Abstract

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The use of genetic interference mechanisms in treatment of human genetic disorders

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Abstract

Gene interference can be defined as the alteration of the activity of specific genes by altering the regulatory sequences. Gene therapy aims at treating genetic diseases through insertion and expression of specific exogenous genetic materials by transfer of nucleic acids either in vivo or in vitro (through modified cells). This results to expression of a normal product thus correcting the abnormal cellular function. Gene therapy involves two approaches which include germ-line and somatic line gene therapy. In germ-line gene therapy, the germ cells are integrated by exogenous functional genes which can be transferred into the offspring's while in somatic line gene therapy, the genes are introduced into the patients' somatic cells and the effects are only experienced on that particular individual. Both methods leads to reduction in the coding mRNA levels. RNA interference not only inhibits transcription, but also induces an RNA degradation process. Gene silencing is enhanced by presence of double stranded RNA (dsRNA) molecules and is initiated by the dicer enzyme, where the dsRNA are cleaved by the protein into small interfering RNA (siRNA) strands which can prime the mRNA of the coding genes to form a duplex (siRNA + mRNA). The duplex activates the RNA-induced silencing complex (RISC) which upon activation degrades the duplex with the critical coding sequences. This is very essential in gene silencing. This gene degradation mechanism thus blocks the process of translation thus inhibiting expression of abnormal proteins responsible for the genetic diseases

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Biography

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