

Gene Therapy: Current Situation and Application Prospect

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Editorial

With the setting up and development of recombinant DNA and gene transfer technology in recent years, gene therapy has been known widely as one of the novel medical measures with huge prospect in the 21st century. Gene therapy means transferring normal genes into the target cells that lose some genes or have some abnormal genes. The transferred normal genes will express in the target cells so as to cure the diseases. Compared with traditional ways, gene therapy is an advanced modern science and technology theoretically possesses the ability to cure recurring diseases such as cancer and hereditary disease. On the one hand, this technology can solve a lot of problems that traditional techniques fail to do. On the other hand, it adopts different strategies against different diseases during the course of therapy in order to overcome the shortcomings of traditional therapeutics. However, no one will deny that this technology is still in its infancy and will bring some security problems. Hundreds of trials have shown that the gene therapy has the potential therapeutic effects with a low risk of adverse reaction, but the efficiency of gene transfer and expression in vivo is still disappointingly low [1].

Cancer is almost the largest killer of mankind and has brought people irresistible fear of death and sufferings. Consequently, how to treat the cancer effectively is a question of great concern to us all. Conventional cancer treatments including surgery, chemotherapy and radiation usually have the characteristics of serious side effects, significant risks, and higher recurrence and mortality rates. In recent years, people place their hopes on the gene therapy. Base on the properties of tumor cells or their distinction from the normal cells, doctors transfer the target gene using the molecular biotechniques into the cells of patient. The target gene will express in the cells and produce gene products (protein or RNA). These products can inhibit the proliferation of the tumor cells or kill them, while have no adverse effect on the normal cells. Thus, this kind of therapy has a better selectivity in the treatment of cancer and will not incur some extra damage to the patients. There is a paradigm that gene therapy has been used to treat leukemia recently. The hospital experts employed certain nucleases altering the genes and created immune cells that can kill the resistant leukemia cells with genetic modification. As a result, the

condition of the patient was improved according to the report. It will be a great promotion to the medical practice if this idea is applied to other scopes.

Genetic diseases such as albinism, thalassemia, color blindness, and congenital heart disease are usually caused by abnormal genes. Conventional therapies including dietary, surgical, and medication treatments do not address the underlying cause. That is to say, they are more likely to tend to solve the superficial problems. In contrast, gene therapy will break through the limitation of traditional therapy. With the help of this science and technology, we will find out abnormal genes or synthesize fantastic molecular vehicles of genetic engineering. These vehicles will carry the normal genes into the cells of patient, and then the genes are widely expressed to produce corresponding product so as to achieve the goal of radically curing genetic diseases. Since the first one was carried out in 1989, over 1900 clinical trials of human gene therapy have been conducted worldwide. Human gene therapy has made substantial progress in the treatment of cancers, inherited monogenic disorders, and cardiovascular disease etc., while encountered with hurdles simultaneously [2].

The key to the success of gene therapy is to transfer genes into target cells efficiently and safely; thus, the transfer efficiency of the foreign genes makes a great influence on their expression. Meanwhile, security issue and moral hazard about gene therapy also gain much attention. Some people are against the therapy for its challenging and unpredictability. In our opinion, uncertainty is one of the characteristics of new techniques, and we should not ignore its enormous potentials. We can take effective measures to improve the technology, and its application will be expanded and developed gradually. Only in this way can we constantly make progress and have a better life.

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