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Outcomes of bone marrow mononuclear cell transplantation combined with interventional education for autism spectrum disorder

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Background and purposes: The prevalence of identified autism spectrum disorder (ASD) is increasing worldwide. Traditional treatment is educational intervention. Unfortunately, many children who receive those treatments remain significantly impaired. The aim of this study was to investigate the safety and clinical outcomes of high-dosage autologous bone marrow mononuclear cell (BMMNC) infusion combined with educational intervention for children with ASD.

Patients and methods: An open-label clinical trial was performed from July 2017 to August 2019 at Vinmec International Hospital, Hanoi, Vietnam. Thirty children who fulfilled the autism criteria of the Diagnostic and Statistical Manual of Mental Disorders, Fifth Edition, and had Childhood Autism Rating Scale (CARS) scores >37 were selected. 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] × 7 mL) for patients above 10 kg. Mononuclear cells were isolated with a Ficoll gradient and then infused intrathecally. The same procedure was repeated 6 months later. After the first transplantation, all patients under weeks of educational intervention based on the Early Start Denver Model.

Results: There were no severe adverse events associated with transplantation. The severity of (ASD) was significantly reduced, with the median CARS score decreasing from 50 (range 40-55.5) to 46.5 (range 33.5-53.5) (P < .05). Adaptive capacity increased, with the median Vineland Adaptive Behavior Scales score rising from 53.5 to 60.5. Social communication, language, and daily skills improved markedly within 18 months after transplantation. Conversely, repetitive behaviors and hyperactivity decreased remarkably.

Conclusion and significant: Autologous bone marrow mononuclear cell transplantation in combination with behavioral intervention was safe and well tolerated in children with ASD. The combination of cell therapy and educational intervention may improve clinical manifestations such as social communication, language, and daily skills in children with ASD.

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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.