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Development of virus vectors for gene therapy

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Gene therapy is a growing field where we find different ways to fix some genetic disorders and diseases, which are very hard to cure or treat. Adeno-associated viruses taken from adenoviruses have been studied intensely for a long time and have been used for various bio-therapies to study the virus-host interactions and to understand the transduction pathway of the virus. The adeno-associated virus vectors are made from recombinant adenoviruses. Recombinant adenoviruses consist of encapsidated genomes that are devoid of all adeno-associated virus protein-coding sequences and have transgene cassettes designed in their place. There are some sequences inserted that are required for genome replication and packaging, which are used during vector production. The production of these vectors is achieved through transduction efficiency by optimising the transgene cassettes and through vector tropism by using capsid engineering and also genetically modifying some components to avoid any host immune system response. These vectors possess the therapeutic transgene specific to the disorder or syndrome, such as haemophilia, Duchenne muscular dystrophy (DMD) and age-related macular degeneration; and then when they enter into the nucleus, the transgene gets transcribed and translated. This viral vector could be able to evade the immune system and have an enhanced translation and transduction to avoid any unwanted side effects which would produce highly diverse mutants and would eventually be a type of cure for the patients with aforestated diseases.

Keywords: Adeno-associated virus, Gene therapy, Vectors, Duchenne muscle dystrophy, Haemophilia

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