehabilitation called Bobath therapy. At scheduled follow-up visits 1, 3, 6, & 12 months after the last dose of respective interventions, the participants were monitored for possible adverse reactions to the interventions as well as for improvements across 3 domains of functioning: Gross Motor Function, Comprehensive Function, Activities of Daily living using respective instruments/scales.

The researchers found the stem cell transplant to be safe, with only mild-moderate adverse reactions reported. They also observed that participants who received the stem cell transplant in combination with physical rehabilitation showed significantly greater improvements across all 3 domains of functioning assessed (Figure 1). These improvements were observed even in the older subjects, providing evidence that the therapeutic window of the transplant extends to

years after the brain injury. Other interesting findings reported include a reduction in inflammatory mediators and an increase in cerebral metabolic activity, as seen on a PET/CT scan, in a subset of patients who received the transplant compared to the placebo group, offering some insight into the possible mechanisms underlying the observed effects.

The findings from this very insightful research provide a much-needed beacon of hope for children like the fictional Shawn McDaniel living with cerebral palsy. With more extensive and large scale translational research built on these insights, perhaps cerebral palsy research in the near or distant future can find a cure for the disorder or at the very least, significantly improve the quality of life of affected individuals.

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