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Commentary

The Importance of Treating Hemophilia A using Induced Pluripotent Stem Cells

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DESCRIPTION

One of the most prevalent hereditary clotting disorders resulting from a factor VIII protein shortage is haemophilia A. Hemophilia A affects 1 in 5,000 males, according to estimates. Numerous genetic changes, including deletions, insertions, inversions, and point mutations in the factor VIII gene, are responsible for its development. Hemophilia A can be classified as severe, moderate, or mild depending on the amount of bleeding and the length of time it takes for the blood to coagulate . Hemophilia A does not currently have a treatment. Recombinant factor VIII infusion or administration is the only therapeutic option for this illness. However, the use of recombinant factor VIII for treatment is constrained by the development of factor VIII inactivating antibodies, the high cost, and the need for repeated injections. Gene therapy has been discovered to be a promising treatment for haemophilia since its launch. The use of patient-derived induced pluripotent stem cells is another Intriguing Therapy Option for Haemophilia A. (iPSCs). Utilizing programmable nucleases such as Zinc Ginger Nucleases (ZFNs), transcription activator-like effector nucleases, and clusters of regularly spaced palindromic repeats, the faulty gene can be repaired in iPSCs. These programmable nucleases in this situation specifically cleave the chromosomal DNA and cause DNA double strand breaks. Endogenous processes like homologous recombination or non-homologous end-joining will be used to repair the nick. Finally, it will cause corrective mutations such inversions, duplications, and deletions. Before being given to patients, these gene-corrected iPSCs are then given the go-ahead to develop into the proper somatic cells in order to guarantee the expression of the functional gene. iPSCs offer benefits and drawbacks of their own. Despite claims that iPSCs would dominate the medical business in the future and supply patient-specific stem cells, there are concerns regarding its use on human beings. Recent innovations face difficulties in harnessing their full potential for biological research and developing successful therapeutic approaches. Since iPSCs are highly popular and are easily available from sources like skin or blood, which are sufficient to produce disease-specific models, it is worthwhile to quantify their potential. The kinetics of illness onset and progression as well as the geographic localization of the disease to create disease models are currently difficult issues, but they can be overcome with cutting-edge techniques like gene editing, biomaterials, reprogramming, etc. Due of its infinite capacity for self-renewal and differentiation, researchers have already started employing patient-specific iPSCs to study the correlation of haemophilia A.

CONCLUSION

In order to properly identify the potentials to treat haemophilia A, it is important to develop an effective method incorporating iPSCs. With priority attention given to this much-needed technology, the therapeutic setting will benefit greatly from its use. One of the most complex proteins known as factor VIII is crucial to the blood coagulation process. Hemophilia A is a severe bleeding illness brought on by defects in the factor VIII protein. Recombinant factor VIII and plasma-derived factor VIII have both been widely utilised to treat haemophilia A patients. Numerous attempts at haemophilia A gene therapy have failed for a variety of unidentified or little-studied causes, including immunological rejection. The development of iPSC-based disease models and the potential of iPSC technology for personalised medicine and cell treatment for haemophilia A are discussed in this article. There is also a brief discussion of the drawbacks of iPSC technology.

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