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Understanding Developmental Disorders through the Lens of Gene Editing and Stem Cells

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Abstract

Due to their role in influencing behaviour, language, learning, as well as bodily health, developmental disorders have considerable concerns. Stem cell therapies as well as gene editing technologies such as CRISPR-Cas9 have been addressed in this research as promising directions toward enhanced understanding as well as treatment. Gene editing makes personalised medicine a reality through the precision fixing of mutations in disorders like autism spectrum disorder as well as Duchenne muscular dystrophy. The stem cells, particularly induced pluripotent stem cells (iPSCs) can be utilised for disease mechanisms to generate customized therapies. Yet still, safe delivery, ethical concerns (germline editing), and equal access continue to be concerning. It points toward the requirement of ethical consideration, rigorous clinical trials, and ongoing research to regulate to use these technologies responsibly for enhanced developmental disorder treatments. This paper explores the use of stem cell therapies, and gene editing to combat developmental disorders. It goes through clinical trials being done with the treatments and evaluates the use of them against developmental disorders.

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Keywords: gene editing, stem cell therapy, developmental disorders, autism spectrum disorder, Duchenne muscular dystrophy.

Introduction

Developmental disorders are disabilities in physical, learning, language, or behaviour areas, caused by cognitive impairments, typically at an earlier age. They result in a lack of function in one or multiple domains, including cognition, motor performance, vision, hearing and speech, and behaviour[1]. Gene editing technologies, like CRISPR-Cas9, enable precise correction of mutations that cause developmental disorders. It offers targeted therapies, potentially providing long-term solutions for conditions like Duchenne muscular dystrophy (DMD) [2], and autism spectrum disorder[3]. While promising, challenges remain in the safe use of gene-editing tools and addressing ethical concerns. Although ongoing advancements point to future breakthroughs in treating these disorders at the genetic level to determine the role of genetics on disease by promoting the creation of more accurate cellular and animal models of pathological processes are still of critical importance [4]. Stem cells are used to cure specific diseases, illnesses, and conditions such as blindness, organ growth for transplants, infertility, dementia etc. They are also easy to get a hold of, effectively reduce pain, and have lower risk of rejection of stem cell treatment in the body. Stem cell treatment, and gene editing tools provide customisable medicine, which makes them potential treatment methods for developmental disorders, but also feature many ethical challenges[5].

Overview of Developmental Disorders

Developmental disorders are chronic conditions which develop due to impairments (physically or mentally) which arise pre-adulthood[6]. The nature vs nurture debate takes a role

in this, as both environmental and biological aspects play a role in the disease progression. Individuals with such disorders are faced with stigma, they are alienated by society. DMD is a severe inherited muscular dystrophy it is characterised by the progressive weakness and degeneration of the muscle[7,8]. It is an X-linked recessive neuromuscular disorder, it is caused by mutations in the DMD gene, which leads to the absence or decreased amount of the dystrophin protein[7,9]. There is no modal treatment, and patients usually die in their twenties due to respiratory muscle weakness or cardiomyopathy[8]. The DMD gene has 79 exons and is estimated to be 2.3 million base pairs long[10] . With the greatest expression in cardiac and skeletal muscle, the gene outperforms tissue-specific transcription and makes up a substantial 14,000 bp mRNA[10,11] . The 427 kDa protein that results from the translation of this mRNA has four primary functional domains: a cysteine-rich domain, a central rod domain with 24 spectrin-like repeats, an N-terminal actin-binding domain, and a C-terminal domain[10 12]. The final dystrophin protein is an essential component of the dystrophin-associated protein complex (DAPC), which connects the extracellular matrix and intracellular actin cytoskeleton by interacting with a variety of proteins, including syntrophins, neuronal nitric oxide synthase (nNOS), and β-dystroglycan[10–12]. Mutations in the DMD gene averts the appropriate construction of the mRNA and/or the production of the dystrophin protein[13]. This leads to membrane instability, calcium overload, and increased oxidative stress in muscle cells, triggering inflammatory cascades and muscle degeneration[14].

Down syndrome is the birth of children with an extra chromosome, which delays their development and increases the risk for other medical conditions[15]. Nature and nurture are both important factors to consider when determining the cause of a disorder. In treating developmental disorders with drug therapies, the nature vs. nurture debate involves an

intricate cross of genetic predispositions and environmental influences. From a nature perspective, genetic factors significantly contribute to the aetiology of disorders like autism spectrum disorder (ASD) and attention-deficit/hyperactivity disorder (ADHD), affecting neurotransmitter systems, alongside brain development[16]. Drug therapies pinpoint these biological pathways and aim to adjust neurotransmitter activity or receptor function. However, nurture factors, such as early life experiences, environmental toxins, and social interactions, can have an influence on gene regulation and neural plasticity, potentially altering the efficacy of drug treatments[17]. The interaction of gene-environment suggests that genetic vulnerabilities may be worsened or alleviated by environmental influences, affecting the development and progression of disorders[18]. Additionally, epigenetic modifications arising from environmental experiences can affect drug metabolism and response, highlighting the intricate interplay between nature and nurture in pharmacological interventions for developmental disorders[19]. This complex relationship underscores the importance of considering both genetic and environmental factors when developing and implementing drug therapies for developmental disorders[20]. They both influence gene expression, though it would not be apparent[21]. Since both genetic and environmental factors contribute to developmental disorders, it is difficult to determine what has a bigger impact to prescribe correct therapeutics. There is a lack of progress and countless failures encountered in targeted drug development for autism spectrum disorder (ASD) and related neurodevelopmental disorders. One in sixty-eight children in Europe[22], and one in thirty-six children in the USA experience ASD[23], and this is why it is crucial to discover targeted ASD drugs. The two FDA-approved medications for autism spectrum disorder (ASD) that are now on the market, risperidone and aripiprazole, both target the aggressive and irritable

behaviours that children with ASD[24–26] exhibit. Other drugs that are being studied or used off-label include naltrexone, which has shown promise in lowering self-harming behaviours[25,27], and selective serotonin reuptake inhibitors (SSRIs), such as fluoxetine, which may lessen anxiety and repetitive behaviours. Sulforaphane, a supplement made from broccoli sprouts, is an emerging treatment. Clinical trials have shown improvements in behavioural symptoms and indicators including oxidative stress and inflammation[28]. Through preclinical investigations and a limited number of human trials, the GABA receptor agonist arbaclofen has shown potential in alleviating agitation and social impairments in ASD[28]. These demonstrate continued attempts to increase the range of available treatments for the primary and related symptoms of ASD.

Role of Gene Editing in Understanding Developmental Disorders

Gene editing technologies such as CRISPR-Cas9 can allow the precise correction of mutations that underlying developmental disorders. The CRISPR system allows rapid and efficient genome modifications in virtually any organism. It permits personalised medicine, treating genetic conditions in many disorders, such as DMD. Although it has benefits, concerns continue due to its safe delivery and ethical impact. And further developments are paving the way for the potential treatment of these disorders at the genetic level[29]. Due to the advantages of the CRISPR-Cas9 system, it is widely used in genetic engineering in biological and therapeutic applications. Additionally, the technology has been used to re-target functional domains that activate gene expression or label specific genomic loci *in situ* within living cells or organisms to investigate developmental mechanisms, gene regulation, and animal behaviour[30]. By identifying and fixing the dystrophin gene mutation, CRISPR-Cas9 can be used to modify the gene that causes DMD. Gene therapy's effectiveness and safety in

treating DMD were evaluated in the clinical trial *NCT02354781*[31]. To restore dystrophin production and enhance muscle function in patients, a modified virus containing a functional form of the dystrophin gene is injected into the patients. Effectively and safely delivering the gene called micro-dystrophin or mini-dystrophin, which are condensed versions of the full-length dystrophin gene modified to fit the limited packaging capacity of AAV vectors[32,33]. Commonly, the AAV vector consists of serotypes rh74 or AAV9, is given systemically through intravenous injection, allowing for systemic transduction of skeletal and cardiac muscles[32,33]. When in the cell, the vector is internalised in clathrin-coated endocytic vesicles, released into the cytoplasm, and translocated to the nucleus where the transgene is released[32]. The exogenous DNA remains primarily in an episomal state, with only a minor fraction integrating into the host chromosome[32]. Transduced cells then express the transgene, producing a truncated but functional dystrophin protein[32]. Delivering to muscle tissue is one of the most difficult tasks. By fixing the dystrophin gene mutations, CRISPR-Cas9 provides a gene-editing method for treating DMD. Nonetheless, issues like delivery and off-target effects need to be resolved[2]. The manipulation of genes raises questions about the boundaries of morality and the potential consequences of altering the building blocks of life – consent, possible failures. Long term effects are unknown, and may result in possible dangers, such as insertional mutagenesis could take years to become apparent, potentially leading to cancer development[34], the body's immune system may attack the newly introduced vectors, causing reactions ranging from swelling to organ failure[35] in future generation[5].

Role of Stem Cells in Treating Developmental Disorders

In the field of neurodevelopment research, induced pluripotent stem cells, iPSCs, have opened a significant new chapter. They make it possible to model specific disorders in

species that were previously difficult or impossible to experiment on, like humans and primates[36]. iPSCs are the type of cells that can be specialised to become almost all adult cells, and they have been reprogrammed back into embryonic-like stem cells. They might be used to replace or repair missing or damaged brain cells that may contribute to autism[3]. Scientists are exploring whether stem cells may alleviate symptoms, including behavioural issues and communication difficulties, or boost brain function[37,38]. Stem cell therapy shows promise in improving social interaction, reducing repetitive behaviours, and enhancing cognitive abilities in individuals with developmental disorders like autism spectrum disorder[39,40]. After obtaining skin cells from autistic individuals, these cells can be reprogrammed to stem cells (such as iPSCs). Next the stem cells are differentiated into neurone cells that could be used to generate and repair the brain. This type of treatment is still considered to be experimental. Scientists are trying to discover whether stem cells are a safe treatment for autism, and whether they can actually mitigate symptoms over time[2]. A clinical trial[41] studying safety and efficacy of stem cell transplantation for children with ASD exhibited that stem cells could repair and regenerate the brain's cells, which potentially represents a new paradigm in treating neurological conditions associated with genetic and developmental disorders. An extensive range of studies have investigated the possibilities of stem cell therapy in treating autism spectrum disorder (ASD). A meta-analysis by Qu et al. (2022) had analysed five studies and found that stem cell therapy markedly enhanced Childhood Autism Rating Scale (CARS) scores compared to the control groups, indicating its safety and efficacy, even with limitations like small sample sizes as well as the lack of uniform protocols were noted[42]. A holistic review by Martínez-Morga et al. (2021) investigated 11 trials involving 461 patients and reported significant improvements in ASD symptoms on scales like the Autism

Behaviour Checklist (ABC) and CARS, with no significant unfavourable events recorded[43]. These findings highlight the potential of stem cell therapy in mitigating ASD symptoms, though further large-scale, long-term studies are required to confirm these results.

Integrating Gene Editing and Stem Cells for Treatment

Personalised medicine is the practice of adjusting medical treatment for each patient according to their environment, lifestyle, and genes, therefore making it appropriate for each patient. This becomes possible by stem cells, which helps to develop personalised therapies for each patient. To safely test new medications and their toxicity, stem cells are used to help create tiny organ models, so called organoids, in place of animal testing. Stem cells can help regenerate damaged tissues (such as heart or spinal cord tissue) using the patient's own stem cells, which lowers the possibility of rejection post-transplantation. It is challenging to translate research into therapies due to the problems arising from the production scaling, safety, and effectiveness[5].

Gene editing can enhance stem cell treatments by enabling mutations to be fixed in hematopoietic stem and progenitor cells (HSPCs) before they differentiate[44]. Scientists have used advanced gene editing techniques to fix HSPC mutations that cause inherited blood disorders and immune deficiency. The immune system and blood depend on haematopoietic stem and progenitor cells (HSPCs). Predominantly found in the bone marrow, HSPCs can self-renew and specialise into every kind of blood cell. HSPCs produce blood cells continuously throughout a life, and therefore are essential for preserving haematopoiesis. They are preferred targets for gene therapy techniques to treat a variety of hereditary blood diseases and immune deficiencies because of their capacity to regenerate the entire blood

system upon transplantation[45]. Once genetically modified, it is possible to grow these HSPCs *ex vivo* and reintroduce them into the body, potentially offering a useful treatment for diseases. The precise modifications made possible by gene editing tools ensure that the cells used in treatments are free of harmful mutations, making cell-based gene editing treatments more powerful. However, there are still concerns about the effectiveness of gene editing and the potential for cellular senescence, limiting the functionality of HSPCs after editing despite the good results[46].

Ethical and Societal Considerations

It is often very difficult for people from lower socioeconomic backgrounds to obtain innovative treatments. For example, research indicates that compared to their wealthier counterparts, African American patients who are dual Medicaid-eligible (qualify for both Medicare and Medicaid in the United States, over 65, and low-income) have a lower likelihood of receiving advanced device-based therapies such as mLVAD (mechanical left ventricular assist device)[47]. Access is primarily concentrated in urban areas, with few facilities providing advanced therapies in rural areas. For instance, there are not many facilities in rural areas that can perform extracorporeal membrane oxygenation, or ECMO[47]. Patients of African American and Hispanic descent are significantly underrepresented in clinical trials for therapies like CAR T-cell therapy. CAR T cells are genetically engineered T cells that express chimeric antigen receptors (CARs) on their surface, allowing them to aim at specific cancer antigens[48,49]. CAR T cell therapy has been investigated using hematopoietic stem/progenitor cells (HSPCs) and induced pluripotent stem cells (iPSCs) to generate more persistent and functional CAR T cells[50,51]. Stem cell-derived CAR T cells have shown greater persistence, trafficking, and antiviral responses compared to conventional CAR T cells[50]. This

underrepresentation exacerbates pre-existing health disparities because these groups often have higher mortality rates from diseases like multiple myeloma but cannot access innovative treatments[52]. Stakeholders and healthcare professionals need to work hard to increase awareness and access within the healthcare system to overcome discrimination [52,53]. Research indicates that most people have a positive opinion of gene editing for therapeutic purposes, particularly when it comes to treating or preventing serious illnesses. A study found that 63% of participants supported embryo editing to prevent diseases that are fatal or severely incapacitating[54]. While support exists for therapeutic uses, public attitudes shift when considering enhancements or non-disease traits. For instance, only 27% supported editing embryos for non-disease characteristics like appearance. Ethical concerns about the moral status of embryos and potential eugenics implications contribute to these hesitations[54]. The World Health Organization (WHO) has established a governance framework for human genome editing, which includes recommendations for oversight at institutional, national, regional, and global levels. This framework addresses ethical, social, and legal challenges associated with human genome editing[55]. The European Parliament has also outlined governance principles for genome editing, emphasising the need for new regulatory frameworks to balance potential benefits against risks associated with these technologies[56].

Conclusion

Treatment options for developmental disorders like DMD and ASD are bright thanks to stem cell therapies and gene editing technologies, particularly CRISPR-Cas9[2,29]. These technologies enable personalised medicine and have the potential to correct genetic mutations and repair damaged tissues. However, challenges remain in safe delivery, addressing ethical concerns such as germline editing[5], and ensuring equitable access[47]. Continued research,

robust clinical trials, ethical deliberation, and comprehensive regulatory frameworks like those discussed by the WHO[55] and European Parliament[56] are essential to responsibly harness the power of gene editing and stem cells for improving the lives of individuals with developmental disorders.

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