The Torch
Vol. 2 (40)

Gene therapy: Adeno-associated virus as a transduction vector

Bhavya Kriti

The last few decades have seen a rise in the study of genetics and molecular biology. With the development of gene therapy, the possibility of curing many monogenic defects and diseases has come forward. Ever since the first application of gene therapy in 1990 by W. French Anderson and his colleagues, many advancements have been made in this field. Every day, research is conducted throughout the world to ensure safer and more effective transfer and expression of genes in humans. Identification and understanding of new gene delivery vectors have led to an increase in the number of vectors that can be used for gene transfer. Therefore, it becomes very important to select a suitable and efficient vector depending on the type of experiment and cells used. Adeno-associated virus (AAV) was first identified in the 1960s in the laboratories of Bob Atchison and Wallace Rowe. It is a small-single strand, non-enveloped DNA virus and does not cause any disease. Its lack of pathogenicity, ability to infect both dividing and non-dividing cells as well as stable integration of genetic material at a specific site (AAVS1 locus) in human chromosome 19 has attracted significant attention among researchers.

The development of recombinant adeno-associated viruses (rAAV) lacking viral genes and containing DNA sequences of interest has proven to be a successful transfection strategy. Currently, there are several ongoing trials with AAV vectors trying to treat muscle and eye disease, especially retinal degeneration. Trials have also been carried out to treat Duchenne muscular dystrophy (DMD) using AAV. Adeno-associated viral vectors have also shown efficiency in neurological gene therapy. Two vectorised AAV serotypes namely AAV1 (glybera gene therapy) and AAV2 (luxturna gene therapy) have gained regulatory approval for commercial use. The rapidly evolving technologies such as gene editing have made it possible to design chimeric recombinant AAVs which are shown to be more efficient than the wild type virus. Many human diseases, such as cardiovascular diseases or neurological diseases could be modulated by gene addition. Recombinant AAV vectors have the potential to tackle such genetic and acquired diseases by using gene addition. Examination and modification of AAV are rapidly growing in the field of genetics to provide more clinically viable viral capsids and optimal genome designs. Continued research in AAV biology and associated limitations will advance the gene therapy technologies which could help in curing various diseases.

Keywords: Gene therapy, Adeno-associated virus, Genetics, Recombinant DNA, Molecular biology, Gene editing, Recombinant adeno-associated virus

Citation:
Bhavya Kriti. Gene therapy: Adeno-associated virus as a transduction vector. The Torch. 2021. 2(40). Available from: https://www.styvalley.com/pub/magazines/torch/read/gene-therapy-adeno-associated-virus-as-a-transduction-vector.