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Viruses as Allies of Man

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At the beginning of the third millennium, man has an opportunity to fulfill the cherished goal of improving the lot of humankind. Newer modalities of medicine are being practiced and daily new breakthroughs are being reported. I would like to talk about gene therapy, a form of molecular medicine, which will have a major impact on human health. At present, gene therapy is being contemplated for both genetic and acquired diseases. These include hemophilia, cystic fibrosis, diabetes, cancer, Parkinson's, Alzheimer's, etc. In the case of genetic diseases, a wide variety of somatic, non-germinal tissues are being explored for the introduction of foreign genes with a view towards gene therapy. A prime requirement for successful gene therapy is the absence of any adverse effect on the recipient. A highly desirable delivery vehicle will be the one that can be generated at high amounts, integrate in non-dividing cells and have little or no associated immune problems. We have recently generated vectors based on a modified, non pathological, safe form of the AIDS virus that have the ability to introduce genes into both dividing and non-dividing cells. The vectors can introduce genes in a variety of cells and hosts. Our current, third-generation viral vectors are devoid of six viral genes and therefore we consider them to be safe vectors. Using third generation lentiviral vectors we can introduce genes directly into brain, liver, muscle, hematopoietic stem cells, and more recently retina and a number of tumor cells. Our data shows that lentiviral vectors can not only efficiently deliver genes, but also have long term sustained production of the foreign protein. We have not observed any untoward immunological consequences due to the vector. My talk will discuss the use of vectors for a wide variety of genetic and acquired diseases. Additionally I will discuss the use of these viral vectors for studying complex biological systems. Finally I will discuss the social and ethical implications of genetic approaches to human health.