MESENCHYMAL STEM CELLS FOR TREATING CEREBRAL PALSY

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Cerebral palsy is a lifelong condition characterized by movement disorders and difficulties in maintaining posture, resulting from non-progressive brain injuries. Currently, there is no cure, but new therapies involving mesenchymal stem cells (MSC) offer hope for potential treatments that could repair central nervous system damage. Some studies suggest that stem cells may improve the damaged environment by acting through paracrine pathways rather than replacing neurons. Recent clinical trials have demonstrated the safety of human umbilical cord (Wharton's Jelly) mesenchymal stem cells (UC-MSC) and their potential to enhance motor functions in children with cerebral palsy, along with improving clinical and imaging outcomes.

In May 2024, a collaborative paper by the Medical University of Lublin and the Polish Stem Cell Bank was accepted by the Stem Cell Reviews and Reports journal and is available ahead of print at PubMed (https://pubmed.ncbi.nlm.nih.gov/38877284/). This paper details the treatment results of 152 children (84 boys and 68 girls, median age 5 years) with cerebral palsy, who received up to two courses of five MSC injections. Product was provided by the The Polish Stem Cell Bank. A previous study by our colleagues: Dariusz Boruczkowski & Izabela Zdolińska-Malinowska reported subjective improvements in quality of life and self-sufficiency following Wharton's Jelly-derived UC-MSC administration. The current study aimed to analyze the impact of UC-MSC on motor function.

In this study, each treatment course included five intravenous or intrathecal UC-MSC injections administered every two months. Depending on the patient's body mass, different doses were used (10, 20, 30, or 40 million cells per injection) to achieve a final dose of 1 million MSC per kilogram of recipient body weight. Motor functions were assessed using the Gross Motor Function Measure (GMFM), 6-Minute Walk Test (6-MWT), Timed Up and Go test (Up&Go test), and Lovett's test for evaluation of muscle strength. Mental abilities were assessed with the Clinical Global Impression (CGI) scale. The stem cell therapy was provided on a compassionate use basis under the legal framework of a hospital exemption procedure of administration pf Advanced Therapy Medicinal Product (ATMP) as classified by the European Medicines Agency Committee for Advanced Therapies.

Following UC-MSC administrations, improvements were observed in all evaluated parameters. The GMFM score increased by a median of 1.9 points (IQR: 0.0-8.0), with changes seen across all GMFM areas. A median increase of 75 meters (IQR: 20.0-115.0) was noted in the 6-MWT, correlating with GMFM score changes. The Up&Go test time decreased by a median of 2 seconds (IQR: -3 to -1), correlating with age, baseline GMFM score, and the 6-MWT results. Slight improvements in muscle strength were noted in Lovett's test. According to the CGI, 75.5% (96/151) of children were seriously or significantly ill at the first visit, with improvements observed in 63.6% (96/151) by the fifth visit, and 23.8% (36/151) showing improvement or great improvement.

In conclusion, mesenchymal stem cell therapy enhanced functional performance in patients, though individual responses varied. The therapy benefited children with high levels of disability but to a lesser extent than those with lower levels of disability. Younger patients responded better, but older children also showed benefits.

Based on earlier studies and this report, early intervention in children at high risk of cerebral palsy is crucial to achieve better outcomes, emphasizing the need to shift MSC interventions from hospital exemptions, experimental procedure to first-line treatments for cerebral palsy, as current treatments are not sufficiently effective.

References:

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