Novel treatment strategies for autism spectrum disorder based on cellular therapy and genomics.

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Background. Autism spectrum disorder (ASD) is a highly heterogeneous neurodevelopmental and behavioral disorder, that still does not have a known treatment for core behavioral disorders. However, there is an increasing amount of ongoing clinical trials, set out to discover novel psychopharmacological mechanisms, cell transplantation methods, epigenetic regulation, and agents affecting the immune system that show promise in the treatment of ASD.
Methods. The current publication is based on narrative review, synthesizing the insight of clinical trials in determining the efficacy of novel ASD treatments from the fields of genomics, cellular therapy, and systems biology. The studies were selected from Science Direct, Springer Link, Oxford Academic, the National Library of Medicine, Nature, MDPI, PubMed, and Genome Medicine. In the study were included only publications with approved clinical trial design methodology, published in the years 2015-2022.
Results. Cell-based therapies have been found efficacious and recommended for ASD to address the neurobiological changes and core behavioral disorders stemming from such changes. The most promising one is stem cell transplantation from autologous umbilical cord blood (AUCB), being proven to be safe and effective in developing social and communication abilities by increasing white matter connectivity in the brains structure, the mechanism is presumed to decrease neural inflammation through the paracrine effect of the stem cells. Another effective novel molecular treatment is the transcriptional regulation targeting agent *tideglusib* which has the potential to alleviate core symptoms. Furthermore, numerous agents targeting synaptic networks, and immune system cells (astrocytes and microglia) have been trialed for ASD and correlated with improvement of symptomatology in clinical trials.
Conclusions. The current research of cellular therapy in ASD treatment shows promising positive outcomes, but there is an ongoing necessity for placebo-controlled double-blind trials to achieve definitive results, likewise, it requires collaboration and access to progressive genetic testing in genomics and measurement methods to further progress toward identifying the genetic pathogenesis and novel therapeutics in ASD.
Keywords: behavioral disorders, ASD, cellular therapy, autologous umbilical blood, genomics