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Chapter 1 : Gene therapy - Wikipedia

The only peer-reviewed journal that focuses on the human aspect of gene therapy, and provides end-to-end coverage of the research, methods, and clinical developments driving today's explosion of gene therapy advances.

Check new design of our homepage! What is Human Gene Therapy Human gene therapy is a milestone in the field of medical science, and it deals with the treatment of chronic health conditions and genetic disorders. There are certain factors related to the effectiveness of this technique, such as autoimmune responses, vectors, multigene disorders, etc. Based on them, specific proteins are synthesized, which in turn, are responsible for the expression and functions of body cells and tissues. If there is any alteration in the genetic sequence, the protein synthesis is disturbed. In such a condition, the cells and tissues are unable to perform their normal function, leading to genetic diseases. Its main principle is to restore the normal functioning of cells and tissues, by replacing abnormal or mutated genes. In simple terms, it is a method of correcting defective genes. There are several ways of implementing this technique; inserting a normal gene into the genome to replace a defective one, and changing the regulation for expression of a specific gene. Another method is to apply selective reverse mutation, so that the defective gene returns to its original form. In majority of cases, a normal therapeutic gene is inserted by using a vector or a carrier molecule. This vector serves the purpose of delivering the normal gene to the target cell. As it incorporates this gene in the cells for example liver cells , it starts producing functional proteins, thus restoring the normal functions of this cell. Various types of viruses such as retrovirus and adenovirus, are used for the purpose of carrier molecules. Before using any of them, the genomic content is changed by removing the defective gene, and replacing it with a therapeutic one. Disadvantages There are many potential side effects of using viruses as vectors, some of which include target-related problems, inflammatory response of the immune system, and toxicity. A more recent technique is to introduce the normal gene directly to the target cell without using vectors. Though it sounds comparatively easy, it is not applicable to all types of such cells. Another disadvantage of this method is that, it requires a large amount of DNA. Another drawback is the short-term effectiveness of the normal gene, after introducing into the target cell. The dividing cells present in the body, are the ones that prevent the expression of therapeutic genes. It is also necessary to regulate the autoimmune responses of the body. This therapy is less effective for multigene problems presence of many defective genes. Though the technology of this therapy is not so advanced, in the present scenario, it has already widened the scope of medical science. All genetic disorders and most of the chronic diseases involve malfunctioning of the genes. Hence, the method given above is a promising technique for treatment of such severe health conditions. Genetic research programs are ongoing to discover treatment options for chronic medical conditions such as heart diseases, cancer, cystic fibrosis, and AIDS. Nowadays, private as well as government grants are provided, so as to encourage studies on this topic.

Chapter 2 : Human Gene Therapy | Mary Ann Liebert, Inc., publishers

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Copyright Introduction Gene therapy is the use of genes to treat disease. It represents a quantum leap in our approach to the treatment of human disease and will have a significant effect on medicine over the next ten years. William French Anderson, Michael Biase, and Ken Culver performed the first successful gene therapy on a human in They developed a protocol for treating Adenosine deaminase ADA deficiency, severe combined immune deficiency, also known as the" Boy in the Bubble disease". ADA deficiency is a result of inheriting two copies of the defective ADA gene in other words it is a recessive disease. Possession of a normal gene leads to the continuous, regular production of ADA in cells throughout the body. Without at least one properly functioning gene, children have no way of converting deoxyadenosine a waste product into inosine. This leads to the rapid build up deoxyadenosine in the system, which becomes phosphoralysed into a toxic triphosphate which kills T-cell. The result is an almost complete failure of the immune system and early death. Concept of Gene Therapy The term gene therapy originally referred to proposed treatments of genetic disorders that would involve replacing a defective gene with its normal counterpart Current usage of the term now extends to include all treatments in which there is an introduction of genetic material into body cells to treat a variety of diseases. Gene therapy utilizes two theoretically possible approaches: The new genetic material cannot be passed on to offspring. Examples of Somatic gene therapy have already proven to be clinically effective. The first successful treatments of adenosine deaminase deficiency took place in in with two patients aged 4 and Both are thriving with continuing treatment. The first successful treatment of familial hypercholesterolemia, a genetic condition, which affects the livers regulation of cholestrols in the blood, took place in of a year-old woman. Her improvement was stable for the 18 months of the study and liver biopsy demonstrated activity of the inserted gene and no discernible abnormalities. Five patients have been treated as of Current research involving Somatic gene therapy is focusing on a number of areas. Clinical trials are being performed on a treatment for cystic fibrosis, a chronic genetic disorder. Such therapy would change the genetic make up of the egg or sperm of an individual and would be carried on to future generations. This would offer the possibility of removing an inherited disorder from a family line forever. This could be achieved by other methods, such as, at present, diagnosis when there is a known risk before embryo implantation during IVF. Germ line therapy is a remote prospect and general opinion is strongly negative; such therapy is currently illegal in most of Europe. Somatic and Germ line gene therapy raise different issues. Somatic gene therapy offers the prospect of effective treatment and cure for previously fatal disorders. Until now it has only been used experimentally for a small range of genetic disorders; even in these cases treatment is complex, difficult and success uncertain. Technical Aspects of Gene Therapy The most fundamental requirement for gene therapy to be successful is that a therapeutic gene can be effectively delivered to a target cell. Once delivered that gene must enter the nucleus of cell wall where it will act as a template for production of protein molecule. The protein then exerts the primary therapeutic effect. This may be, for example, cell killing in the case of tumor therapy or cell preservation in the case of neurodegenerative disease. There are several ways to get genes into cells. The most efficient of these uses disabled, engineered viruses. These systems are efficient because viruses have evolved over long periods of time to deliver their own genes to cells. Whenever, we get a viral disease, be it a cold or AIDS, the particular virus concerned is placing its genes into our cells in order to reprogrammed our cells to produce more virus. When we use viruses for gene therapy we disable them so that they are unable to cause disease and we engineer them in such a way that they pick up and deliver the genes of our choice rather than their own genes. These derivatives of viruses that are used for gene delivery are known as viral vectors. The most frequently used viral vectors are of two types. The vectors based on adenovirus are generally used for therapeutic strategies that require the therapeutic gene to be active for only a short time. Gene delivery by adenoviruses is very efficient but because the gene does not become integrated into the chromosomes of the target cell the gene is lost overtime. This not a disadvantage for some therapeutic strategies such as cell destruction in the treatment of some cancers, restinosis or inflammatory

disease. However, it is a disadvantage where sustained gene activity required for many months such as in the treatment of some tumors, neurodegenerative disease and HIV infection. The second major type of vector is generally used and this is based on the retrovirus, murine leukemia virus MLV. When genes are delivered by derivatives of MLV they become integrated into the chromosomes of target cell and are maintained for as long as the cell remains alive. Gene activity is easy to control and continues over long periods of time. Many clinical trials have been conducted with these MLV based systems and has been shown to be well tolerated with no adverse side effects. One of the major difference between adenovirus vectors and MLV vectors is that the former can deliver genes to cells that are not multiplying by cell division whereas the latter cannot. Until recently this has meant gene therapy strategies that demand long term gene activity in cells that are not dividing have been feasible. Examples of important target cells that do not divide are neurons, certain cells of the immune system and certain epithelial cells. Lentiviruses are a subgroup with in the general family of retroviruses but they are distinct from the MLV like viruses in that they are able to infect non-dividing cells. The best studied of the lentiviruses is HIV and when observation was made, about 10 years ago, that HIV could infect terminally differentiated macrophages, which do not divide, there was a move within the research community to develop gene delivery vectors from HIV. There were number of early technical difficulties and first generation vectors could not be used in the clinic as they had potential to generate infectious HIV. Over past two years we have seen new HIV based vectors emerge that are severely disabled containing only the few HIV components that are required for efficient gene delivery to non dividing cells. These so called minimal vectors are now candidates for gene delivery vehicles for clinical use in gene therapy. The technique, called Chimeraplasty, was developed for mammalian gene therapy. It has an advantage over current genetic engineering methods in that it can seek out any specific gene and cause tiny mutations with high precision. Instead of adding a new gene to trick a plant into doing something it would not normally do, Chimeraplasty simply switches on or off function for which the plant already has a gene. Until now, an entire gene had to hitch a ride into the nucleus on a defused viruses, which has the ability to insert itself into the genome. However, the virus could settle anywhere on the genome, sometimes choosing a location that is less than optimal for the replication of new gene. Technique also eliminates the danger from inserting large sections of genes with potentially undesirable side- effects, such as poisoning beneficial insects. For Chimeraplasty, researchers start with small chunks of artificial genetic material, called oligonucleotides or "oligos", with about 25 bases each. They mirror one specific plant gene except for a mismatch of a few bases. The chunks are hooked up to tiny gold particles, which are then shot into nucleus of cell with a particle gun. When the oligos attach their counterparts in the cell, the DNA repair machinery tries to "fix" the mismatch, using the new sequence of the bases as blueprint. Boosting blood cell production does little good for patients, whose blood cells are malformed, such as those of sickle cell anemic. The ultimate goal of gene therapy is not to compensate for genetic diseases but to erase them completely. Preliminary work published in the September 6 issue of science offers a reason to hope that goal may be possible. A team led by Allyson Cole-strauss and Kyonggeum Yoon of Thomas Jefferson University in Philadelphia experimented on cells containing a mutant gene that causes sickle cell anemia. The cells normal DNA repair machinery then apparently replaced the mutation with the normal code thus permanently curing 10 to 20 percent of cells. The researchers still have to demonstrate that this technique works in human cells and in human bodies. In about half of lung cancer cases, a gene called p53 has mutated and thus falls to encode a protein that oversees programmed cell death. In the absence of this protein, Which helps to curb the growth of damaged or abnormal cells, cancer can gain a foothold. Replacing such defective p53 genes with fresh ones has shown promise against a variety of cancers in animal experiments and studies of a few patients. Scientists now report further progress in such localized gene therapy. By enlisting a virus to deliver p53 to tumor sites in 28 people with lung cancer, they temporarily stabilized or reversed the course of the cancer in more than half patients. The patients, average age 65, had lung cancer that was either inoperable or was no longer responding to radiation treatment or chemotherapy. The researchers injected the tumors with an adenovirus engineered to contain p53 genes. The virus was modified to prevent it from replicating and thus causing the upper respiratory infection that it might otherwise bring about. During the 6 months treatment period patients received one to six monthly injections of the

modified virus. The researchers delivered a range of doses -from 1 million to billion viral units to gauge any toxicity of the treatment. Among the 25 others, tumors shrank in 2 patients, stabilized in 16 and continued to grow in the other 7. The dose of virus mattered; cancer progressed unabated in three of five patients who received injections of 10 million or fewer viral units. In contrast, only 4 of 20 patients getting larger dose experienced cancer growth. Argument of Human Gene Therapy Consider what a nation would gain by permitting parents to genetically enhance their children. By assumption, the genetic enhancement technology increases the ability of children to learn and perform cognitive tasks, and thus to acquire and generate knowledge. Permitting or facilitating genetic enhancement would therefore increase the collective human capital embodied in nations workers. The increasing prevalence of high ability genotypes would also multiply the effects of other national investments in education, training, and scientific or engineering research. Because genetic enhancements are heritable, the effect of these investments on the stock of human capital are cumulative unless enhanced offspring or their descendants emigrate. Finally, permitting genetic enhancement would be a cheap way for a state to increase aggregate human capital, because competition between parents would lead some parents to pay for it out of their own pockets. If expanding stock of nations human capital brings increasing returns in productivity and economic growth, it means that in economic competitions among nations, small initial differences in the distribution of able people can multiply, over time, to large international differences in the rate of economic growth. Thus nations have an incentive to defect from an international ban on genetic enhancement to get a jump on others in the accumulation of human capital. It is said that the day is not far of when parents will be able to browse through gene catalogs to special order a hazel eyed, red headed extrovert with perfect pitch. Every new discovery gives shape and bracing focus to a debate we have barely begun. If you could make your kids smatter, would you? If everyone else did, would it be fair not to? Which side effects would we tolerate? What if making smarter kids also made them meaner? What if only the rich could afford the advantage? Does god give us both the power to re-create ourselves and moral muscles to resist?

Chapter 3 : Medical Xpress - Human Gene Therapy

Human Gene Therapy is the definitive peer-reviewed rapid-publication journal covering all aspects of human gene therapy. The Journal publishes scientific papers on original investigations into the transfer and expression of genes in mammals, including humans with in-depth coverage of DNA, RNA, and cell therapies.

See other articles in PMC that cite the published article. Abstract Human gene therapy has made substantial progress since the initiation of the first clinical trials 20 years ago. Here, we summarized important applications of gene transfer protocols in the treatment of various human diseases using different viral vectors. Recent successful trials on the treatment of ocular diseases and inherited immune deficiencies are particularly encouraging and have raised hopes that human gene therapy as a standard treatment option will finally become a reality. While immune responses and insertional mutagenesis pose obstacles for this novel form of molecular medicine, continuous progress suggests that a wider range of diseases can be treated with gene therapy in the future. Introduction With advances in molecular cloning, gene transfer emerged in the late s as a potentially revolutionary new form of molecular medicine, holding promise for novel therapies for many genetic and acquired diseases. Similarly, Time magazine, reporting on recent high-impact publications in clinical gene therapy, asked the question: In principle, this can be accomplished using ex vivo gene transfer to cells that had been removed from the patient. Alternatively, the vector carrying the functional gene copy is directly injected into the body to achieve in vivo gene transfer. The first clinical trials were initiated in and published in , summarizing ex vivo gene transfer to umbilical cord blood cells or to autologous T lymphocytes of children with severe combined immune deficiency SCID due to mutations in the adenosine deaminase ADA gene Blaese et al. In these early studies, gene transfer protocols based on a murine retroviral vector were inefficient and unable to transduce hematopoietic stem cells HSC , thereby limiting extent and duration of gene transfer. The first decade of clinical gene therapy sought to establish safety of this novel treatment modality and to gain first experience in patients. In the late s, superior gene transfer protocols and vectors were rapidly developed, and numerous successful treatments in small and large animal models of human diseases were reported. In the clinic, the dawn of the new millennium brought the first successes as well as setbacks. Immune responses and insertional mutagenesis emerged as main hurdles for gene therapy. A violent innate immune response to an intravenously delivered adenoviral vector caused the death of a patient with a rare metabolic disorder in Somia and Verma, In , the first clearly successful gene therapy was reported. However, 4 of 10 children in this French trial and 1 of 10 children in a similar trial in the UK developed leukemia Hacein-Bey-Abina et al. It is likely that the combination of the growth advantage of the gene-corrected T cells and the activation of LMO2 was responsible for this outcome. Retroviral vectors integrate randomly into the host genome, but show a preference for transcriptionally active genes, and they contain sequences that are prone to activating nearby genes encoded by the host chromosome.

Chapter 4 : Two Decades of Clinical Gene Therapy â€“ Success Is Finally Mounting

The simplest method of human gene therapy would be to administer purified DNA directly to tissue using methods similar to those used for other drug molecules. Manthorpe et al. have examined in detail the transfection of mouse muscle tissue after intramuscular injection of microgram amounts of purified reporter DNA.

Retroviruses go a stage further by having their genetic material copied into the genome of the host cell. A number of viruses have been used for human gene therapy, including retroviruses , adenoviruses , herpes simplex , vaccinia , and adeno-associated virus. Non-viral[edit] Non-viral methods present certain advantages over viral methods, such as large scale production and low host immunogenicity. However, non-viral methods initially produced lower levels of transfection and gene expression , and thus lower therapeutic efficacy. Later technology remedied this deficiency. Short-lived nature â€" Before gene therapy can become a permanent cure for a condition, the therapeutic DNA introduced into target cells must remain functional and the cells containing the therapeutic DNA must be stable. Problems with integrating therapeutic DNA into the genome and the rapidly dividing nature of many cells prevent it from achieving long-term benefits. Patients require multiple treatments. Immune response â€" Any time a foreign object is introduced into human tissues, the immune system is stimulated to attack the invader. Stimulating the immune system in a way that reduces gene therapy effectiveness is possible. Problems with viral vectors â€" Viral vectors carry the risks of toxicity, inflammatory responses, and gene control and targeting issues. Some therapies may breach the Weismann barrier between soma and germ-line protecting the testes, potentially modifying the germline, falling afoul of regulations in countries that prohibit the latter practice. This has occurred in clinical trials for X-linked severe combined immunodeficiency X-SCID patients, in which hematopoietic stem cells were transduced with a corrective transgene using a retrovirus , and this led to the development of T cell leukemia in 3 of 20 patients. This may be problematic since the longer the DNA is, the harder it is to integrate into cell genomes. The first was that of Jesse Gelsinger , who died in because of immune rejection response. Please consider summarizing the material while citing sources as needed. November s and earlier[edit] In Friedmann and Roblin authored a paper in Science titled "Gene therapy for human genetic disease? Production of the missing enzyme was temporarily stimulated, but the new cells with functional genes were not generated. She led a normal life only with the regular injections performed every two months. The effects were successful, but temporary. This therapy also represents the beginning of cancer immunogene therapy, a treatment which proves to be effective due to the anti-tumor mechanism of IGF-I antisense, which is related to strong immune and apoptotic phenomena. In Claudio Bordignon , working at the Vita-Salute San Raffaele University , performed the first gene therapy procedure using hematopoietic stem cells as vectors to deliver genes intended to correct hereditary diseases. Clinical trials were halted temporarily in , but resumed after regulatory review of the protocol in the US, the United Kingdom, France, Italy, and Germany. The allele that codes for adenosine deaminase ADA was obtained and inserted into a retrovirus. Retroviruses and stem cells were mixed, after which the viruses inserted the gene into the stem cell chromosomes. Injections of the ADA enzyme were also given weekly. After four years more treatment was needed. The approach has shown promising results in the treatment of six different malignant tumors: Trojan Trojan et al. In humans, the use of hydroxyurea to stimulate the production of HbF temporarily alleviates sickle cell symptoms. The researchers demonstrated this treatment to be a more permanent means to increase therapeutic HbF production. This technique has the potential to treat thalassaemia , cystic fibrosis and some cancers. They used liposomes coated in a polymer called polyethylene glycol , which unlike viral vectors, are small enough to cross the bloodâ€"brain barrier. If a siRNA is designed to match the RNA copied from a faulty gene, then the abnormal protein product of that gene will not be produced. The study is the first to show that gene therapy can treat the myeloid system. The immune system normally recognizes the new gene as foreign and rejects the cells carrying it. The research utilized a newly uncovered network of genes regulated by molecules known as microRNAs. This natural function selectively obscured their therapeutic gene in immune system cells and protected it from discovery. Mice infected with the gene containing an immune-cell microRNA target sequence did not reject the gene. In

August scientists successfully treated metastatic melanoma in two patients using killer T cells genetically retargeted to attack the cancer cells. In a phase I clinical trial , five subjects with chronic HIV infection who had failed to respond to at least two antiretroviral regimens were treated. All patients had stable or decreased viral load; four of the five patients had stable or increased CD4 T cell counts. All five patients had stable or increased immune response to HIV antigens and other pathogens. This was the first evaluation of a lentiviral vector administered in a US human clinical trial. The first operation was carried out on a year-old British male, Robert Johnson, in early The results of a small clinical trial in children were published in April. In May two more groups reported positive results in independent clinical trials using gene therapy to treat the condition. In all three clinical trials, patients recovered functional vision without apparent side-effects. Cone function and day vision were restored for at least 33 months in two young specimens. The therapy was less efficient for older dogs. About a third of the hemoglobin contained the form introduced by the viral vector and blood transfusions were not needed. This cure was accepted by the medical community in In August two of three subjects of a pilot study were confirmed to have been cured from chronic lymphocytic leukemia CLL. The therapy used genetically modified T cells to attack cells that expressed the CD19 protein to fight the disease. The treatment used Alipogene tiparvovec Glybera to compensate for lipoprotein lipase deficiency , which can cause severe pancreatitis. They were also given bone marrow. One patient relapsed and died and one died of a blood clot unrelated to the disease. The therapy was designed to increase the levels of SERCA 2, a protein in heart muscles, improving muscle function. Three of the children had metachromatic leukodystrophy , which causes children to lose cognitive and motor skills. Another three children were making progress. Over two years later all six were producing clotting factor. Over a six-month to two-year period all had improved their sight. In March researchers reported that 12 HIV patients had been treated since in a trial with a genetically engineered virus with a rare mutation CCR5 deficiency known to protect against HIV with promising results. The technique is named immunoprophylaxis by gene transfer IGT. Animal tests for antibodies to ebola, malaria, influenza, and hepatitis were underway. One year after the treatment she was still free of her cancer a highly aggressive form of acute lymphoblastic leukaemia [ALL]. This was the second gene therapy treatment to be approved in Europe. One of the four trials did find weak evidence that liposome-based CFTR gene transfer therapy may lead to a small respiratory improvement for people with CF. This weak evidence is not enough to make a clinical recommendation for routine CFTR gene therapy. The T cells are engineered to target a protein called CD19 that is common on B cells. This is the first form of gene therapy to be approved in the United States. In October, a similar therapy called axicabtagene ciloleucel was approved for non-Hodgkin lymphoma. Six of the seven patients on the high dose regime increased the level of the blood clotting VIII to normal levels.

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Chapter 5 : Human Gene Therapy

The definitive rapid-publication journal covering all aspects of gene therapy through original investigations into the transfer and expression of genes in mammals, with in-depth coverage of DNA, RNA, and cell therapies.

Gene therapy holds promise for treating a wide range of diseases, such as cancer, cystic fibrosis, heart disease, diabetes, hemophilia and AIDS. Researchers are still studying how and when to use gene therapy. Currently, in the United States, gene therapy is available only as part of a clinical trial. Researchers are investigating several ways to do this, including: Some cells become diseased because certain genes work incorrectly or no longer work at all. Replacing the defective genes may help treat certain diseases. For instance, a gene called p53 normally prevents tumor growth. Several types of cancer have been linked to problems with the p53 gene. If doctors could replace the defective p53 gene, that might trigger the cancer cells to die. Mutated genes that cause disease could be turned off so that they no longer promote disease, or healthy genes that help prevent disease could be turned on so that they could inhibit the disease. Making diseased cells more evident to the immune system. Doctors could use gene therapy to train your immune system to recognize the cells that are a threat. Rather, it usually has to be delivered using a carrier, called a vector. Researchers remove the original disease-causing genes from the viruses, replacing them with the genes needed to stop disease. This technique presents the following risks: Unwanted immune system reaction. This may cause inflammation and, in severe cases, organ failure. Targeting the wrong cells. If this happens, healthy cells may be damaged, causing other illness or diseases, such as cancer. Infection caused by the virus. Possibility of causing a tumor. If the new genes get inserted in the wrong spot in your DNA, there is a chance that the insertion might lead to tumor formation. The gene therapy clinical trials underway in the U. What you can expect Currently, the only way for you to receive gene therapy is to participate in a clinical trial. Clinical trials are research studies that help doctors determine whether a gene therapy approach is safe for people. They also help doctors understand the effects of gene therapy on the body. Your specific procedure will depend on the disease you have and the type of gene therapy being used. For example, in one type of gene therapy: You may have blood drawn or you may need bone marrow removed from your hipbone with a large needle. Then, in a lab, cells from the blood or bone marrow are exposed to a virus or another type of vector that contains the desired genetic material. Once the vector has entered the cells in the lab, those cells are injected back into your body into a vein or into tissue, where your cells take up the vector along with the altered genes. Other vectors being studied in clinical trials include: Stem cells are the cells from which all other cells in your body are created. For gene therapy, stem cells can be trained in a lab to become cells that can help fight disease. Results The possibilities of gene therapy hold much promise. Clinical trials of gene therapy in people have shown some success in treating certain diseases, such as: Severe combined immune deficiency Blindness caused by retinitis pigmentosa Leukemia But several significant barriers stand in the way of gene therapy becoming a reliable form of treatment, including: Finding a reliable way to get genetic material into cells Targeting the correct cells Reducing the risk of side effects Gene therapy continues to be a very important and active area of research aimed at developing new, effective treatments for a variety of diseases. Clinical trials Explore Mayo Clinic studies testing new treatments, interventions and tests as a means to prevent, detect, treat or manage this disease.

Chapter 6 : ZNF zinc finger protein [(human)]

Gene therapy is an experimental technique that uses genes to treat or prevent disease. In the future, this technique may allow doctors to treat a disorder by inserting a gene into a patient's cells instead of using drugs or surgery.

Facts Targeting Disease What is gene therapy? Gene therapy is when DNA is introduced into a patient to treat a genetic disease. The new DNA usually contains a functioning gene to correct the effects of a disease-causing mutation. Gene therapy uses sections of DNA usually genes to treat or prevent disease. The DNA is carefully selected to correct the effect of a mutated gene that is causing disease. The technique was first developed in but has, so far, had limited success in treating human diseases. Gene therapy may be a promising treatment option for some genetic diseases , including muscular dystrophy and cystic fibrosis. There are two different types of gene therapy depending on which types of cells are treated: Gene therapy techniques There are several techniques for carrying out gene therapy. Gene augmentation therapy This is used to treat diseases caused by a mutation that stops a gene from producing a functioning product, such as a protein. This therapy adds DNA containing a functional version of the lost gene back into the cell. The new gene produces a functioning product at sufficient levels to replace the protein that was originally missing. This is only successful if the effects of the disease are reversible or have not resulted in lasting damage to the body. For example, this can be used to treat loss of function disorders such as cystic fibrosis by introducing a functional copy of the gene to correct the disease see illustration below. Gene inhibition therapy Suitable for the treatment of infectious diseases, cancer and inherited disease caused by inappropriate gene activity. The aim is to introduce a gene whose product either: The basis of this therapy is to eliminate the activity of a gene that encourages the growth of disease-related cells. For example, cancer is sometimes the result of the over-activation of an oncogene gene which stimulates cell growth. So, by eliminating the activity of that oncogene through gene inhibition therapy, it is possible to prevent further cell growth and stop the cancer in its tracks. Killing of specific cells Suitable for diseases such as cancer that can be treated by destroying certain groups of cells. This can be achieved in one of two ways: It is essential with this method that the inserted DNA is targeted appropriately to avoid the death of cells that are functioning normally. How is DNA transfer done? The vector acts as a vehicle to carry the new DNA into the cells of a patient with a genetic disease. An illustration to show the transfer of a new gene into the nucleus of a cell via a viral vector. Avoiding the immune response: The role of the immune system is to fight off intruders. Sometimes new genes introduced by gene therapy are considered potentially-harmful intruders. This can spark an immune response in the patient, that could be harmful to them. This is usually by using vectors that are less likely to trigger an immune response. Ideally, a new gene introduced by gene therapy will integrate itself into the genome of the patient and continue working for the rest of their lives. There is a risk that the new gene will insert itself into the path of another gene, disrupting its activity. This could have damaging effects, for example, if it interferes with an important gene involved in regulating cell division, it could result in cancer. The cost of gene therapy: Many genetic disorders that can be targeted with gene therapy are extremely rare. Gene therapy therefore often requires an individual, case-by-case approach. This may be effective, but may also be very expensive. This page was last updated on Related Content:

Chapter 7 : Impact Factor of Human Gene Therapy - | | | | | | | |

In the medicine field, gene therapy (also called human gene transfer) is the therapeutic delivery of nucleic acid into a patient's cells as a drug to treat disease.

Chapter 8 : Human Gene Therapy Journal | British Society for Gene and Cell Therapy

Gene therapy replaces a faulty gene or adds a new gene in an attempt to cure disease or improve your body's ability to fight disease. Gene therapy holds promise for treating a wide range of diseases, such as cancer, cystic fibrosis, heart

disease, diabetes, hemophilia and AIDS.

Chapter 9 : What is Human Gene Therapy

but human gene therapy dreams of treating diseases by replacing or supplementing the product of defective or introducing novel therapeutic genes. So definitely human gene therapy is an effective addition to the arsenal of.