Stem Cell Transplantation Therapy as a Treatment for Autism Spectrum Disorder

By Angelina Tupikova

Autism spectrum disorder (ASD) is a multifactorial disorder that afflicts 1 in 59 individuals nationwide. ASD is prevalent across many ethnicities and socioeconomic backgrounds and tends to occur more often in males [1]. It is characterized by abnormalities in social interactions and communication as well as repetitive behaviors. There are hundreds of genes that are linked to the development of ASD. Most are responsible for the proper development and function of the central nervous system. Currently, there are over 2 million people living with ASD and, unfortunately, there is no cure. The increasing prevalence of ASD and the financial burden associated with ASD requires a creative approach to treatment [2].

Children who are currently diagnosed with ASD are treated with behavioral interventions for example: speech-therapy, play-based therapy, and behavioral therapy. These interventions tend to focus on the associated symptoms of the disorder rather than the underlying cause. Although this type of therapy has shown signs of progress and improvement, it is insufficient [3]. We need to be doing more. The field is still in dire need of a more effective treatment for ASD and this is where stem cells come into play!

Let's talk stem cells! Stem cell research has been a popular topic of discussion for over two decades. It seems like the possibilities for stem cell-based therapies are endless. Here, I'd like to discuss their potential for therapeutic applications for neurodevelopmental disorders like ASD.

Stem cells are cells that have the potential to develop into specialized cell types of the body—like muscle cells, blood cells, and neurons.. There is also a type of stem cell called mesenchymal stem/stromal cells (MSCs), which have a variety of roles. In the body, MSCs are able to develop into connective tissue cells types, like bone, cartilage, fat and muscle cells. MSCs can also release factors that help regulate the immune system by decreasing inflammation. Interestingly, when MSCs are introduced to patients they seem to modulate the immune system, but are culled from the body before they differentiate into any cell types. There is evidence that some ASD patients suffer from increased inflammation in their central nervous system. What if we used MSC therapy to treat the underlying cause of ASD by focusing on treating the neurological disease instead of the behavioral symptoms [4]?

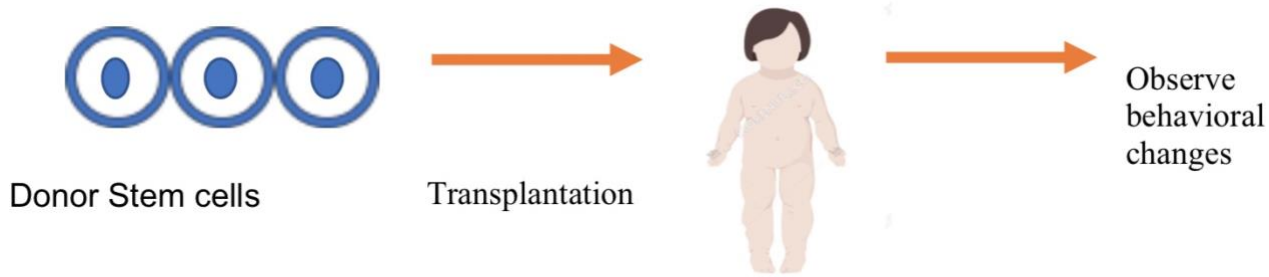

Figure 1: Visual representation of donor stem cell transplantation into humans for therapeutic treatment

Often transplantation therapies involve injecting an individual with healthy stem cells that can be used to replace cells that are not functioning properly (Fig.1). In this case, the MSCs would not replace cells; rather, they would serve to reduce inflammation and thereby enable the brain to restore normal neural connections. In a small phase I clinical trial at Duke University involving 25 children, the intravenous transplantation of MSCs in umbilical cord blood was shown to be safe for the recipient (no adverse effects were reported), and the children exhibited improved socialization, communication and adaptive behavior scores up to a year following the treatment [4]. This is an incredibly promising finding! Scientists will need to expand the number of trial participants and continue assessing the patients to see how long lasting the impact might be. But it is already apparent that MSC immunomodulation is a very promising and safe treatment!

Some stem cell transplantation therapies aimed at introducing new cells have been also tested in clinical trials and the results are promising! For example, in a clinical trial in Italy scientists introduced fetal neural progenitor cells (cells in between neural stem cells and fully formed neurons) and fetal hematopoietic stem cells (HSCs; blood-forming stem cells) to 45 children [6]. Neural progenitors act as a source of new neurons and the HSCs impact the immune system. No adverse side effects were reported, and at the one-year follow-up, the children had significantly improved scores on all behavioral tests! The use of fetal stem cells is much more controversial than MSCs, and safety is a bigger concern. That being said, given how many people are afflicted with ASD, any potentially effective treatment feels like a win for scientists working in the field.

A number of other exciting stem cell trials have been conducted in India, Ukraine, China and the United States with participants ranging from children to young adults [3]. In all of these, the long-term results from the clinical trials demonstrated that there were significant improvements in social communication skills, speech, and decreases in repetitive behavior.

These initial results are exciting and promising for the potential of treating neurological disorders with stem cell-based therapies. Many of the authors note that the trials should be extended beyond a year before they can definitively conclude these treatments are safe, but the results thus far are an exciting step in the right direction. By treating the underlying neurological condition with stem cell transplantation therapy, we could positively impact the lives of millions of people!

References

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