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Outcomes of autologous bone marrow mononuclear cell infusions for cerebral palsy in children

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Background and purposes: Cerebral palsy (CP) is common disability in children. The results of traditional treatments are very limited. Recently, cell therapy is emerging as a treatment for many neurological diseases. The purpose of this report is to present the outcomes of autologous bone marrow mononuclear (BMMNC) infusions in management of children with cerebral palsy.

Methods: The Gross Motor Function Measure (GMFM)-88, which consists of 88 items categorized into five domains: (1) lying and rolling; (2) sitting; (3) crawling and kneeling; (4) standing; and (5) walking, running and jumping, and, the muscular spasticity [Modified Ashworth Scale (MMAS) were used to assess the motor function for CP patients. German Coma Remission Scale and Gross Motor Function Measure (GMFM)-88 were used to assess the recovery of patients with vegetative state after nearly death drowning.

Bone marrow was harvested by anterior iliac crest puncture under general anesthesia. The volume collected was as follows: 8 mL/kg for patients under 10 kg (80 mL + [body weight in kg - 10] \times 7 mL) for patients above 10 kg. Mononuclear cells were isolated with a Ficoll gradient and then infused intrathecally.

Results: From 2015-2019, 64 patients with CP received autologous bone marrow mononuclear cell infusions including 30 children with CP due to oxygen deprivation, 25 children with CP related to neonatal kernicterus, 4 patients with CP due to intracranial hemorrhage (ICH) incidence during the neonatal period, 5 patients with vegetative state after nearly death drowning. All infusions were performed safety without any severe adverse event. Significant changes were observed in the children's gross motor function and muscle spasticity, as evidenced by the GMFM-88 total score, scores for each of its domains, the GMFM-66 percentile and the muscle tone. All five patients with with vegetative state after nearly death drowning displayed improvement across the major parameters of gross motor function, cognition, and muscle spasticity. Three patients displayed improved communication including the expression of words. In particular, one patient remarkably reduced cerebral atrophy, with nearly normal cerebral parenchyma after BMMNC transplantation.

Conclusion: Autologous BMMNC is safe and effective for children with CP related to oxygen deprivation, to neonatal kernicterus, intracranial hemorrhage in neonatal period and vegetative state after nearly death drowning.

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Biography

Prof. Nguyen Thanh Liem is a pioneer and innovator in pediatric endoscopic surgery and stem cell transplantation. He is internationally recognized inventor by his seven advanced operative techniques in the Pediatric Surgery. Regarding the stem cell studies, Prof. Liem is a pioneer of using stem cell therapy for unmet diseases in children such as cerebral palsy due to oxygen deprivation, due to neonatal icterus, due to intracranial hemorrhage, neurological sequelae after nearly death drowning, Autism, bronchopulmonary dysplasia, and liver cirrhosis due to biliary atresia. He was Director of Vietnam National Children Hospital. Currently, Prof. Liem is the director of Vinmec Research Institute of Stem Cell and Gene Technology. He has published 100 studies on the international journals and over 200 papers on domestic journals. He is also the co-author of surgery pediatrics textbooks published in UK and US such as Operative Pediatric Surgery, Ashcraft's Pediatric Surgery, and Pediatric Laparoscopic and Thoracoscopic Surgery. By his dedicated and delightful contributions to the medical practices and research, he has been honored many prestige awards conferred by the Vietnam government and Nikkei Asia Prize in 2018 in the Science and Technology category thank to his contribution to develop and popularize pediatric endoscopic surgery in Asia and the world. He is categorized in top 100 scientists of Asia by Singapore journal in 2019.