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**Gene Therapy**

First, we must understand that genes are sequences of bases that, when decoded, carry instruction on how to make proteins. These proteins both make up the majority of cellular structures as well as perform most life functions. The problem with this is that sometimes these genes are somehow modified for the worse, and the resulting proteins cannot carry out their normal functions, resulting in genetic disorders. This is where gene therapy comes in. Gene therapy is a technique designed to correct defective genes responsible for disease development. There are multiple approaches in which gene therapy can be done:

* A normal, healthy gene can be inserted into a location in the genome, thereby replacing a nonfunctional gene.
* By using homologous recombination, an abnormal gene can be swapped for a normal gene.
* Selective Reverse Mutation can be used to repair an abnormal gene, returning the gene to its normal function.

(<http://www.ornl.gov/sci/techresources/Human_Genome/medicine/genetherapy.shtml>)

It is also possible to simply introduce therapeutic DNA into the target cells, but this is limited to certain tissues and requires large amounts of DNA.

Gene Therapy is not without its issues, however. There are several reasons why it has not become a hugely effective treatment for genetic disease. Gene therapy is often short-lived, since the cells the therapeutic DNA is introduced to is so rapidly dividing. This often means that gene therapy produces no long term results unless patients undergo multiple rounds of gene therapy. There is also the problem of immune response. Just like any other foreign object, trying to inject therapeutic DNA into the cells has the risk of stimulating the immune system in a way that will reduce the effectiveness of the gene therapy. Along these lines, viruses (the main DNA carrier of choice in most studies) also bring potential problems to the patient. Not only are there risks with inflammatory and immune responses due to the virus, but it is also feared that once the virus is inside the patient, it may recover its ability to cause disease.

One specific application of gene therapy that is especially interesting is its work in genetic diseases of the eye. More specifically, genetic optic nerve diseases that can cause devastating vision loss, and many times blindness. According to a study at Cambridge university, they have achieved "highly efficient transfection of retinal ganglion cells in a rat model of glaucoma following a single intravitreal injection of adeno-associated virus" (<http://www.nature.com/eye/journal/v18/n11/full/6701579a.html>). Their research also shows how gene therapy is an especially good candidate for the eye. First, the eye is a much smaller organ than most, making it possible to transfect a significantly large proportion of the cells, making gene therapy more likely to be successful. Also, the eye is isolated, relatively speaking. This reduces the risk of unwanted transfection to other cells. The third, and most significant bonus, is that most of the target cells for ocular gene therapy are not currently undergoing cell division, which is one of the largest enemies to gene therapy. As an example of what gene therapy could do for eye diseases, take Glaucoma. Glaucoma is the second leading cause of blindness in the world, and it involves multiple genetic as well as environmental factors. What gene therapy can do for glaucoma is slow the rate of RGC death. By slowing down RGC death, the damage done by glaucoma is lessened. This means that gene therapy for eyes can have a significant impact on one of the leading causes of blindness, which is an amazing thing.

Sources:

<http://www.ornl.gov/sci/techresources/Human_Genome/medicine/genetherapy.shtml>

<http://www.nature.com/eye/journal/v18/n11/full/6701579a.html>