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Can we really cure Genetic Diseases

Nazima Ameen[1], Sabeeha Shafi[2]*

[1]Department of Pharmacy, Sheri Kashmir Institute of Medical Sciences, SKIMS, Soura, Srinagar, Kashmir, India
[1]Department of Pharmaceutical sciences, University of Kashmir, Hazratbal, Srinagar, Kashmir, 190006, India

Abstract : Advances in biochemistry, molecular biology and biotechnology have paved a new way to understand the genetic basis of inherited diseases. It always remained a dream of researchers to replace the defective genes with good ones and cure the genetic disorders. Gene defects result in failure to synthesize a functional protein or in the synthesis of a dysfunctional protein. Gene therapy is defined as the insertion of functional genes into target cells to replace or supplement defective genes so as to achieve the therapeutic goals. The newly introduced genes will encode proteins and correct the deficiencies that occur in genetic diseases. Apart from inherited genetic disorders with single nucleotide polymorphism (SNP),the major thrust area of gene therapy are a number of acquired diseases such as malignancies, immunological disorders , including AIDS, cardiovascular neurological and infective diseases in many of which even short-term expression of the introduced gene could be therapeutic. Safe methods have been devised to do this, using several viral and non-viral vectors. Two main approaches emerged: in-vivo gene therapy and ex-vivo gene therapy. The important vectors employed in ex-vivo modification are Viruses (Retrovirus, Adenovirus, Adeno-associated virus, Herpes Simplex Virus), Human Artificial Chromosome (HAC), Bone Marrow Cells etc. The major concern with viral gene delivery is immunogenicity. The residual viral elements can be immunogenic, cytopathic, and/or recombinogenic. Non-viral vectors are far less efficient than viral vectors in terms of transfection efficiency, but they have advantages due to their low immunogenicity and their large capacity for therapeutic DNA. Gene transfer protocols have been approved for human use in inherited diseases, cancers and acquired disorders. In 1990, the first successful clinical trial of gene therapy was initiated for adenosine deaminase deficiency. Since then, the number of clinical protocols initiated worldwide has increased exponentially. Although preliminary results of these trials are somewhat disappointing, but human gene therapy dreams of treating diseases by replacing or supplementing the product of defective or introducing novel therapeutic genes. Unlike conventional treatments, most of which provide symptomatic alleviation, gene therapy eliminates genetic defects that cause a variety of diseases including genetic disorders, cancers and cardiovascular diseases, that is, gene therapy promises “cure” in a sense.

Key Words : Gene therapy, viral and non-viral vectors, in-vivo and ex-vivo gene therapy, Human artificial chromosome(HAC), Immunogenicity, Transfection efficiency.

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