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Autologous stem cell-based gene therapy

Priasha Dutta

Gene therapy using a patient's stem cells is increasingly becoming a better alternative to allogeneic stem cell transplantation. This is especially useful when the patient's compatible donors cannot be found. The increasing number of efficient virus-based methods for delivering therapeutic genes has allowed the development of genetic medicines for inherited disorders of the immune system, haemoglobinopathies and a number of lethal metabolic diseases. Novel gene editing approaches using haematopoietic stem cells (HSCs) have been developed for the treatment of paediatric diseases. One such approach is using the cluster of differentiation 34 (CD34) which is a transmembrane phosphoglycoprotein that is encoded by the CD34 gene present in humans. HSCs enriched in CD34+ can be harvested from the patients, either from bone marrow or from mobilised peripheral blood. The CD34+ cells are cultured in laboratories with cytokines and viral vectors, after which they are harvested and then processed under a series of quality control steps before they are reinfused into the patient. The cell product is usually cryopreserved, thereby allowing time for the quality control tests and the shipment of cells to clinical transplantation centres which may be present far from the production site. After reinfusion, HSCs find their niches and differentiate into mature blood cells, thus preserving the clinical effects. A significant number of viral vectors have been used for such gene therapy applications. Retroviruses are usually used to transform HSCs because they have the ability to introduce viral genes into the host human genome in a stable manner. The retroviruses used in gene therapy are devoid of replication and only have the most vital genes. Some of these genes are given via co-transfection with various plasmids into these trans-complementing cell lines. Stable packaging cell lines have been used for the operation of gamma-retroviral vectors and HIV-derived lentiviral (LV) vectors on them. Therefore, gene therapy using HSCs has the potential for further progress and the development of more efficient methods.

Keywords: Stem cells, CD34+ cells, Genes, Gene therapy, Gene editing, Vectors

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