Efficacy of stem cell therapy in ambulatory and non-ambulatory children with Duchenne muscular dystrophy: Phase I–II

Alper Ibrahim Dai[1], Osman Baspinar[1], Ahmet Yeşilyurt[2], Eda Sun[3], Çigdem Inci Aydemir[4], Olga Nehir Öztel[4], Davut Unsal Capkan[5], Ferda Pinarli[2], Abdullah Agar[6] and **Erdal Karaöz[3, 4, 7]**
[1]Gaziantep University, Turkey
[2]Diskapi Yildirim Beyazit Research and Education Hospital, Turkey
[3]Istinye University, Center for Stem Cell Research and Application, Turkey
[4]Liv Hospital – Center for Regenerative Medicine and Stem Cell Research and Manufacturing, Turkey
[5]Deva Hospital, Turkey
[6]University of Travnik, Bosnia and Herzegovina
[7]Istinye University, Turkey

Purpose: Duchenne muscular dystrophy (DMD) is an X-linked recessive paediatric disorder that ultimately leads to progressive muscle degeneration. It has been known that cell-based therapies were used to promote muscle regeneration. The main purpose of this study was to investigate the effects of allogeneic Wharton jelly-derived mesenchymal stem cells therapy in Duchene muscular dystrophy.

Patients & Methods: Four ambulatory and five non-ambulatory male patients were assessed as having acceptance criteria. Gene expression and immunohistochemical analysis were performed for *dystrophin* gene expression. The fluorescent *in situ* hybridization method was used for detection of chimerism and donor–recipient compatibility. Complement dependent lymphocytotoxic crossmatch test and detection of panel reactive antigen were performed. All patients were treated with 2 × 106 cells/kg dose of allogeneic Wharton jelly derived mesenchymal stem cells via intra-arterial and intramuscular administration. Stability was maintained in patient follow up tests, which are respiratory capacity tests, cardiac measurements and muscle strength tests.

Results: The vastus intermedius muscle was observed in one patient with MRI. Chimerism was detected by fluorescent *in situ* hybridization and mean gene expression was increased to 3.3-fold. An increase in muscle strength measurements and pulmonary function tests was detected. Additionally, we observed two of nine patients with positive panel reactive antigen result.

Conclusion: All our procedures are well tolerated and we have not seen any application related complications so far. Our main purpose of this study was to investigate the effects of allogeneic mesenchymal stem cell therapy and determine its suitability and safety as a form of treatment in this untreatable disorder.

Biography

Alper İbrahim Dai, graduated from Istanbul University, School of Medicine. He completed his paediatric residency at Jackson Memorial Hospital, University of Miami / Florida and Children's hospital of West Virginia at Morgantown. As a fellowship he had 3 years of paediatric neurology at SUNY, Children's Hospital at Buffalo / New York. He had 1 year of EEG / Epilepsy fellowship at Vanderbilt University, Nashville / Tennessee. He has been working at Division of Pediatrics Neurology at Gaziantep University Hospital, Gaziantep / Turkey for 15 years. His major interests are, phase III clinical trial in anti-epileptic medications and phase I and II clinical trials in stem cell therapy in children with neurological problems..

dai.alper@gmail.com