Gene Therapy for Genetic Disorders
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Abstract — Genetic disorders are rare but serious illnesses that stem from mistakes or mutations in a person’s genes. These disorders can vary in severity however they are usually untreatable. Gene therapy is a potential way to combat such illnesses as it replaces defective genes with their correct DNA interpretations.

I. INTRODUCTION

Medicine is always evolving to try and cure the illnesses and various conditions that affect people every day. Genetic disorders have for a long time been extremely difficult to address. They are not caused by a simple bacteria or virus entering the body, but instead are caused by the body itself making DNA replication errors. The errors in the genome can cause the body to lack specific proteins vital to functioning. Previously there had been no direct way to treat such illnesses but gene therapy has opened up the door to a new method of treatment. Gene therapy inserts corrected genes into a patient where they can be properly replicated and begin to help fight the illness.

II. METHODS

Gene therapy is relatively simple in that an injection to the afflicted area or into the blood will get the therapeutic gene into the body. To introduce a corrected gene into a cell though, there needs to be a special type of delivery system. This is found in the form of a vector. Traditionally, vectors are viruses that have been given a therapeutic gene to insert in the cell nucleus where it can be reproduced. To prevent infection from the virus, they are usually inhibited so that only the helping gene can be transferred.

Besides viral vectors, new non-viral vectors are being developed. They pose a risk free alternative to introducing genes into cells, however, getting through the cell membrane is a significant challenge. Multiple methods have been created such as gene guns that use gold particles coated in DNA, electroporation that electrically alters the membrane briefly so DNA can pass through, and ultrasounds with microbubbles to increase cell permeability.

III. RESULTS

. While gene therapy is still considered to be in its trial stages, it has been approved to treat many diseases even ones outside the range of genetic disorders such as cardiovascular diseases, cancer, and neurological diseases. Results from some of the treatments have been successful and beneficial towards patients, however they do not completely cure an individual. In cases such as chronic granulomatous disease, a genetic immunodeficiency disease, therapy has had a large impact and greatly improved life conditions for patients who maintain continued therapy. Other cases like cystic fibrosis also require continued use of therapy but as inhalers. Although there have been successes, therapy still seems to be somewhat patient and case specific. As technology and understanding of genes improves, the success rate of gene therapy will improve and the treatment as a whole should be applicable to an even large group of diseases.

IV. DISCUSSION

Currently gene therapy is being used to treat everything from cystic fibrosis to adenovirus infections to heart failure and even cancers. As medical science evolves, addressing the specific error in patients’ genes will become more precise and the delivery mechanism will be more effective and less reliant on viral vectors. The transition from viral vectors to non-viral vectors such as gold particles fired from a gene gun will negate any risks such as infection. Other safe and efficient delivery methods such as, hydrodynamic gene transfer will be completely developed. This type of gene transfer uses a build up of pressure from excess DNA in the blood to force its way into cells where there it can be copied instead of an error bearing gene. Methods like this are considered safe and simple, but are currently unpractical until better technology is created. In conclusion, gene therapy is a viable treatment for not just genetic disorders but many forms of illness. As time progresses, new technologies should be expected to even better treat patients and eventually completely cure them.

REFERENCES