EFFICACY OF FETAL STEM CELL TRANSPLANTATION IN AUTISM SPECTRUM DISORDERS: A PILOT STUDY

Introduction: Autism spectrum disorders (ASDs) are heterogeneous complex neuro-developmental pathologies characterized by abnormalities in social interactions, with the presence of impaired communication, restricted interests, and repetitive stereotypic behaviours. Prevalence rate of autism is quickly increasing. Despite much research efforts, there are still no defined mechanisms of ASDs pathogenesis, rendering autism management very difficult, without defined standard approach. Current pharmacological treatments are directed at the associated behavioural symptoms, without affecting all core symptoms of ASDs. Cell therapy represents the great promise for the future of molecular and regenerative medicine. Several types of cells offer a valid approach to curing several untreatable human neurodegenerative diseases. New perspectives for ASDs therapy are provided by stem cells. Based on recent advances in the understanding of immunological pathologies associated with ASDs, it appears stem cell therapies could be designed to target the observed molecular mechanisms of these disorders. Among stem cell populations, fetal stem cells (FSCs) have immune-regulatory functions found in mesenchymal stem cells, but in addition, exhibit a potent expansion capacity and plasticity, showing a great potential for clinical use. Indeed, FSCs are more rapidly, easily, and efficiently reprogrammed to pluripotency than neonatal and adult cells. Furthermore, these cells are more primitive, thus having greater multi-potentiality than their adult counterparts.

This study investigated the safety and efficacy of FSC transplantations in treating children with ASDs.

Methods: 15 children (7 male, age: 7.43±4.89 and 8 female, age: 6.39±3.29) diagnosed with ASDs in accordance to Diagnostic and Statistical Manual of Mental Disorders (DSM-IV) were enrolled. ASDs patients received two FSC injections: intravenously and subcutaneously, respectively. Autistic children were timely monitored at 0, 1, 3, 6, and 12 months after the stem cell transplantations. Autism Treatment Evaluation Checklist (ATEC) test scores were performed to evaluate improvement in autistic behaviours. Laboratory examinations and clinical assessment of adverse effects were performed in order to evaluate treatment safety.

Results: ASD children treated with FSCs observed no adverse effects. There were no significant safety issues related to the treatment. Statistically significant differences were shown on ATEC scores evaluation regarding speech, sociability, sensory and health, as well as the total score, in the treated patients compared to the pre-treatment values (time 0) at several time-points (one, three and six months) post-treatment (p<0.05). ATEC total score values + sem were: 95.2±5.99 at time 0; 84.8±6.12 at 1 month post-treatment; 73.0±6.00 three months post-treatment; 74.0±6.36 at six months post-treatment.

Conclusions: transplantations of FSCs were safety and effective in improve autistic syndromes. Stem cell therapy offers a new therapeutic option for ASD management.